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As filed with the Securities and Exchange Commission on November 8, 2013.

Registration No. 333-

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM S-1

REGISTRATION STATEMENT UNDER THE SECURITIES ACT OF 1933

ULTRAGENYX PHARMACEUTICAL INC.

(Exact name of Registrant as specified in its charter)

Delaware	**2834**	**27-2546083**
(State or other jurisdiction of incorporation or organization)	*(Primary Standard Industrial Classification Code Number)*	*(I.R.S. Employer Identification Number)*

60 Leveroni Court
Novato, CA 94949
(415) 483-8800

(Address, including zip code, and telephone number, including area code, of Registrant's principal executive offices)

Emil D. Kakkis, M.D., Ph.D.
President and Chief Executive Officer
Ultragenyx Pharmaceutical Inc.
60 Leveroni Court
Novato, CA 94949
(415) 483-8800

(Name, address, including zip code, and telephone number, including area code, of agent for service)

Copies to:

Ryan A. Murr, Esq. **Lisa M. Kahle, Esq.** **Ropes & Gray LLP** **Three Embarcadero Center, Third Floor** **San Francisco, CA 94111** **Telephone: (415) 315-6300** **Facsimile: (415) 315-6350**	**Shalini Sharp** **Chief Financial Officer** **Ultragenyx Pharmaceutical Inc.** **60 Leveroni Court** **Novato, CA 94949** **Telephone: (415) 483-8800** **Facsimile: (415) 483-8810**	**B. Shayne Kennedy, Esq.** **Brian J. Cuneo, Esq.** **Latham & Watkins LLP** **650 Town Center Drive, 20th Floor** **Costa Mesa, CA 92626** **Telephone: (714) 540-1235** **Facsimile: (714) 755-8290**

Approximate date of commencement of proposed sale to the public:
As soon as practicable after the effective date of this Registration Statement.

If any of the securities being registered on this Form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, check the following box. ☐

If this Form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, please check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this Form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this Form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer ☐ Accelerated filer ☐ Non-accelerated filer ☒ Smaller reporting company ☐
(Do not check if a smaller reporting company)

CALCULATION OF REGISTRATION FEE

Title of each class of securities to be registered	**Proposed maximum aggregate offering price(1)**	**Amount of registration fee**
Common Stock, $0.001 par value per share	$86,250,000	$11,109

(1) Estimated solely for the purpose of calculating the amount of the registration fee in accordance with Rule 457(o) under the Securities Act of 1933, as amended. Includes shares that the underwriters have the option to purchase to cover overallotments, if any.

The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the Registration Statement shall become effective on such date as the Commission, acting pursuant to said Section 8(a), may determine.

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The information in this preliminary prospectus is not complete and may be changed. We may not sell these securities until the registration statement filed with the Securities and Exchange Commission is effective. This prospectus is not an offer to sell these securities, and it is not soliciting an offer to buy these securities in any jurisdiction where the offer or sale is not permitted.

Subject to completion, dated November 8, 2013

Prospectus

shares

Common Stock

This is an initial public offering of common stock by Ultragenyx Pharmaceutical Inc. We are selling shares of common stock. The initial public offering price is between $ and $ per share.

Prior to this offering, there has been no public market for our common stock. We have applied for listing of our common stock on The NASDAQ Global Market under the symbol “RARE”.

We are an “emerging growth company” under applicable Securities and Exchange Commission rules and will be subject to reduced public company reporting requirements.

	Per share	Total
Initial public offering price	$	$
Underwriting discounts and commissions(1)	$	$
Proceeds to Ultragenyx Pharmaceutical Inc., before expenses	$	$

(1) See “Underwriting” for additional disclosure regarding underwriting discounts, commissions and estimated offering expenses.

We have granted the underwriters an option for a period of 30 days to purchase up to additional shares of common stock.

Investing in our common stock involves a high degree of risk. See “Risk Factors” beginning on page 10.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed on the adequacy or accuracy of this prospectus. Any representation to the contrary is a criminal offense.

The underwriters expect to deliver the shares of common stock to investors on or about , 201 .

J.P. Morgan **Morgan Stanley**

Cowen and Company

Canaccord Genuity

, 201

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We have not authorized anyone to provide you with information other than that contained in this prospectus or in any free writing prospectus prepared by or on behalf of us or to which we have referred you. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give to you. We are offering to sell, and seeking offers to buy, shares of our common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or any sale of our common stock. Our business, financial condition, results of operations, and prospects may have changed since that date.

No action is being taken in any jurisdiction outside the United States to permit a public offering of our common stock or possession or distribution of this prospectus in that jurisdiction. Persons who come into possession of this prospectus in jurisdictions outside the United States are required to inform themselves about and to observe any restrictions as to this offering and the distribution of this prospectus applicable to that jurisdiction.

PROSPECTUS SUMMARY

The items in the following summary are described in more detail later in this prospectus. This summary provides an overview of selected information and does not contain all of the information you should consider before buying our common stock. Therefore, you should read the entire prospectus carefully, especially the "Risk Factors" section beginning on page 10 and our financial statements and the related notes appearing at the end of this prospectus, before deciding to invest in our common stock. In this prospectus, unless the context otherwise requires, references to "the Company," "we," "us," "our," or "Ultragenyx" refer to Ultragenyx Pharmaceutical Inc.

Overview

We are a development-stage biopharmaceutical company focused on the identification, acquisition, development, and commercialization of novel products for the treatment of rare and ultra-rare diseases, with an initial focus on serious, debilitating metabolic genetic diseases. We focus on diseases for which the unmet medical need is high, the biology for treatment is clear, and for which there are no approved therapies. Since our inception in 2010, we have in-licensed potential treatments for five different diseases that are or we expect will be in Phase 2 clinical studies by early 2014. Our strategy, which is predicated upon time- and cost-efficient drug development, allows us to pursue multiple programs in parallel with the goal of delivering safe and effective therapies to patients with the utmost urgency.

Our current pipeline consists of two product categories: biologics, including a monoclonal antibody and enzyme replacement therapies; and small-molecule substrate replacement therapies. Enzymes are proteins that the body uses to process materials needed for normal cellular function, and substrates are the materials upon which enzymes act. When enzymes or substrates are missing, the body is unable to perform its normal cellular functions, often leading to significant clinical disease. Several of our therapies are intended to replace deficient enzymes or substrates.

The following table summarizes our product candidate pipeline:

Candidate	Description	Indication	Pre-clinical	Phase 1	Phase 1/2 or Phase 2	Phase 3 or pivotal	Status / Anticipated milestones	Ultragenyx commercial rights
Biologics								
KRN23 (UX023)	Anti-FGF23 monoclonal antibody	XLH					▪ Expect to initiate pediatric clinical development in 2014	▪ U.S. and Canada: Joint with KHK (profit share) ▪ Mexico, Central and South America
rhGUS (UX003)	Enzyme replacement	MPS 7					▪ Expect to initiate a Phase 1/2 clinical study by end of 2013	▪ Worldwide
rhPPCA (UX004)	Enzyme replacement	Galactosialidosis					▪ Expect to continue preclinical development during 2014	▪ Worldwide
Small molecules								
Triheptanoin (UX007)	Substrate replacement	LC-FAOD					▪ Expect to initiate Phase 2 study by end of 2013	▪ Worldwide
Triheptanoin	Substrate replacement	Glut1 DS					▪ Expect to initiate Phase 2 clinical study in early 2014	▪ Worldwide
SA-ER (UX001)	Substrate replacement	HIBM					▪ Expect data from ongoing Phase 2 study by end of 2013	▪ Worldwide (excluding Japan and certain other Asian territories)

Our current product candidate pipeline has been either in-licensed from academic institutions or derived from partnerships with other pharmaceutical companies. Where possible, our strategy is to acquire and retain

global commercialization rights to our products to maximize long-term value. Over time, we intend to build our own commercial organization, which we believe will be of modest size due to the relatively small number of specialists who treat patients with rare and ultra-rare diseases.

The patients we seek to treat have diseases with limited or no treatment options, and we recognize that their lives and well-being are highly dependent upon our efforts to develop new therapies. For this reason, we are passionate about developing these therapies with the utmost urgency and care. We strive to build a company that is faster, better, and smarter about advancing multiple product candidates through approval.

We were founded in April 2010 by our current President and Chief Executive Officer, Dr. Emil Kakkis, M.D., Ph.D., who is the former Chief Medical Officer of BioMarin Pharmaceutical Inc. We have assembled an experienced team with extensive drug development and commercialization capabilities, particularly in the orphan drug area. Dr. Kakkis and the team at Ultragenyx have been previously involved, at other companies, in the development and/or commercialization of many therapies approved or in development for rare metabolic genetic diseases, including Aldurazyme, Naglazyme, Kuvan, and Vimizim (BioMarin); Lumizyme/Myozyme (Sanofi-Genzyme); and asfotase alpha (Enobia; now Alexion). Our investors include, but are not limited to, the following entities, their affiliates or funds advised by them: TPG Biotech, Fidelity Biosciences (Beacon Bioventures), HealthCap, Pappas Ventures, Adage Capital Partners, L.P., Capital Research Global Investors, Columbia Wanger Asset Management, Jennison Associates LLC, BlackRock, Inc., Genzyme Corporation, Shire LLC, and Ramius LLC.

Product Candidates

KRN23 for the treatment of XLH

KRN23 is a fully human monoclonal antibody administered via subcutaneous injection that is designed to bind and reduce the biological activity of fibroblast growth factor, or FGF23, to increase abnormally low phosphate levels in patients with X-linked hypophosphatemia, or XLH. Patients with XLH have low serum phosphate levels due to excessive phosphate loss into the urine, which is directly caused by the effect on kidney function of excess FGF23 production in bone cells. Low phosphate levels lead to poor bone mineralization and a variety of clinical manifestations, including skeletal deformity, bone pain, short stature, gross motor impairment, muscle weakness, and lower than normal bone density. There is no approved drug therapy or treatment for the underlying cause of XLH. Most patients are managed using oral phosphate replacement and vitamin D therapy, which is only partially effective at restoring bone physiology and growth and has significant side effects.

In August 2013, we formed a collaboration with Kyowa Hakko Kirin Co., Ltd., or KHK, to jointly develop and commercialize KRN23 for the treatment of XLH. KHK has conducted one Phase 1, one Phase 1/2 study and one Phase 1/2 extension study of KRN23 in adults with XLH. We expect to receive data for the Phase 1/2 studies in 2014. Results from the Phase 1 single dose study demonstrated that KRN23 was well tolerated. The data suggest efficacy in increasing serum phosphate levels, while reducing urinary excretion of phosphate. We expect to continue to develop KRN23 in adults with XLH. In addition, we expect to initiate a Phase 2 pediatric study in 2014. Given the high turnover and growth of bone during childhood and the critical role phosphate plays in bone growth, pediatric XLH patients have the highest morbidity and potential for benefit.

rhGUS for the treatment of MPS 7

Recombinant human beta-glucuronidase, or rhGUS, is an intravenous, or IV, enzyme replacement therapy for the treatment of mucopolysaccharidosis 7, or MPS 7, also known as Sly Syndrome. Patients with MPS 7 suffer from severe cellular and organ dysfunction that typically leads to death in the teens or early adulthood. MPS 7 is caused by a deficiency of the lysosomal enzyme beta-glucuronidase, which is required for the breakdown of certain complex carbohydrates known as glycosaminoglycans, or GAGs. The inability to properly break down GAGs leads to their accumulation in many tissues, resulting in a serious multi-system disease. There are currently no approved drug therapies for MPS 7.

We licensed exclusive worldwide rights to rhGUS-related know-how and cell lines from Saint Louis University in November 2010. We have conducted preclinical studies to support the chronic IV administration of rhGUS. We plan to initiate an open-label, Phase 1/2 study to evaluate the safety, tolerability, efficacy, and dose of IV administration every other week of rhGUS in five patients with MPS 7 who are between five and 30 years of age. The initial 12-week treatment period will be followed by a dose-titration period and a long-term extension study. We expect to commence this study by the end of 2013. If results from the initial 12-week treatment period from this study are supportive, we plan to initiate a pivotal Phase 3 study enrolling approximately 12 patients.

rhPPCA for the treatment of galactosialidosis

Recombinant human protective protein cathepsin-A, or rhPPCA, which we in-licensed from St. Jude Children's Research Hospital in September 2012, is in preclinical development as an enzyme replacement therapy for galactosialidosis, a rare lysosomal storage disease for which there are no currently approved drug therapies. Similar to MPS patients, patients with galactosialidosis present with both soft tissue storage in the liver, spleen, and other tissues, as well as connective tissue (bone and cartilage) related disease. As with MPS 7, an enzyme deficiency results in accumulation of substrates in the lysosomes, causing skeletal and organ dysfunction, and death. We plan to continue preclinical development of rhPPCA during 2014.

Triheptanoin for the treatment of LC-FAOD

We are developing triheptanoin for oral liquid administration intended as a substrate replacement therapy for patients with long-chain fatty acid oxidation disorders, or LC-FAOD. Triheptanoin is a medium-chain triglyceride of three seven-carbon fatty acids designed to provide substrate replacement for fatty acid metabolism and restore production of energy. Patients with LC-FAOD have a deficiency that impairs the ability to produce energy from fat, which can lead to depletion of all glucose in the body, and severe liver, muscle, and heart disease, as well as death. There are currently no approved drugs or treatments specifically for LC-FAOD. The current standard of care for LC-FAOD includes diligent prevention of fasting combined with the use of low-fat/high-carbohydrate diets, carnitine supplementation in some cases, and medium-chain triglyceride, or MCT, oil supplementation. Despite treatment with the current standard of care, many patients continue to suffer significant morbidity and mortality.

Triheptanoin has been studied clinically for 13 years in approximately 150 human subjects affected by a variety of diseases, including 65 patients with LC-FAOD. Multiple investigator-sponsored open-label studies suggest clinical improvements with triheptanoin treatment, even for patients who were on standard of care. We recently completed a retrospective medical record review study to assess the clinical outcome of triheptanoin treatment on LC-FAOD subjects who have been participating in a compassionate use program at the University of Pittsburgh Medical Center. The data showed that treatment with triheptanoin appeared to reduce the frequency and severity of hospitalizations previously experienced by these patients.

We licensed certain intellectual property rights relating to triheptanoin from Baylor Research Institute in September 2012. Triheptanoin is in an ongoing investigator-sponsored Phase 2 study for the treatment of LC-FAOD. We plan to initiate a prospective open-label Phase 2 study of triheptanoin treatment in approximately 20 to 30 severely affected LC-FAOD patients by the end of 2013. The effects of treatment on clinical and physiologic disease will be assessed in three areas: skeletal myopathy, liver disease, and cardiac disease. A principal goal of the study is to determine the appropriate clinical endpoints and patient population for testing in potential later-stage pivotal studies.

Triheptanoin for the treatment of Glut1 DS

We are also developing triheptanoin for patients with glucose transporter type-1 deficiency syndrome, or Glut1 DS. Glut1 DS is caused by a mutation affecting the gene that codes for Glut1, which is a protein that transports glucose from blood into the brain. Because glucose is the primary source of energy for the brain,

Glut1 DS results in a chronic state of energy deficiency in the brain and is characterized by seizures, developmental delay, and movement disorder. There are currently no approved drugs specific to Glut1 DS. The current standard of care for Glut1 DS is the ketogenic diet, an extreme high-fat (70-80% of daily calories as fat)/low-carbohydrate diet, which generates ketone bodies as an alternative energy source to glucose. The ketogenic diet is difficult to comply with and has demonstrated limited effectiveness in the treatment of developmental delay and movement disorder.

Triheptanoin is intended as a substrate replacement therapy to provide an alternative source of energy to the brain in Glut1 DS patients. Although an open-label investigator-sponsored clinical study is ongoing and the results have not yet been reported, there are anecdotal reports of benefit in terms of reduced seizures and improved development rate in some Glut1 DS subjects taking triheptanoin. We are planning to initiate a clinical development program to study the effects of triheptanoin in Glut1 DS by early 2014. We anticipate that the program will initially consist of a Phase 2 adaptive, randomized, double-blind, placebo-controlled, parallel-group study of approximately 50 pediatric subjects who are currently not on ketogenic diet.

SA-ER for the treatment of HIBM

We are developing an extended-release, oral formulation of sialic acid, or SA-ER, for the treatment of hereditary inclusion body myopathy, or HIBM, which is also known as GNE myopathy. HIBM is characterized by severe progressive muscular myopathy, or disease in which muscle fibers do not function properly, with onset in the late teens or twenties. Patients with HIBM have a genetic defect in the gene coding for a particular enzyme that is involved in the first step in the biosynthesis of sialic acid. Therefore, HIBM patients have a sialic acid deficiency, which interferes with muscle function, leading to myopathy and atrophy. Patients typically become wheelchair bound within ten to 20 years from onset. There is no approved drug therapy for HIBM.

SA-ER is intended as a substrate replacement therapy designed to address sialic acid deficiency and restore muscle function in HIBM patients. We are currently conducting a randomized, double-blind, placebo-controlled Phase 2 study of SA-ER in 47 HIBM patients. An interim analysis at 24 weeks of treatment showed modest dose-dependent improvement in muscle strength compared to declines in placebo-treated subjects in some muscle groups, particularly in the upper extremities at the higher dose. SA-ER appeared to be well tolerated with no serious adverse events observed to date in either dose group. Patients will be evaluated again at 48 weeks, with that data anticipated by the end of 2013. Following the 48-week analysis, we plan to continue to treat these patients in an extension study with an increased dosage of sialic acid based on the dose dependence observed at week 24. We anticipate that data from the extension study should be available in late 2014.

Our Strategy

Our strategy is to identify, acquire, develop, and commercialize novel products for the treatment of rare and ultra-rare diseases in the United States, the European Union, and select international markets, with the goal of becoming a leading rare disease company. The critical components of our business strategy include the following:

- Focus on rare and ultra-rare diseases with significant unmet medical need;
- Focus on diseases and therapies with clear mechanisms of action;
- Leverage our experience and relationships to in-license promising product candidates;
- Develop and commercialize multiple product candidates in parallel;
- Focus on excellent and rapid clinical and regulatory execution; and
- Seek to retain global commercialization rights to product candidates.

Risks Associated with Our Business

Our ability to implement our business strategy is subject to numerous risks that you should be aware of before making an investment decision. These risks are described more fully in the section entitled "Risk Factors" immediately following this prospectus summary. These risks include, among others:

- We are a development-stage company and have a limited operating history on which to assess our business, have incurred significant losses since our inception, and anticipate that we will continue to incur significant losses for the foreseeable future;
- Even if this offering is successful, we expect that we will need to raise additional funding before we can expect to become profitable from sales of our products;
- We are heavily dependent upon the success of our product candidates, which are in the early stages of clinical development, and we cannot provide any assurance that any of our product candidates will receive regulatory approval;
- Because the target patient populations of our product candidates are small, we must be able to successfully identify patients and acquire a significant market share to achieve profitability and growth;
- The insurance coverage and reimbursement status of newly-approved orphan products is uncertain and failure to obtain or maintain adequate coverage and reimbursement for our product candidates could limit our ability to market those products and decrease our ability to generate revenue;
- If we are unable to obtain and maintain effective intellectual property rights for our technologies, product candidates, or any future product candidates, we may not be able to compete effectively in our markets; and
- Our future success depends in part upon our ability to retain our Founder, President, and Chief Executive Officer and to attract, retain, and motivate other qualified personnel.

Our Corporate Information

We were founded in April 2010 as a California corporation, and we reincorporated as a Delaware corporation in June 2011. Our principal executive offices are located at 60 Leveroni Court, Novato, CA 94949, and our telephone number is (415) 483-8800. Our web site address is www.ultragenyx.com. The information on, or that can be accessed through, our web site is not part of this prospectus. We have included our web site address as an inactive textual reference only.

We have filed trademark applications with the U.S. Patent and Trademark Office for the marks Ultragenyx™ and Ultragenyx Pharmaceutical™, and we are developing commercial names for our product candidates. This prospectus also contains trademarks of others, including Aldurazyme®, Naglazyme®, Kuvan®, Vimizim™, Lumizyme®, Myozyme® and asfotase alpha. We do not intend our use or display of other companies' trade names or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any other companies.

THE OFFERING

Common stock offered by us	shares
Common stock to be outstanding after this offering	shares
Underwriters' option to purchase additional shares	shares
Use of proceeds	We estimate that the net proceeds from this offering will be approximately $ million, or approximately $ million if the underwriters exercise their option to purchase additional shares in full, at an assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. We intend to use the net proceeds from this offering to fund our preclinical and clinical programs and any remaining proceeds for personnel-related costs, working capital, and other general corporate purposes. See "Use of Proceeds."
Risk factors	You should read the "Risk Factors" section of this prospectus for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.
Directed share program	At our request, the underwriters have reserved up to shares of our common stock offered by this prospectus for sale, at the initial public offering price, to our directors and officers and certain employees and other parties related to us. Shares purchased by our directors and officers will be subject to the 180-day lock-up restriction described in the "Underwriting" section of this prospectus. The number of shares of common stock available for sale to the general public will be reduced to the extent these individuals purchase such reserved shares. Any reserved shares that are not so purchased will be offered by the underwriters to the general public on the same basis as the other shares offered by this prospectus.
Proposed NASDAQ Global Market symbol	"RARE"

The number of shares of common stock to be outstanding after this offering is based on 11,607,173 shares of common stock outstanding as of September 30, 2013 and 61,431,574 additional shares of our common stock issuable upon conversion of all of our outstanding shares of preferred stock upon closing of this offering.

The number of shares of our common stock to be outstanding after this offering excludes the following:

- 4,785,720 shares of common stock issuable upon the exercise of outstanding stock options as of September 30, 2013 having a weighted-average exercise price of $0.27 per share;
- 1,027,662 shares of common stock issuable upon the exercise of outstanding warrants as of September 30, 2013 having a weighted-average exercise price of $1.034 per share;

- 4,906,484 shares of common stock reserved for issuance pursuant to future equity awards under our 2011 Equity Incentive Plan, as amended, as of September 30, 2013, which will become available for issuance under our 2013 Incentive Plan after the completion of this offering;
- shares of common stock reserved for issuance (including the above-referenced shares reserved for issuance under our 2011 Equity Incentive Plan, as amended) pursuant to future equity awards under our 2013 Incentive Plan, as well as any future increases in the number of shares of our common stock reserved for future issuance under this plan, which will become effective immediately prior to the completion of this offering; and
- shares of common stock reserved for future issuance under our 2013 Employee Stock Purchase Plan, or 2013 ESPP, as well as any future increases in the number of shares of our common stock reserved for future issuance under the 2013 ESPP, which will become effective immediately prior to the completion of this offering.

Except as otherwise indicated, all information contained in this prospectus:

- reflects the conversion of all of our outstanding shares of preferred stock into an aggregate of 61,431,574 shares of common stock immediately prior to the completion of this offering;
- assumes the effectiveness of our amended and restated certificate of incorporation and amended and restated by-laws immediately prior to the completion of this offering;
- assumes that the underwriters do not exercise their option to purchase additional shares; and
- assumes no exercise of outstanding options or warrants after September 30, 2013.

SUMMARY FINANCIAL DATA

The following table summarizes our statements of operations and balance sheet data. We have derived the following statements of operations data for the years ended December 31, 2011 and 2012 from our audited financial statements appearing elsewhere in this prospectus. The statements of operations data for the nine months ended September 30, 2012 and 2013 and the balance sheet data as of September 30, 2013 are derived from our unaudited financial statements appearing elsewhere in this prospectus. In our opinion, these unaudited financial statements have been prepared on a basis consistent with our audited financial statements and contain all adjustments, consisting only of normal and recurring adjustments, necessary for a fair presentation of such financial data. You should read this data together with our financial statements and related notes appearing elsewhere in this prospectus and the information under the captions "Selected Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations." Our historical results are not necessarily indicative of our future results, and our operating results for the nine-month period ended September 30, 2013 are not necessarily indicative of the results that may be expected for the year ending December 31, 2013 or any other interim periods or any future year or period.

	Year Ended December 31,		Nine Months Ended September 30,	
	2011	2012	2012	2013
	(In thousands, except share and per share amounts)		(unaudited)	
Statements of Operations Data:				
Operating expenses:				
Research and development	$ 4,717	$ 12,641	$ 8,866	$ 19,625
General and administrative	1,844	3,344	2,441	3,130
Total operating expenses	6,561	15,985	11,307	22,755
Loss from operations	(6,561)	(15,985)	(11,307)	(22,755)
Interest income	4	1	—	157
Interest expense	(270)	—	—	—
Other expense	(22)	(350)	(97)	(1,155)
Net loss	$ (6,849)	$ (16,334)	$ (11,404)	$ (23,753)
Net loss attributable to common stockholders [1]	$ (7,466)	$ (19,561)	$ (12,749)	$ (31,624)
Net loss per share attributable to common stockholders, basic and diluted	$ (1.47)	$ (4.53)	$ (3.94)	$ (3.09)
Shares used to compute net loss per share attributable to common stockholders, basic and diluted	5,069,694	4,316,868	3,235,308	10,220,034
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited) [1]		$		$
Shares used to compute pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited) [1]				

	As of September 30, 2013		
	Actual	Pro Forma[2]	Pro Forma as Adjusted[3][4]
		(unaudited) (in thousands)	
Balance Sheet Data:			
Cash, cash equivalents and marketable securities	$ 63,657	$ 63,657	$
Working capital	61,067	57,652	
Total assets	68,592	68,592	
Convertible preferred stock warrant liability	1,596	—	
Convertible preferred stock	118,002	—	
Deficit accumulated during the development stage	(56,846)	(56,846)	
Total stockholders' (deficit) equity	(56,848)	59,335	

(1) See Notes 2 and 14 to our audited financial statements and Note 8 of our unaudited financial statements included elsewhere in this prospectus for an explanation of the calculations of our basic and diluted net loss per share attributable to common stockholders and pro forma net loss per share attributable to common stockholders.

(2) Pro forma to reflect (i) the conversion of all of our outstanding shares of convertible preferred stock into an aggregate of 61,431,574 shares of our common stock, (ii) the reclassification to additional paid-in capital of our preferred stock warrant liability in connection with the conversion of our outstanding preferred stock warrants into common stock warrants, and (iii) a dividend of $3.4 million payable concurrent with the conversion of our preferred stock to common stock to the holders of our preferred stock, which has been calculated as if the conversion of preferred stock into common stock occurred as of November 8, 2013, in each case, immediately prior to the completion of this offering.

(3) Pro forma as adjusted to further reflect the sale of shares of common stock in this offering at an assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

(4) A $1.00 increase (decrease) in the assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus, would increase (decrease) each of cash, cash equivalents and marketable securities, working capital, total assets, and total stockholders' equity by approximately $ million, assuming that the number of shares offered by us, as set forth on the cover of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. A 1,000,000 share increase in the number of shares offered by us would increase each of cash, cash equivalents and marketable securities, working capital, total assets, and total stockholders' equity by approximately $ million after deducting estimated underwriting discounts and commissions and any estimated offering expenses payable by us. Conversely, a 1,000,000 share decrease in the number of shares offered by us would decrease each of cash, cash equivalents and marketable securities, working capital, total assets, and total stockholders' equity by approximately $ million after deducting estimated underwriting discounts and commissions and any estimated offering expenses payable by us.

RISK FACTORS

An investment in shares of our common stock involves a high degree of risk. You should carefully consider the following information about these risks, together with the other information appearing elsewhere in this prospectus, including our financial statements and related notes thereto, before deciding to invest in our common stock. The occurrence of any of the following risks could have a material adverse effect on our business, financial condition, results of operations and future growth prospects. In these circumstances, the market price of our common stock could decline, and you may lose all or part of your investment.

Risks Related to Our Financial Condition and Capital Requirements

We are a development-stage company and have a limited operating history on which to assess our business, have incurred significant losses since our inception, and anticipate that we will continue to incur significant losses for the foreseeable future.

We are a development-stage biopharmaceutical company with a limited operating history. We have incurred net losses in each year since our inception in April 2010, including net losses of $6.8 million and $16.3 million for the years ended December 31, 2011 and 2012, respectively, and $23.8 million for the nine months ended September 30, 2013. As of September 30, 2013, we had a deficit accumulated during the development stage of $56.8 million.

We have devoted substantially all of our financial resources to identify, acquire, and develop our product candidates, including conducting clinical studies and providing general and administrative support for these operations. To date, we have financed our operations primarily through the sale of equity securities and convertible promissory notes. The amount of our future net losses will depend, in part, on the rate of our future expenditures and our ability to obtain funding through equity or debt financings, strategic collaborations, or grants. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We are in the early stages of clinical development for our product candidates, we have not yet commenced pivotal clinical studies for any product candidate and it may be several years, if ever, before we complete pivotal clinical studies and have a product candidate approved for commercialization. If we obtain regulatory approval to market a product candidate, our future revenue will depend upon the size of any markets in which our product candidates may receive approval, and our ability to achieve sufficient market acceptance, pricing, reimbursement from third-party payors, and adequate market share for our product candidates in those markets. However, even if we obtain adequate market share for our product candidates, because the potential markets in which our product candidates may ultimately receive regulatory approval are very small, we may never become profitable despite obtaining such market share and acceptance of our products.

We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if and as we:

- continue our research and nonclinical and clinical development of our product candidates;
- expand the scope of our current clinical studies for our product candidates;
- advance our programs into more expensive clinical studies;
- initiate additional nonclinical, clinical, or other studies for our product candidates;
- change or add additional manufacturers or suppliers;
- seek regulatory and marketing approvals for our product candidates that successfully complete clinical studies;
- establish a sales, marketing, and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- seek to identify, assess, acquire, and/or develop other product candidates;
- make milestone or other payments under any license agreements;

- seek to maintain, protect, and expand our intellectual property portfolio;
- seek to attract and retain skilled personnel;
- create additional infrastructure to support our operations as a public company and our product development and planned future commercialization efforts; and
- experience any delays or encounter issues with any of the above, including but not limited to failed studies, complex results, safety issues, or other regulatory challenges that require longer follow-up of existing studies, additional major studies, or additional supportive studies in order to pursue marketing approval.

Further, the net losses we incur may fluctuate significantly from quarter to quarter and year to year, such that a period-to-period comparison of our results of operations may not be a good indication of our future performance.

We have never generated any revenue from product sales and may never be profitable.

We have no products approved for commercialization and have never generated any revenue. Our ability to generate revenue and achieve profitability depends on our ability, alone or with strategic collaboration partners, to successfully complete the development of, and obtain the regulatory and marketing approvals necessary to commercialize one or more of our product candidates. We do not anticipate generating revenue from product sales for the foreseeable future. Our ability to generate future revenue from product sales depends heavily on our success in many areas, including but not limited to:

- completing research and nonclinical and clinical development of our product candidates;
- obtaining regulatory and marketing approvals for product candidates for which we complete clinical studies;
- developing a sustainable and scalable manufacturing process for any approved product candidates and establishing and maintaining supply and manufacturing relationships with third parties that can conduct the process and provide adequate (in amount and quality) products to support clinical development and the market demand for our product candidates, if approved;
- launching and commercializing product candidates for which we obtain regulatory and marketing approval, either directly or with a collaborator or distributor;
- obtaining market acceptance of our product candidates as viable treatment options;
- addressing any competing technological and market developments;
- identifying, assessing, acquiring and/or developing new product candidates;
- negotiating favorable terms in any collaboration, licensing, or other arrangements into which we may enter;
- maintaining, protecting, and expanding our portfolio of intellectual property rights, including patents, trade secrets, and know-how; and
- attracting, hiring, and retaining qualified personnel.

Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our expenses could increase beyond expectations if we are required by the U.S. Food and Drug Administration, or the FDA, the European Medicines Agency, or the EMA, or other regulatory agencies, domestic or foreign, to change our manufacturing processes or assays, or to perform clinical, nonclinical, or other types of studies in addition to those that we currently anticipate. In cases where we are successful in obtaining regulatory approvals to market one or more of our product candidates, our revenue will be dependent, in part, upon the size of the markets in the territories for which we gain regulatory approval, the accepted price for the product, the ability to get reimbursement at any price, and whether we own the commercial rights for that territory. If the number of our

addressable rare disease patients is not as significant as we estimate, the indication approved by regulatory authorities is narrower than we expect, or the reasonably accepted population for treatment is narrowed by competition, physician choice or treatment guidelines, we may not generate significant revenue from sales of such products, even if approved. For example, the development of KRN23, rhGUS, and triheptanoin for pediatric use is an important part of our current business strategy; if we are unable to obtain regulatory approval for the desired age ranges, our business may suffer. Additionally, if we are not able to generate revenue from the sale of any approved products, we may never become profitable.

Even if this offering is successful, we expect that we will need to raise additional funding before we can expect to become profitable from sales of our products. This additional financing may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit, or terminate our product development efforts or other operations.

We are currently advancing our KRN23, rhGUS, triheptanoin, and SA-ER product candidates through clinical development and our other product candidate, rhPPCA, as well as our other early stage research projects, through preclinical development. Developing our product candidates is expensive, and we expect our research and development expenses to increase substantially in connection with our ongoing activities, particularly as we advance our product candidates through clinical studies.

As of September 30, 2013, our cash, cash equivalents and marketable securities were $63.7 million. We expect that our existing cash, cash equivalents and marketable securities, not including the proceeds we receive from this offering, will be sufficient to fund our current operations for at least the next 12 months; however, we expect that we will require additional capital to obtain regulatory approval for, and to commercialize, our product candidates. In addition, our operating plans may change as a result of many factors that may currently be unknown to us, and we may need to seek additional funds sooner than planned. Our future funding requirements will depend on many factors, including but not limited to:

- the scope, rate of progress, results and cost of our clinical studies, nonclinical testing, and other related activities;
- the cost of manufacturing clinical supplies, and establishing commercial supplies, of our product candidates and any products that we may develop;
- the number and characteristics of product candidates that we pursue;
- the cost, timing, and outcomes of regulatory approvals;
- the cost and timing of establishing sales, marketing, and distribution capabilities; and
- the terms and timing of any collaborative, licensing, and other arrangements that we may establish, including any required milestone and royalty payments thereunder.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. In addition, we cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. Moreover, the terms of any financing may adversely affect the holdings or the rights of our stockholders and the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our shares to decline. The sale of additional equity or convertible securities would dilute all of our stockholders. The incurrence of indebtedness could result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell, or license intellectual property rights, and other operating restrictions that could adversely impact our ability to conduct our business. We could also be required to seek funds through arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be desirable and we may be required to relinquish rights to some of our technologies or product candidates or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results, and prospects. Even if we believe we have sufficient funds for our current or future operating plans, we may seek additional capital if market conditions are favorable or if we have specific strategic considerations.

If we are unable to obtain funding on a timely basis, we may be required to significantly curtail, delay, or discontinue one or more of our research or development programs or the commercialization of any product candidates or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition, and results of operations.

Risks Related to the Discovery and Development of Our Product Candidates

We are heavily dependent on the success of our product candidates, which are in the early stages of clinical development. We cannot give any assurance that any of our product candidates will receive regulatory approval, which is necessary before they can be commercialized.

To date, we have invested substantially all of our efforts and financial resources to identify, acquire, and develop our product candidates, including conducting clinical studies and providing general and administrative support for these operations. Our future success is dependent on our ability to successfully develop, obtain regulatory approval for, and then successfully commercialize one or more product candidates. We currently generate no revenue from sales of any drugs, and we may never be able to develop or commercialize a marketable drug.

Each of our product candidates is in the early stages of development and will require additional clinical development, management of nonclinical, clinical, and manufacturing activities, regulatory approval, obtaining adequate manufacturing supply, building of a commercial organization, and significant marketing efforts before we generate any revenue from product sales. We currently have three product candidates in Phase 1/2 or Phase 2 clinical studies. None of our product candidates have advanced into a pivotal study and it may be years before such study is initiated, if at all. We are not permitted to market or promote any of our product candidates before we receive regulatory approval from the FDA or comparable foreign regulatory authorities, and we may never receive such regulatory approval for any of our product candidates.

Although certain of our employees have prior experience with submitting marketing applications to the FDA or comparable foreign regulatory authorities, we as a company have not submitted such applications for our product candidates. We cannot be certain that any of our product candidates will be successful in clinical studies or receive regulatory approval. Further, our product candidates may not receive regulatory approval even if they are successful in clinical studies. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations.

We generally plan to seek regulatory approval to commercialize our product candidates in the United States, the European Union, or EU, and in additional foreign countries where we have commercial rights. To obtain regulatory approval in other countries, we must comply with numerous and varying regulatory requirements of such other countries regarding safety, efficacy, chemistry, manufacturing and controls, clinical studies, commercial sales, pricing, and distribution of our product candidates. Even if we are successful in obtaining approval in one jurisdiction, we cannot ensure that we will obtain approval in any other jurisdictions. If we are unable to obtain approval for our product candidates in multiple jurisdictions, our revenue and results of operations could be negatively affected.

The regulatory approval processes of the FDA and comparable foreign authorities are lengthy, time consuming, and inherently unpredictable. If we are ultimately unable to obtain regulatory approval for our product candidates, our business will be substantially harmed.

The time required to obtain approval by the FDA and comparable foreign authorities is unpredictable, typically takes many years following the commencement of clinical studies, and depends upon numerous factors. In addition, approval policies, regulations, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate’s clinical development and may vary among jurisdictions, which may cause delays in the approval or the decision not to approve an application. We have not obtained regulatory approval for any product candidate, and it is possible that none of our existing product candidates or any product candidates we may seek to develop in the future will ever obtain regulatory approval.

Applications for our product candidates could fail to receive regulatory approval for many reasons, including but not limited to the following:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical studies;
- the population studied in the clinical program may not be sufficiently broad or representative to assure safety in the full population for which we seek approval;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from nonclinical studies or clinical studies;
- the data collected from clinical studies of our product candidates may not be sufficient to support the submission of a new drug application, or NDA, or biologics license application, or BLA, or other submission or to obtain regulatory approval in the United States or elsewhere;
- we may be unable to demonstrate to the FDA or comparable foreign regulatory authorities that a product candidate's risk-benefit ratio for its proposed indication is acceptable;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes, test procedures and specifications, or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

This lengthy approval process, as well as the unpredictability of the results of clinical studies, may result in our failing to obtain regulatory approval to market any of our product candidates, which would significantly harm our business, results of operations, and prospects.

Clinical drug development involves a lengthy and expensive process with an uncertain outcome, and results of earlier studies may not be predictive of future study results.

Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical study process. The results of preclinical studies and early clinical studies of our product candidates may not be predictive of the results of later-stage clinical studies. Product candidates that have shown promising results in early-stage clinical studies may still suffer significant setbacks in subsequent registration clinical studies. For example, the safety or efficacy results generated to date in clinical studies for KRN23, triheptanoin, and SA-ER do not ensure that later clinical studies will demonstrate similar results. There is a high failure rate for drugs and biologics proceeding through clinical studies, and product candidates in later stages of clinical studies may fail to show the desired safety and efficacy traits despite having progressed through preclinical studies and initial clinical studies. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical studies due to lack of efficacy or adverse safety profiles, notwithstanding promising results in earlier studies. Moreover, nonclinical and clinical data are often susceptible to varying interpretations and analyses. We do not know whether any Phase 2, Phase 3, or other clinical studies we may conduct will demonstrate consistent or adequate efficacy and safety sufficient to obtain regulatory approval to market our drug candidates.

We may find it difficult to enroll patients in our clinical studies given the limited number of patients who have the diseases for which our product candidates are being studied. Difficulty in enrolling patients could delay or prevent clinical studies of our product candidates.

Identifying and qualifying patients to participate in clinical studies of our product candidates is critical to our success. The timing of our clinical studies depends in part on the speed at which we can recruit patients to participate in testing our product candidates, and we may experience delays in our clinical studies if we encounter difficulties in enrollment.

Each of the conditions for which we plan to evaluate our current product candidates is a rare genetic disease. Accordingly, there are limited patient pools from which to draw for clinical studies. For our current product candidates:

- we estimate that several thousand patients in the United States suffer from XLH, for which KRN23 is being studied;
- we estimate that up to approximately 200 patients in the developed world may suffer from MPS 7, for which rhGUS is being studied;
- we estimate that several thousand patients in the United States suffer from LC-FAOD, for which triheptanoin is being studied;
- we estimate that several thousand patients in the United States suffer from Glut1 DS, for which triheptanoin is being studied; and
- we estimate that about 1,200 to 2,000 patients in the developed world suffer from HIBM, for which SA-ER is being studied.

In addition to the rarity of these diseases, the eligibility criteria of our clinical studies will further limit the pool of available study participants as we will require that patients have specific characteristics that we can measure or to assure their disease is either severe enough or not too advanced to include them in a study. Additionally, the process of finding and diagnosing patients may prove costly. We also may not be able to identify, recruit, and enroll a sufficient number of patients to complete our clinical studies because of the perceived risks and benefits of the product candidate under study, the availability and efficacy of competing therapies and clinical studies, the proximity and availability of clinical study sites for prospective patients, and the patient referral practices of physicians. If patients are unwilling to participate in our studies for any reason, the timeline for recruiting patients, conducting studies, and obtaining regulatory approval of potential products may be delayed.

If we experience delays in the completion of, or termination of, any clinical study of our product candidates, the commercial prospects of our product candidates will be harmed, and our ability to generate product revenue from any of these product candidates could be delayed or prevented. In addition, any delays in completing our clinical studies will increase our costs, slow down our product candidate development and approval process, and jeopardize our ability to commence product sales and generate revenue. Any of these occurrences may harm our business, financial condition, and prospects significantly. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical studies may also ultimately lead to the denial of regulatory approval of our product candidates.

We may encounter substantial delays in our clinical studies, or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical studies to demonstrate the safety and efficacy of the product candidates in humans. Clinical testing is expensive, time consuming, and uncertain as to outcome. We cannot guarantee that any clinical studies will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical studies can occur at any stage of testing, and our future clinical studies may not be successful. Events that may prevent successful or timely completion of clinical development include but are not limited to:

- inability to generate sufficient preclinical, toxicology, or other *in vivo* or *in vitro* data to support the initiation of human clinical studies;
- delays in reaching a consensus with regulatory agencies on study design;
- delays in reaching agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical study sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and clinical study sites;

- delays in obtaining required Institutional Review Board, or IRB, approval at each clinical study site;
- imposition of a clinical hold by regulatory agencies, after review of an investigational new drug, or IND, application or amendment, or equivalent application or amendment, or an inspection of our clinical study operations or study sites;
- delays in recruiting suitable patients to participate in our clinical studies;
- difficulty collaborating with patient groups and investigators;
- failure by our CROs, other third parties, or us to adhere to clinical study requirements;
- failure to perform in accordance with the FDA's good clinical practices requirements, or applicable regulatory guidelines in other countries;
- delays in having patients complete participation in a study or return for post-treatment follow-up;
- patients dropping out of a study;
- occurrence of adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;
- the cost of clinical studies of our drug candidates being greater than we anticipate;
- clinical studies of our drug candidates producing negative or inconclusive results, which may result in us deciding, or regulators requiring us, to conduct additional clinical studies or abandon drug development programs; and
- delays in manufacturing, testing, releasing, validating, or importing/exporting sufficient stable quantities of our product candidates for use in clinical studies or the inability to do any of the foregoing.

Any inability to successfully complete nonclinical and clinical development could result in additional costs to us or impair our ability to generate revenue. In addition, if we make manufacturing or formulation changes to our product candidates, such as our plan to manufacture a combination extended release and immediate release version of sialic acid, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical study delays could also shorten any periods during which our products have patent protection and may allow our competitors to bring products to market before we do, which could impair our ability to obtain orphan exclusivity and to successfully commercialize our product candidates and may harm our business and results of operations.

Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any.

Undesirable side effects caused by our product candidates could cause us or regulatory authorities to interrupt, delay, or halt clinical studies and could result in a more restrictive label or the delay or denial of regulatory approval by the FDA or other comparable foreign authorities. Patients treated with triheptanoin have experienced drug-related side effects such as cramping, diarrhea, and loose stools. In addition, during a 13-year study of approximately 150 human subjects, including 65 with LC-FAOD, three serious adverse events were classified as possibly related to triheptanoin treatment (muscle cell rupture and elevated creatine kinase reported for two subjects and myoglobinuria in one subject); however, these serious adverse events can be considered typical of the underlying disease. Additionally, patients treated with SA-ER have experienced drug-related side effects including mild gastrointestinal discomfort. While we have not initiated our own clinical studies for triheptanoin, there may be other side effects associated with its use that we discover. Only single-dose Phase 1

data for KRN23 has been reported to date. Other side effects may result from repeated dosing and/or longer-term exposure. Enzyme replacement therapies have been associated with infusion-associated reactions due to a developing allergy to the product, which can cause rashes, pain, significant clinical disease, or even death. Our rhGUS and rhPPCA product candidates may also cause these or similar side effects when clinical trials are initiated. Results of our studies could reveal a high and unacceptable severity and prevalence of these or other side effects. In such an event, our studies could be suspended or terminated, and the FDA or comparable foreign regulatory authorities could order us to cease further development of or deny or withdraw approval of our product candidates for any or all targeted indications.

The drug-related side effects could affect patient recruitment, the ability of enrolled patients to complete the study, or result in potential product liability claims. We currently carry product liability insurance in the amount of $5.0 million in the aggregate, and we are required to maintain product liability insurance pursuant to certain of our license agreements. We believe our product liability insurance coverage is sufficient in light of our current clinical programs; however, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses due to liability. A successful product liability claim or series of claims brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could adversely affect our results of operations and business. In addition, regardless of merit or eventual outcome, product liability claims may result in impairment of our business reputation, withdrawal of clinical study participants, costs due to related litigation, distraction of management's attention from our primary business, initiation of investigations by regulators, substantial monetary awards to patients or other claimants, the inability to commercialize our product candidates, and decreased demand for our product candidates, if approved for commercial sale.

Additionally, if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by such products, a number of potentially significant negative consequences could result, including but not limited to:

- regulatory authorities may withdraw approvals of such product;
- regulatory authorities may require additional warnings on the label;
- we may be required to create a Risk Evaluation and Mitigation Strategy, or REMS, plan, which could include a medication guide outlining the risks of such side effects for distribution to patients, a communication plan for healthcare providers, and/or other elements to assure safe use;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved, and could significantly harm our business, results of operations, and prospects.

Even if we obtain regulatory approval for a product candidate, our products will remain subject to regulatory scrutiny.

If our product candidates are approved, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping, conduct of post-marketing studies, and submission of safety, efficacy, and other post-market information, including both federal and state requirements in the United States and requirements of comparable foreign regulatory authorities.

Manufacturers and manufacturers' facilities are required to comply with extensive FDA, and comparable foreign regulatory authority, requirements, including ensuring that quality control and manufacturing procedures conform to current Good Manufacturing Practices, or cGMP, regulations. As such, we and our contract manufacturers will be subject to continual review and inspections to assess compliance with cGMP and adherence to commitments made in any NDA, BLA or marketing authorization application, or MAA. Accordingly, we and others with whom we work must continue to expend time, money, and effort in all areas of regulatory compliance, including manufacturing, production, and quality control.

Any regulatory approvals that we receive for our product candidates may be subject to limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase IV clinical trials, and surveillance to monitor the safety and efficacy of the product candidate. We will be required to report certain adverse reactions and production problems, if any, to the FDA and comparable foreign regulatory authorities. Any new legislation addressing drug safety issues could result in delays in product development or commercialization, or increased costs to assure compliance. We will have to comply with requirements concerning advertising and promotion for our products. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved label. As such, we may not promote our products for indications or uses for which they do not have approval. The holder of an approved NDA, BLA, or MAA must submit new or supplemental applications and obtain approval for certain changes to the approved product, product labeling, or manufacturing process. We could also be asked to conduct post-marketing clinical studies to verify the safety and efficacy of our products in general or in specific patient subsets. If original marketing approval were obtained via the accelerated approval pathway, we could be required to conduct a successful post-marketing clinical study to confirm clinical benefit for our products. An unsuccessful post-marketing study or failure to complete such a study could result in the withdrawal of marketing approval.

If a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, or disagrees with the promotion, marketing or labeling of a product, such regulatory agency may impose restrictions on that product or us, including requiring withdrawal of the product from the market. If we fail to comply with applicable regulatory requirements, a regulatory agency or enforcement authority may, among other things:

- issue warning letters;
- impose civil or criminal penalties;
- suspend or withdraw regulatory approval;
- suspend any of our ongoing clinical studies;
- refuse to approve pending applications or supplements to approved applications submitted by us;
- impose restrictions on our operations, including closing our contract manufacturers' facilities; or
- seize or detain products, or require a product recall.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response, and could generate negative publicity. Any failure to comply with ongoing regulatory requirements may significantly and adversely affect our ability to commercialize and generate revenue from our products. If regulatory sanctions are applied or if regulatory approval is withdrawn, the value of our company and our operating results will be adversely affected.

Risks Related to our Reliance on Third Parties

We rely on third parties to conduct our nonclinical and clinical studies and perform other tasks for us. If these third parties do not successfully carry out their contractual duties, meet expected deadlines, or comply with regulatory requirements, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business could be substantially harmed.

We have relied upon and plan to continue to rely upon third-party CROs to monitor and manage data for our ongoing nonclinical and clinical programs. We rely on these parties for execution of our nonclinical and clinical studies, and control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal, regulatory, and scientific standards and our reliance on the CROs does not relieve us of our regulatory responsibilities. We and our CROs and other vendors are required to comply with cGMP, current good clinical practices, or cGCP, and Good Laboratory Practices, or

GLP, which are regulations and guidelines enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area, or EEA, and comparable foreign regulatory authorities for all of our product candidates in clinical development. Regulatory authorities enforce these regulations through periodic inspections of study sponsors, principal investigators, study sites, and other contractors. If we or any of our CROs or vendors fail to comply with applicable regulations, the data generated in our nonclinical and clinical studies may be deemed unreliable and the FDA, EMA, or comparable foreign regulatory authorities may require us to perform additional nonclinical and clinical studies before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical studies comply with cGCP regulations. In addition, our clinical studies must be conducted with product produced under cGMP regulations. Our failure to comply with these regulations may require us to repeat clinical studies, which would delay the regulatory approval process.

If any of our relationships with these third-party CROs terminate, we may not be able to enter into arrangements with alternative CROs or do so on commercially reasonable terms. In addition, our CROs are not our employees, and except for remedies available to us under our agreements with such CROs, we cannot control whether or not they devote sufficient time and resources to our on-going nonclinical and clinical programs. If CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the data they obtain is compromised due to the failure to adhere to our protocols, regulatory requirements, or for other reasons, our clinical studies may be extended, delayed, or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. CROs may also generate higher costs than anticipated. As a result, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase, and our ability to generate revenue could be delayed.

Switching or adding additional CROs involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, there can be no assurance that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition, and prospects.

We are dependent on KHK for the development and commercialization of KRN23 in certain major markets, and KHK's failure to commercialize KRN23 in those markets would result in a material adverse effect on our business and operating results.

Under our agreement with KHK, KHK has the sole right to commercialize KRN23 in Europe and, at a specified time, in the United States and Canada subject to a limited promotion right retained by us. Our development partnership with KHK may not be successful, and we may not realize the expected benefits from such partnership, due to a number of important factors, including but not limited to the following:

- KHK has no obligation under our agreement to use diligent efforts to commercialize KRN23 in Europe. The timing and amount of any royalty payments we may receive under our agreement will depend on, among other things, the efforts, allocation of resources, and successful commercialization of KRN23 by KHK in Europe. Additionally, if KHK were to decide not to commercialize KRN23 in Europe, and we nevertheless wished to commercialize KRN23 in Europe, we would need to renegotiate with KHK certain terms of our agreement but may be unable to do so on reasonable terms, in a timely manner, or at all;
- the timing and amount of any royalty payments we may receive under our agreement with KHK will depend on, among other things, the efforts, allocation of resources, and successful commercialization of KRN23 by KHK in the United States and Canada under our agreement;
- KHK may change the focus of its commercialization efforts or pursue higher-priority programs;
- KHK may fail to manufacture or supply sufficient drug product of KRN23 for our development and clinical use, which could result in program delays;

- KHK may fail to manufacture or supply sufficient drug product of KRN23 for our commercial use, if approved, which could result in lost revenue;
- KHK may elect to develop and commercialize KRN23 indications with a larger market than XLH and at a lower price, thereby reducing the profit margin on sales of KRN23 for any orphan indications, including XLH;
- if KHK were to breach or terminate the agreement with us, we would no longer have any rights to develop or commercialize KRN23 or such rights would be limited to non-terminated countries;
- KHK may terminate its agreement with us, adversely impacting our potential revenue from licensed products; and
- the timing and amounts of expense reimbursement that we may receive are uncertain, and the total expenses for which we are obligated to reimburse KHK may be greater than anticipated.

We rely completely on third parties to manufacture our nonclinical and clinical drug supplies. Our business could be harmed if those third parties fail to provide us with sufficient quantities of drug product, or fail to do so at acceptable quality levels or prices.

We do not currently have, nor do we plan to acquire, the infrastructure or capability internally to manufacture our nonclinical and clinical drug supplies for use in the conduct of our clinical studies, and we lack the resources and the capability to manufacture any of our product candidates on a clinical or commercial scale. We rely on our manufacturers to purchase from third-party suppliers the materials necessary to produce our product candidates for our clinical studies. There are a limited number of suppliers for raw materials that we use to manufacture our drugs, and there may be a need to identify alternate suppliers to prevent a possible disruption of the manufacture of the materials necessary to produce our product candidates for our clinical studies, and, if approved, ultimately for commercial sale. We do not have any control over the process or timing of the acquisition of these raw materials by our manufacturers. Although we generally do not begin a clinical study unless we believe we have a sufficient supply of a product candidate to complete such study, any significant delay or discontinuity in the supply of a product candidate, or the raw material components thereof, for an ongoing clinical study due to the need to replace a third-party manufacturer could considerably delay completion of our clinical studies, product testing, and potential regulatory approval of our product candidates, which could harm our business and results of operations.

We are subject to a multitude of manufacturing risks, any of which could substantially increase our costs and limit supply of our product candidates.

The process of manufacturing our product candidates is complex, highly regulated, and subject to several risks, including but not limited to:

- the process of manufacturing our product candidates is extremely susceptible to product loss due to contamination, equipment failure or improper installation or operation of equipment, or vendor or operator error. Even minor deviations from normal manufacturing processes for any of our product candidates could result in reduced production yields, product defects, and other supply disruptions. If microbial, viral, or other contaminations are discovered in our product candidates or in the manufacturing facilities in which our product candidates are made, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination; and
- the manufacturing facilities in which our product candidates are made could be adversely affected by equipment failures, labor shortages, natural disasters, power failures, and numerous other factors.

Although we have not experienced any contaminations, equipment failures, or other similar manufacturing problems, any adverse developments affecting manufacturing operations for our product candidates may result in shipment delays, inventory shortages, lot failures, withdrawals or recalls, or other interruptions in the supply of

our product candidates. We may also have to take inventory write-offs and incur other charges and expenses for product candidates that fail to meet specifications, undertake costly remediation efforts, or seek more costly manufacturing alternatives.

The drug substance and drug product for our product candidates are currently acquired from single-source suppliers. The loss of these suppliers, or their failure to supply us with the drug substance or drug product, could materially and adversely affect our business.

The drug substance and drug product for KRN23 are made by KHK pursuant to our license and collaboration agreement with KHK. The drug substance and drug product for rhGUS are manufactured by Rentschler Biotechnologie GmbH under a development and clinical supply agreement and accompanying purchase orders. The pharmaceutical-grade drug substance for triheptanoin is manufactured by Cremer Oleo GmbH & Co. KG, or Cremer, pursuant to our supply agreement with Cremer, and the drug product for triheptanoin is prepared by Haupt Pharma AG pursuant to purchase orders. The drug substance for SA-ER is manufactured by Sanyo Fine Co., Ltd. under our license agreement and accompanying purchase orders with Nobelpharma Co., Ltd., and the drug product for SA-ER is manufactured by AAIPharma Services Corp., or AAIPharma, pursuant to our license agreement and accompanying purchase orders with AAIPharma. We do not currently have any other suppliers for the drug substance or drug product of our product candidates and, although we believe that there are alternate sources of supply that could satisfy our clinical and commercial requirements, we cannot assure you that identifying alternate sources and establishing relationships with such sources would not result in significant delay in the development of our product candidates. Additionally, we may not be able to enter into supply arrangements with alternative suppliers on commercially reasonable terms, or at all. A delay in the development of our product candidates or having to enter into a new agreement with a different third party on less favorable terms than we have with our current suppliers could have a material adverse impact upon on our business.

We and our collaborators and contract manufacturers are subject to significant regulation with respect to manufacturing our product candidates. The manufacturing facilities on which we rely may not continue to meet regulatory requirements or may not be able to meet supply demands.

All entities involved in the preparation of therapeutics for clinical studies or commercial sale, including our existing contract manufacturers for our product candidates, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical studies must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of contaminants or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We, our collaborators, or our contract manufacturers must supply all necessary documentation in support of an NDA, BLA, or MAA on a timely basis and must adhere to GLP and cGMP regulations enforced by the FDA and other regulatory agencies through their facilities inspection program. Some of our contract manufacturers have never produced a commercially approved pharmaceutical product and therefore have not obtained the requisite regulatory authority approvals to do so. The facilities and quality systems of some or all of our collaborators and third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of our product candidates or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted. Although we oversee the contract manufacturers, we cannot control the manufacturing process of, and are completely dependent on, our contract manufacturing partners for compliance with the regulatory requirements. If these facilities do not pass a pre-approval plant inspection, regulatory approval of the products may not be granted or may be substantially delayed until any violations are corrected to the satisfaction of the regulatory authority, if ever.

The regulatory authorities also may, at any time following approval of a product for sale, audit the manufacturing facilities of our collaborators and third-party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time consuming for us or a third party to implement, and that may include the temporary or permanent suspension of a clinical study or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could materially harm our business.

If we, our collaborators, or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA or other applicable regulatory authority can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new drug product or biologic product, withdrawal of an approval, or suspension of production. As a result, our business, financial condition, and results of operations may be materially harmed.

Additionally, if supply from one approved manufacturer is interrupted, an alternative manufacturer would need to be qualified through an NDA or BLA supplement or MAA variation, or equivalent foreign regulatory filing, which could result in further delay. The regulatory agencies may also require additional studies if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause us to incur higher costs and could cause the delay or termination of clinical studies, regulatory submissions, required approvals, or commercialization of our product candidates. Furthermore, if our suppliers fail to meet contractual requirements and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical studies may be delayed or we could lose potential revenue.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Because we rely on third parties to develop and manufacture our product candidates, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements, or other similar agreements with our collaborators, advisors, employees, and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, such as trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business.

Risks Related to Commercialization of Our Product Candidates

If the market opportunities for our product candidates are smaller than we believe they are, our revenue may be adversely affected, and our business may suffer. Because the target patient populations of our product candidates are small, we must be able to successfully identify patients and acquire a significant market share to achieve profitability and growth.

We focus our research and product development on treatments for rare and ultra-rare genetic diseases. Given the small number of patients who have the diseases that we are targeting, it is critical to our ability to grow and become profitable that we continue to successfully identify patients with these rare and ultra-rare genetic diseases. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on

our beliefs and estimates. These estimates have been derived from a variety of sources, including the scientific literature, surveys of clinics, patient foundations, or market research, and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these diseases. The number of patients may turn out to be lower than expected. The effort to identify patients with diseases we seek to treat is in early stages, and we cannot accurately predict the number of patients for whom treatment might be possible. Additionally, the potentially addressable patient population for each of our product candidates may be limited or may not be amenable to treatment with our product candidates, and new patients may become increasingly difficult to identify or gain access to, which would adversely affect our results of operations and our business. Further, even if we obtain significant market share for our product candidates, because the potential target populations are very small, we may never achieve profitability despite obtaining such significant market share.

We intend to rely on third-party manufacturers to produce our product candidates, but we have not entered into binding agreements with any such manufacturers to support commercialization. Additionally, these manufacturers do not have experience producing our product candidates at commercial levels and may not achieve the necessary regulatory approvals or produce our product candidates at the quality, quantities, locations, and timing needed to support commercialization.

We have not yet secured manufacturing capabilities for commercial quantities of our product candidates. Although we intend to rely on third-party manufacturers for commercialization, we have only entered into agreements with such manufacturers to support our clinical studies. We may be unable to negotiate binding agreements with the manufacturers to support our commercialization activities at commercially reasonable terms.

Manufacturers may not have the experience or ability to produce our product candidates at commercial levels. We may run into technical or scientific issues related to manufacturing or development that we may be unable to resolve in a timely manner or with available funds. We also have not completed all of the characterization and validation activities necessary for commercialization and regulatory approvals. If our manufacturing partners do not conduct all such necessary activities in accordance with applicable regulations, our commercialization efforts will be harmed.

Even if our third-party product manufacturers develop an acceptable manufacturing process, if such third-party manufacturers are unable to produce the necessary quantities of our product candidates, or in compliance with cGMP or other pertinent regulatory requirements, and within our planned timeframe and cost parameters, the development and sales of our products, if approved, may be materially harmed.

We face intense competition and rapid technological change and the possibility that our competitors may develop therapies that are similar, more advanced, or more effective than ours, which may adversely affect our financial condition and our ability to successfully commercialize our product candidates.

The biotechnology and pharmaceutical industries are intensely competitive and subject to rapid and significant technological change. We are currently aware of various existing therapies that may compete with our product candidates. For example, XLH is currently treated with oral phosphate and Vitamin D therapy, which may compete with KRN23. Furthermore, B. Braun Medical Inc., or B. Braun, has received orphan drug designation for triheptanoin in Europe for certain LC-FAOD indications and we believe that B. Braun is evaluating whether or not to initiate clinical development; triheptanoin is also available and is currently being studied in food-grade form, which may compete with our pharmaceutical-grade product. LC-FAOD is currently treated with diet therapy and medium-chain triglyceride oil, which may compete with triheptanoin. Glut1 DS is currently treated primarily with the ketogenic diet and anti-epileptic drugs, which may also compete with triheptanoin. Additionally, we are aware of a program at the National Institutes of Health, whose intellectual property rights are licensed to a company in New Zealand that is investigating the use of another metabolite in the sialic acid pathway, ManNAc, for the treatment of HIBM, which could compete with SA-ER. ManNAc may have a potential advantage over SA-ER in that it is not a charged molecule like sialic acid is, which might improve its distribution and uptake. Gene therapy and other approaches may also emerge for the treatment of any of the disease areas in which we focus.

We have competitors both in the United States and internationally, including major multinational pharmaceutical companies, specialty pharmaceutical companies, and biotechnology companies. Some of the pharmaceutical and biotechnology companies we expect to compete with include Shire, Sanofi, BioMarin, Alexion, and Roche, as well as other smaller companies or biotechnology startups and large multinational pharmaceutical companies. Many of our competitors have substantially greater financial, technical, and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations. Additional mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. As a result, these companies may obtain regulatory approval more rapidly than we are able to and may be more effective in selling and marketing their products as well. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors may succeed in developing, acquiring, or licensing on an exclusive basis, products that are more effective or less costly than any product candidate that we may develop, or achieve earlier patent protection, regulatory approval, product commercialization, and market penetration than we do. Additionally, technologies developed by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against competitors.

We currently have no marketing and sales organization. If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, we may be unable to generate any revenue.

Although our employees may have sold other similar products in the past while employed at other companies, we as a company have no experience selling and marketing our product candidates and we currently have no marketing or sales organization. To successfully commercialize any products that may result from our development programs, we will need to develop these capabilities, either on our own or with others. If our product candidates receive regulatory approval, we intend to establish a sales and marketing organization with technical expertise and supporting distribution capabilities to commercialize our product candidates in major markets, which will be expensive, difficult, and time consuming. Any failure or delay in the development of our internal sales, marketing, and distribution capabilities would adversely impact the commercialization of our products.

Further, given our lack of prior experience in marketing and selling biopharmaceutical products, our initial estimate of the size of the required sales force may be materially more or less than the size of the sales force actually required to effectively commercialize our product candidates. As such, we may be required to hire substantially more sales representatives to adequately support the commercialization of our product candidates or we may incur excess costs as a result of hiring more sales representatives than necessary. With respect to certain geographical markets, we may enter into collaborations with other entities to utilize their local marketing and distribution capabilities, but we may be unable to enter into such agreements on favorable terms, if at all. If our future collaborators do not commit sufficient resources to commercialize our future products, if any, and we are unable to develop the necessary marketing capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We may be competing with companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

The commercial success of any current or future product candidate will depend upon the degree of market acceptance by physicians, patients, third-party payors, and others in the medical community.

Even with the requisite approvals from the FDA and comparable foreign regulatory authorities, the commercial success of our product candidates will depend in part on the medical community, patients, and third-party payors accepting our product candidates as medically useful, cost-effective, and safe. Any product that we bring to the market may not gain market acceptance by physicians, patients, third-party payors, and others in the

medical community. The degree of market acceptance of any of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy of the product as demonstrated in clinical studies and potential advantages over competing treatments;
- the prevalence and severity of any side effects, including any limitations or warnings contained in a product's approved labeling;
- the clinical indications for which approval is granted;
- relative convenience and ease of administration;
- the cost of treatment, particularly in relation to competing treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support and timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party insurance coverage and reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in nonclinical and clinical studies, market acceptance of the product will not be fully known until after it is launched. Our efforts to educate the medical community and third-party payors on the benefits of the product candidates may require significant resources and may never be successful. If our product candidates are approved but fail to achieve an adequate level of acceptance by physicians, patients, third-party payors, and others in the medical community, we will not be able to generate sufficient revenue to become or remain profitable.

The insurance coverage and reimbursement status of newly-approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for new or current products could limit our ability to market those products and decrease our ability to generate revenue.

Our target patient populations are small, and accordingly the pricing, coverage, and reimbursement of our product candidates, if approved, must be adequate to support our commercial infrastructure. Our per-patient prices must be sufficient to recover our development and manufacturing costs and potentially achieve profitability. Accordingly, the availability and adequacy of coverage and reimbursement by governmental and private payors are essential for most patients to be able to afford expensive treatments such as ours, assuming approval. Sales of our product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid for by health maintenance, managed care, pharmacy benefit, and similar healthcare management organizations, or reimbursed by government authorities, private health insurers, and other third-party payors. If coverage and reimbursement are not available, or are available only to limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a return on our investment.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, the principal decisions about coverage and reimbursement for new drugs are typically made by the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, as CMS decides whether and to what extent a new drug will be covered and reimbursed under Medicare. Private payors tend to follow the coverage reimbursement policies established by CMS to a substantial degree. It is difficult to predict what CMS will decide with respect to reimbursement for products such as ours.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in

Europe, Canada, and other countries has and will continue to put pressure on the pricing and usage of our product candidates. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. Other countries allow companies to fix their own prices for medicinal products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our products may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenue and profits.

Moreover, increasing efforts by governmental and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations, and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain effective patent rights for our product candidates or any future product candidates, we may not be able to compete effectively in our markets.

We rely upon a combination of patents, trade secret protection, and confidentiality agreements to protect the intellectual property related to our technologies and product candidates. Our success depends in large part on our and our licensors' ability to obtain and maintain patent and other intellectual property protection in the United States and in other countries with respect to our proprietary technology and products.

We have sought to protect our proprietary position by filing patent applications in the United States and abroad related to our novel technologies and products that are important to our business. This process is expensive and time consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain and involves complex legal and factual questions for which legal principles remain unsolved. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates in the United States or in other foreign countries. There is no assurance that all potentially relevant prior art relating to our patents and patent applications has been found, which can invalidate a patent or prevent a patent from issuing from a pending patent application. Even if patents do successfully issue, and even if such patents cover our product candidates, third parties may challenge their validity, enforceability, or scope, which may result in such patents being narrowed, found unenforceable or invalidated. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual property, provide exclusivity for our product candidates, or prevent others from designing around our claims. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

We, independently or together with our licensors, have filed several patent applications covering various aspects of our product candidates. We cannot offer any assurances about which, if any, patents will issue, the breadth of any such patent or whether any issued patents will be found invalid and unenforceable or will be threatened by third parties. Any successful opposition to these patents or any other patents owned by or licensed to us after patent issuance could deprive us of rights necessary for the successful commercialization of any product candidates that we may develop. Further, if we encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced.

Although we have a number of patents covering methods of use and certain compositions of matter, we do not have complete patent protection for our product candidates. For example, there is no patent coverage for

KRN23 in Latin America, where we have rights to commercialize the compound. Therefore, a competitor could develop the same or similar antibody as well as other approaches that target FGF23. Additionally, none of the current intellectual property relating to rhGUS covers composition of matter, and there are currently no patents that cover rhPPCA. Therefore, it is possible that a competitor could develop the same or similar enzyme with respect to rhGUS and/or rhPPCA, subject to any regulatory exclusivities. With respect to triheptanoin, although some of the patents relating to triheptanoin cover aspects of composition of matter, it is possible that a competitor could develop the same or similar molecule. With respect to SA-ER, none of the patents relating to SA-ER cover composition of matter. Therefore, it is possible that a competitor could develop the same or similar molecule. If we cannot obtain and maintain effective patent rights for our product candidates, we may not be able to compete effectively and our business and results of operations would be harmed.

We may not have sufficient patent terms to effectively protect our products and business.

Patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after it is filed. Although various extensions may be available, the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired for a product, we may be open to competition from generic medications.

While patent term extensions under the Hatch-Waxman Act in the United States and under supplementary protection certificates in Europe may be available to extend the patent exclusivity term for KRN23, rhGUS, triheptanoin, and SA-ER, we cannot provide any assurances that any such patent term extension will be obtained and, if so, for how long. In addition, upon issuance in the United States any patent term can be adjusted based on certain delays caused by the applicant(s) or the United States Patent and Trademark Office, or USPTO. For example, a patent term can be reduced based on certain delays caused by the patent applicant during patent prosecution. If we do not have sufficient patent terms or regulatory exclusivity to protect our products, our business and results of operations will be adversely affected.

Patent policy and rule changes could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. The laws of foreign countries may not protect our rights to the same extent as the laws of the United States. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. We therefore cannot be certain that we or our licensors were the first to make the invention claimed in our owned and licensed patents or pending applications, or that we or our licensor were the first to file for patent protection of such inventions. Assuming the other requirements for patentability are met, in the United States prior to March 15, 2013, the first to make the claimed invention is entitled to the patent, while outside the United States, the first to file a patent application is entitled to the patent. After March 15, 2013, under the Leahy-Smith America Invents Act, or the Leahy-Smith Act, enacted on September 16, 2011, the United States has moved to a first to file system. The Leahy-Smith Act also includes a number of significant changes that affect the way patent applications will be prosecuted and may also affect patent litigation. The effects of these changes are currently unclear as the USPTO must still implement various regulations, the courts have yet to address any of these provisions and the applicability of the act and new regulations on specific patents discussed herein have not been determined and would need to be reviewed. In general, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

If we are unable to maintain effective proprietary rights for our product candidates or any future product candidates, we may not be able to compete effectively in our markets.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors, and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

Although we expect all of our employees and consultants to assign their inventions to us, and all of our employees, consultants, advisors, and any third parties who have access to our proprietary know-how, information, or technology to enter into confidentiality agreements, we cannot provide any assurances that all such agreements have been duly executed or that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Misappropriation or unauthorized disclosure of our trade secrets could impair our competitive position and may have a material adverse effect on our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret.

Third-party claims of intellectual property infringement may prevent or delay our development and commercialization efforts.

Our commercial success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. There have been many lawsuits and other proceedings involving patent and other intellectual property rights in the biotechnology and pharmaceutical industries, including patent infringement lawsuits, interferences, oppositions, and reexamination proceedings before the USPTO and corresponding foreign patent offices. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may be subject to claims of infringement of the patent rights of third parties.

Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture, or methods for treatment related to the use or manufacture of our product candidates. We have conducted freedom to operate analyses with respect to only certain of our product candidates, and therefore we do not know whether there are any third-party patents that would impair our ability to commercialize these product candidates. We also cannot guarantee that any of our analyses are complete and thorough, nor can we be sure that we have identified each and every patent and pending application in the United States and abroad that is relevant or necessary to the commercialization of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our product candidates may infringe.

In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover aspects of our formulations, the manufacturing process of any of our product candidates, methods of use, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire or are finally determined to be invalid or unenforceable. Such a license may not be available on commercially reasonable terms, or at all.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

We may not be successful in obtaining or maintaining necessary rights to our product candidates through acquisitions and in-licenses.

We currently have rights to the intellectual property, through licenses from third parties and under patents that we own, to develop our product candidates. Because our programs may require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to acquire, in-license, or use these proprietary rights. For example, our product candidates may require specific formulations to work effectively and efficiently and the rights to these formulations may be held by others. We may be unable to acquire or in-license any compositions, methods of use, processes, or other third-party intellectual property rights from third parties that we identify as necessary for our product candidates. The licensing and acquisition of third-party intellectual property rights is a competitive area, and a number of more established companies are also pursuing strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources, and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment.

We sometimes collaborate with U.S. and foreign academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program.

If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of that program and our business and financial condition could suffer.

We may face competition from biosimilars, which may have a material adverse impact on the future commercial prospects of KRN23, rhGUS, and rhPPCA.

Even if we are successful in achieving regulatory approval to commercialize a product candidate faster than our competitors, we may face competition from biosimilars with respect to KRN23, rhGUS, and rhPPCA. In the United States, the Biologics Price Competition and Innovation Act of 2009 created an abbreviated approval pathway for biological products that are demonstrated to be "highly similar," or biosimilar, to or "interchangeable" with an FDA-approved biological product. This new pathway could allow competitors to reference data from innovative biological products 12 years after the time of approval of the innovative biological product. This data exclusivity does not prevent another company from developing a product that is highly similar to the innovative product, generating its own data, and seeking approval. Data exclusivity only assures that another company cannot rely upon the data within the innovator's application to support the biosimilar product's approval. In his proposed budget for fiscal year 2014, President Obama proposed to cut this 12-year period of exclusivity down to seven years. He also proposed to prohibit additional periods of exclusivity due to minor changes in product formulations, a practice often referred to as "evergreening." It is possible that Congress may take these or other measures to reduce or eliminate periods of exclusivity. The Biologics Price Competition and Innovation Act of 2009 is complex and only beginning to be interpreted and implemented by

the FDA. As a result, its ultimate impact, implementation, and meaning is subject to uncertainty. While it is uncertain when any such processes may be fully adopted by the FDA, any such processes could have a material adverse effect on the future commercial prospects for KRN23, rhGUS, and rhPPCA.

In Europe, the European Commission has granted marketing authorizations for several biosimilars pursuant to a set of general and product class-specific guidelines for biosimilar approvals issued over the past few years. In Europe, a competitor may reference data supporting approval of an innovative biological product, but will not be able to get on the market until 10 years after the time of approval of the innovative product. This 10-year marketing exclusivity period will be extended to 11 years if, during the first eight of those 10 years, the marketing authorization holder obtains an approval for one or more new therapeutic indications that bring significant clinical benefits compared with existing therapies. In addition, companies may be developing biosimilars in other countries that could compete with our products.

If competitors are able to obtain marketing approval for biosimilars referencing our products, our products may become subject to competition from such biosimilars, with the attendant competitive pressure and consequences.

Additional competitors could enter the market with generic versions of our small-molecule product candidates, which may result in a material decline in sales of triheptanoin and SA-ER.

Under the Hatch-Waxman Act, a pharmaceutical manufacturer may file an abbreviated new drug application, or ANDA, seeking approval of a generic copy of an approved innovator product. Under the Hatch-Waxman Act, a manufacturer may also submit an NDA under section 505(b)(2) that references the FDA's finding of safety and effectiveness of a previously approved drug. A 505(b)(2) NDA product may be for a new or improved version of the original innovator product. Innovative small molecule drugs may be eligible for certain periods of regulatory exclusivity (e.g., five years for new chemical entities, three years for changes to an approved drug requiring a new clinical study, seven years for orphan drugs), which preclude FDA approval (or in some circumstances, FDA filing and review of) an ANDA or 505(b)(2) NDA relying on the FDA's finding of safety and effectiveness for the innovative drug. In addition to the benefits of regulatory exclusivity, an innovator NDA holder may have patents claiming the active ingredient, product formulation or an approved use of the drug, which would be listed with the product in the FDA publication, "Approved Drug Products with Therapeutic Equivalence Evaluations," known as the "Orange Book." If there are patents listed in the Orange Book, a generic applicant that seeks to market its product before expiration of the patents must include in the ANDA or 505(b)(2) what is known as a "Paragraph IV certification," challenging the validity or enforceability of, or claiming non-infringement of, the listed patent or patents. Notice of the certification must be given to the innovator, too, and if within 45 days of receiving notice the innovator sues to protect its patents, approval of the ANDA is stayed for 30 months, or as lengthened or shortened by the court.

Accordingly, if triheptanoin and SA-ER are approved, competitors could file ANDAs for generic versions of triheptanoin and SA-ER, or 505(b)(2) NDAs that reference triheptanoin and SA-ER, respectively. If there are patents listed for triheptanoin and SA-ER in the Orange Book, those ANDAs and 505(b)(2) NDAs would be required to include a certification as to each listed patent indicating whether the ANDA applicant does or does not intend to challenge the patent. We cannot predict whether any patents issuing from our pending patent applications will be eligible for listing in the Orange Book, how any generic competitor would address such patents, whether we would sue on any such patents, or the outcome of any such suit.

We may not be successful in securing or maintaining proprietary patent protection for products and technologies we develop or license. Moreover, if any patents that are granted and listed in the Orange Book are successfully challenged by way of a Paragraph IV certification and subsequent litigation, the affected product could more immediately face generic competition and its sales would likely decline materially. Should sales decline, we may have to write off a portion or all of the intangible assets associated with the affected product and our results of operations and cash flows could be materially and adversely affected.

The patent protection and patent prosecution for some of our product candidates is dependent on third parties.

While we normally seek and gain the right to fully prosecute the patents relating to our product candidates, there may be times when patents relating to our product candidates are controlled by our licensors. This is the case with our agreement with KHK, who is primarily responsible for the prosecution of patents and patent applications licensed to us under the collaboration agreement. If KHK or any of our future licensing partners fail to appropriately prosecute and maintain patent protection for patents covering any of our product candidates, our ability to develop and commercialize those product candidates may be adversely affected and we may not be able to prevent competitors from making, using, and selling competing products. In addition, even where we now have the right to control patent prosecution of patents and patent applications we have licensed from third parties, we may still be adversely affected or prejudiced by actions or inactions of our licensors and their counsel that took place prior to us assuming control over patent prosecution.

If we fail to comply with our obligations in the agreements under which we license intellectual property and other rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

We are a party to a number of intellectual property license agreements that are important to our business and expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, milestone payment, royalty, and other obligations on us. If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, we may be required to make certain payments to the licensor, we may lose the exclusivity of our license, or the licensor may have the right to terminate the license, in which event we would not be able to develop or market products covered by the license. Additionally, the milestone and other payments associated with these licenses will make it less profitable for us to develop our drug candidates. See “Business—License Agreements” for a description of our license agreements with KHK, Baylor Research Institute, Nobelpharma, AAIPharma, HIBM Research Group, St. Louis University and St. Jude Children’s Research Hospital, which includes a description of the termination provisions of these agreements.

In some cases, patent prosecution of our licensed technology is controlled solely by the licensor. If our licensors fail to obtain and maintain patent or other protection for the proprietary intellectual property we license from them, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, and our competitors could market competing products using the intellectual property. In certain cases, we control the prosecution of patents resulting from licensed technology. In the event we breach any of our obligations related to such prosecution, we may incur significant liability to our licensing partners. Licensing of intellectual property is of critical importance to our business and involves complex legal, business, and scientific issues. Disputes may arise regarding intellectual property subject to a licensing agreement, including but not limited to:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our collaborators; and
- the priority of invention of patented technology.

If disputes over intellectual property and other rights that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

Although we are not currently involved in any litigation, we may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time consuming, and unsuccessful.

Competitors may infringe our patents or the patents of our licensors. Although we are not currently involved in any litigation, if we or one of our licensing partners were to initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO, or made a misleading statement, during prosecution. The outcome following legal assertions of invalidity and unenforceability is unpredictable.

Interference proceedings provoked by third parties or brought by us or declared by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Our defense of litigation or interference proceedings may fail and, even if successful, may result in substantial costs and distract our management and other employees. In addition, the uncertainties associated with litigation could have a material adverse effect on our ability to raise the funds necessary to continue our clinical trials, continue our research programs, license necessary technology from third parties, or enter into development partnerships that would help us bring our product candidates to market.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. There could also be public announcements of the results of hearings, motions, or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of our common stock.

We may be subject to claims that our employees, consultants, or independent contractors have wrongfully used or disclosed confidential information of third parties or that our employees have wrongfully used or disclosed alleged trade secrets of their former employers.

We employ individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants, and independent contractors do not use the proprietary information or know-how of others in their work for us, and we are not currently subject to any claims that our employees, consultants, or independent contractors have wrongfully used or disclosed confidential information of third parties, we may in the future be subject to such claims. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel, which could adversely impact our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

We may be subject to claims challenging the inventorship of our patents and other intellectual property.

Although we are not currently experiencing any claims challenging the inventorship of our patents or ownership of our intellectual property, we may in the future be subject to claims that former employees, collaborators or other third parties have an interest in our patents or other intellectual property as an inventor or co-inventor. For example, we may have inventorship disputes arise from conflicting obligations of consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if

we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology industry involves both technological and legal complexity. Therefore, obtaining and enforcing biotechnology patents is costly, time consuming, and inherently uncertain. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on future actions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting, and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Further, licensing partners such as KHK may not prosecute patents in certain jurisdictions in which we may obtain commercial rights, thereby precluding the possibility of later obtaining patent protection in these countries. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and may also export infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets, and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Risks Related to Our Business Operations

Our future success depends in part on our ability to retain our Founder, President, and Chief Executive Officer and to attract, retain, and motivate other qualified personnel.

We are highly dependent on Emil D. Kakkis, M.D., Ph.D., our Founder, President, and Chief Executive Officer, the loss of whose services may adversely impact the achievement of our objectives. Dr. Kakkis could

leave our employment at any time, as he is an "at will" employee. Recruiting and retaining other qualified employees, consultants, and advisors for our business, including scientific and technical personnel, will also be critical to our success. There is currently a shortage of skilled personnel in our industry, which is likely to continue. As a result, competition for skilled personnel is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for individuals with similar skill sets. In addition, failure to succeed in preclinical or clinical studies may make it more challenging to recruit and retain qualified personnel. The inability to recruit and retain qualified personnel, or the loss of the services of Dr. Kakkis, may impede the progress of our research, development, and commercialization objectives.

If we fail to obtain or maintain orphan drug exclusivity for our products, our competitors may sell products to treat the same conditions and our revenue will be reduced.

Our business strategy focuses on the development of drugs that are eligible for FDA and EU orphan drug designation. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is intended to treat a rare disease or condition, defined as a patient population of fewer than 200,000 in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the European Union, the EMA's Committee for Orphan Medicinal Products, or COMP, grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention, or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union. Additionally, designation is granted for products intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating or serious and chronic condition when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biological product or where there is no satisfactory method of diagnosis, prevention, or treatment, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition.

In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages, and user-fee waivers. In addition, if a product receives the first FDA approval for the indication for which it has orphan designation, the product is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity or where the manufacturer is unable to assure sufficient product quantity. In the European Union, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity following drug or biological product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity.

Because the extent and scope of patent protection for our products may in some cases be limited, orphan drug designation is especially important for our products for which orphan drug designation may be available. For eligible drugs, we plan to rely on the exclusivity period under the Orphan Drug Act to maintain a competitive position. If we do not obtain orphan drug exclusivity for our drug products and biologic products that do not have broad patent protection, our competitors may then sell the same drug to treat the same condition sooner than if we had obtained orphan drug exclusivity and our revenue will be reduced.

Even though we have orphan drug designation for KRN23 in the United States, and rhGUS and SA-ER in the United States and Europe, we may not be the first to obtain marketing approval for any particular orphan indication due to the uncertainties associated with developing pharmaceutical products. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs with different active moieties can be approved for the same condition. Even after an orphan drug is approved, the FDA or EMA can subsequently approve the same drug with the same active moiety for the same condition if the FDA or EMA concludes that the later drug is safer, more effective, or makes

a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

We will need to expand our organization and we may experience difficulties in managing this growth, which could disrupt our operations.

As of September 30, 2013, we had 46 full-time employees. As our development and commercialization plans and strategies develop, we expect to need additional managerial, operational, sales, marketing, financial, legal, and other resources. Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees, and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize product candidates and compete effectively will depend, in part, on our ability to effectively manage any future growth.

We may not be successful in our efforts to identify, license, discover, develop, or commercialize additional product candidates.

Although a substantial amount of our effort will focus on the continued clinical testing, potential approval, and commercialization of our existing product candidates, the success of our business also depends upon our ability to identify, license, discover, develop, or commercialize additional product candidates. Research programs to identify new product candidates require substantial technical, financial, and human resources. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful. Our research programs or licensing efforts may fail to yield additional product candidates for clinical development and commercialization for a number of reasons, including but not limited to the following:

- our research or business development methodology or search criteria and process may be unsuccessful in identifying potential product candidates;
- we may not be able or willing to assemble sufficient resources to acquire or discover additional product candidates;
- our product candidates may not succeed in preclinical or clinical testing;
- our potential product candidates may be shown to have harmful side effects or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval;
- competitors may develop alternatives that render our product candidates obsolete or less attractive;
- product candidates we develop may be covered by third parties' patents or other exclusive rights;
- the market for a product candidate may change during our program so that such a product may become unreasonable to continue to develop;
- a product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; and
- a product candidate may not be accepted as safe and effective by patients, the medical community, or third-party payors.

If any of these events occur, we may be forced to abandon our development efforts for a program or programs, or we may not be able to identify, license, discover, develop, or commercialize additional product candidates, which would have a material adverse effect on our business and could potentially cause us to cease operations.

We will incur significant increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.

As a public company, we will incur significant legal, accounting, and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act, as well as rules subsequently implemented by the Securities and Exchange Commission, or SEC, and The NASDAQ Global Market have imposed various requirements on public companies. In July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as "say on pay" and pay parity. Recent legislation permits smaller "emerging growth companies" to implement many of these requirements over a longer period and up to five years from the pricing of this offering. We intend to take advantage of this new legislation but cannot guarantee that we will not be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment, and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time consuming and costly. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain our current levels of such coverage.

The Sarbanes-Oxley Act requires, among other things, that we maintain effective internal controls for financial reporting and disclosure controls and procedures. In particular, we will be required to perform system and process evaluation and testing of our internal controls over financial reporting to allow management to report, commencing in our annual report on Form 10-K for the year ending December 31, 2014, on the effectiveness of our internal controls over financial reporting, if then required by Section 404 of the Sarbanes-Oxley Act. Our testing may reveal deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses. Our compliance with Section 404 will require that we incur substantial accounting expense and expend significant management efforts. We currently do not have an internal audit group, and we will need to hire additional accounting and financial staff with appropriate public company experience and technical accounting knowledge. Moreover, if we are not able to comply with the requirements of Section 404 in a timely manner or if we identify or our independent registered public accounting firm identifies deficiencies in our internal controls over financial reporting that are deemed to be material weaknesses, the market price of our stock could decline and we could be subject to sanctions or investigations by NASDAQ, the SEC, or other regulatory authorities, which would require additional financial and management resources.

New laws and regulations as well as changes to existing laws and regulations affecting public companies, including the provisions of the Sarbanes-Oxley Act and rules adopted by the SEC and by NASDAQ, would likely result in increased costs to us as we respond to their requirements.

Healthcare legislative reform measures may have a material adverse effect on our business and results of operations.

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or the Health Care Reform Law, was passed, which substantially changes the way health care is financed by both governmental and private insurers, and significantly impacts the U.S. pharmaceutical industry. The Health Care Reform Law, among other things, subjects biologic products to potential competition by lower-cost biosimilars, addresses a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted, or injected, increases the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extends the rebate program to individuals enrolled in Medicaid managed

care organizations, establishes annual fees and taxes on manufacturers of certain branded prescription drugs, and promotes a new Medicare Part D coverage gap discount program.

In addition, other legislative changes have been proposed and adopted in the United States since the Health Care Reform Law was enacted. On August 2, 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers up to 2% per fiscal year. On January 2, 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, or the ATRA, which, among other things, delayed for another two months the budget cuts mandated by these sequestration provisions of the Budget Control Act of 2011. On March 1, 2013, the President signed an executive order implementing sequestration, and on April 1, 2013, the 2% Medicare payment reductions went into effect. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

We may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws, and health information privacy and security laws. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

If we obtain FDA approval for any of our product candidates and begin commercializing those products in the United States, our operations may be directly, or indirectly through our customers, subject to various federal and state fraud and abuse laws, including, without limitation, the federal Anti-Kickback Statute, the federal False Claims Act, and physician sunshine laws and regulations. These laws may impact, among other things, our proposed sales, marketing, and education programs. In addition, we may be subject to patient privacy regulation by both the federal government and the states in which we conduct our business. The laws that may affect our ability to operate include:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, to induce, or in return for, the purchase or recommendation of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs;
- federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid, or other third-party payors that are false or fraudulent;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal criminal statutes that prohibit executing a scheme to defraud any healthcare benefit program and making false statements relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology and Clinical Health Act, or HITECH, and its implementing regulations, which imposes certain requirements relating to the privacy, security, and transmission of individually identifiable health information;
- the federal physician sunshine requirements under the Health Care Reform Laws requires manufacturers of drugs, devices, biologics, and medical supplies to report annually to the U.S. Department of Health and Human Services information related to payments and other transfers of value to physicians, other healthcare providers, and teaching hospitals, and ownership and investment interests held by physicians and other healthcare providers and their immediate family members and applicable group purchasing organizations; and
- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws that may apply to items or services reimbursed by any third-party payor, including commercial insurers, state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary

compliance guidelines and the relevant compliance guidance promulgated by the federal government, or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures, and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. In addition, recent health care reform legislation has strengthened these laws. For example, the Health Care Reform Law, among other things, amends the intent requirement of the federal anti-kickback and criminal healthcare fraud statutes. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. Moreover, the Health Care Reform Law provides that the government may assert that a claim including items or services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the False Claims Act.

If our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, imprisonment, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

International expansion of our business exposes us to business, regulatory, political, operational, financial, and economic risks associated with doing business outside of the United States.

We currently have limited international operations, but our business strategy incorporates potentially significant international expansion, particularly in anticipation of approval of our product candidates. We plan to maintain sales representatives and conduct physician and patient association outreach activities, as well as clinical trials, outside of the United States. Doing business internationally involves a number of risks, including but not limited to:

- multiple, conflicting, and changing laws and regulations such as privacy regulations, tax laws, export and import restrictions, employment laws, regulatory requirements, and other governmental approvals, permits, and licenses;
- failure by us to obtain and maintain regulatory approvals for the use of our products in various countries;
- additional potentially relevant third-party patent rights;
- complexities and difficulties in obtaining protection and enforcing our intellectual property;
- difficulties in staffing and managing foreign operations;
- complexities associated with managing multiple payor reimbursement regimes, government payors, or patient self-pay systems;
- limits in our ability to penetrate international markets;
- financial risks, such as longer payment cycles, difficulty collecting accounts receivable, the impact of local and regional financial crises on demand and payment for our products, and exposure to foreign currency exchange rate fluctuations;
- natural disasters, political and economic instability, including wars, terrorism, and political unrest, outbreak of disease, boycotts, curtailment of trade, and other business restrictions;
- certain expenses including, among others, expenses for travel, translation, and insurance; and

- regulatory and compliance risks that relate to maintaining accurate information and control over sales and activities that may fall within the purview of the U.S. Foreign Corrupt Practices Act, or FCPA, its books and records provisions, or its anti-bribery provisions.

Any of these factors could significantly harm our future international expansion and operations and, consequently, our results of operations.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

Our research and development activities and our third-party manufacturers' and suppliers' activities involve the controlled storage, use, and disposal of hazardous materials, including the components of our product candidates and other hazardous compounds. We and our manufacturers and suppliers are subject to laws and regulations governing the use, manufacture, storage, handling, and disposal of these hazardous materials. In some cases, these hazardous materials and various wastes resulting from their use are stored at our and our manufacturers' facilities pending their use and disposal. We cannot eliminate the risk of contamination, which could cause an interruption of our commercialization efforts, research and development efforts and business operations, environmental damage resulting in costly clean-up and liabilities under applicable laws and regulations governing the use, storage, handling, and disposal of these materials and specified waste products. Although we believe that the safety procedures utilized by us and our third-party manufacturers for handling and disposing of these materials generally comply with the standards prescribed by these laws and regulations, we cannot guarantee that this is the case or eliminate the risk of accidental contamination or injury from these materials. In such an event, we may be held liable for any resulting damages and such liability could exceed our resources and state or federal or other applicable authorities may curtail our use of certain materials and/or interrupt our business operations. Furthermore, environmental laws and regulations are complex, change frequently, and have tended to become more stringent. We cannot predict the impact of such changes and cannot be certain of our future compliance. We do not currently carry biological or hazardous waste insurance coverage.

We or the third parties upon whom we depend may be adversely affected by earthquakes or other natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Our corporate headquarters and laboratory are located in the San Francisco Bay Area, and our collaboration partner for KRN23, KHK, is located in Japan, which have both in the past experienced severe earthquakes and other natural disasters. We do not carry earthquake insurance. Earthquakes or other natural disasters could severely disrupt our operations or those of our collaborators, and have a material adverse effect on our business, results of operations, financial condition, and prospects. If a natural disaster, power outage, or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure (such as the manufacturing facilities of our third-party contract manufacturers) or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place currently are limited and are unlikely to prove adequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which, particularly when taken together with our lack of earthquake insurance, could have a material adverse effect on our business.

Risks Related to this Offering and Ownership of Our Common Stock

The market price of our common stock may be highly volatile, and you may not be able to resell your shares at or above the initial public offering price.

Prior to this offering, there has not been a public market for our common stock. An active trading market for our common stock may not develop following this offering. You may not be able to sell your shares quickly or at

the market price if trading in our common stock is not active. The initial public offering price for the shares will be determined by negotiations between us and the representative of the underwriters and may not be indicative of prices that will prevail in the trading market.

The market price of our common stock is likely to be volatile. Our stock price could be subject to wide fluctuations in response to a variety of factors, including but not limited to the following:

- adverse results or delays in preclinical or clinical studies;
- any inability to obtain additional funding;
- any delay in filing an IND, NDA, BLA, or other regulatory submission for any of our product candidates and any adverse development or perceived adverse development with respect to the applicable regulatory agency's review of that IND, NDA, BLA, or other regulatory submission;
- the perception of limited market sizes or pricing for our product candidates;
- failure to successfully develop and commercialize our product candidates;
- post-marketing safety issues;
- failure to maintain our existing strategic collaborations or enter into new collaborations;
- failure by us or our licensors and strategic collaboration partners to prosecute, maintain, or enforce our intellectual property rights;
- changes in laws or regulations applicable to our products;
- any inability to obtain adequate product supply for our product candidates or the inability to do so at acceptable prices;
- adverse regulatory decisions;
- introduction of new products, services, or technologies by our competitors;
- failure to meet or exceed financial projections we may provide to the public;
- failure to meet or exceed the financial projections of the investment community;
- the perception of the pharmaceutical industry by the public, legislatures, regulators, and the investment community;
- announcements of significant acquisitions, strategic partnerships, joint ventures, or capital commitments by us, our strategic collaboration partner, or our competitors;
- disputes or other developments relating to proprietary rights, including patents, litigation matters, and our ability to obtain patent protection for our technologies;
- additions or departures of key scientific or management personnel;
- significant lawsuits, including patent or stockholder litigation;
- if securities or industry analysts do not publish research or reports about our business, or if they issue an adverse or misleading opinion regarding our stock;
- changes in the market valuations of similar companies;
- general market or macroeconomic conditions;
- sales of our common stock by us or our stockholders in the future; and
- trading volume of our common stock.

In addition, biotechnology and biopharmaceutical companies in particular have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance.

Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

As of November 1, 2013, our executive officers, directors, five percent stockholders, and their affiliates beneficially owned approximately 73% of our voting stock and, upon closing of this offering, that same group will beneficially own approximately % of our outstanding voting stock (assuming no exercise of the underwriters' option to purchase additional shares and no exercise of outstanding options and warrants). Therefore, even after this offering, these stockholders will have the ability to influence us through their ownership positions, which may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may believe are in your best interest as one of our stockholders.

We are an "emerging growth company" and, due to the reduced reporting requirements applicable to emerging growth companies, certain investors may find investing in our common stock less attractive.

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company for up to five years, although circumstances could cause us to lose that status earlier, including if the market value of our common stock held by non-affiliates exceeds $700.0 million as of any June 30 before that time or if we have total annual gross revenue of $1.0 billion or more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following December 31 or, if we issue more than $1.0 billion in non-convertible debt during any three-year period before that time, we would cease to be an emerging growth company immediately. We cannot predict if investors will find our common stock less attractive because we may rely on this exemption. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

If you purchase our common stock in this offering, you will incur immediate and substantial dilution in the book value of your shares.

Investors purchasing shares of common stock in this offering will pay a price per share that substantially exceeds the pro forma book value per share of our tangible assets after subtracting our liabilities and the preferred stock dividend that we will pay concurrent with this offering. As a result, investors purchasing shares of common stock in this offering will incur immediate dilution of $ per share, based on an assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus, and our pro forma net tangible book value as of September 30, 2013. For information on how the foregoing amounts were calculated, see "Dilution."

This dilution is due to the substantially lower price paid by our investors who purchased shares prior to this offering as compared to the price offered to the public in this offering, and the exercise of stock options granted to our employees. In addition, as of September 30, 2013, we had outstanding options and warrants to purchase 5,813,382 shares of our capital stock; the exercise of any of these options or warrants would result in additional dilution. As a result of the dilution to investors purchasing shares in this offering, investors may receive significantly less than the purchase price paid in this offering, if anything, in the event of our liquidation.

Sales of a substantial number of shares of our common stock in the public market could cause our stock price to fall.

If our existing stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after the lock-up and other legal restrictions on resale discussed in this prospectus lapse, the

market price of our common stock could decline. Based upon the number of shares of common stock, on an as-converted basis, outstanding as of , 2013, upon the closing of this offering we will have outstanding a total of shares of common stock, assuming no exercise of the underwriters' option to purchase additional shares. Of these shares, as of the date of this prospectus, approximately shares of our common stock, plus any shares sold upon exercise of the underwriters' option to purchase additional shares, will be freely tradable, without restriction, in the public market immediately following this offering, assuming that current stockholders do not purchase shares in this offering.

The lock-up agreements pertaining to this offering will expire 180 days from the date of this prospectus. After the lock-up agreements expire, based upon the number of shares of common stock, on an as-converted basis, outstanding as of , 2013, up to an additional shares of common stock will be eligible for sale in the public market, of which shares are held by directors, executive officers and other affiliates and will be subject to Rule 144 under the Securities Act of 1933, as amended, or the Securities Act. J.P. Morgan Securities LLC and Morgan Stanley & Co. LLC, however, may, in their sole discretion, permit our officers, directors, and other stockholders who are subject to these lock-up agreements to sell shares prior to the expiration of the lock-up agreements.

In addition, as of , 2013, shares of common stock that are either subject to outstanding options, reserved for future issuance under our equity incentive plans, or subject to outstanding warrants will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, the lock-up agreements, and Rule 144 and Rule 701 under the Securities Act. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the market price of our common stock could decline.

After this offering, the holders of approximately shares of our common stock will be entitled to rights with respect to the registration of their shares under the Securities Act, subject to the lock-up agreements described above. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares purchased by affiliates. Any sales of securities by these stockholders could have a material adverse effect on the market price of our common stock.

Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plans, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.

We will need additional capital in the future to continue our planned operations. To the extent we raise additional capital by issuing equity securities, our stockholders may experience substantial dilution. We may sell common stock, convertible securities, or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities, or other equity securities in more than one transaction, investors may be materially diluted by subsequent sales. These sales may also result in material dilution to our existing stockholders, and new investors could gain rights superior to our existing stockholders.

Pursuant to our 2013 Incentive Plan, or the 2013 Plan, which will become effective immediately prior to the completion of this offering, our management is authorized to grant stock options and other equity-based awards to our employees, directors, and consultants. The number of shares available for future grant under the 2013 Plan will automatically increase on January 1 of each year by up to the least of shares and % of all shares of our capital stock outstanding as of December 31 of the prior calendar year, subject to the ability of our compensation committee to take action to reduce the size of the increase in any given year. Currently, we plan to register the increased number of shares available for issuance under the 2013 Plan each year. Pursuant to our 2013 Employee Stock Purchase Plan, or 2013 ESPP, which will become effective immediately prior to the completion of this offering, eligible employees will be able to acquire shares of our common stock at a discount to the prevailing market price, and an aggregate of shares will be available for issuance under the 2013 ESPP. The number of shares available for issuance under the 2013 ESPP will automatically increase on January 1 of each year by up to the least of shares and % of all shares of our capital stock

outstanding as of December 31 of the prior calendar year, subject to the ability of our compensation committee to take action to reduce the size of the increase in any given year. If our board of directors elects to increase the number of shares available for future grant under the 2013 Plan or the 2013 ESPP, our stockholders may experience additional dilution, which could cause our stock price to fall.

We have broad discretion in the use of the net proceeds from this offering and may not use them effectively.

Our management will have broad discretion in the application of the net proceeds from this offering, including for any of the purposes described in the section entitled "Use of proceeds," and you will not have the opportunity as part of your investment decision to assess whether the net proceeds are being used appropriately. Because of the number and variability of factors that will determine our use of the net proceeds from this offering, their ultimate use may vary from their currently intended use. The failure by our management to apply these funds effectively could harm our business. Pending their use, we may invest the net proceeds from this offering in investment-grade, interest-bearing securities. These investments may not yield a favorable return to our stockholders.

Our ability to use our net operating loss carryforwards and certain other tax attributes may be limited.

We have incurred substantial losses during our history and do not expect to become profitable in the near future and we may never achieve profitability. To the extent that we continue to generate taxable losses, unused losses will carry forward to offset future taxable income, if any, until such unused losses expire. Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards, or NOLs, and other pre-change tax attributes (such as research tax credits) to offset its post-change income may be limited. We may experience ownership changes in the future as a result of subsequent shifts in our stock ownership. As a result, if we earn net taxable income, our ability to use our pre-change net operating loss carryforwards to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

We do not intend to pay dividends on our common stock so any returns will be limited to the value of our stock.

We have never declared or paid any cash dividends on our common stock. Although we will pay a cash dividend to our existing holders of preferred stock, which was agreed to at the time of the private placement financings, in connection with the consummation of this offering, we currently anticipate that we will retain future earnings for the development, operation, and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will therefore be limited to the appreciation of their stock.

Provisions in our amended and restated certificate of incorporation and by-laws, as well as provisions of Delaware law, could make it more difficult for a third party to acquire us or increase the cost of acquiring us, even if doing so would benefit our stockholders or remove our current management.

Our amended and restated certificate of incorporation, amended and restated by-laws, and Delaware law contain provisions that may have the effect of delaying or preventing a change in control of us or changes in our management. Our amended and restated certificate of incorporation and by-laws, which will become effective upon the closing of this offering, include provisions that:

- authorize "blank check" preferred stock, which could be issued by our board of directors without stockholder approval and may contain voting, liquidation, dividend, and other rights superior to our common stock;

- create a classified board of directors whose members serve staggered three-year terms;
- specify that special meetings of our stockholders can be called only by our board of directors or the chairperson of our board of directors;
- prohibit stockholder action by written consent;
- establish an advance notice procedure for stockholder approvals to be brought before an annual meeting of our stockholders, including proposed nominations of persons for election to our board of directors;
- provide that our directors may be removed only for cause;
- provide that vacancies on our board of directors may be filled only by a majority of directors then in office, even though less than a quorum;
- specify that no stockholder is permitted to cumulate votes at any election of directors;
- expressly authorize our board of directors to modify, alter or repeal our amended and restated by-laws; and
- require holders of 75% of our outstanding common stock to amend specified provisions of our amended and restated certificate of incorporation and amended and restated by-laws.

These provisions, alone or together, could delay, deter, or prevent hostile takeovers and changes in control or changes in our management.

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which limits the ability of stockholders owning in excess of 15% of our outstanding voting stock to merge or combine with us.

Any provision of our amended and restated certificate of incorporation or amended and restated by-laws or Delaware law that has the effect of delaying or deterring a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock, and could also affect the price that some investors are willing to pay for our common stock.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements that involve risks and uncertainties. All statements other than statements of historical facts contained in this prospectus are forward-looking statements. In some cases, you can identify forward-looking statements by words such as “anticipate,” “believe,” “contemplate,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “seek,” “should,” “target,” “will,” “would,” or the negative of these words or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- our expectations regarding the timing of reporting results from our clinical studies of triheptanoin and SA-ER;
- our expectations regarding the timing of commencing clinical studies with respect to KRN23, rhGUS, rhPPCA, and triheptanoin;
- the likelihood of regulatory approvals for our product candidates;
- the potential market opportunities for commercializing our product candidates;
- our expectations regarding the potential market size and the size of the patient populations for our product candidates, if approved for commercial use;
- estimates of our expenses, future revenue, capital requirements, and our needs for additional financing;
- our ability to develop, acquire, and advance product candidates into, and successfully complete, clinical studies;
- the implementation of our business model and strategic plans for our business and product candidates;
- the initiation, timing, progress, and results of future preclinical studies and clinical studies, and our research and development programs;
- the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates;
- our ability to maintain and establish collaborations or obtain additional funding;
- our expectations regarding the time during which we will be an emerging growth company under the JOBS Act;
- our expectations regarding the composition of our board of directors;
- our use of proceeds from this offering;
- our financial performance; and
- developments and projections relating to our competitors and our industry.

Any forward-looking statements in this prospectus reflect our current views with respect to future events or to our future financial performance and involve known and unknown risks, uncertainties, and other factors that may cause our actual results, performance, or achievements to be materially different from any future results, performance, or achievements expressed or implied by these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under “Risk Factors” and elsewhere in this prospectus. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

This prospectus also contains estimates, projections, and other information concerning our industry, our business, and the markets for certain diseases, including data regarding the estimated size of those markets, and the incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research, or similar methodologies is inherently subject to uncertainties and actual events or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained this industry, business, market, and other data from reports, research surveys, studies, and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data, and similar sources.

USE OF PROCEEDS

We estimate that the net proceeds from the sale of shares of common stock in this offering will be approximately $ million at an assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters exercise their option to purchase additional shares in full, we estimate that the net proceeds will be approximately $ million after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. Each $1.00 increase (decrease) in the assumed initial public offering price of $ per share would increase (decrease) our net proceeds by $ million, assuming the number of shares offered by us, as set forth on the cover of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. We may also increase or decrease the number of shares we are offering. An increase (decrease) of 1,000,000 in the number of shares we are offering would increase (decrease) the net proceeds to us from this offering, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us, by approximately $ million, assuming the assumed initial public offering price stays the same.

We are undertaking this offering in order to access the public capital markets and to increase our liquidity. We intend to use the net proceeds of this offering as follows:

- Approximately $ million to fund our clinical program for KRN23;
- Approximately $ million to fund our clinical program for rhGUS;
- Approximately $ million to fund our ongoing clinical program for triheptanoin in both LC-FAOD and Glut1 DS;
- Approximately $ million to fund our ongoing clinical program for SA-ER; and
- The remainder for personnel-related costs, preclinical research, working capital, and other general corporate purposes.

Our expected use of net proceeds from this offering represents our current intentions based upon our present plans and business condition. As of the date of this prospectus, we cannot predict with complete certainty all of the particular uses for the net proceeds to be received upon the completion of this offering or the actual amounts that we will spend on the uses set forth above. We may also use a portion of the net proceeds to in-license, acquire, or invest in additional businesses, technologies, products, or assets. Although we have no specific agreements, commitments, or understandings with respect to any in-license or acquisition, we evaluate such opportunities and engage in related discussions with other companies from time to time. Due to the many variables inherent to the development of our product candidates, we cannot currently predict the stage of development we expect the net proceeds of this offering to achieve for our clinical studies and product candidates. We also will pay a cash dividend of approximately $3.4 million to the holders of our preferred stock, assuming a conversion of our preferred stock on November 8, 2013, utilizing our existing cash on hand at the time of the closing of the offering.

The amount and timing of our actual expenditures will depend upon numerous factors, including the results of our research and development efforts, the timing and success of preclinical studies, our ongoing clinical studies or clinical studies we may commence in the future and the timing of regulatory submissions. As a result, our management will have broad discretion over the use of the net proceeds from this offering.

Pending the use of the proceeds from this offering, we intend to invest the net proceeds in interest-bearing, investment-grade securities, certificates of deposit, or government securities.

DIVIDEND POLICY

We have never declared or paid cash dividends on our common stock. Although we will pay a cash dividend to our existing holders of preferred stock, which was agreed to at the time of the private placement financings, in connection with the consummation of this offering, we currently intend to retain all available funds and any future earnings, if any, to fund the development and expansion of our business, and we do not anticipate paying any cash dividends on our common stock in the foreseeable future. Any future determination to pay dividends will be made at the discretion of our board of directors or any authorized committee thereof.

CAPITALIZATION

The following table sets forth our cash, cash equivalents and marketable securities and capitalization as of September 30, 2013:

- on an actual basis;
- on a pro forma basis to reflect (i) the conversion of all outstanding shares of our convertible preferred stock into an aggregate of 61,431,574 shares of our common stock, (ii) the reclassification to additional paid-in capital of our preferred stock warrant liability in connection with the conversion of our outstanding preferred stock warrants into common stock warrants, and (iii) a dividend of $3.4 million payable concurrent with the conversion of our preferred stock to common stock to the holders of our preferred stock, which has been calculated as if the conversion of preferred stock into common stock occurred as of November 8, 2013, in each case, immediately prior to the completion of this offering; and
- on a pro forma as adjusted basis to additionally reflect the issuance and sale of shares of common stock in this offering at an assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus, after deducting the estimated underwriting discount and commissions and estimated offering expenses payable by us.

You should read this information together with our audited financial statements and related notes appearing elsewhere in this prospectus and the information set forth under the heading “Selected Financial Data” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations.”

	As of September 30, 2013		
	Actual	Pro Forma	Pro Forma as Adjusted
		(in thousands, except share and per share data) (unaudited)	
Cash, cash equivalents and marketable securities	$ 63,567	$ 63,567	$
Preferred stock warrant liability	$ 1,596	—	
Convertible preferred stock, par value $0.001 per share: 62,459,236 shares authorized, 61,431,574 shares issued and outstanding, actual; no shares authorized, issued and outstanding, pro forma and pro forma as adjusted	118,002	—	
Stockholders’ deficit:			
Preferred stock, par value $0.001 per share: no shares authorized, issued or outstanding, actual; shares authorized and no shares issued or outstanding, pro forma and pro forma as adjusted	—	—	
Common stock, par value $0.001 per share: 85,000,000 shares authorized, 11,607,173 shares issued and outstanding, actual; shares authorized, 73,038,747 shares issued and outstanding pro forma, and shares issued and outstanding, pro forma as adjusted	12	73	
Additional paid-in capital	—	116,122	
Accumulated other comprehensive loss	(14)	(14)	
Deficit accumulated during the development stage	(56,846)	(56,846)	
Total stockholders’ (deficit) equity	(56,848)	59,335	
Total capitalization	$ 62,750	$ 59,335	$

Each $1.00 increase (decrease) in the assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus, would increase (decrease) each of pro forma as adjusted cash, cash equivalents and marketable securities, additional paid-in capital, total stockholders’ equity, and total capitalization by approximately $ million, assuming that the number of shares offered by us, as set

forth on the cover of this prospectus, remains the same. We may also increase or decrease the number of shares we are offering. An increase (decrease) of 1,000,000 in the number of shares we are offering would increase (decrease) each of pro forma as adjusted cash, cash equivalents and marketable securities, additional paid-in capital, total stockholders' equity, and total capitalization by approximately $ million, assuming the assumed initial public offering price per share, as set forth on the cover of this prospectus, remains the same. The pro forma as adjusted information is illustrative only, and we will adjust this information based on the actual initial public offering price and other terms of this offering determined at pricing.

The number of shares of common stock issued and outstanding in the table above excludes the following shares as of September 30, 2013:

- 4,785,720 shares of common stock issuable upon the exercise of outstanding stock options having a weighted-average exercise price of $0.27 per share;
- 1,027,662 shares of common stock issuable upon the exercise of outstanding warrants having a weighted-average exercise price $1.034 per share;
- 4,906,484 shares of common stock reserved for issuance pursuant to future equity awards under our 2011 Equity Incentive Plan, as amended, which will become available for issuance under our 2013 Incentive Plan after completion of this offering;
- shares of common stock reserved for issuance (including the above-referenced shares reserved for issuance under our 2011 Equity Incentive Plan, as amended) pursuant to future equity awards under our 2013 Incentive Plan, as well as any future increases in the number of shares of our common stock reserved for future issuance under this plan, which will become effective immediately prior to the completion of this offering; and
- shares of common stock reserved for future issuance under our 2013 Employee Stock Purchase Plan, or 2013 ESPP, as well as any future increases in the number of shares of our common stock reserved for future issuance under the 2013 ESPP, which will become effective immediately prior to the completion of this offering.

DILUTION

If you invest in our common stock in this offering, your ownership interest will be diluted to the extent of the difference between the assumed initial public offering price per share of our common stock and the pro forma as adjusted net tangible book value per share of our common stock immediately after this offering.

Net tangible book value per share is determined by dividing our total tangible assets less our total liabilities by the number of shares of common stock outstanding. Our historical net tangible book value as of September 30, 2013 was $ million, or $ per share. Our pro forma net tangible book value as of September 30, 2013 was $ million, or $ per share, based on the total number of shares of our common stock outstanding as of September 30, 2013. Pro forma net tangible book value, before the issuance and sale of shares in this offering, gives effect to:

- the conversion of all outstanding shares of our convertible preferred stock into an aggregate of 61,431,574 shares of our common stock;
- the reclassification to additional paid-in capital of our preferred stock warrant liability in connection with the conversion of our outstanding preferred stock warrants into common stock warrants; and
- the payment in cash to the preferred stock holders of a $ million dividend concurrent with the conversion of the convertible preferred stock.

Dilution per share to new investors represents the difference between the amount per share paid by purchasers of shares of common stock in this offering and the pro forma as adjusted net tangible book value per share of common stock immediately after completion of this offering. After giving effect to our sale of shares of common stock in this offering at an assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus, and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of September 30, 2013 would have been $, or $ per share. This represents an immediate increase in pro forma net tangible book value of $ per share to existing stockholders and an immediate dilution of $ per share to investors participating in this offering, as illustrated in the following table:

Assumed initial public offering price per share		$
Historical net tangible book value per share as of September 30, 2013	$	
Pro forma increase in net tangible book value per share as of September 30, 2013		
Pro forma net tangible book value per share as of September 30, 2013		
Increase in pro forma net tangible book value per share attributable to new investors		
Pro forma as adjusted net tangible book value per share after this offering		
Dilution per share to investors participating in this offering		$

Each $1.00 increase (decrease) in the assumed public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus, would increase (decrease) our pro forma as adjusted net tangible book value by $ million, or $ per share, and the dilution per share to investors in this offering by $, assuming that the number of shares offered by us, as set forth on the cover of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. We may also increase or decrease the number of shares we are offering. An increase (decrease) of 1,000,000 in the number of shares we are offering would increase (decrease) our pro forma as adjusted net tangible book value as of September 30, 2013 after this offering by approximately $ million, or approximately $ per share, and would decrease (increase) dilution per share to investors in this offering by approximately $, assuming the assumed initial public offering price per share remains the same, after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. The pro forma as adjusted information is illustrative only, and we will adjust this information based on the actual initial public offering price and other terms of this offering determined at pricing.

If the underwriters' option to purchase additional shares from us is exercised in full, the pro forma as adjusted net tangible book value per share after this offering would be $ per share, the increase in pro forma

net tangible book value per share to existing stockholders would be $ per share and the dilution to investors participating in this offering would be $ per share.

The following table presents, on a pro forma as adjusted basis described above, the differences between the existing stockholders and the purchasers of shares in this offering with respect to the number of shares purchased from us, the total consideration paid, which includes net proceeds received from the issuance of common and convertible preferred stock, cash received from the exercise of stock options and warrants, and the value of any stock issued for services and the average price paid per share (in thousands, except per share amounts and percentages):

	Shares Purchased		Total Consideration		Average Price per Share
	Number	Percent	Amount	Percent	
Existing stockholders		%		%	$
New investors					
Totals					

The foregoing calculations exclude the following shares as of September 30, 2013:

- 4,785,720 shares of common stock issuable upon the exercise of outstanding stock options having at a weighted-average exercise price of $0.27 per share;
- 1,027,662 shares of common stock issuable upon the exercise of outstanding warrants having a weighted-average exercise price $1.034 per share;
- 4,906,484 shares of common stock reserved for issuance pursuant to future awards under our 2011 Equity Incentive Plan, as amended, which will become available for issuance under our 2013 Incentive Plan after completion of this offering;
- shares of common stock reserved for issuance (including the above-referenced shares reserved for issuance under our 2011 Equity Incentive Plan, as amended) pursuant to future equity awards under our 2013 Equity Incentive Plan, as well as any future increases in the number of shares of our common stock reserved for future issuance under this plan, which will become effective immediately prior to the completion of this offering; and
- shares of common stock reserved for future issuance under our 2013 Employee Stock Purchase Plan, or 2013 ESPP, as well as any future increases in the number of shares of our common stock reserved for future issuance under the 2013 ESPP, which will become effective immediately prior to the completion of this offering.

Furthermore, we may choose to raise additional capital through the sale of equity or convertible debt securities due to market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. New investors will experience further dilution if any of our outstanding options or warrants are exercised, new options are issued and exercised under our equity incentive plans or we issue additional shares of common stock, other equity securities or convertible debt securities in the future.

SELECTED FINANCIAL DATA

The selected statements of operations data for the years ended December 31, 2011 and 2012 and the selected balance sheet data as of December 31, 2011 and 2012 are derived from our audited financial statements included elsewhere in this prospectus. The selected statements of operations data for the nine months ended September 30, 2012 and 2013 and the selected balance sheet data as of September 30, 2013 have been derived from our unaudited financial statements included elsewhere in this prospectus. The unaudited interim financial information has been prepared on the same basis as the annual financial information and, in the opinion of management, reflects all adjustments, which include only normal recurring adjustments, necessary to present fairly our financial position as of September 30, 2013 and the results of operations for the nine months ended September 30, 2012 and 2013. Our historical results are not necessarily indicative of the results that may be expected in the future and interim results are not necessarily indicative of results to be expected for the full year. You should read the selected historical financial data below in conjunction with the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations" and the financial statements and related notes included elsewhere in this prospectus.

	Year Ended December 31,		Nine Months Ended September 30,	
	2011	2012	2012	2013
	(in thousands, except share and per share amounts)		(unaudited)	
Statements of Operations Data:				
Operating expenses:				
Research and development	$ 4,717	$ 12,641	$ 8,866	$ 19,625
General and administrative	1,844	3,344	2,441	3,130
Total operating expenses	6,561	15,985	11,307	22,755
Loss from operations	(6,561)	(15,985)	(11,307)	(22,755)
Interest income	4	1	—	157
Interest expense	(270)	—	—	—
Other expense	(22)	(350)	(97)	(1,155)
Net loss	$ (6,849)	$ (16,334)	$ (11,404)	$ (23,753)
Net loss attributable to common stockholders[1]	$ (7,466)	$ (19,561)	$ (12,749)	$ (31,624)
Net loss per share attributable to common stockholders, basic and diluted	$ (1.47)	$ (4.53)	$ (3.94)	$ (3.09)
Shares used to compute net loss per share attributable to common stockholders, basic and diluted	5,069,694	4,316,868	3,235,308	10,220,034
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)[1]		$		$
Shares used to compute pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)[1]				

(1) See Notes 2 and 14 to our audited financial statements and Note 8 of our unaudited financial statements included elsewhere in this prospectus for an explanation of the calculations of basic and diluted net loss per share attributable to common stockholders and pro forma net loss per share attributable to common stockholders.

	As of December 31,		As of September 30,
	2011	2012	2013
		(in thousands)	(unaudited)
Balance Sheet Data:			
Cash, cash equivalents and marketable securities	$10,645	$ 86,190	$ 63,657
Working capital	9,954	83,257	61,067
Total assets	12,129	88,316	68,592
Convertible preferred stock warrant liability	216	518	1,596
Convertible preferred stock	18,604	111,387	118,002
Deficit accumulated during the development stage	(8,155)	(27,058)	(56,846)
Total stockholders' deficit	(7,961)	(27,047)	(56,848)

MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with the section of this prospectus entitled "Selected Financial Data" and our financial statements and related notes included elsewhere in this prospectus. This discussion and other parts of this prospectus contain forward-looking statements that involve risk and uncertainties, such as statements of our plans, objectives, expectations, and intentions. As a result of many factors, including those factors set forth in the "Risk Factors" section of this prospectus, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a development-stage biopharmaceutical company focused on the identification, acquisition, development, and commercialization of novel products for the treatment of rare and ultra-rare diseases, with an initial focus on serious, debilitating metabolic genetic diseases. We focus on diseases for which the unmet medical need is high, the biology for treatment is clear, and for which there are no approved therapies. Since our inception in 2010, we have in-licensed potential treatments for five different diseases that are or we expect will be in Phase 2 clinical studies by early 2014. Our strategy, which is predicated upon time- and cost-efficient drug development, allows us to pursue multiple programs in parallel with the goal of delivering safe and effective therapies to patients with the utmost urgency.

Our current pipeline consists of two product categories: biologics, including a monoclonal antibody and enzyme replacement therapies; and small-molecule substrate replacement therapies. Enzymes are proteins that the body uses to process materials needed for normal cellular function, and substrates are the materials upon which enzymes act. When enzymes or substrates are missing, the body is unable to perform its normal cellular functions, often leading to significant clinical disease. Several of our therapies are intended to replace deficient enzymes or substrates.

Our biologics pipeline includes the following three product candidates:

- KRN23, or UX023, is an antibody targeting fibroblast growth factor 23, or FGF23, intended for the treatment of X-linked hypophosphatemia, or XLH, a rare genetic disease that impairs bone growth. We are developing KRN23 pursuant to our collaboration with Kyowa Hakko Kirin Co., Ltd., or KHK. KHK has conducted one Phase 1 study, one Phase 1/2 study and one Phase 1/2 extension study of KRN23 in adults with XLH. We expect to continue the clinical development of KRN23 in adults as well as initiate pediatric clinical development in 2014.
- rhGUS, or UX003, is an enzyme replacement therapy we are developing for the treatment of mucopolysaccharidosis 7, or MPS 7, a rare lysosomal storage disease that often leads to multi-organ dysfunction, pervasive skeletal disease, and death. We plan to initiate a Phase 1/2 clinical study in MPS 7 by the end of 2013.
- rhPPCA, or UX004, is an enzyme replacement therapy in preclinical development for galactosialidosis, a rare lysosomal storage disease that can cause multi-system clinical disease similar to MPS 7 including enlarged liver, joint disease, abnormal bone development, short stature, and death. We plan to continue preclinical development of rhPPCA during 2014.

Our substrate replacement therapy pipeline includes the following product candidates in development for three diseases:

- Triheptanoin, or UX007, is a synthetic oil with a specifically designed chemical composition being studied in an investigator-sponsored Phase 2 study for the treatment of long-chain fatty acid oxidation disorders, or LC-FAOD. This is a set of rare metabolic diseases that prevent the conversion of fat into energy and can cause low blood sugar, muscle rupture, and heart and liver disease. We plan to initiate our own Phase 2 study in LC-FAOD by the end of 2013.

- Triheptanoin is also in an investigator-sponsored Phase 2 study for the treatment of glucose transporter type-1 deficiency syndrome, or Glut1 DS, a rare metabolic disease of brain energy deficiency that can result in seizures, developmental delay, and movement disorder. We plan to initiate our own Phase 2 clinical study in Glut1 DS in early 2014.
- SA-ER, or UX001, is an extended-release form of sialic acid in a Phase 2 study for the treatment of hereditary inclusion body myopathy, or HIBM, a neuromuscular disorder that causes muscle weakness and wasting. We reported 24-week data from our ongoing Phase 2 study in HIBM in July 2013 and anticipate top-line 48-week data by the end of 2013.

We are considered a development-stage company under U.S. generally accepted accounting principles, or U.S. GAAP, and have only a limited operating history. Since our inception in 2010, we have devoted substantially all of our resources to identify, acquire, and develop our product candidates, including conducting clinical studies and providing general and administrative support for these operations. We have funded our operations to date primarily from the issuance and sale of convertible preferred stock and convertible promissory notes.

We have never been profitable and have incurred net losses in each year since inception. Our net losses were $6.8 million and $16.3 million for the years ended December 31, 2011 and 2012, and $11.4 million and $23.8 million for the nine months ended September 30, 2012 and 2013. As of September 30, 2013 we had a deficit accumulated during the development stage of $56.8 million. Substantially all of our net losses have resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations.

Financial Operations Overview

Revenue

To date, we have not generated any revenue. We do not expect to receive any revenue from any product candidates that we develop until we obtain regulatory approval and commercialize our products or enter into collaborative agreements with third parties.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for the development of our product candidates, which include:

- expenses incurred under agreements with clinical study sites that conduct research and development activities on our behalf;
- expenses incurred under license agreements with third parties;
- employee and consultant-related expenses, which include salaries, benefits, travel, and stock-based compensation;
- laboratory and vendor expenses related to the execution of preclinical, non-clinical, and clinical studies;
- the cost of acquiring, developing, and manufacturing clinical study materials; and
- facilities, depreciation, and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance, and other supply costs.

We expense all research and development costs in the periods in which they are incurred. Costs for certain development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and clinical sites. Nonrefundable advance payments for goods or services to be received in future periods for use in research and development activities are deferred and capitalized. The capitalized amounts are then expensed as the related goods are delivered and the services are performed.

The largest component of our total operating expenses has historically been our investment in research and development activities, including the clinical development of our product candidates. We allocate research and development salaries, benefits, stock-based compensation, and indirect costs to our product candidates on a program-specific basis, and we include these costs in the program-specific expenses. We expect our research and development expenses will increase in absolute dollars in future periods as we continue to invest in research and development activities related to developing our product candidates, and as programs advance into later stages of development and we enter into larger clinical studies. The process of conducting the necessary clinical research to obtain FDA approval is costly and time consuming and the successful development of our product candidates is highly uncertain. As a result, we are unable to determine the duration and completion costs of our research and development projects or when and to what extent we will generate revenue from the commercialization and sale of any of our product candidates.

General and Administrative Expenses

General and administrative expenses consist primarily of personnel costs, allocated facilities costs, and other expenses for outside professional services, including legal, human resources, audit, and accounting services. Personnel costs consist of salaries, benefits, and stock-based compensation. We expect that our general and administrative expenses will increase in the future to support continued research and development activities, and as a result of operating as a public company, including expenses related to compliance with the rules and regulations of the Securities and Exchange Commission, or SEC, and those of any national securities exchange on which our securities are traded, additional insurance expenses, investor relations activities, and other administration and professional services.

Interest income

Interest income consists of interest earned on our cash, cash equivalents, and marketable securities.

Interest expense

Interest expense consists of interest on outstanding borrowings under convertible promissory notes. We had no outstanding debt as of December 31, 2011 and thereafter as our convertible promissory notes and accrued interest were all converted into shares of Series A convertible preferred stock in 2011.

Other expense

Other expense primarily consists of gains and losses resulting from the remeasurement of our convertible preferred stock warrant liability. We will continue to record adjustments to the estimated fair value of the convertible preferred stock warrants until they are exercised, expired, or converted into warrants to purchase shares of our common stock upon the completion of a liquidity event, including the completion of an initial public offering. At that time, we will reclassify the convertible preferred stock warrant liability as additional paid-in capital and we will no longer record any related periodic fair value adjustments.

Critical Accounting Policies and Significant Judgments and Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with U.S. GAAP. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported expenses incurred during the reporting periods. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. We

believe that the accounting policies discussed below are critical to understanding our historical and future performance, as these policies relate to the more significant areas involving management's judgments and estimates.

Accrued Research and Development Expenses

As part of the process of preparing financial statements, we are required to estimate and accrue expenses, the largest of which is related to accrued research and development expenses. This process involves reviewing contracts and purchase orders, identifying services that have been performed on our behalf, and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of the actual costs.

We record accruals for estimated costs of research, preclinical and clinical studies, and manufacturing development. These costs are a significant component of our research and development expenses. A substantial portion of our ongoing research and development activities is conducted by third-party service providers. We accrue the costs incurred under our agreements with these third parties based on actual work completed in accordance with agreements established with these third parties. We determine the actual costs through discussions with internal personnel and external service providers as to the progress or stage of completion of the services and the agreed-upon fee to be paid for such services. We make significant judgments and estimates in determining the accrual balance in each reporting period. As actual costs become known, we adjust our accruals. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and could result in us reporting amounts that are too high or too low in any particular period. Our accrual is dependent, in part, upon the receipt of timely and accurate reporting from clinical research organizations and other third-party vendors. To date, there have been no material differences from our accrued estimated expenses to the actual clinical trial expenses.

Estimated Fair Value of Convertible Preferred Stock Warrant Liability

Warrants for the purchase of Series A convertible preferred stock that are contingently redeemable are classified as liabilities on the balance sheet at their estimated fair value. At the end of each reporting period, changes in estimated fair value during the period are recorded in other expense, net. We will continue to adjust the carrying value of the warrants until the earlier of the exercise of the warrants or the completion of a liquidity event, including the completion of an initial public offering, at which time the liabilities will be reclassified to additional paid-in capital.

We estimate the fair values of our convertible preferred stock warrants using an option-pricing model based on inputs as of the valuation measurement dates, including our estimated equity value at the valuation measurement dates, the estimated volatility of the price of our convertible preferred stock, the remaining contractual terms of the warrants, and the risk-free interest rates.

Stock-Based Compensation

Stock-based compensation costs related to stock options granted to employees are measured at the date of grant based on the estimated fair value of the award, net of estimated forfeitures. We estimate the grant date fair value, and the resulting stock-based compensation expense, using the Black-Scholes option-pricing model. The grant date fair value of the stock-based awards is recognized on a straight-line basis over the requisite service period, which is generally the vesting period of the respective awards.

The Black-Scholes option-pricing model requires the use of highly subjective assumptions which determine the fair value of stock-based awards. These assumptions include:

- *Expected term* — The expected term represents the period that the stock-based awards are expected to be outstanding and is determined using the simplified method (based on the midpoint between the vesting date and the end of the contractual term).

- *Expected volatility* — Since we are privately held and do not have any trading history for our common stock, the expected volatility was estimated based on the average volatility for comparable publicly traded biopharmaceutical companies over a period equal to the expected term of the stock option grants. When selecting comparable publicly traded biopharmaceutical companies on which we based our expected stock price volatility, we selected companies with comparable characteristics to us, including enterprise value, risk profiles, position within the industry, and with historical share price information sufficient to meet the expected life of the stock-based awards. The historical volatility data was computed using the daily closing prices for the selected companies' shares during the equivalent period of the calculated expected term of the stock-based awards. We will continue to apply this process until a sufficient amount of historical information regarding the volatility of our own stock price becomes available.
- *Risk-free interest rate* — The risk-free interest rate is based on the U.S. Treasury zero coupon issues in effect at the time of grant for periods corresponding with the expected term of option.
- *Expected dividend* — We have never paid dividends on our common stock and have no plans to pay dividends on our common stock. Therefore, we used an expected dividend yield of zero.

In addition to the assumptions used in the Black-Scholes option-pricing model, we must also estimate a forfeiture rate to calculate the stock-based compensation for our awards. We will continue to use judgment in evaluating the expected volatility, expected terms, and forfeiture rates utilized for our stock-based compensation calculations on a prospective basis.

For the years ended December 31, 2011 and 2012, stock-based compensation expense was $0.3 million and $0.9 million, respectively. For the nine month periods ended September 30, 2012 and 2013, stock-based compensation expense was $0.7 million and $0.4 million, respectively. As of September 30, 2013, we had $0.8 million of total unrecognized stock-based compensation costs, net of estimated forfeitures, which we expect to recognize over a weighted-average period of 2.4 years.

Fair Value of Common Stock

We are required to estimate the fair value of the common stock underlying our stock-based awards when performing the fair value calculations with the Black-Scholes option-pricing model. The fair value of the common stock underlying our stock-based awards was determined on each grant date by our board of directors, with input from management. Given the absence of a public trading market of our common stock, and in accordance with the American Institute of Certified Public Accountants, or AICPA, Practice Guide, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*, our board of directors exercised reasonable judgment and considered numerous objective and subjective factors to estimate the fair value of our common stock.

All options to purchase shares of our common stock have been granted with an exercise price per share no less than the fair value per share of our common stock underlying those options on the date of grant. To assist our board of directors with the determination of the exercise price of our stock options and the fair value of the common stock underlying the options, we obtained third-party valuations of our common stock as of June 30, 2012, December 31, 2012, June 30, 2013, and September 30, 2013. Our board of directors considered the fair values of the common stock derived in the third-party valuations as one of the factors it considered when setting the exercise prices for options granted. Our board of directors also considered a range of objective and subjective factors and assumptions in estimating the fair value of our common stock on the date of grant, including:

- progress of our research and development efforts;
- our operating results and financial condition, including our levels of available capital resources;
- rights and preferences of our common stock compared to the rights and preferences of our other outstanding equity securities;
- our stages of development and material risks related to our business;

- the achievement of enterprise milestones, including entering into collaboration or license agreements and our progress in clinical trials;
- the valuation of publicly-traded companies in the life sciences and biotechnology sectors, as well as recently completed mergers and acquisitions of peer companies;
- equity market conditions affecting comparable public companies;
- the likelihood of achieving a liquidity event for the shares of common stock, such as an initial public offering given prevailing market and biotechnology sector conditions; and
- that the grants involved illiquid securities in a private company.

Contemporaneous Valuations

We obtained third-party valuations of our common stock as of June 30, 2012, December 31, 2012, June 30, 2013, and September 30, 2013 to assist our board of directors in estimating the fair value of our common stock at subsequent grant dates.

June 2012 and December 2012 Contemporaneous Valuations. The June 2012 and December 2012 valuations used the Back-Solve Method of the option-pricing method, or OPM, which derives the implied equity value for one type of equity security from a contemporaneous transaction involving another equity security. The June 2012 valuation, which was completed in July 2012, was based on the price of Series A convertible preferred stock that we sold to investors in July 2012. The December 2012 valuation was based on the price of Series B convertible preferred stock that we sold in December 2012. In both valuations, the contemporaneous transaction occurred in close proximity and involved third-party investors. Given the arm's-length nature of these financings, the close proximity of the Series A and Series B convertible preferred stock financings to the respective valuation dates, and the fair value hierarchy as described in FASB Accounting Standards Codification Topic 820, *Fair Value*, we believe the per share issuance prices of the Series A and Series B convertible preferred stock provide an indication of our equity value, as well as the fair value of common stock, as of June 30, 2012 and December 31, 2012, respectively.

The OPM treats common stock and preferred stock as call options on the total equity value of a company, with exercise prices based on the liquidation preference of the preferred stock. Under this method, the common stock has value only if the funds available for distribution to the stockholders exceed the value of the liquidation preference at the time of a liquidity event such as a merger, sale, or IPO, assuming the enterprise has funds available to make a liquidation preference meaningful and collectible by the stockholders. The common stock is modeled as a call option on the underlying equity value at a predetermined exercise price. In the model, the exercise price is based on a comparison with the total equity value, rather than, as in the case of a regular call option, a comparison with a per share stock price. The OPM uses the Black-Scholes option-pricing model to price the call option. This model defines the securities' fair values as functions of the current fair value of a company and uses assumptions such as the anticipated timing of a potential liquidity event and the estimated volatility of the equity securities.

For purposes of the June 2012 valuation, we estimated the time to liquidity as 1.5 years based on then-current plans and estimates of our board of directors and management regarding a liquidity event. The volatility assumption was based on an analysis of guideline companies' historical equity volatility factors for a period of 1.5 years, which is the term assumption. Based on this analysis of the guideline companies, a volatility assumption of 67% was selected and utilized. The risk-free rate was estimated as the interpolated 1.5 year U.S. Treasury yield. A discount for lack of marketability of 31% was then applied to the value indicated in our common stock. Based on these factors, the third party valuation concluded that our common stock had a fair value of $0.26 per share as of June 30, 2012.

For purposes of the December 2012 valuation, we estimated the time to liquidity as 2.0 years based on then-current plans and estimates of our board of directors and management regarding a liquidity event. The time to

liquidity increased from 1.5 years from June 2012 to 2.0 years in December 2012 due to the completion of the Series B convertible preferred stock financing in December 2012, which provided us with the funds to be able to have a liquidity event at a later date. The volatility assumption was based on an analysis of guideline companies' historical equity volatility factors for a period of 2.0 years, which is the term assumption. Based on this analysis of the guideline companies, a volatility assumption of 75% was selected and utilized. The risk-free rate was estimated as the interpolated 2.0 year U.S. Treasury yield. A discount for lack of marketability of 40% was then applied to the value indicated in our common stock. Based on these factors, the third-party valuation concluded that our common stock had a fair value of $0.58 per share as of December 31, 2012.

June 2013 Contemporaneous Valuation. For purposes of the June 2013 valuation, a hybrid method was used to determine our equity value, which is a hybrid between the probability-weighted return methodology, or PWERM, and the OPM. The PWERM is a scenario-based analysis that estimates the value per share based on the probability-weighted present value of expected future investment returns, considering each of the possible outcomes available to us, as well as the economic and control rights of each share class. In the hybrid method, the OPM is used to estimate the allocation of value within one or more of PWERM scenarios. The hybrid method can be a useful alternative to explicitly modeling all PWERM scenarios in situations when the company has transparency into one or more near-term exits but is unsure about what will occur if the current plans fall through. The hybrid model was selected at this time for the reasons described below relating to our plans for a potential IPO.

The OPM was used to allocate the equity value to the various securities under two scenarios. The first scenario assumed we would complete an IPO within 12 months and the second scenario assumed we would remain private beyond 12 months with a potential sale or merger in 2.0 years. The estimated time to liquidity was 1.0 year and 2.0 years based on timing of a liquidity event in the two scenarios. Based on an analysis of the guideline companies, a volatility assumption of 75% was selected and utilized for both scenarios. The risk-free rate was estimated based on the applicable U.S. Treasury yield. A discount for lack of marketability of 20% and 35% was applied to the value indicated in our common stock under the first scenario and the second scenario, respectively. As of June 30, 2013 our board of directors had not authorized our management to begin preparations for a potential IPO. The board made this decision in late July, and that is when we initiated our preparation process. The increased probability of an IPO was retroactively taken into consideration in the June 30, 2013 valuation, which is a critical factor contributing to the increase in the fair value of our common stock as of that date. Based on these factors, the third-party valuation concluded that our common stock had a fair value of $1.30 per share as of June 30, 2013.

September 2013 Contemporaneous Valuation. For purposes of the September 2013 valuation, a hybrid method was used to determine our equity value, which is consistent with the method used in the June 2013 valuation.

The OPM was used to allocate the equity value to the various securities under two scenarios. The first scenario assumed we would complete an IPO within six months and the second scenario assumed we would remain private beyond 12 months with a potential sale or merger in 1.5 years. We estimated a probability of 60% for the first scenario and 40% for the second scenario compared to a 50% weighting for each in the June 2013 valuation. The increase in the probability of an IPO was because, at the July 2013 meeting of our board of directors, preparations for a potential IPO were authorized, we subsequently selected a banking syndicate, and on September 6, 2013 an organizational meeting was held in order to begin the IPO process. The estimated time to liquidity was six months and 1.5 years based on timing of a liquidity event in the two scenarios. Based on an analysis of the guideline companies, a volatility assumption of 75% was selected and utilized for both scenarios. The risk-free rate was estimated based on the applicable U.S. Treasury yield. A discount for lack of marketability of 10% and 30% was applied to the value indicated in our common stock under the first scenario and the second scenario, respectively. In addition, in July 2013, we announced that we would initiate development of triheptanoin for a new indication, Glut1 DS, which was incorporated into the valuation. In August 2013, we entered into a collaboration agreement with KHK, which was also incorporated into the valuation. Based on these factors, the third-party valuation concluded that our common stock had a fair value of $2.19 per share as of September 30, 2013.

Stock Option Grants

Information regarding our stock option grants along with the estimated fair value per share of the underlying common stock, for stock options granted since January 1, 2012 is summarized in the table below:

Grant date	Number of common shares underlying options granted	Exercise price per common share	Estimated fair value per share of common stock
August 2, 2012	1,415,000	$ 0.26	$ 0.26
September 13, 2012	90,000	0.26	0.26
October 25, 2012	60,000	0.26	0.26
March 21, 2013	260,000	0.58	0.58
April 25, 2013	250,000	0.58	0.58
May 23, 2013	895,000	0.58	0.58
August 15, 2013	170,000	1.30	1.30
November 1, 2013	1,629,000	2.19	2.19

The intrinsic value of all outstanding options as of September 30, 2013 was $ million based on the estimated fair value of our common stock of $ per share, the midpoint of the estimated price range set forth on the cover of this prospectus.

The estimated fair value per share of the common stock in the table above represents the determination by our board of directors of the fair value of our common stock as of the date of the grant, as discussed below.

Stock Options Granted in August, September, and October 2012. Our board of directors granted stock options on August 2, 2012, September 13, 2012, and October 25, 2012, each having an exercise price of $0.26 per share. In establishing this exercise price, our board of directors considered input from management, the results of our June 30, 2012 third-party valuation, the objective and subjective criteria discussed above as well as the following: (i) in September 2012, we obtained the rights to rhPPCA and in-licensed North American rights to triheptanoin; (ii) triheptanoin had primarily been studied in academic settings with data available only from uncontrolled clinical studies or in anecdotal form; and (iii) rhPPCA has only ever been studied in mice and *in vitro*, and we have made very limited investments in the development of this compound to date. As each of these compounds was speculative, early stage, and would require significant funds and risk on our part to advance, our board of directors did not believe that these events increased our equity value.

In the judgment of our board of directors, there were no internal or external developments that would indicate that the fair value of our common stock would have increased from June 30, 2012. As a result, our board of directors concluded that the fair value of our common stock at each of these grant dates was $0.26 per share.

Stock Options Granted in March, April, and May 2013. Our board of directors granted stock options on March 21, 2013, April 25, 2013, and May 23, 2013, each having an exercise price of $0.58 per share. In establishing this exercise price, our board of directors considered input from management, the results of our December 31, 2012 third-party valuation, the objective and subjective criteria discussed above as well as the following: (i) a toxicology study for rhGUS, a retrospective study for triheptanoin, and the Phase 2 study for SA-ER were all ongoing during this time period, but no results had yet been generated; and (ii) these studies were all either underway or already contemplated at the time of the financing upon which the December 31, 2012 valuation was based. Given the lack of clarity around a future liquidity event, and the lack of any significant clinical data in the first five months of 2013, in the judgment of our board of directors, there were no internal or external developments that would indicate that the fair value of our common stock had increased from December 31, 2012. As a result, our board of directors concluded that the fair value of our common stock at each of these grant dates was $0.58 per share.

Stock Options Granted in August 2013. Our board of directors granted stock options on August 15, 2013, each having an exercise price of $1.30 per share. In establishing this exercise price, our board of directors considered input from management, the results of our June 30, 2013 third-party valuation, the objective and

subjective criteria discussed above as well as the following: (i) the probability of an IPO was already taken into consideration in the June 30, 2013 valuation and there was no change in probability as of August 15, 2013; (ii) the interim analysis of the 24-week data in our study of SA-ER showed only modest efficacy; (iii) although we had announced development of triheptanoin for GLUT1 DS between June 30, 2013 and August 15, 2013, no clinical development activity had yet commenced as of the latter date; and (iv) our acquisition of ex-U.S. rights to triheptanoin, which was announced in July 2013, was already taken into consideration in the valuation as of June 30, 2013. As a result, our board of directors concluded that the fair value of our common stock at this grant date was $1.30 per share.

Stock Options Granted in November 2013. Our board of directors granted stock options on November 1, 2013, each having an exercise price of $2.19 per share. In establishing this exercise price, our board of directors considered input from management, the results of our September 30, 2013 third-party valuation, and the objective and subjective criteria discussed above. The board of directors considered the increase in the probability of an IPO from 50% as of June 30, 2013 to 60% as of September 30, 2013 and also concluded that no further change in this probability had taken place as of November 1, 2013. In the judgment of our board of directors, there were no internal or external developments that would indicate the fair value of our common stock would have increased from September 30, 2013. As a result, our board of directors concluded that the fair value of our common stock at this grant date was $2.19 per share.

Income Taxes

We use the liability method of accounting for income taxes. Under this method, deferred tax assets and liabilities are determined based on the differences between the financial reporting and the tax bases of assets and liabilities and are measured using the enacted tax rates and laws that will be in effect when the differences are expected to reverse. We assess the likelihood that the resulting deferred tax assets will be realized. A valuation allowance is provided when it is more likely than not that some portion or all of a deferred tax asset will not be realized.

As of December 31, 2012, our total deferred tax assets were $10.1 million. Due to our lack of earnings history and uncertainties surrounding our ability to generate future taxable income, the net deferred tax assets have been fully offset by a valuation allowance. The deferred tax assets were primarily comprised of federal and state tax net operating losses and tax credit carryforwards. Utilization of the net operating loss and tax credit carryforwards may be subject to an annual limitation due to historical or future ownership percentage change rules provided by the Internal Review Code of 1986, and similar state provisions. The annual limitation may result in the expiration of certain net operating loss and tax credit carryforwards before their utilization.

Results of Operations

Comparison of the nine months ended September 30, 2012 and 2013

	Nine Months Ended September 30,		Dollar Change	% Change
	2012	2013		
	(dollars in thousands)			
Operating expenses:				
Research and development	$ 8,866	$ 19,625	$ 10,759	121%
General and administrative	2,441	3,130	689	28%
Total operating expenses	11,307	22,755	11,448	101%
Loss from operations	(11,307)	(22,755)	(11,448)	101%
Interest income	—	157	157	*
Other expense	(97)	(1,155)	(1,058)	*
Net loss	$ (11,404)	$ (23,753)	$ (12,349)	108%

Research and Development Expenses. Research and development expenses increased $10.8 million, or 121%, for the nine months ended September 30, 2013 compared to the same period in 2012. The following table summarizes our research and development expenses for the nine months ended September 30, 2012 and 2013 and for the period from April 22, 2010 (inception) through September 30, 2013.

	Nine Months Ended September 30,		Period from April 22, 2010 (Inception) Through September 30, 2013
	2012	2013	
	(dollars in thousands)		
Development candidate:			
rhGUS	$ 1,903	$ 5,915	$ 9,561
rhPPCA	141	205	384
Triheptanoin (LC-FAOD)	281	4,074	4,620
Triheptanoin (Glut1 DS)	—	1,032	1,032
SA-ER	5,223	5,942	17,268
KRN23	—	124	124
Preclinical and other research costs	1,318	2,333	4,952
Total research and development expenses	$ 8,866	$ 19,625	$ 37,941

The increase in research and development expenses above is primarily due to:

- for rhGUS, an increase of $4.0 million related to the development and manufacturing associated with supplying the product candidate for our toxicology and clinical studies;
- for triheptanoin (LC-FAOD), an increase of $3.8 million related to the initiation of our program in 2013, which included $0.8 million we paid to exercise the option with Baylor Research Institute, or BRI, pursuant to our license agreement with BRI to license the rights to triheptanoin in all territories outside of North America and $1.0 million in personnel-related costs as we allocated resources to this program, and costs related to the manufacture of clinical supplies;
- for triheptanoin (Glut1 DS), an increase of $1.0 million related to costs associated with the start up of clinical activities, including $0.4 million in personnel costs as we allocated resources to this program;
- for SA-ER, an increase of $0.7 million related to the increase in clinical activities for this program; and
- an increase of $1.0 million in preclinical and other product development costs for various other potential product candidates.

General and Administrative Expenses. General and administrative expenses increased $0.7 million, or 28%, for the nine months ended September 30, 2013 compared to the same period in 2012. The increase in general and administrative expenses was primarily due to increases in professional services costs and in personnel costs in support of our research and development activities.

Interest Income. Interest income increased $0.2 million for the nine months ended September 30, 2013 compared to the same period in 2012, primarily due to funds invested from our Series B convertible stock financing completed in December 2012.

Other Expense. Other expense increased $1.1 million for the nine months ended September 30, 2013 compared to the same period in 2012. The increase was due to the fair value remeasurement of the liability related to our convertible preferred stock warrants.

Comparison of Years Ended December 31, 2011 and 2012

	Year ended December 31,		Dollar Change	% Change
	2011	2012		
	(dollars in thousands)			
Operating expenses:				
Research and development	$ 4,717	$ 12,641	$ 7,924	168%
General and administrative	1,844	3,344	1,500	81%
Total operating expenses	6,561	15,985	9,424	144%
Loss from operations	(6,561)	(15,985)	(9,424)	144%
Interest income	4	1	(3)	*
Interest expense	(270)	—	270	*
Other expense	(22)	(350)	(328)	*
Net loss	$(6,849)	$(16,334)	$ 9,485	138%

* *not meaningful*

Research and Development Expenses. Research and development expenses increased $7.9 million, or 168%, for the year ended December 31, 2012 compared to the same period in 2011. The following table summarizes our research and development expenses for the years ended December 31, 2011 and 2012 and for the period from April 22, 2010 (inception) through September 30, 2013.

	Year Ended December 31,		Period from April 22, 2010 (Inception) through December 31, 2012
	2011	2012	
	(in thousands)		
Development candidate:			
rhGUS	$ 438	$ 3,198	$ 3,646
rhPPCA	7	172	179
Triheptanoin (LC-FAOD)	—	546	546
SA-ER	3,570	6,840	11,326
Preclinical and other research costs	702	1,885	2,619
Total research and development expenses	$4,717	$12,641	$ 18,316

The increase in research and development expenses above is primarily due to:

- for SA-ER, an increase of $3.3 million related to the development and manufacturing costs for our various clinical studies, increase in personnel costs, and the costs for our Phase 2 clinical study;
- for rhGUS, an increase of $2.8 million related to the development and manufacturing costs associated with supplying rhGUS for our various clinical studies;
- for triheptanoin, an increase of $0.5 million related to the increase in clinical activities, as well as $0.3 million paid to BRI to license the rights to triheptanoin; and
- an increase of $1.2 million in preclinical and other research costs for various other potential product candidates.

General and Administrative Expenses. General and administrative expenses increased $1.5 million, or 81%, for the year ended December 31, 2012 compared to the same period in 2011. The increase in general and administrative expenses was primarily due to increases in professional services costs and in personnel costs in support of our research and development activities.

Interest Expense. Interest expense decreased $0.3 million for the year ended December 31, 2012 compared to the same period in 2011. The decrease is due to the conversion of the outstanding convertible promissory notes and related accrued interest into shares of our Series A convertible preferred stock in June 2011.

Other Expense. Other expense increased $0.3 million for the year ended December 31, 2012 compared to the same period in 2011. The increase was primarily due to the fair value remeasurement of the liability related to our convertible preferred stock warrants.

Liquidity and Capital Resources

Since our inception, we have funded our operations primarily with $103.9 million in net proceeds from the sale of convertible preferred stock and $3.6 million in proceeds received from convertible promissory notes. As of September 30, 2013, we had $63.7 million in cash, cash equivalents, and marketable securities and a deficit accumulated during the development stage of $56.8 million.

Our primary use of cash is to fund operating expenses, which consist primarily of research and development expenditures. Due to our significant research and development expenditures, we have generated significant operating losses since our inception. Cash used to fund operating expenses is impacted by the timing of when we pay these expenses, as reflected in the change in our outstanding accounts payable and accrued expenses.

The following table summarizes our cash flows for the periods indicated (in thousands):

	Year Ended December 31,		Nine Months Ended September 30,	
	2011	2012	2012	2013
			(unaudited)	
Cash used in operating activities	$ (5,825)	$ (12,504)	$ (8,790)	$ (21,427)
Cash used in investing activities	(924)	(1,191)	(1,067)	(58,847)
Cash provided by financing activities	17,329	89,240	15,228	133
Net increase (decrease) in cash and cash equivalents	$ 10,580	$ 75,545	$ 5,371	$ (80,141)

Cash Used in Operating Activities

Cash used in operating activities for the nine months ended September 30, 2013 was $21.4 million and reflected net loss of $23.8 million, offset by non-cash charges of $0.3 million for depreciation and amortization, $1.0 million for the amortization of premium paid on purchased marketable securities, $0.4 million for stock-based compensation and $1.1 million for the revaluation of convertible preferred stock warrant liability. Cash used in operating activities also reflected a $2.7 million increase in prepaid expenses and other current assets primarily due to deferred offering costs related to our IPO and an increase in interest income receivable as our invested funds increased with the sale of our Series B convertible preferred stock in December 2012. The increase was offset by a $2.4 million increase in accounts payable and accrued liabilities primarily due to higher clinical study and related costs as we continued to increase our research and development activities.

Cash used in operating activities for the nine months ended September 30, 2012 was $8.8 million and reflected net loss of $11.4 million, offset by non-cash charges of $0.2 million for depreciation and amortization, $0.7 million for stock-based compensation and $0.1 million for the revaluation of convertible preferred stock warrant liability. Cash used in operating activities reflected an increase of $1.6 million in accounts payable and accrued liabilities related to higher clinical study and related costs and other research and development activities.

Cash used in operating activities for the year ended December 31, 2012 was $12.5 million and reflected net loss of $16.3 million, offset by non-cash charges of $0.9 million for stock-based compensation, $0.3 million for depreciation and amortization, and $0.3 million expense for the revaluation of the convertible preferred stock warrant liability. Cash used in operating activities also reflected an increase in accounts payable and accrued and other liabilities of $2.4 million related to higher clinical study and related costs and other research and development activities.

Cash used in operating activities for the year ended December 31, 2011 was $5.8 million and reflected net loss of $6.8 million, offset by non-cash charges of $0.3 million for interest expense related to the convertible promissory notes and $0.3 million for stock-based compensation. Cash used in operating activities also reflected

an increase in accounts payable and accrued and other liabilities of $0.7 million as we continued to increase our research and development activities and the timing of payments made to our vendors, which was partially offset by an increase in prepaid expenses and other current assets of $0.2 million.

Cash Used in Investing Activities

Cash used in investing activities for the nine months ended September 30, 2013 was $58.8 million and related to purchases of marketable securities of $63.1 million, proceeds from maturities of marketable securities of $4.5 million and purchases of property and equipment of $0.3 million.

Cash used in investing activities for the nine months ended September 30, 2012 was $1.1 million and primarily related to purchases of property and equipment related to our move to a new leased facility in March 2012.

Cash used in investing activities for the year ended December 31, 2012 was $1.2 million and related to purchases of property and equipment of $1.1 million related to our move to a new leased facility in March 2012 and an increase in restricted cash of $0.1 million.

Cash used in investing activities for the year ended December 31, 2011 was $0.9 million and was related to purchases of property and equipment of $0.5 million and an increase in restricted cash of $0.4 million.

Cash Flows Provided by Financing Activities

Cash provided by financing activities for the nine months ended September 30, 2013 was $0.1 million related to proceeds from the issuance of common stock for the exercise of stock options.

Cash provided by financing activities for the nine months ended September 30, 2012 was $15.2 million and primarily related to net proceeds from the issuance of convertible preferred stock of $15.1 million.

Cash provided by financing activities for the year ended December 31, 2012 was $89.2 million and primarily related to net proceeds from the issuance of convertible preferred stock of $89.0 million.

Cash provided by financing activities for the year ended December 31, 2011 was $17.3 million and related to net proceeds from the issuance of $14.9 million of convertible preferred stock and $2.4 million of promissory notes.

Funding Requirements

We believe that our existing capital resources, not including the proceeds we receive from this offering, will be sufficient to meet our projected operating requirements for at least the next 12 months. We anticipate that we will continue to generate losses for the foreseeable future, and we expect the losses to increase as we continue the development of, and seek regulatory approvals for, our product candidates, and begin to commercialize any approved products. If we need to raise additional capital to fund our operations and complete our ongoing and planned clinical studies, funding may not be available to us on acceptable terms, or at all. We expect to finance future cash needs through public or private equity or debt offerings. If we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may be required to delay, limit, reduce, or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Our future funding requirements will depend on many factors, including the following:

- the scope, rate of progress, results and cost of our clinical studies, nonclinical testing, and other related activities;
- the cost of manufacturing clinical supplies, and establishing commercial supplies, of our product candidates and any products that we may develop;
- the number and characteristics of product candidates that we pursue;
- the cost, timing, and outcomes of regulatory approvals;

- the cost and timing of establishing sales, marketing, and distribution capabilities; and
- the terms and timing of any collaborative, licensing, and other arrangements that we may establish, including any required milestone and royalty payments thereunder.

Contractual Obligations

The following table summarizes our contractual obligations at December 31, 2012 (in thousands):

	Payments due by period				
	Less than 1 year	1 to 3 years	3 to 5 years	More than 5 years	Total
Operating leases	$ 345	$570	$332	$ —	$1,247

JOBS Act Accounting Election

The Jumpstart our Business Startups Act of 2012, or the JOBS Act, permits an "emerging growth company" such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies. We are choosing to "opt out" of this provision and, as a result, we will comply with new or revised accounting standards as required when they are adopted. This decision to opt out of the extended transition period under the JOBS Act is irrevocable.

Newly Adopted Accounting Pronouncements

In February 2013, the Financial Accounting Standards Board issued Accounting Standards Update No. 2013-02, or ASU 2013-02, Reporting of Amounts Reclassified Out of Accumulated Other Comprehensive Income. ASU 2013-02 requires reporting and disclosure about changes in accumulated other comprehensive income balances and reclassifications out of accumulated other comprehensive income. We adopted this guidance as of January 1, 2013 on a prospective basis, and this adoption did not have an impact on our financial statements.

Off-Balance Sheet Arrangements

Since our inception, we have not engaged in any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

Quantitative and Qualitative Disclosures About Market Risk

Our exposure to market risk for changes in interest rates relates primarily to interest earned on our cash equivalents and marketable securities. The primary objective of our investment activities is to preserve our capital to fund operations. A secondary objective is to maximize income from our investments without assuming significant risk. Our investment policy provides for investments in low-risk, investment-grade debt instruments. As of September 30, 2013, we had cash, cash equivalents, and marketable securities totaling $63.7 million consisting of bank deposits, money market funds, and investment-grade corporate bonds which are subject to default, changes in credit rating, and changes in market value. The securities in our investment portfolio are classified as available for sale and are subject to interest rate risk and will decrease in value if market interest rates increase. A hypothetical 10% change in interest rates during any of the period presented would not have had a material impact on our financial statements. To date, we have not experienced a loss of principal on any of our investments.

We face foreign exchange risk as a result of entering into transactions denominated in currencies other than U.S. dollars. Due to the uncertain timing of expected payments in foreign currencies, we do not utilize any forward exchange contracts. All foreign transactions settle on the applicable spot exchange basis at the time such payments are made. An adverse movement in foreign exchange rates could have a material effect on payments made to foreign suppliers and for license agreements. A hypothetical 10% change in foreign exchange rates during any of the periods presented would not have had a material impact on our financial statements.

BUSINESS

Overview

We are a development-stage biopharmaceutical company focused on the identification, acquisition, development, and commercialization of novel products for the treatment of rare and ultra-rare diseases, with an initial focus on serious, debilitating metabolic genetic diseases. We focus on diseases for which the unmet medical need is high, the biology for treatment is clear, and for which there are no approved therapies. Since our inception in 2010, we have in-licensed potential treatments for five different diseases that are or we expect will be in Phase 2 clinical studies by early 2014. Our strategy, which is predicated upon time- and cost-efficient drug development, allows us to pursue multiple programs in parallel with the goal of delivering safe and effective therapies to patients with the utmost urgency.

Our current pipeline consists of two product categories: biologics, including a monoclonal antibody and enzyme replacement therapies; and small-molecule substrate replacement therapies. Enzymes are proteins that the body uses to process materials needed for normal cellular function, and substrates are the materials upon which enzymes act. When enzymes or substrates are missing, the body is unable to perform its normal cellular functions, often leading to significant clinical disease. Several of our therapies are intended to replace deficient enzymes or substrates. Our biologics pipeline includes the following three product candidates:

- KRN23, or UX023, is an antibody targeting fibroblast growth factor 23, or FGF23, intended for the treatment of X-linked hypophosphatemia, or XLH, a rare genetic disease that impairs bone growth. We are developing KRN23 pursuant to our collaboration with Kyowa Hakko Kirin Co., Ltd., or KHK. KHK has conducted one Phase 1 study, one Phase 1/2 study and one Phase 1/2 extension study of KRN23 in adults with XLH. Data from the Phase 1/2 studies should be available in 2014. We expect to continue the clinical development of KRN23 in adults as well as initiate pediatric clinical development in 2014.
- rhGUS, or UX003, is an enzyme replacement therapy we are developing for the treatment of mucopolysaccharidosis 7, or MPS 7, a rare lysosomal storage disease that often leads to multi-organ dysfunction, pervasive skeletal disease, and death. We plan to initiate a Phase 1/2 clinical study in MPS 7 by the end of 2013.
- rhPPCA, or UX004, is an enzyme replacement therapy in preclinical development for galactosialidosis, a rare lysosomal storage disease that can cause multi-system clinical disease similar to MPS 7 including enlarged liver, joint disease, abnormal bone development, short stature, and death. We plan to continue preclinical development of rhPPCA during 2014.

Our substrate replacement therapy pipeline includes the following product candidates in development for three diseases:

- Triheptanoin, or UX007, is a synthetic oil with a specifically designed chemical composition being studied in an investigator-sponsored Phase 2 study for the treatment of long-chain fatty acid oxidation disorders, or LC-FAOD. This is a set of rare metabolic diseases that prevent the conversion of fat into energy and can cause low blood sugar, muscle rupture, and heart and liver disease. We plan to initiate our own Phase 2 study in LC-FAOD by the end of 2013.
- Triheptanoin is also in an investigator-sponsored Phase 2 study for the treatment of glucose transporter type-1 deficiency syndrome, or Glut1 DS, a rare metabolic disease of brain energy deficiency that can result in seizures, developmental delay, and movement disorder. We plan to initiate our own Phase 2 clinical study in Glut1 DS in early 2014.
- SA-ER, or UX001, is an extended-release form of sialic acid in a Phase 2 study for the treatment of hereditary inclusion body myopathy, or HIBM, a neuromuscular disorder that causes muscle weakness and wasting. We reported 24-week data from our ongoing Phase 2 study in HIBM in July 2013 and anticipate top-line 48-week data by the end of 2013.

Our current product candidate pipeline has been either in-licensed from academic institutions or derived from partnerships with other pharmaceutical companies. Where possible, our strategy is to acquire and retain

global commercialization rights to our products to maximize long-term value. Over time, we intend to build our own commercial organization, which we believe will be of modest size due to the relatively small number of specialists who treat patients with rare and ultra-rare diseases.

The patients we seek to treat have diseases with limited or no treatment options, and we recognize that their lives and well-being are highly dependent upon our efforts to develop new therapies. For this reason, we are passionate about developing these therapies with the utmost urgency and care. We strive to build a company that is faster, better, and smarter about advancing multiple product candidates through approval.

We were founded in April 2010 by our current President and Chief Executive Officer, Dr. Emil Kakkis, M.D., Ph.D., who is the former Chief Medical Officer of BioMarin Pharmaceutical Inc. We have assembled an experienced team with extensive drug development and commercialization capabilities, particularly in the orphan drug area. Dr. Kakkis and the team at Ultragenyx have been previously involved at other companies, in the development and/or commercialization of many therapies approved or in development for rare metabolic genetic diseases, including Aldurazyme, Naglazyme, Kuvan, and Vimizim (BioMarin); Lumizyme/Myozyme (Sanofi-Genzyme); and asfotase alpha (Enobia; now Alexion). Our investors include, but are not limited to, the following entities or their affiliates or funds advised by them: TPG Biotech, Fidelity Biosciences (Beacon Bioventures), HealthCap, Pappas Ventures, Adage Capital Partners, L.P., Capital Research Global Investors, Columbia Wanger Asset Management, Jennison Associates LLC, BlackRock, Inc., Genzyme Corporation, Shire LLC, and Ramius LLC.

Our Strategy

Our strategy is to identify, acquire, develop, and commercialize novel products for the treatment of rare and ultra-rare diseases in the United States, the European Union, and select international markets, with the goal of becoming a leading rare disease biotechnology company. The critical components of our business strategy include the following:

- ***Focus on rare and ultra-rare diseases with significant unmet medical need.*** There are numerous rare and ultra-rare metabolic genetic diseases that currently have no approved drug therapy and for which no therapies are currently in development. Patients suffering from these diseases often have a high unmet medical need with significant morbidity and/or mortality. We are focused on developing and commercializing therapies for multiple such indications with the utmost urgency.
- ***Focus on diseases and therapies with clear mechanisms of action.*** We also focus on diseases that have biology and root causes that are well understood. For example, several of our product candidates are replacement therapies for a single deficient enzyme or substrate in the body. We believe that developing drugs that directly impact known disease pathways will increase the probability of success of our development programs.
- ***Leverage our experience and relationships to in-license promising product candidates.*** Our management team has strong relationships with key opinion leaders in the metabolic genetic field, as well as a history of success in the development and commercialization of therapies for rare and ultra-rare genetic diseases. Accordingly, we enjoy unique access to many in-licensing opportunities. All of our current product candidates are in-licensed from academic institutions or derived from partnerships with other pharmaceutical companies on attractive terms. We believe these parties have agreed to license product candidates to us because they are confident in our drug development capabilities and experience in bringing rare disease therapies to market.
- ***Develop and commercialize multiple product candidates in parallel.*** Clinical studies for rare and ultra-rare diseases can often be smaller, fewer in number, and less expensive than those for larger market indications. Development of multiple programs in the metabolic genetics field also generates organizational efficiencies and economies of scale. As a result of these efficiencies, we can feasibly develop multiple clinical-stage product candidates in parallel, resulting in a more diversified portfolio that provides multiple opportunities to create value.

- ***Focus on excellent and rapid clinical and regulatory execution.*** We believe that building a successful and sustainable rare disease-focused company requires very specific expertise in the areas of patient identification, clinical study design and conduct, and regulatory strategy. We have assembled a team with a successful track record in managing global clinical development activities in an efficient manner, and with multinational experience in obtaining regulatory approvals for rare disease products.
- ***Seek to retain global commercialization rights to product candidates.*** We intend to seek and retain global commercialization rights to our product candidates whenever possible to maximize the potential value of our product portfolio. Our plan is to establish our own commercial organization in major pharmaceutical markets and develop a network of third-party distributors in smaller markets. We believe this commercial organization can be modest and targeted due to the relatively small number of specialists who typically treat patients with the diseases to be addressed by our product candidates. As a result, we do not expect that we will require pharmaceutical partners for commercialization of our product candidates, although we may consider partnering for certain territories or indications or for other strategic purposes.

Product Candidates

The following table summarizes our product candidate pipeline:

Candidate	Description	Indication	Pre-clinical	Phase 1	Phase 1/2 or Phase 2	Phase 3 or pivotal	Status / Anticipated milestones	Ultragenyx commercial rights
Biologics								
KRN23 (UX023)	Anti-FGF23 monoclonal antibody	XLH					▪ Expect to initiate pediatric clinical development in 2014	▪ U.S. and Canada: Joint with KHK (profit share) ▪ Mexico, Central and South America
rhGUS (UX003)	Enzyme replacement	MPS 7					▪ Expect to initiate a Phase 1/2 clinical study by end of 2013	▪ Worldwide
rhPPCA (UX004)	Enzyme replacement	Galactosialidosis					▪ Expect to continue preclinical development during 2014	▪ Worldwide
Small molecules								
Triheptanoin (UX007)	Substrate replacement	LC-FAOD					▪ Expect to initiate Phase 2 study by end of 2013	▪ Worldwide
Triheptanoin	Substrate replacement	Glut1 DS					▪ Expect to initiate Phase 2 clinical study in early 2014	▪ Worldwide
SA-ER (UX001)	Substrate replacement	HIBM					▪ Expect data from ongoing Phase 2 study by end of 2013	▪ Worldwide (excluding Japan and certain other Asian territories)

Biologics product candidates

KRN23 for the treatment of XLH

KRN23 is a fully human monoclonal antibody administered via subcutaneous injection that is designed to bind and reduce the biological activity of FGF23 to increase abnormally low phosphate levels in patients with XLH. In August 2013, we formed a collaboration with and licensed certain intellectual property from Kyowa Hakko Kirin Co., Ltd., or KHK, to jointly develop and commercialize KRN23 for the treatment of XLH. KHK has conducted one Phase 1 study, one Phase 1/2 study and one Phase 1/2 extension study of KRN23 in adults with XLH. We expect to receive data for the Phase 1/2 studies in 2014. We expect to continue the clinical development of KRN23 in adults as well as initiate pediatric clinical development in 2014.

XLH disease background

Patients with XLH have low serum phosphate levels due to excessive phosphate loss into the urine, which is directly caused by the effect on kidney function of excess FGF23 production in bone cells. FGF23 is produced by cells responsible for bone formation to manage calcification as part of bone growth and remodeling by signaling to the kidney to promote phosphate excretion and to suppress vitamin D production when phosphate levels are too high. Low phosphate levels lead to poor bone mineralization and a variety of clinical manifestations, including skeletal deformity, bone pain, short stature, gross motor impairment, muscle weakness, and lower than normal bone density. XLH is an inherited genetic disease that can be genetically confirmed and affects both males and females. Diagnosis of the disease typically takes place via assessment of clinical presentation, x-ray of the bones, and urine and blood tests to confirm renal phosphate wasting.

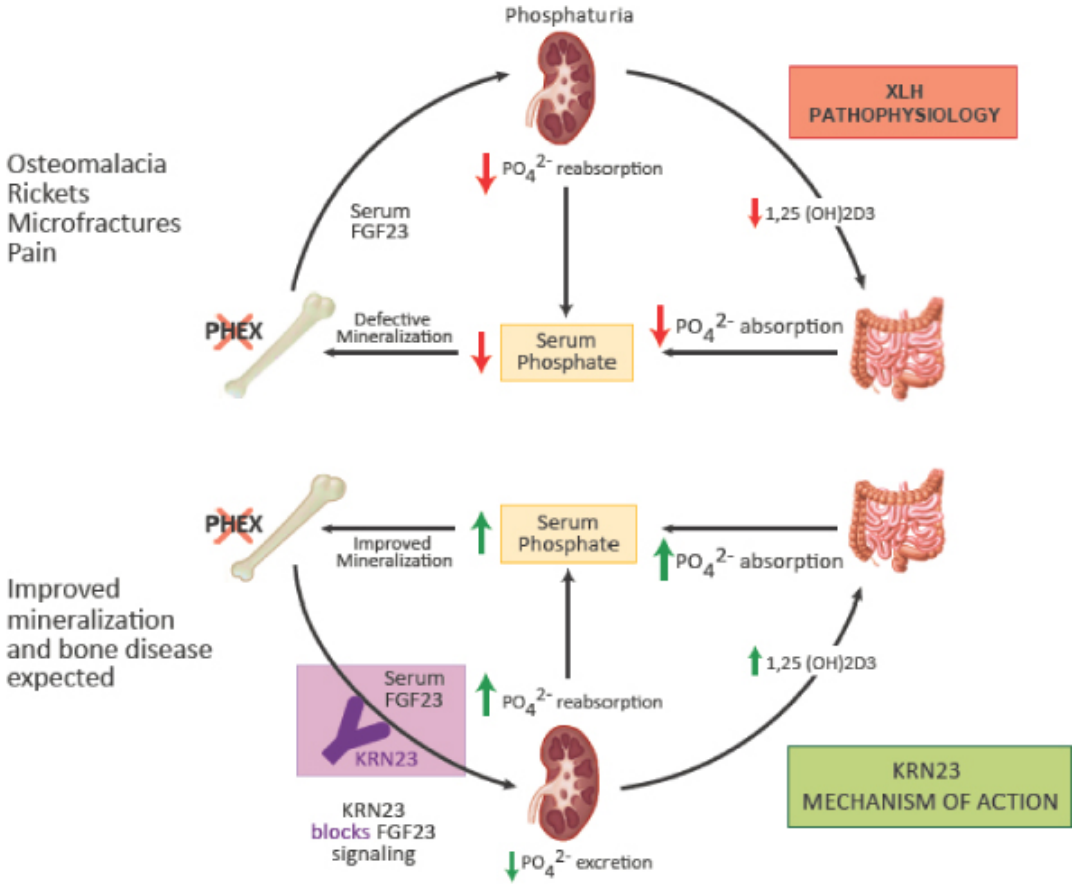
XLH patients have a genetic deficiency in a regulatory system that senses phosphate levels and uses FGF23 to control serum phosphate. The deficiency interferes with this regulatory process and leads to abnormal up-regulation of FGF23, which causes phosphate wasting by the kidney. FGF23 induces profound reductions in serum phosphate levels by two mechanisms simultaneously: reducing renal reabsorption via reducing expression of a sodium phosphate co-transporter, as well as reducing intestinal phosphate reabsorption via a reduction in active vitamin D production.

There is no approved drug therapy or treatment for the underlying cause of XLH. Most patients are managed using oral phosphate replacement and vitamin D therapy, which is only partially effective at restoring bone physiology and growth and has significant side effects. These therapies require extremely close monitoring due to the potential for excessive spikes in phosphate levels, which can result in severe damage to the kidneys from excess calcium and phosphate deposits and other complications. Additionally, some patients are unable to tolerate the regimen due to the chalky stool that results from taking large amounts of oral phosphate or the high frequency of dosing required. We believe XLH patients need a better treatment option that is more specific to the underlying cause of the disease and safer in how it manages phosphate levels.

KRN23 background and clinical development

KRN23 is designed to bind FGF23 and interfere with a required co-stimulatory signaling mechanism. The interference in FGF23 signaling is intended to prevent phosphate wasting in the urine and increase serum phosphate levels. Increased serum phosphate levels are intended to improve bone mineralization and reduce the severity of the clinical manifestations of XLH.

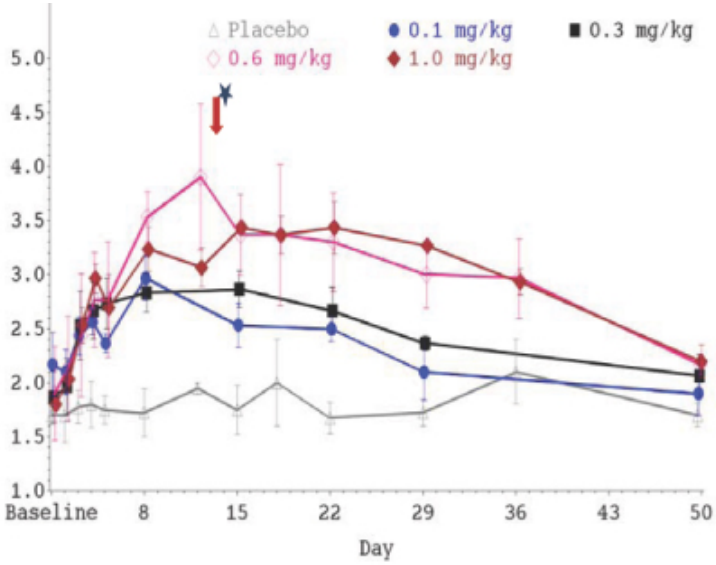
The following graphic illustrates the pathophysiology of XLH and the mechanism of action of KRN23.



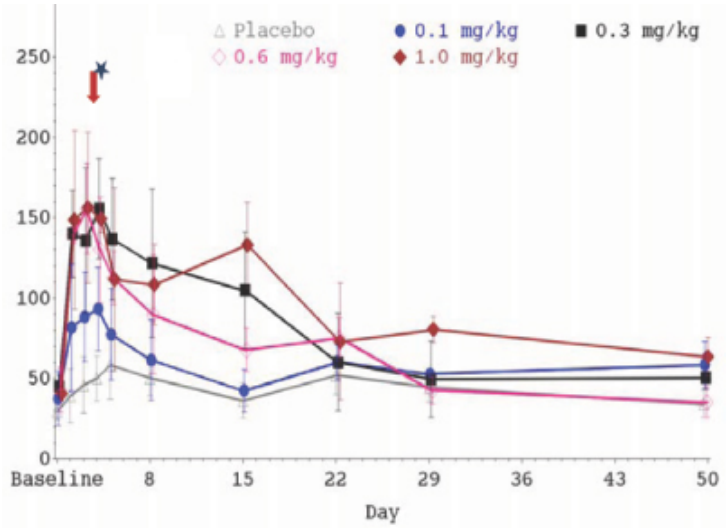
KHK has conducted one Phase 1 study, one Phase1/2 study, and one Phase 1/2 extension study of KRN23 in adults with XLH. Results from the Phase 1 single dose study in 38 adult XLH patients were presented at the American Society for Bone and Mineral Research in October 2013 and demonstrated that KRN23 was well tolerated and increased serum phosphate, or phosphate in the blood, and vitamin D levels. Of the 38 adult XLH patients, 12 received a single subcutaneous injection of KRN23 (at doses of 0.1, 0.3, 0.6, or 1.0 mg/kg), 17 received a single intravenous injection of KRN23 (at doses of 0.003, 0.01, 0.03, 0.1, or 0.3 mg/kg) and 9 received placebo. The effect of KRN23 on the increase in serum phosphate levels was comparable between intravenous and subcutaneous administration; however, time to reach peak effect was slower and duration of effect was greater with subcutaneous administration compared with intravenous administration. Although the phosphate levels did not reach the normal range, the demonstrated improvement suggests that significant benefit could be expected. No serious adverse events were reported in the Phase 1 study, although some patients experienced non-serious treatment-emergent adverse events. The most common non-serious treatment-emergent adverse events in the study overall were nausea and headache, although no patients in the placebo or subcutaneous treatment arms reported these events. In the subcutaneous arm, two patients experienced elevated levels of the enzyme amylase in the blood, and two patients experienced back pain. There did not appear to be a relationship between the incidence and types of adverse events and the dose administered following a single dose of study drug. The following graphics illustrate the results of KHK's Phase 1 single dose study relating to serum phosphate and vitamin D levels after subcutaneous injection, which is the route of administration planned for use in all subsequent clinical trials. Increases were statistically significant at the 0.3, 0.6, and 1.0 mg/kg subcutaneous dose

levels, with p-values less than 0.001 for serum phosphate and less than 0.01 for vitamin D. P-values are an indication of statistical significance reflecting the probability of an observation occurring due to chance alone. Differences with a p-value of less than 0.05 are considered statistically significant.

Serum Phosphate (mg/dL)



Vitamin D (pg/mL)



We expect to continue to develop KRN23 in adults with XLH. We are beginning clinical development for a pediatric indication for KRN23 and plan to initiate a Phase 2 pediatric study in 2014. Depending on the results of our Phase 2 pediatric study, we intend to conduct a Phase 3 pediatric trial. Given the high turnover and growth of bone during childhood and the critical role phosphate plays in bone growth, pediatric XLH patients have the

highest morbidity and potential for benefit. As a result, pediatric XLH patients may also have the greatest potential for improvement based on third-party data regarding enzyme replacement therapy in hypophosphatasia, which is another genetic bone disease with poor bone mineralization related to phosphate metabolism caused by a different, unrelated mechanism. Furthermore, given the more rapid turnover of bone in children as compared to adults, the timeframe to observe improvement is expected to be more rapid as well. We also plan to conduct an adult Phase 2b study in parallel with our Phase 3 pediatric trial.

Potential market opportunity

Based on incidence and prevalence rates published in a Danish epidemiologic study and surveys of physicians in the United States, we estimate that there are approximately 3,000 cases of XLH in pediatric patients in the United States. Further, there are an estimated 9,000 cases of XLH in adult patients in the United States. However, we expect that many of these adult patients may not seek treatment if their bone disease is not too severe.

rhGUS for the treatment of MPS 7

We are developing recombinant human beta-glucuronidase, or rhGUS, as an intravenous, or IV, enzyme replacement therapy for the treatment of MPS 7, also known as Sly Syndrome. Patients with MPS 7 suffer from severe cellular and organ dysfunction that typically leads to death in the teens or early adulthood. MPS 7 is caused by a deficiency of the lysosomal enzyme beta-glucuronidase, or GUS, which is required for the breakdown of certain complex carbohydrates known as glycosaminoglycans, or GAGs. The inability to properly break down GAGs leads to their accumulation in many tissues, resulting in a serious multi-system disease. We licensed exclusive worldwide rights to rhGUS-related know-how and cell lines from Saint Louis University in November 2010. We expect to initiate a Phase 1/2 study of rhGUS in MPS 7 patients by the end of 2013 and report interim data in 2014.

MPS 7 disease background

Patients with MPS 7 may have abnormal coarsened facial features, enlargement of the liver and spleen, airway obstruction, lung disease, cardiovascular complications, joint stiffness, short stature, and a skeletal disease known as dysostosis multiplex. In addition, many patients experience progressive lung problems as a result of airway obstruction and mucous production, often leading to sleep apnea and pulmonary insufficiency, and eventually requiring tracheostomy. Significant enlargement of the liver and spleen and an abnormal ribcage, when combined with frequent recurrent and chronic infections, can lead to progressive respiratory compromise and failure. Heart disease is also common in patients with severe MPS 7, although it may not develop or manifest until later in life. Lysosomal storage of GAGs within the joint tissues can lead to significant stiffness and restriction of mobility in the hip, shoulder, elbow, and knee joints. All of these disease complications can result in severe pain or the inability to walk, often resulting in the use of a wheelchair. Additional symptoms can include corneal clouding, hernias, visual loss, hearing loss, and developmental delay.

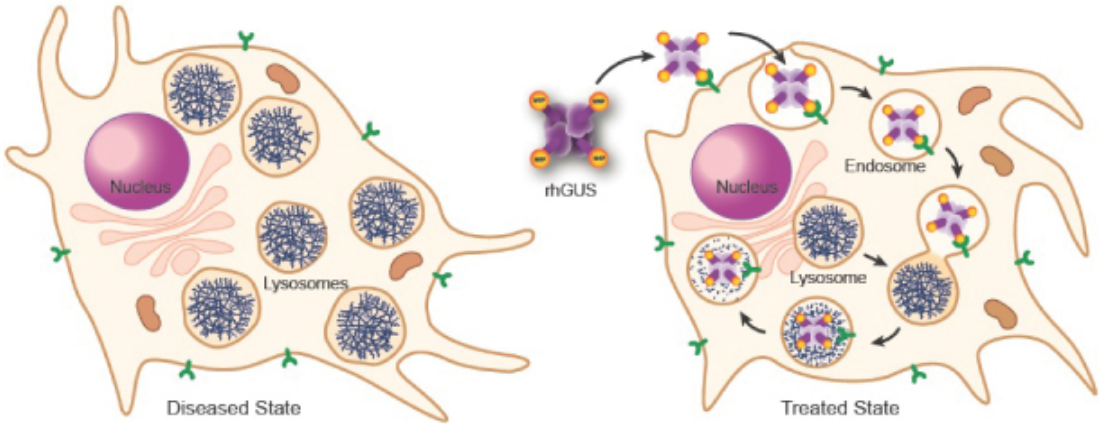
Most MPS 7 patients die between their teenage years and thirties, though some may live longer. The most severe form of the disease can uniquely present at birth with non-immune hydrops fetalis, which is almost always fatal and may account for as much as half of the disease incidence. Non-immune hydrops fetalis is a very severe neonatal condition in which the child retains an enormous amount of fluid throughout the body. Infants with hydrops fetalis rarely survive beyond a few weeks to a few months of age.

MPS 7 is often suspected from coarsened facial features, physical disease, and liver enlargement on clinical examination. Urine tests demonstrate excess GAG excretion, and the specific MPS disease is definitively diagnosed through tests that demonstrate a deficiency in GUS enzyme activity. Prenatal diagnostic tools have also been used to verify if a fetus has a deficiency of the enzyme and is affected with the disease.

There are currently no approved drug therapies for MPS 7. A few patients have been given bone marrow or hematopoetic stem cell transplants, but their skeletal and connective tissue disease is not effectively treated by these transplants, and the morbidity and mortality of the transplant procedure can be significant, particularly if not conducted during the first two years of life.

rhGUS background

GUS is an enzyme found in the lysosome, a digestive compartment inside the cell needed to process sugars, fats, and proteins. As with other lysosomal enzymes, uptake of GUS into cells and tissues occurs by a particular receptor that recognizes a certain marker on the enzyme, known as mannose-6-phosphate, which is critical for optimal tissue distribution and rapid clearance of GAGs. Studies in cell culture and *in vivo* have demonstrated that our rhGUS product candidate is taken up into cells efficiently by this receptor, resulting in the delivery of the enzyme into the lysosomes and clearance of stored GAGs in MPS 7 animal models. The following graphic illustrates the receptor-mediated uptake of the rhGUS enzyme by the diseased cell and the subsequent clearance of storage of accumulated GAGs in the lysosomes.

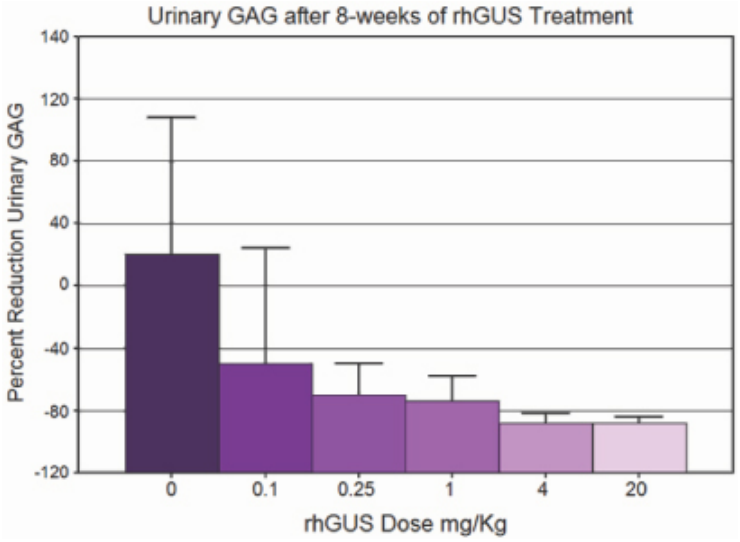


Historical studies in preclinical models of MPS 7 have shown that human or mouse GUS enzyme replacement achieves distribution to many tissues, including the brain, and significantly reduces or prevents lysosomal storage during treatment. The reduced lysosomal storage correlates with significant improvements in bone development, growth, cognitive ability, hearing, immune function, and survival.

Efficacy data from randomized controlled Phase 3 studies conducted by third parties for other enzyme deficiencies treated with other enzyme replacement therapies have been published for other related lysosomal diseases, including MPS 1, MPS 6, and MPS 2. All three of these enzyme replacement therapies reduced lysosomal storage in Phase 3 clinical studies resulting in reduction in liver size to normal or near normal, as well as significant reduction in spleen size. Additionally, the three enzyme replacement therapies showed improvement in pulmonary function, walking ability, joint range of motion in patients with more severe joint mobility restriction, and sleep apnea for those patients with abnormal airway function during sleep at baseline. Urinary GAG excretion also decreased significantly in these studies. All three products have been approved in the United States and the European Union as well as other territories worldwide based on safety and efficacy presented in these Phase 3 programs. Based on these results, we believe there is a strong rationale for our approach to treat MPS 7 with rhGUS.

Preclinical results

We have conducted preclinical studies to support the chronic intravenous, or IV, administration of rhGUS. Administration of rhGUS resulted in substantial distribution of enzyme, as well as reduction in tissue pathology in a wide variety of tissues, including the liver, spleen, lung, heart, kidney, muscle, bone, and brain. No adverse toxicology was noted in these studies. The following graph shows dose-dependent urinary GAG reduction in mice with the administration of rhGUS at eight weeks.



For the toxicology program, rhGUS was administered in two species, with no infusion-associated reactions or clinical problems noted.

Clinical development

We plan to initiate an open-label, Phase 1/2 study to evaluate the safety, tolerability, efficacy, and dose of IV administration every other week of rhGUS in five patients with MPS 7 who are between five and 30 years of age. The initial 12-week treatment period will be followed by a dose-titration period and a long-term extension study. The United Kingdom Medicines and Healthcare products Regulatory Agency has accepted our request for clinical study authorization and we expect to commence this study by the end of 2013. If results from the initial 12-week treatment period from this study are supportive, we plan to initiate a pivotal Phase 3 study enrolling approximately 12 patients.

With respect to our rhGUS program, although we have not yet filed an investigational new drug, or IND, application, we have held meetings regarding the pathway for potential approval of the program for MPS 7 with the United States Food and Drug Administration, or the FDA, and the European Medicines Agency, or EMA. The EMA agreed that approval under exceptional circumstances could be possible for a proposed 12-patient placebo-controlled pivotal study in this disease with urinary GAG levels as a surrogate primary endpoint provided the data was strongly supportive of a favorable benefit/risk ratio. The EMA requested that some evidence or trend in improvement in clinical endpoints be observed to support the primary endpoint, but recognized that a statistically significant result on clinical endpoints was unlikely given the small number of patients expected to be enrolled in the study. The FDA would like to see additional data correlating GAG levels with other clinical endpoints, which we plan to collect both through our Phase 1/2 study as well as through an ongoing retrospective study correlating GAG levels with clinical endpoints in other MPS diseases.

In addition to the above development plan, we intend to study MPS 7 patients under the age of five years, including younger infants born with hydrops fetalis. Currently, these infants often die within a few months to one

year, but enzyme replacement therapy might be able to reduce GAG storage and improve health and survival in these patients. We are also supplying rhGUS to an investigator who is treating a single U.S. patient under an emergency IND.

Potential market opportunity

Through our ongoing survey work with metabolic clinics, we have identified approximately 90 potential MPS 7 patients worldwide to date, including approximately 15 in the United States. Based on our experiences with other MPS diseases, we expect that, over time, more patients will be identified during patient identification efforts globally, potentially resulting in up to approximately 200 patients worldwide. Based on published literature, we also estimate that approximately 20 patients per year worldwide are born with non-immune hydrops fetalis due to MPS 7. The establishment of efficacy and safety with our therapy may help drive ascertainment of MPS 7 patients.

rhPPCA

We licensed the rights to recombinant human protective protein cathepsin-A, or rhPPCA, from St. Jude Children's Research Hospital in September 2012. rhPPCA is in preclinical development as an enzyme replacement therapy for galactosialidosis, a rare lysosomal storage disease for which there are no currently approved drug therapies. As with MPS 7, an enzyme deficiency results in accumulation of substrates in the lysosomes, causing skeletal and organ dysfunction, and death. In galactosialidosis, the missing protease, cathepsin A, is a lysosomal stabilizing agent for the two other enzymes: sialidase and beta-galactosidase. Cathepsin A is therefore also called "protective protein," or PPCA. PPCA enzyme deficiency leads to an accumulation of oligosaccharides, or short sugar chains. Enzyme replacement therapy *in vitro* and in the mouse model of this condition has shown that the replacement of PPCA results in reduction in storage of oligosaccharides in multiple organs, just as has been observed in other enzyme replacement therapies. In the 2014 to 2015 timeframe, we intend to pursue process development and manufacture development-scale quantities of rhPPCA in order to conduct proof-of-concept experiments in preclinical animal models of galactosialidosis. Depending on the results of our proof-of-concept preclinical studies, we may begin clinical studies in 2015 or 2016.

Small-molecule product candidates

Triheptanoin for the treatment of LC-FAOD

We are developing triheptanoin for oral liquid administration intended as a substrate replacement therapy for patients with long-chain fatty acid oxidation disorders, or LC-FAOD. Patients with LC-FAOD have a deficiency that impairs both fatty acid metabolism and the Krebs cycle, which is a series of chemical reactions inside the body's cells that generate energy from fat. Triheptanoin is a medium-chain triglyceride of three seven-carbon fatty acids designed to provide substrate replacement for both fatty acid metabolism and the Krebs cycle and restore production of energy. We have exclusively in-licensed global rights to triheptanoin from Baylor Research Institute. Triheptanoin is in an ongoing investigator-sponsored Phase 2 study, and we plan to initiate a Phase 2 study in LC-FAOD patients in 2013.

LC-FAOD background

Without sufficient fatty acid oxidation and Krebs cycle function, LC-FAOD patients cannot rely on fatty acids for energy as would normally occur, making such patients more reliant on glucose metabolism and more susceptible to severe energy crises during periods of fasting and illness. The inability to produce energy from fat can lead to depletion of all glucose in the body, and severe liver, muscle, and heart disease. These symptoms can lead to serious hospitalizations or early death. Due to altered energy balance in skeletal muscle, many patients experience low muscle tone, weakness, exercise intolerance, muscle pain and fatigue, low-grade chronic rhabdomyolysis (muscle rupture), and severe acute episodes of rhabdomyolysis requiring hospitalization. As LC-FAOD is a known cause of sudden infant death syndrome, patients born with all types of LC-FAOD in the

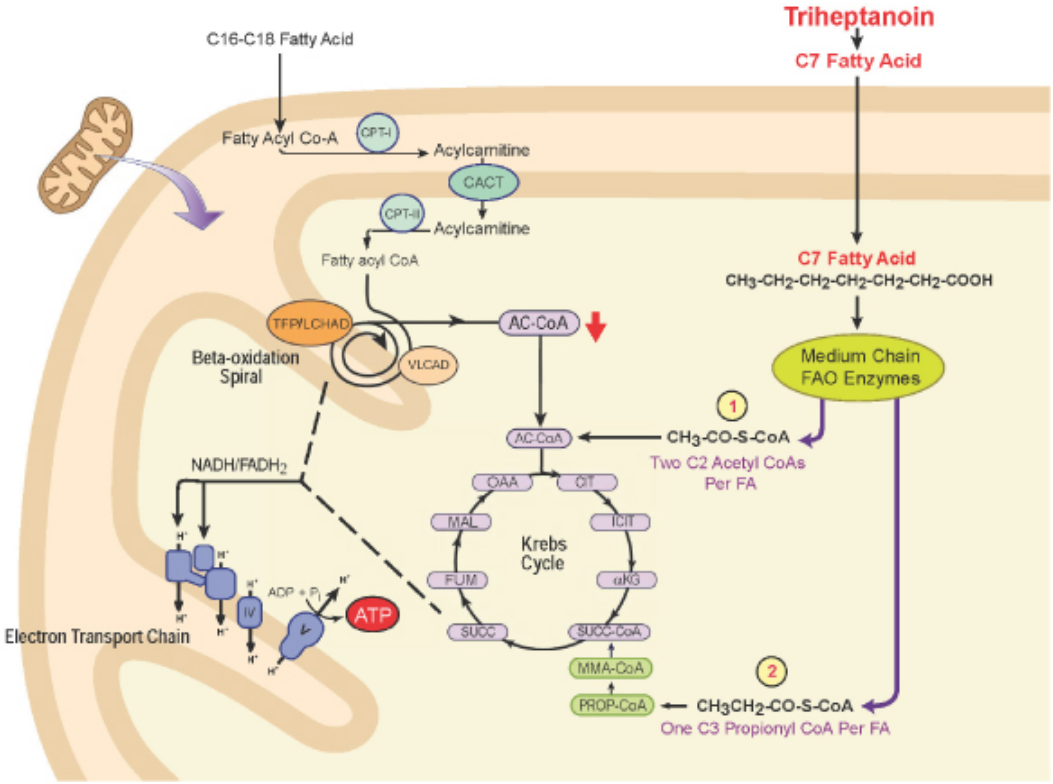
United States are now diagnosed via newborn screening in all 50 states. Outside of the United States, patients are increasingly diagnosed by newborn screening, though can also be diagnosed as a result of symptomatic presentations such as serious liver, muscle, and heart disease.

Patients with LC-FAOD have defects in genes that code for multiple enzymes involved in converting long chains of fat into energy. LC-FAOD patients are also thought to become deficient in the intermediate compounds that are required for the normal metabolic function of the Krebs cycle. This deficiency may be caused by damage to the mitochondria due to the accumulation of long-chain fatty acids, or due to the consumption of the intermediates in the production of glucose to overcome low blood glucose levels.

There are currently no approved drugs or treatments specifically for LC-FAOD. The current standard of care for LC-FAOD includes diligent prevention of fasting combined with the use of low-fat/high-carbohydrate diets, carnitine supplementation in some cases, and medium-chain triglyceride, or MCT, oil supplementation. MCT oil has medium even-chain fatty acids that can be metabolized by medium-chain fatty acid oxidation enzymes and can bypass long-chain fatty acid oxidation enzyme blocks, but does not provide odd-chain fatty acids that can refill the Krebs cycle or be converted to new glucose. In many patients, the current standard of care, including MCT oil, does not prevent all hypoglycemic events, exercise intolerance, muscle weakness, rhabdomyolysis, and cardiomyopathy, as well as associated mortality. For example, a mortality rate of more than 50% has been observed in spite of treatment with standard of care. Major metabolic decompensations also persist despite newborn screening.

Triheptanoin background and clinical development

Triheptanoin consists of medium-length odd-chain fatty acids that should bypass blocks in the long-chain fatty acid oxidation pathway and also restore function of the Krebs cycle. Triheptanoin is converted into heptanoate and ketone bodies; once these metabolites enter the mitochondria, they are metabolized by the medium-chain oxidation enzymes, bypassing the defective long-chain enzymes. These metabolites are converted into two-carbon units (pathway 1 in the figure below) and three-carbon units (pathway 2 in the figure below) required to fuel and operate the Krebs cycle. With these two substrates entering the Krebs cycle, the energy production process can continue and generate the adenosine triphosphate, or ATP, used to support cellular metabolism.



Triheptanoin has been studied by academic researchers for over a decade in a large cohort of human FAOD subjects. Multiple investigator-sponsored open-label studies suggest clinical improvements with triheptanoin treatment, even for patients who were on standard of care. In 2006, a cumulative summary was reported for 48 FAOD subjects who were initially on standard of care but were then treated with triheptanoin, demonstrating a large decrease in liver enlargement and low blood sugar in nearly all subjects, a substantial decrease in frequency of muscle rupture, and a decrease in cardiac disease. Below is a chart summarizing the data reported in the 2006 study.

	# Symptomatic Patients	
Symptoms	**Before triheptanoin**	**After triheptanoin**
Cardiac	10	1
Muscle rupture	36	15
Weakness/fatigue	44	10
Low blood sugar	24	1
Liver enlargement	26	2
Retinopathy	3	3

Triheptanoin has been studied clinically for 13 years in approximately 150 human subjects affected by a variety of diseases, including 65 patients with LC-FAOD. Over this 13-year period, six deaths have been reported among the LC-FAOD patients, but none of these were considered by investigators to be related to triheptanoin. Triheptanoin has been generally well tolerated when administered to subjects with all subtypes of LC-FAOD. Some patients have had non-serious treatment-emergent adverse events involving the gastrointestinal, or GI, system, such as cramping, diarrhea, and loose stools. These GI side effects can be managed by slowly increasing the dose when initiating therapy and by mixing triheptanoin with food or drink, but some patients have discontinued therapy due to the GI upset. In addition, excess weight gain has been reported and can be prevented with careful monitoring of total caloric intake.

The serious adverse events reported for the 65 subjects generally involved events consistent with the underlying LC-FAOD disease, including muscle weakness or pain, myoglobinuria, or muscle protein in the urine, muscle cell rupture, metabolic crisis, cardiomyopathy, hypoglycemia and elevated creatine kinase, or CK, often in association with infections, exercise, or during periods of limited triheptanoin treatment. Other serious adverse events involved events such as infections, fever and vomiting from unspecified cause, respiratory distress/breathing problems, falling oxygen saturation rate, seizure, or medical procedures apparently unrelated to FAOD such as reaction to injection, cleft palate repair, ear tube placement, tonsillectomy/adenoidectomy, cardiac stent placement.

Only three serious adverse events (6%) were classified as possibly related to triheptanoin treatment: muscle cell rupture and elevated CK reported for two subjects, and myoglobinuria in conjunction with exercise, suboptimal triheptanoin dose, and no fluid intake reported for one subject. All three of these serious adverse events were reported during treatment with low dose triheptanoin, and these serious adverse events can be considered typical of the underlying disease.

We have completed a retrospective medical record review study to assess the clinical outcome of triheptanoin treatment on LC-FAOD subjects who have been participating in a compassionate use program at the University of Pittsburgh Medical Center. The data were presented at the 12th International Conference of Inborn Errors of Metabolism in September 2013. The study evaluated the impact of triheptanoin treatment on the rate and extent of hospitalizations in 20 of 24 patients who have been treated with triheptanoin for up to 13 years as

part of compassionate use and who consented to be part of the study. The study involved an intensive medical record collection and review of patients to capture major medical events available, during the period before and after initiation of treatment until the present time. A total of 120 individual charts were evaluated, which covered 241 years of patient data and included a total of 319 hospitalizations. The study compared the major medical event rate before and after initiation of triheptanoin treatment including the total number of hospitalizations and hospital days per year due to all causes, muscle rupture, hypoglycemia, or cardiomyopathy. The total number of events or hospital days for each patient was divided by the total number of years pre-treatment or post-treatment initiation to calculate an annual rate. The preliminary results of our retrospective medical record review are as follows:

Description	Pre-treatment	Post-treatment	% decrease	n	p-value
Mean total hospitalizations/year(1)	1.94	1.26	36%	16	0.1126
Mean total hospital days/year(1),(2)	17.55	5.4	69%	15	0.0242
Mean infant total hospitalizations/year(3)	13.01	1.37	89%	4	0.0892
Mean hypoglycemia events/year(1),(4)	0.92	0.04	96%	9	0.0091
Mean hypoglycemia total hospital days/year(1),(2),(4)	8.42	0.18	98%	9	0.0257
Mean rhabdomyolysis events/year(1),(5)	1.05	0.68	35%	11	0.4604
Mean rhabdomyolysis total hospital days/year(1),(5)	5.94	2.16	64%	9	0.1224
Mean peak creatine kinase (units) for rhabdomyolysis events(1),(5)	85,855	25,797	68%	7	0.1279

(1) Excludes data for four infants dosed within first six months of life.

(2) Excludes hospitalizations with unknown discharge dates.

(3) Four infants were dosed within the first six months of life.

(4) Includes only those patients with hypoglycemia events prior to treatment.

(5) Includes only those patients with rhabdomyolysis events prior to treatment.

We have conducted a pre-investigational new drug, or pre-IND, meeting with the FDA and agreed on the immediate non-clinical and clinical development plan. Accordingly, we can proceed with our clinical program in patients aged six years and older. An IND is in effect, which allows us to commence human clinical studies, and we have initiated a non-rodent toxicology study to be able to study triheptanoin in patients between six months and six years of age.

By the end of 2013, we plan to initiate a prospective open-label Phase 2 study of triheptanoin treatment in approximately 20 to 30 severely affected LC-FAOD subjects exhibiting significant clinical symptoms despite current therapy. Subjects will continue current therapy for four weeks to establish their baseline condition and then begin treatment with triheptanoin. We anticipate that dosing will be gradually increased to an effective dose, expected to be 25-35% of total caloric intake, while ensuring tolerability. The subjects will be followed over 24 weeks, then may continue treatment for an additional 54 weeks. The effects of treatment on clinical and physiologic disease will be assessed in three areas: skeletal myopathy, liver disease, and cardiac disease. A principal goal of the study is to determine the appropriate clinical endpoints and patient populations for testing in potential later-stage pivotal studies.

In addition to advancing our own development program toward potential approval, we are supporting multiple compassionate use and independent investigator-sponsored clinical studies in FAOD and other indications.

Potential market opportunity

Based upon data from the National Newborn Screening Information System, we estimate that there are approximately 2,000 to 3,500 LC-FAOD patients in the United States, depending on the assumed mortality rate. It is unclear how many of these patients are currently diagnosed because the availability of newborn screening in all 50 states in the United States is a relatively new development. Furthermore, until further clinical development of triheptanoin is conducted, it is not clear which subsets of diagnosed patients would be considered by clinicians to be good candidates for triheptanoin treatment. Outside of the United States, where newborn screening is not consistently done, figures regarding the prevalence of LC-FAOD are more uncertain. To further understand the patient population, we have begun a survey of individual metabolic clinics, and to date we have individually identified over 1,300 LC-FAOD patients worldwide, including over 600 in the United States.

Triheptanoin for the treatment of Glut1 DS

We are also developing triheptanoin for patients with glucose transporter type-1 deficiency syndrome, or Glut1 DS. Glut1 DS is caused by a mutation affecting the gene that codes for Glut1, which is a protein that transports glucose from blood into the brain. Because glucose is the primary source of energy for the brain, Glut1 DS results in a chronic state of energy deficiency in the brain. Triheptanoin in this indication is intended as a substrate replacement therapy to provide an alternative source of energy to the brain in Glut1 DS patients. We plan to initiate an adaptive 52-week Phase 2 study in Glut1 DS patients by early 2014, in which the study enrollment may be adapted based on an interim analysis of efficacy and seizure reduction data. If the magnitude of benefit is substantial, the study enrollment may increase, whereas if it is not, then the target enrollment will remain the same.

Glut1 DS background

Glut1 DS is characterized by seizures, developmental delay, and movement disorder in approximately 90%, 75%, and 90% of patients, respectively. The seizures experienced by patients with Glut1 DS can be of many types, and more than one type of seizure can occur in the same patient. In infants and young children, the phenotype is dominated by the seizure disorder and developmental delay. The majority of children with Glut1 DS experience problems with language, both in speaking and in understanding what is said to them. In older patients and in some less severe patients, motor dysfunction has been the dominant symptom.

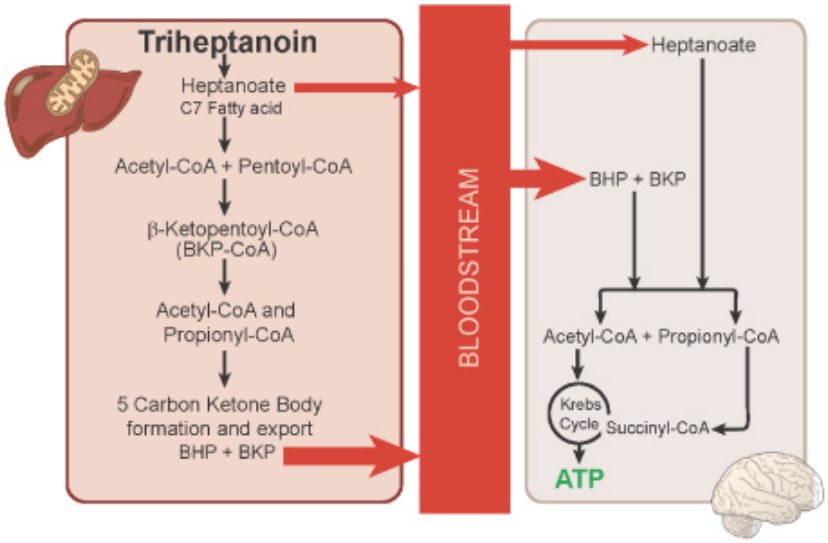
Patients are suspected of having Glut1 DS based on clinical grounds and a low cerebrospinal fluid, or CSF, glucose level. The diagnosis can be confirmed using a red cell glucose uptake test, but this test is not universally available. Currently, genetic testing is readily available and constitutes the main diagnostic test in suspected cases.

There are currently no approved drugs specific to Glut1 DS. Patients may be treated with antiepileptic drugs, or AEDs, for seizure control, although the seizures of Glut1 DS are generally considered resistant to existing AEDs. The current standard of care for Glut1 DS is the ketogenic diet, an extreme high-fat (70-80% of daily calories as fat)/low-carbohydrate diet, which generates ketone bodies as an alternative energy source to glucose. The ketogenic diet is effective in controlling or reducing the seizures of Glut1 DS in most cases. However, seizures are not controlled completely in all patients on the ketogenic diet, and many patients have difficulties fully complying with the diet. Some patients continue to have significant problems with developmental delay and motor dysfunction despite improvement in seizures. We believe that a treatment that does not require an extreme diet therapy could help reduce the burden of Glut1 DS and potentially improve the developmental and other outcomes, without the adverse effects of the diet.

Triheptanoin background and clinical development

The rationale for using triheptanoin as a therapeutic treatment for Glut1 DS is that triheptanoin is metabolized to heptanoate, which in turn is further metabolized to four- and five-carbon ketone bodies. These

metabolites bypass the Glut1 transporter to cross the blood-brain-barrier and provide an alternative energy source to the brain. Heptanoate also crosses the blood-brain-barrier and can be converted to glucose. As in LC-FAOD, some of these metabolites have the ability to restore proper functioning of the Krebs cycle within the brain. The following illustrates the mechanism of action of triheptanoin in the treatment of Glut1 DS.



There are a number of third-party publications on triheptanoin that provide data on its efficacy in epilepsy models and its absorption and metabolism when administered intravenously and orally at doses up to 40% of recommended daily caloric intake.

To date, there are no reports of toxicity on triheptanoin when used as an alternate source of energy in the diet. The metabolites of triheptanoin are essential as a source of energy in patients with Glut1 DS. They are therefore not expected to be toxic. Two toxicity studies have been reported in the literature; toxicities were not observed in either study, other than increased liver fat, which may have been related to high fat intake.

Triheptanoin has been studied clinically for 13 years in approximately 150 human subjects affected by a variety of diseases. Of these, 51 subjects were pediatric subjects with some as young as neonates, and 23 of the 51 pediatric subjects received over five years of treatment with triheptanoin. These data support the safety of triheptanoin when administered at approximately 35% of daily caloric intake in pediatric patients. Although an open-label investigator-sponsored clinical study is ongoing and the results have not yet been reported, there are anecdotal reports of benefit in terms of reduced seizures and improved development rate in some Glut1 DS subjects taking triheptanoin.

We are planning to initiate a clinical development program to study the effects of triheptanoin in Glut1 DS. We anticipate that the program will initially consist of a Phase 2 adaptive, randomized, double-blind, placebo-controlled, parallel-group study of approximately 50 pediatric subjects who are currently not on ketogenic diet. Enrolled subjects will be allowed to maintain standard of care treatment with up to three AEDs and will record their seizure frequency during the baseline period. At the end of the baseline period, eligible subjects will be randomized to either placebo or triheptanoin and will gradually increase their dose to approximately 35% of total daily calories. Subjects will then maintain a stable dose of study drug for six weeks. When 30 subjects have completed the treatment period, an independent data monitoring committee will review the efficacy and seizure reduction data to consider modifying the target patient enrollment. After the initial double-blind treatment period, the open-label extension period will begin, wherein all subjects will be treated with triheptanoin through week 48 of the study, and the 30 initial subjects will enter a dose-exploration period. The study will evaluate the impact of treatment with triheptanoin on seizure frequency, cognitive and other developmental delay, and movement disorder/motor abnormalities, as well as safety and tolerability. We expect to initiate this study by early 2014.

Potential market opportunity

While a comprehensive genetic analysis of birth incidence has not been conducted, published literature suggests a range of 3,000 to 7,000 Glut1 DS patients in the United States based on evaluations of generalized or absence seizures. The increasing recognition of alternative or variable motor forms of the disease suggests that older patients may be discovered over time. Given that the disease can be inherited as an autosomal dominant disease, the discovery of one patient may be used to identify other affected relatives in some cases, which can be important in marketing of the product.

Our clinic survey in Glut1 DS has only recently begun. To date, we have identified over 200 individual patients worldwide, including more than 80 in the United States, and we continue to contact clinics and expand the survey.

SA-ER for the treatment of HIBM

We are developing an extended-release, oral formulation of sialic acid, or SA-ER, for the treatment of HIBM, which is also known as GNE myopathy. Sialic acid is an essential, naturally occurring amino sugar found in humans and most organisms. SA-ER is intended as a substrate replacement therapy designed to address sialic acid deficiency and restore muscle function in HIBM patients. We are the licensee or owner of patents and patent applications relating to sialic acid and its use for HIBM. SA-ER is in Phase 2 clinical studies with top-line results expected by the end of 2013. We expect to continue to evaluate SA-ER in the extension portion of the ongoing Phase 2 study during 2014 and expect data from the extension portion of the study will be available in late 2014.

HIBM background

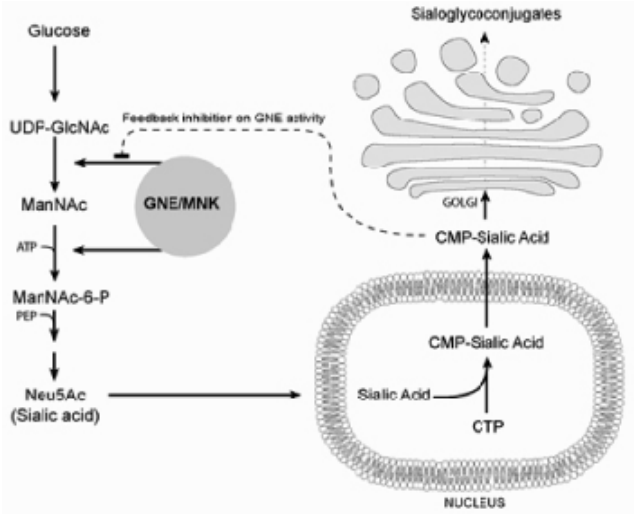
HIBM can be genetically confirmed and is characterized by severe progressive muscular myopathy, or disease in which muscle fibers do not function properly, with onset in the late teens or twenties. In HIBM patients, distal motor function in the legs is first affected by muscle weakness and atrophy but nearly all muscles become progressively weaker, leading to patients becoming wheelchair bound within ten to twenty years from onset. Although all muscles in HIBM patients are generally affected to some degree, the quadriceps and certain facial and diaphragm muscles may be relatively spared from severe disease. Diagnosis of HIBM typically takes place via assessment of clinical presentation and muscle biopsy.

Patients with HIBM have a genetic defect in the gene coding for a particular enzyme. The enzyme is involved in the first step in the biosynthesis of sialic acid, which is required for the glycosylation of proteins and lipids. Therefore, patients with these mutations typically have a sialic acid deficiency. Sialic acid is needed for many proteins and lipids in the body for normal function. In patients with HIBM, a partial deficiency of this enzyme leads to a sialic acid deficiency in the muscle, which interferes with muscle function, leading to myopathy and atrophy.

There is no approved drug therapy for HIBM. Patients typically become wheelchair-bound within ten to 20 years from onset.

SA-ER background and clinical development

SA-ER is designed to replace deficient sialic acid in HIBM patients. The underlying pathophysiology of HIBM has been subject to some debate, whereby it has been hypothesized that the deficient enzyme may have functions other than sialic acid biosynthesis. However, no alternative functions have been definitively established, and subsequent studies have shown that sialic acid substrate replacement in mouse models has profound beneficial effects on the phenotype with little residual clinical disease or pathology remaining after treatment. Therefore, we believe that sialic acid biosynthesis is a key function of the deficient enzyme, and we do not anticipate that theories regarding other functions of the deficient enzyme will impact our SA-ER development program. The following graphic illustrates the biosynthetic pathway of sialic acid in the body.



We have conducted a Phase 1 single-dose and repeat-dose safety and pharmacokinetics study of SA-ER in 26 HIBM patients at doses up to six grams per day. No serious adverse events were reported in the study, and adverse events were considered mild to moderate. SA-ER was absorbed and provided steady and significant drug levels over a period of 8–16 hours, depending on the dose level, regardless of inter-patient variability in the degree of absorption. At the six-gram per day repeat dose, taking SA-ER with food seemed to extend the time period in terms of elevated drug levels. At higher dose levels, mean sialic acid concentrations reached levels that were two to three times normal sialic acid levels.

We are currently conducting a Phase 2 randomized, double-blind, placebo-controlled study testing SA-ER for safety, pharmacodynamics and efficacy in 47 HIBM patients. The primary objective of the Phase 2 study is to evaluate safety, dose, and potential pharmacodynamic effect of restoring sialylation of muscle in patients with a confirmed genetic mutation for HIBM. The study is also evaluating clinical measures of muscle strength, mobility, function, self-reported disability, and changes in quality of life. Patients were randomized to receive placebo, 3 grams, or 6 grams per day of SA-ER. At 24 weeks, placebo patients were randomized and crossed over into either of the two dose groups on a blinded basis and followed for an additional 24 weeks.

An interim analysis of the Phase 2 study was performed following 24 weeks of treatment. The data showed modest dose-dependent improvement in muscle strength compared to declines in placebo-treated subjects in some muscle groups, particularly in the upper extremities at the 6-gram dose. These changes were more pronounced in those patients that had greater walking ability at baseline (greater than 200 meters in the six-minute walk test, a predefined subset). The lower extremities showed a trend of improved strength only in the group that walked greater than 200 meters at baseline. In some muscle groups, the p-value was less than 0.05, which is considered statistically significant. In other muscle groups, improvement was observed with a p-value between 0.06 and 0.15, which we consider to be trended toward statistical significance. The p-value is the probability that the reported result was achieved purely by chance (e.g., a p-value < 0.05 means that there is a less than 5% chance that improved strength

was purely due to chance). In some muscle groups no improvement was observed. Creatine kinase levels showed a trend to improvement (a decline) in the 6-gram dose group compared with a rise in placebo. Other clinical endpoints did not reveal changes at this interim assessment. SA-ER appeared to be well tolerated with no serious adverse events observed to date in either dose group. Mild GI discomfort was observed in the treated patients, but was not dose-dependent. Patients will be evaluated again at 48 weeks, with that top-line data anticipated by the end of 2013. A detailed presentation or publication of the 24- and 48-week data is expected in 2014. Following the 48-week analysis, we plan to continue to treat these patients in an extension study with an increased dosage of sialic acid based on the dose dependence observed at week 24. We anticipate that data from the extension study should be available in late 2014. We are also pursuing development of preclinical prodrugs of sialic acid, which may have better penetration into muscle tissue.

We have also initiated a disease monitoring program that is intended to improve the body of knowledge about HIBM and its typical course. This program is being conducted in partnership with the University of Newcastle's TREAT-NMD organization, a global neuromuscular physician network in Newcastle, England. The program is designed to integrate an online registry capturing patient-reported information, a fully monitored physician-driven natural history study, and potentially any post-approval patient follow-up into a single cohesive program.

Potential market opportunity

Approximately 400 HIBM cases have been reported in the published literature. HIBM is expected to occur in one in every 1,600 persons of Persian Jewish descent. Patients have also been identified in Asian Indian, European, Chinese, Japanese, Korean, and Middle Eastern populations. To better understand the patient population, we conducted an initial survey of 420 myopathy clinics in the United States, and the extrapolated results suggest a patient population of 300 to 400 in the United States and 1,200 to 2,000 worldwide. Our recent patient identification efforts have resulted in over 300 HIBM patients in the United States to date, and over 800 worldwide.

Competition

The commercialization of new drugs is competitive, and we may face worldwide competition from individual investigators, major pharmaceutical companies, specialty pharmaceutical companies, biotechnology companies, nutraceutical companies, and ultimately biosimilar and generic companies. Our competitors may develop or market therapies that are more effective, safer, or less costly than any that may be commercialized by us, or may obtain regulatory approval for their therapies more rapidly than we may obtain approval for ours.

The acquisition or licensing of pharmaceutical products is also very competitive, and a number of more established companies, which have acknowledged strategies to license or acquire products, may have competitive advantages as may other emerging companies taking similar or different approaches to product acquisitions. These established companies may have a competitive advantage over us due to their size, cash flows, and institutional experience.

With respect to KRN23, although we are not aware of any other products currently in clinical development for the treatment of XLH, it is possible that competitors may produce, develop, and commercialize therapeutics, or utilize other approaches such as gene therapy, to treat XLH. Most pediatric patients with XLH are managed using oral phosphate replacement and vitamin D therapy, which is relatively inexpensive and therefore may adversely affect our ability to commercialize KRN23, if approved, in some countries.

With respect to rhGUS and rhPPCA, we are not aware of any other compounds currently in clinical development for MPS 7 or galactosialidosis, but it is possible that other companies may produce, develop, and commercialize compounds that might treat these diseases. Additionally, gene therapy and other therapeutic approaches may emerge for the treatment of lysosomal diseases. Bone marrow or stem cell transplants have also been used in MPS 7 and in other lysosomal storage diseases and represent a potential competing therapy. Stem cell transplants have been effective in treating soft tissue storage and in having an impact on brain disease, but have not to date proven effective in treating bone and connective tissue disease. Enzyme replacement therapy can have an impact on bone and connective tissue disease if patients are treated early.

With respect to triheptanoin, there are currently no approved drugs or treatments for patients with LC-FAOD or Glut1 DS. LC-FAOD is commonly treated with diet therapy and medium chain triglycerides, and triheptanoin would compete with MCT. Glut1 DS is commonly treated with ketogenic diet and anti-epileptic drugs. Triheptanoin may compete with these approaches, though it may also be used in combination. Although we believe that triheptanoin should be considered a drug and will be regulated that way, it is possible that other companies or individuals may attempt to produce triheptanoin for use by LC-FAOD, Glut1 DS, and other patients by attempting to sell the product via a nutraceutical or food pathway. It is also possible that other companies may produce, develop, and commercialize other medium-chain odd-chain fatty acids, or completely different compounds, to treat LC-FAOD and Glut1 DS. For example, B. Braun Medical Inc., or B. Braun, has applied for and received orphan drug designation for triheptanoin in Europe; we are not, however, aware of any ongoing development activities by B. Braun. Other companies may also utilize other approaches, such as gene therapy, to treat LC-FAOD and Glut1 DS.

With respect to SA-ER, although there are currently no approved drug therapies for the treatment of HIBM, it is possible that others may develop alternative approaches to the treatment of HIBM, including other metabolites from the sialic acid pathway, prodrugs, other drug therapies, and gene therapy. We are aware of a program at the National Institutes of Health that is investigating the use of another metabolite in the sialic acid pathway, N-acetyl mannosamine, or ManNAc, for the treatment of HIBM. This program is licensed to New Zealand Pharma, which manufactures ManNAc. The program recently completed a Phase 1 clinical study, and we anticipate that it will advance into Phase 2 testing.

Many of our competitors have substantially greater financial, technical, and human resources than we have. Additional mergers and acquisitions in the pharmaceutical industry may result in even more resources being concentrated in our competitors. Competition may increase further as a result of advances made in the commercial applicability of technologies and greater availability of capital for investment in these fields. Our success will be based in part on our ability to build and actively manage a portfolio of drugs that addresses unmet medical needs and creates value in patient therapy.

License Agreements

Kyowa Hakko Kirin

In August 2013, we entered into a collaboration and license agreement with Kyowa Hakko Kirin Co., Ltd., or KHK, pursuant to which we and KHK will collaborate on the development and commercialization of certain products containing KRN23, an antibody directed towards FGF23, in the field of orphan diseases in the United States and Canada, or the profit share territory, and in the European Union, Switzerland, and Turkey, or the European territory, and we will have the right to develop and commercialize such products in the field of orphan diseases in Mexico and Central and South America, or Latin America. Under the agreement, we also have a right of first negotiation with KHK to receive a license to develop and commercialize products in any non-diagnostic field, or in the field of orphan drugs outside of the profit share territory, the European territory, and Latin America.

In the field of orphan diseases, and except for ongoing studies being conducted by KHK, we will be the lead party for development activities in the profit share territory and in the European territory until, with respect to the profit share territory, the fifth anniversary of the first commercial sale in the United States in the first indication and, with respect to the European territory, the date on which marketing approval for a licensed product for the first indication is obtained in the European territory on a country-by-country basis; each such date is referred to herein as the applicable transition date. We will share the costs for development activities in the profit share territory and European territory conducted pursuant to the development plan before the applicable transition date equally with KHK. On the applicable transition date in the relevant territory, KHK will become the lead party and be responsible for these costs. However, we will continue to share the costs of the studies commenced prior to the applicable transition date equally with KHK. While we are the lead development party in the profit share territory, we must use commercially reasonable efforts to conduct development activities in at least one orphan disease indication other than XLH, as mutually agreed upon by KHK and us.

In the profit share territory, KHK will book sales of products and we will have the sole right to promote the products for a specified period of time, with KHK increasingly participating in the promotion of the products until five years from commercial launch, after which KHK will have the sole right to promote the products, subject to a limited promotion right retained by us. In the European territory, KHK will book sales of products and have the sole right to promote and sell the products. In Latin America, we will book sales of products and have the sole right to promote and sell the products.

The profit or loss from commercializing products in the profit share territory until the applicable transition date will be shared between us and KHK on a 50/50 basis. Thereafter, we will be entitled to receive a tiered double-digit revenue share in the mid to high twenty percent range in the profit share territory, intended to approximate the profit share. We will also be entitled to receive a royalty of up to 10% on net sales in the European territory. In Latin America, we will pay to KHK a low single-digit royalty on net sales. Our and KHK's obligations to pay royalties will continue on a country-by-country basis for so long as we or KHK, as applicable, are selling products in such country.

KHK will supply all quantities of product for clinical studies. KHK will also supply all quantities of product for commercial sales in the profit share territory and in Latin America. The supply price to us for commercial sales in the profit share territory and in Latin America will be determined based on a fixed percentage of net sales.

The collaboration and license agreement will continue for as long as products in the field of orphan diseases are sold in the profit share territory, European territory, or Latin America, unless the agreement is terminated in accordance with its terms.

KHK may terminate the entire agreement if we do not timely initiate a first pediatric study in XLH. In addition, KHK may terminate the agreement in certain countries or territories based upon our failure to meet certain milestones. Specifically, if we do not obtain U.S. or European marketing approval of KRN23 for the treatment of XLH by a certain date, or make a first commercial sale, on a country-by-country basis, in Latin America by certain deadlines, KHK may terminate the agreement only with respect to the applicable territory or country in which the milestone was not timely met. In certain circumstances, we have the right to obtain an extension of the applicable deadline by making a payment to KHK in the low single-digit to low double-digit millions of dollars, depending on the milestone. Also, in the event of the occurrence of certain excusable delays, the deadline for meeting the applicable milestone above is extended to account for the period of the delay. Furthermore, either party may terminate the agreement for the material breach or bankruptcy of the other party. In any event of termination by KHK, unless such termination is the result of KHK's termination for certain types of breach of the agreement by us, we may receive low single-digit to low double-digit royalties on net post-termination sales by KHK in one or more countries or territories, the amount of which varies depending on the timing of, and reason for, such termination. In any event of termination, our rights to KRN23 under the agreement and our obligations to share development costs will cease, and the program will revert to KHK, worldwide if the agreement is terminated as a whole or solely in the terminated countries if the agreement is terminated solely with respect to certain countries.

Saint Louis University

In November 2010, we entered into a license agreement with Saint Louis University, or SLU, wherein SLU granted us certain exclusive rights to intellectual property related to GUS. Under the terms of the license agreement, SLU granted us an exclusive worldwide license to make, have made, use, import, offer for sale, and sell therapeutics related to SLU's beta-glucuronidase product, such as our rhGUS product candidate, for use in the treatment of human diseases. Under this agreement, we agreed to use best efforts to develop and commercialize a licensed product as soon as practicable consistent with sound and reasonable business practices and judgment.

Under the license agreement, we paid SLU an up-front fee of $10,000, which was recorded as a research and development expense. We will make a milestone payment of $100,000 upon approval of a glucuronidase-based enzyme therapy for treatment of MPS 7. Additionally, upon reaching a certain level of cumulative worldwide sales of the product, we will pay to SLU a low single-digit royalty on net sales of the licensed products in any country or region, subject to certain potential deductions. Our obligation to pay royalties to SLU continues on a country-by-country basis until the expiration of the last-to-expire licensed patent covering the product in such

country or, in the United States, Japan, and the European Union, until the later expiration of any orphan drug exclusivity. We may deduct a portion of the royalty owed if a third-party license is required. We may terminate the agreement for convenience at any time and SLU may terminate the agreement for our material breach, bankruptcy, or challenge of the licensed patents or technology, and SLU may terminate the agreement or render our license non-exclusive if we fail to meet our diligence obligations. Unless terminated as set forth above, this license agreement continues in full force and effect until the latest of expiration of the last patent based on technology licensed under the agreement, at which point our license becomes fully paid.

St. Jude Children's Research Hospital

In September 2012, we entered into a license agreement with St. Jude Children's Research Hospital, or St. Jude, wherein St. Jude granted us certain exclusive rights to intellectual property related to rhPPCA. Under the terms of the license agreement, St. Jude granted us an exclusive license under certain know-how to research, develop, make, use, offer to sell, import, and otherwise commercialize and exploit certain PPCA protein products to treat, prevent, and/or diagnose galactosialidosis and other monogenetic diseases. We agreed to make commercially reasonable efforts to develop and commercialize at least one licensed product.

Under the license agreement, we paid St. Jude an up-front fee of $10,000, which was recorded as research and development expense. Additionally, we will pay to St. Jude a royalty of less than 1% on net sales of these products for so long as such products retain orphan drug exclusivity, on a country-by-country basis. We also received a right of first negotiation to receive an exclusive license under patents and know-how related to other uses of these products. We may terminate the agreement for convenience at any time and St. Jude may terminate the agreement for our material breach of the agreement. Unless terminated for convenience or material breach, as applicable, this license agreement continues in full force and effect, until our royalty obligations expire, at which point our license becomes irrevocable, perpetual, fully paid, and royalty-free.

Baylor Research Institute

In September 2012, we entered into a license agreement with Baylor Research Institute, or BRI, whereby we exclusively licensed certain intellectual property related to triheptanoin for North America and paid BRI an up-front fee of $250,000. The license includes patents, patent applications, know-how, and intellectual property related to the composition and formulation of triheptanoin as well as its use in treating a number of orphan diseases, including FAOD. The license grant includes the sole right to develop, manufacture, and commercialize licensed products for all human and animal uses. In June 2013, we exercised our option to license this intellectual property outside of North America and paid BRI the $750,000 fee associated with this option exercise. Under the license agreement, we are obligated to use commercially reasonable efforts to develop and commercialize licensed products in select orphan indications. If we fail to meet our diligence obligations with respect to a specified orphan indication or set of orphan indications, BRI may convert our license to a non-exclusive license with respect to such orphan indication or set of orphan indications until we receive regulatory approval for licensed products in the applicable orphan indication or set of orphan indications. We are also obligated to pay a mid-single digit royalty on net sales to BRI, subject to certain reductions and offsets. Our obligation to pay royalties to BRI continues on a licensed product-by-licensed product and country-by-country basis until the later of the expiration of the first regulatory exclusivity granted with respect to such product in such country or the expiration of the last-to-expire licensed patent claiming such product in such country, in each case in connection with approval in such country for FAOD or an orphan disease covered by our license from BRI. We may make future payments of up to $10.5 million contingent upon attainment of certain development milestones and $7.5 million if certain sales milestones are achieved. We may terminate the agreement for convenience at any time and either we or BRI may terminate the agreement for the material breach or bankruptcy of the other party. If we terminate for BRI's breach or bankruptcy, our license from BRI will remain in effect, subject to our continued payment of reduced milestones and royalties. Unless terminated for convenience or material breach or bankruptcy, as applicable, this license agreement continues in full force and effect, on a product-by-product and country-by-country basis, until our royalty obligations expire, at which point our license from BRI with respect to such product in such country becomes irrevocable, perpetual, fully paid and royalty-free.

Nobelpharma

In September 2010, we entered into a collaboration and license agreement with Nobelpharma Co., Ltd., or Nobelpharma. Under the terms of the collaboration and license agreement, each party granted the other party a worldwide exclusive license under certain of that party's intellectual property related to the compound identified as N-acetylneuraminic acid, also known as sialic acid, to develop, manufacture, and commercialize products. Nobelpharma's licensed territory includes Japan and certain other Asian countries, and our licensed territory includes the rest of the world. The parties conduct development independently, and each party is obligated to make commercially reasonable efforts to file an investigational new drug application for licensed products in its territory and, in our case, to obtain patent term extensions and data exclusivity in Europe and North America, and share with the other party all data, documentation, and information that is generated in conducting such activities. Nobelpharma must use commercially reasonable efforts to supply us with the sialic acid drug substance. Either Nobelpharma or we can terminate this supply arrangement for convenience, at which point Nobelpharma would provide technical assistance to allow us to manufacture the sialic acid drug substance ourselves. If we choose to manufacture the sialic acid drug substance, Nobelpharma will have the right to purchase the sialic acid drug substance from us and we will use commercially reasonable efforts to supply Nobelpharma with the sialic acid drug substance.

Under the collaboration and license agreement, we have paid Nobelpharma approximately $110,000 as an upfront fee and approximately $495,000 in development milestone payments and also issued 240,000 shares of common stock to Nobelpharma. We are required to pay Nobelpharma a high single digit royalty on net sales of products in our territory and Nobelpharma is required to pay us a mid-single digit royalty on net sales in their territory (with the exception of Japan). Each party's obligation to pay royalties is subject to certain offsets and deductions, and is payable on a product-by-product and country-by-country basis until the expiration of the collaboration and license agreement. In addition, as of September 30, 2013, we are obligated to make a future payment to Nobelpharma of ¥200 million (approximately $2.0 million U.S. dollars) based upon achievement of a certain approval milestone. Either party may terminate the agreement for the material breach or bankruptcy of the other party. If either party terminates the agreement, the terminating party's license will become irrevocable and royalty-free. Unless terminated for material breach or bankruptcy, as applicable, this license agreement continues in full force and effect, on a country-by-country basis, until the date of the first launch of a generic product of the licensed product in a country.

AAI Pharma

In March 2011, we entered into a license agreement with AAIPharma Services Corp., or AAI Pharma. Under the terms of this license agreement, AAI Pharma granted us a fully paid-up, royalty-free, exclusive, perpetual, and irrevocable license to research, develop, make, have made, use, import, offer for sale, and sell products incorporating AAI Pharma's controlled release matrix solid dose oral tablet technology for use in connection with sialic acid for the treatment of HIBM or distal myopathy with rimmed vacuoles. Under the license agreement, we will pay a mid-single digit percentage of any sublicense revenue received by us related to the sublicense of AAI Pharma technology. As consideration, we agreed to provide preclinical and clinical data to AAI Pharma. AAI Pharma is responsible for patent prosecution and maintenance, subject to our right to review and comment on such prosecution and maintenance. We may terminate the agreement for convenience at any time and either party may terminate the agreement for the material breach or bankruptcy of the other party.

HIBM Research Group

In April 2012, we entered into an exclusive license agreement with HIBM Research Group, or HRG, wherein HRG granted us an exclusive, worldwide license to certain intellectual property related to the treatment of HIBM and related conditions using substrate replacement therapy.

Under the terms of license agreement, we paid HRG an up-front fee of $25,000, which was recorded as a research and development expense. We will make future payments contingent upon attainment of various development and approval milestones of up to $300,000 in the aggregate. Additionally, we will pay to HRG a

royalty of less than 1% of net sales of products, if any. Our obligation to pay royalties to HRG continues on a product-by-product and country-by-country basis until the expiration of the last-to-expire licensed patent claiming such product in such country, or the later expiration of orphan drug exclusivity in certain countries. We are obligated to make commercially reasonable efforts to develop and commercialize a substrate replacement therapy for HIBM. We may terminate the agreement for convenience at any time and either party may terminate the agreement for the material breach or bankruptcy of the other party. We must also terminate the agreement if we terminate our HIBM substrate replacement therapy program. Unless terminated for convenience, for our termination of our HIBM substrate replacement therapy program, or for material breach or bankruptcy, as applicable, this license agreement continues in full force and effect, on a product-by-product and country-by-country basis, until the expiration date of the last-to-expire licensed patent claiming such product in such country, or the later expiration of orphan drug exclusivity in certain countries, at which point our license becomes irrevocable, perpetual, fully paid, and royalty-free.

Patents and Proprietary Rights

The proprietary nature of, and protection for, our product candidates, processes, and know-how are important to our business. Our success depends in part on our ability to protect the proprietary nature of our product candidates, technology, and know-how, to operate without infringing on the proprietary rights of others, and to prevent others from infringing our proprietary rights. We seek patent protection in the United States and internationally for our product candidates and other technology. Our policy is to patent or in-license the technology, inventions and improvements that we consider important to the development of our business. In addition to patent protection, we intend to use other means to protect our proprietary rights, including pursuing marketing or data exclusivity periods, orphan drug status, and similar rights that are available under regulatory provisions in certain countries, including the United States, Europe, Japan, and China. See “U.S. Government Regulation — Orphan Designation and Exclusivity,” “U.S. Government Regulation — Pediatric Studies and Exclusivity,” “U.S. Government Regulation — Patent Term Restoration,” “U.S. Government Regulation — Biosimilars and Exclusivity,” “U.S. Government Regulation — Abbreviated New Drug Applications for Generic Drugs,” “U.S. Government Regulation — Hatch-Waxman Patent Certification and the 30-Month Stay,” and “European Union/Rest of World Government Regulation — Orphan Designation and Exclusivity” below for additional information.

We also rely on trade secrets, know-how, and continuing innovation to develop and maintain our competitive position. We cannot be certain that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications filed by us in the future, nor can we be sure that any of our existing patents or any patents granted to us in the future will be commercially useful in protecting our technology.

We seek regulatory approval for our products in disease areas with high unmet medical need, great market potential, and where we have a proprietary position through patents covering various aspects of our products, such as composition, dosage, formulation, use, and manufacturing process, among others. Our success depends on an intellectual property portfolio that supports our future revenue streams and erects barriers to our competitors. We are maintaining and building our patent portfolio through filing new patent applications, prosecuting existing applications, and licensing and acquiring new patents and patent applications.

Despite these measures, any of our intellectual property and proprietary rights could be challenged, invalidated, circumvented, infringed or misappropriated, or such intellectual property and proprietary rights may not be sufficient to permit us to take advantage of current market trends or otherwise to provide competitive advantages. For more information, please see “Risks Related to our Intellectual Property.”

As of November 1, 2013, we own 9 pending U.S. patent applications and corresponding patents and patent applications internationally. In addition, as of November 1, 2013, we have licensed 10 issued U.S. patents and 14 pending U.S. patent applications as well as corresponding foreign patents and applications from third parties, on an exclusive basis. With respect to our issued patents in the United States and Europe, we are also entitled to obtain a patent term extension to extend the patent expiration date. For example, in the United States, we can apply for a patent term extension of up to five years for one of the patents covering a product once the product is approved by the FDA. The exact duration of the extension depends on the time we spend in clinical studies as

well as getting a new drug application approval from the FDA. The patent portfolios for our five leading product candidates as of November 1, 2013 are summarized below.

KRN23

We have rights from KHK to patents and patent applications relating to KRN23, a fully human monoclonal antibody against FGF23, and its use for the treatment of XLH and various other hypophosphatemic conditions. Pursuant to this license, we share rights to 20 issued patents, including 3 U.S. patents and 1 pending U.S. application and patents and applications in other jurisdictions covering generic and specific antibodies against FGF23 as well as their use for the treatment of XLH and related conditions. The patent terms for issued patents in the United States are from 2022 to 2029 (without patent term extension). The projected patent term for pending applications in the United States is 2028. We intend to pursue marketing and orphan drug exclusivity periods that are available to us under regulatory provisions in certain countries. KRN23 has received orphan drug designation in the United States.

rhGUS

We have no issued patents covering rhGUS but we are in the process of filing patent applications directed to compositions with certain characteristics that are useful for the enzyme replacement therapy for the treatment of multi-system lysosomal storage disease. Throughout clinical research and development, we also intend to file patent applications directed to various aspects of the treatment therapy including dosage, regiment, formulation, manufacturing, etc. We intend to pursue marketing and orphan drug exclusivity periods that are available under regulatory provisions in certain countries. rhGUS has received orphan drug designation in both the United States and Europe.

rhPPCA

We have no issued patents or patent applications filed for rhPPCA, although it is partially protected by proprietary know-how licensed from St. Jude Children's Hospital. We intend to build a patent portfolio directed to compositions with certain characteristics that are useful for the enzyme replacement therapy for the treatment of autosomal recessive lysosomal storage disease as well as various aspects of the treatment therapy including dosage, regimen, formulation, manufacturing, etc. We intend to pursue marketing and orphan drug exclusivity periods that are available under regulatory provisions in certain countries.

Triheptanoin

We are the licensee or owner of patents and patent applications relating to triheptanoin and its use for a number of diseases including FAOD and Glut1 DS. In particular, we have an exclusive license from Baylor Research Institute, or BRI, with respect to its triheptanoin patent portfolio. We have licensed from BRI 24 issued patents, including 7 U.S. patents and 9 pending U.S. applications and patents and applications in other jurisdictions covering composition, formulation, use and manufacturing of triheptanoin and related odd carbon fatty acids. The patent terms for issued patents in the United States are from 2020 to 2024 (without patent term extension). The projected patent terms for pending applications in the United States are from 2020 to 2034. We intend to pursue marketing and orphan drug exclusivity periods that are available under regulatory provisions in certain countries.

SA-ER

We are the licensee or owner of patents and patent applications relating to sialic acid and its use for the treatment of HIBM. We have 9 pending U.S. applications and patents and applications in other jurisdictions covering the use of sialic acid for the treatment of HIBM, biomarkers useful for such treatment as well as extended release formulations of sialic acid. The projected patent terms for pending applications in the United States are from 2028 to 2033.

We intend to pursue marketing and orphan drug exclusivity periods that are available under regulatory provisions in certain countries. SA-ER has received orphan drug designation in both the United States and the European Union.

Trademarks

We have filed U.S. trademark applications for ULTRAGENYX and ULTRAGENYX PHARMACEUTICAL.

Other

We rely upon unpatented trade secrets, know-how, and continuing technological innovation to develop and maintain our competitive position. We seek to protect our ownership of know-how and trade secrets through an active program of legal mechanisms including assignments, confidentiality agreements, material transfer agreements, research collaborations, and licenses.

Manufacturing

We currently contract with third parties for the manufacturing and testing of our product candidates for preclinical studies and clinical studies and intend to do so in the future. We do not own or operate manufacturing facilities for the production of clinical quantities of our product candidates. We currently have no plans to build our own clinical or commercial scale manufacturing capabilities. The use of contracted manufacturing and reliance on collaboration partners is relatively cost-efficient and has eliminated the need for our direct investment in manufacturing facilities and additional staff early in development. Although we rely on contract manufacturers, we have personnel with extensive manufacturing experience to oversee our contract manufacturers.

To date, our third-party manufacturers have met our manufacturing requirements. We expect third-party manufacturers to be capable of providing sufficient quantities of our product candidates to meet anticipated full scale commercial demands. To meet our projected needs for commercial manufacturing, third parties with whom we currently work might need to increase their scale of production or we will need to secure alternate suppliers. We believe that there are alternate sources of supply that can satisfy our clinical and commercial requirements, although we cannot be certain that identifying and establishing relationships with such sources, if necessary, would not result in significant delay or material additional costs.

KRN23

The drug substance and drug product for KRN23 are made by KHK in Japan under the collaboration and license agreement with KHK. The cell line to produce KRN23 is specific for this product and is in KHK's control. All other raw materials are commercially available.

rhGUS

rhGUS drug substance and drug product are manufactured by Rentschler Biotechnologie GmbH, or Rentschler, under a development and clinical supply agreement executed in August 2012. Pursuant to the supply agreement, we have agreed not to source larger quantities of drug substance or drug product from another supplier than from Rentschler in any given year. The supply agreement will continue in full force and effect until all services have been completed or terminated per the terms of the supply agreement. Either party may terminate the supply agreement if the other party fails to pay any sum payable under the supply agreement within 30 days after a written demand is issued after the original due date, if the other party makes a material misrepresentation or commits a material breach of its obligations under the supply agreement and fails to cure such breach within specified time periods if curable, if the other party ceases to carry on its business for a period no less than 60 days, or if a party experiences certain insolvency events. Additionally, either party may terminate the supply agreement upon 30 days' prior written notice if the Steering Committee concludes that the services required under the supply agreement cannot be performed and we may terminate the agreement at any time before

completion of the services rendered pursuant to the agreement upon 60 days' prior written notice. The cell line to produce rhGUS is specific for this product and is in our control and stored in multiple secure locations. All other raw materials are commercially available.

rhPPCA

No supplier has yet been selected for rhPPCA. The cell line to produce rhPPCA is specific for this product and is in our control and stored in multiple secure locations. The process to produce rhPPCA will only contain commercially available materials.

Triheptanoin

The pharmaceutical-grade drug substance for triheptanoin is manufactured by Cremer Oleo GmbH & Co. KG in Germany under an exclusive worldwide supply agreement, subject to certain limitations, executed in 2012. The supply agreement has an initial term of three years; thereafter, the agreement shall be automatically renewed for additional two-year periods unless either party notifies the other party of its intention not to renew in writing at least three calendar months before the expiration of the then current term. Additionally, if a party materially breaches an obligation under the agreement and does not cure such breach within 60 days of receiving notice of the breach from the non-breaching party, the non-breaching party may terminate the agreement immediately upon written notice to the breaching party. Triheptanoin drug product manufacturing has been done with more than one party and is not considered a very specialized task.

SA-ER

The drug substance for SA-ER is currently manufactured by Sanyo Fine Co., Ltd. in Japan through the license agreement with Nobelpharma. The SA-ER drug product is manufactured by AAI Pharma under our license agreement and accompanying purchase orders with AAI Pharma. We are in the process of identifying secondary sources of drug substance and drug product for SA-ER. Manufacture of the drug substance requires a specialized enzyme-catalyzed step, and a secondary source of the enzyme itself is also under development. All raw materials to produce the drug substance and drug product are commercially available. The cell line to produce the specialized enyme is under our contol and is stored in multiple secured locations.

Sales and Marketing

We currently intend to build the commercial infrastructure in the United States and Europe necessary to effectively support the commercialization of all of our product candidates, if and when we believe a regulatory approval of the first of such product candidates in a particular geographic market appears imminent. The commercial infrastructure for orphan products typically consists of a targeted, specialty sales force that calls on a limited and focused group of physicians supported by sales management, medical liaisons, internal sales support, an internal marketing group, and distribution support. One challenge unique to commercializing therapies for rare diseases is the difficulty in identifying eligible patients due to the very small and sometimes heterogeneous disease populations. Our management team is experienced in maximizing patient identification for both clinical development and commercialization purposes in rare diseases.

Additional capabilities important to the orphan marketplace include the management of key accounts such as managed care organizations, group-purchasing organizations, specialty pharmacies, and government accounts. To develop the appropriate commercial infrastructure, we will have to invest significant amounts of financial and management resources, some of which will be committed prior to any confirmation that any of our product candidates will be approved.

Outside of the United States and Europe, where appropriate, we may elect in the future to utilize strategic partners, distributors, or contract sales forces to assist in the commercialization of our products. In certain instances we may consider building our own commercial infrastructure.

Government Regulation

Government authorities in the United States (including federal, state, and local authorities) and in other countries, extensively regulate, among other things, the manufacturing, research and clinical development, marketing, labeling and packaging, storage, distribution, post-approval monitoring and reporting, advertising and promotion, pricing, and export and import of pharmaceutical products, such as those we are developing. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local, and foreign statutes and regulations require the expenditure of substantial time and financial resources.

U.S. Government Regulation

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, and its implementing regulations, and biologics under the FDCA and the Public Health Service Act, or PHSA, and its implementing regulations. FDA approval is required before any new unapproved drug or dosage form, including a new use of a previously approved drug, can be marketed in the United States. Drugs and biologics are also subject to other federal, state, and local statutes and regulations. If we fail to comply with applicable FDA or other requirements at any time during the drug development process, clinical testing, the approval process or after approval, we may become subject to administrative or judicial sanctions. These sanctions could include the FDA's refusal to approve pending applications, license suspension or revocation, withdrawal of an approval, warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties or criminal prosecution. Any FDA enforcement action could have a material adverse effect on us.

The process required by the FDA before product candidates may be marketed in the United States generally involves the following:

- completion of extensive preclinical laboratory tests and preclinical animal studies, all performed in accordance with the Good Laboratory Practices, or GLP, regulations;
- submission to the FDA of an investigational new drug application, or IND, which must become effective before human clinical studies may begin and must be updated annually;
- approval by an independent institutional review board, or IRB, or ethics committee representing each clinical site before each clinical study may be initiated;
- performance of adequate and well-controlled human clinical studies to establish the safety and efficacy of the product candidate for each proposed indication;
- preparation of and submission to the FDA of a new drug application, or NDA, or biologics license application, or BLA, after completion of all pivotal clinical studies;
- potential review of the product application by an FDA advisory committee, where appropriate and if applicable;
- a determination by the FDA within 60 days of its receipt of an NDA or BLA to file the application for review;
- satisfactory completion of an FDA pre-approval inspection of the manufacturing facilities where the proposed product drug substance is produced to assess compliance with current Good Manufacturing Practices, or cGMP; and
- FDA review and approval of an NDA or BLA prior to any commercial marketing or sale of the drug in the United States.

The preclinical and clinical testing and approval process requires substantial time, effort, and financial resources, and we cannot be certain that any approvals for our product candidates will be granted on a timely basis, if at all.

An IND is a request for authorization from the FDA to administer an investigational new drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol(s) for

human studies. The IND also includes results of animal and *in vitro* studies assessing the toxicology, pharmacokinetics, pharmacology, and pharmacodynamic characteristics of the product; chemistry, manufacturing, and controls information; and any available human data or literature to support the use of the investigational new drug. An IND must become effective before human clinical trials may begin. An IND will automatically become effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to the proposed clinical studies. In such a case, the IND may be placed on clinical hold and the IND sponsor and the FDA must resolve any outstanding concerns or questions before clinical studies can begin. Accordingly, submission of an IND may or may not result in the FDA allowing clinical studies to commence.

Clinical Studies

Clinical studies involve the administration of the investigational new drug to human subjects under the supervision of qualified investigators in accordance with current Good Clinical Practices, or cGCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical study. Clinical studies are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety, and the efficacy criteria to be evaluated. A protocol for each clinical study and any subsequent protocol amendments must be submitted to the FDA as part of the IND. Additionally, approval must also be obtained from each clinical study site's institutional review board, or IRB, before the studies may be initiated, and the IRB must monitor the study until completed. There are also requirements governing the reporting of ongoing clinical studies and clinical study results to public registries.

The clinical investigation of a drug is generally divided into three or four phases. Although the phases are usually conducted sequentially, they may overlap or be combined.

- *Phase 1*. The drug is initially introduced into healthy human subjects or patients with the target disease or condition. These studies are designed to evaluate the safety, dosage tolerance, metabolism and pharmacologic actions of the investigational new drug in humans, the side effects associated with increasing doses, and if possible, to gain early evidence on effectiveness.
- *Phase 2*. The drug is administered to a limited patient population to evaluate dosage tolerance and optimal dosage, identify possible adverse side effects and safety risks, and preliminarily evaluate efficacy.
- *Phase 3*. The drug is administered to an expanded patient population, generally at geographically dispersed clinical study sites to generate enough data to statistically evaluate dosage, clinical effectiveness and safety, to establish the overall benefit-risk relationship of the investigational new drug product, and to provide an adequate basis for product approval.
- *Phase 4*. In some cases, the FDA may condition approval of an NDA or BLA for a product candidate on the sponsor's agreement to conduct additional clinical studies after approval. In other cases, a sponsor may voluntarily conduct additional clinical studies after approval to gain more information about the drug. Such post-approval studies are typically referred to as Phase 4 clinical studies.

A pivotal study is a clinical study that adequately meets regulatory agency requirements for the evaluation of a drug candidate's efficacy and safety such that it can be used to justify the approval of the product. Generally, pivotal studies are Phase 3 studies, but the FDA may accept results from Phase 2 studies if the study design provides a well-controlled and reliable assessment of clinical benefit, particularly in situations where there is an unmet medical need and the results are sufficiently robust.

The FDA, the IRB, or the clinical study sponsor may suspend or terminate a clinical study at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk. Additionally, some clinical studies are overseen by an independent group of qualified experts organized by the clinical study sponsor, known as a data safety monitoring board or committee. This group provides authorization for whether or not a study may move forward at designated check points based on access to certain data from the study. We may also suspend or terminate a clinical study based on evolving business objectives and/or competitive climate.

The clinical study process can take three to ten years or more to complete, and there can be no assurance that the data collected will support FDA approval or licensure of the product.

Submission of an NDA or BLA to the FDA

Assuming successful completion of all required testing in accordance with all applicable regulatory requirements, detailed investigational new drug product information is submitted to the FDA in the form of an NDA or BLA requesting approval to market the product for one or more indications. Under federal law, the submission of most NDAs and BLAs is subject to an application user fee. For fiscal year 2014, the application user fee exceeds $2.1 million, and the sponsor of an approved NDA or BLA is also subject to annual product and establishment user fees, set at $104,060 per product and $554,600 per establishment. These fees are typically increased annually. Applications for orphan drug products are exempted from the NDA and BLA user fees and may be exempted from product and establishment user fees, unless the application includes an indication for other than a rare disease or condition.

An NDA or BLA must include all relevant data available from pertinent preclinical and clinical studies, including negative or ambiguous results as well as positive findings, together with detailed information relating to the product's chemistry, manufacturing, controls, and proposed labeling, among other things. Data can come from company-sponsored clinical studies intended to test the safety and effectiveness of a use of a product, or from a number of alternative sources, including studies initiated by investigators. To support marketing approval, the data submitted must be sufficient in quality and quantity to establish the safety and effectiveness of the investigational new drug product to the satisfaction of the FDA.

Once an NDA or BLA has been submitted, the FDA's goal is to review the application within ten months after it accepts the application for filing, or, if the application relates to an unmet medical need in a serious or life-threatening indication, six months after the FDA accepts the application for filing. The review process is often significantly extended by FDA requests for additional information or clarification.

Before approving an NDA or BLA, the FDA typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA or BLA, the FDA will typically inspect one or more clinical sites to assure compliance with cGCP.

The FDA is required to refer an application for a novel drug to an advisory committee or explain why such referral was not made. Typically, an advisory committee is a panel of independent experts, including clinicians and other scientific experts, that reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

The FDA's Decision on an NDA or BLA

After the FDA evaluates the NDA or BLA and conducts inspections of manufacturing facilities where the drug product and/or its drug substance will be produced, it may issue an approval letter or a Complete Response Letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. A Complete Response Letter indicates that the review cycle of the application is complete and the application is not ready for approval. A Complete Response Letter may require additional clinical data and/or an additional pivotal Phase 3 clinical study(ies), and/or other significant, expensive and time-consuming requirements related to clinical studies, preclinical studies or manufacturing. Even if such additional information is submitted, the FDA may ultimately decide that the NDA or BLA does not satisfy the criteria for approval. The FDA could also approve the NDA or BLA with a Risk Evaluation and Mitigation Strategy, or REMS, plan to mitigate risks, which could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. The FDA also may condition approval on, among other things, changes to proposed labeling, development of adequate

controls and specifications, or a commitment to conduct one or more post-market studies or clinical studies. Such post-market testing may include Phase 4 clinical studies and surveillance to further assess and monitor the product's safety and effectiveness after commercialization. Also, new government requirements, including those resulting from new legislation, may be established, or the FDA's policies may change, which could delay or prevent regulatory approval of our products under development.

Expedited Review and Accelerated Approval Programs

A sponsor may seek approval of its product candidate under programs designed to accelerate FDA's review and approval of NDAs and BLAs. For example, Fast Track Designation may be granted to a drug intended for treatment of a serious or life-threatening disease or condition that has potential to address unmet medical needs for the disease or condition. The key benefits of fast track designation are the eligibility for priority review, rolling review (submission of portions of an application before the complete marketing application is submitted), and accelerated approval, if relevant criteria are met. Based on results of the Phase 3 clinical study(ies) submitted in an NDA or BLA, upon the request of an applicant, the FDA may grant the NDA or BLA a priority review designation, which sets the target date for FDA action on the application at six months after the FDA accepts the application for filing. Priority review is granted where there is evidence that the proposed product would be a significant improvement in the safety or effectiveness of the treatment, diagnosis, or prevention of a serious condition. If criteria are not met for priority review, the application is subject to the standard FDA review period of ten months after FDA accepts the application for filing. Priority review designation does not change the scientific/medical standard for approval or the quality of evidence necessary to support approval.

Under the accelerated approval program, the FDA may approve an NDA or BLA on the basis of either a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. Post-marketing studies or completion of ongoing studies after marketing approval are generally required to verify the drug's clinical benefit in relationship to the surrogate endpoint or ultimate outcome in relationship to the clinical benefit. In addition, the Food and Drug Administration Safety and Innovation Act, or FDASIA, which was enacted and signed into law in 2012, established the new Breakthrough Therapy designation. A sponsor may seek FDA designation of its product candidate as a breakthrough therapy if the drug is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development.

Post-Approval Requirements

Drugs manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims are subject to prior FDA review and approval. There also are continuing, annual user fee requirements for any marketed products and the establishments at which such products are manufactured, as well as new application fees for supplemental applications with clinical data.

Drug manufacturers are subject to periodic unannounced inspections by the FDA and state agencies for compliance with cGMP requirements. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon us and any third-party manufacturers that we may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

We rely, and expect to continue to rely, on third parties for the production of clinical quantities of our product candidates, and expect to rely in the future on third parties for the production of commercial quantities. Future FDA and state inspections may identify compliance issues at our facilities or at the facilities of our contract manufacturers that may disrupt production or distribution, or require substantial resources to correct. In addition, discovery of previously unknown problems with a product or the failure to comply with applicable requirements may result in restrictions on a product, manufacturer or holder of an approved NDA or BLA, including withdrawal or recall of the product from the market or other voluntary, FDA-initiated or judicial action that could delay or prohibit further marketing. Also, new government requirements, including those resulting from new legislation, may be established, or the FDA's policies may change, which could delay or prevent regulatory approval of our products under development.

The FDA may withdraw approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical studies to assess new safety risks; or imposition of distribution restrictions or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or holds on post-approval clinical studies;
- refusal of the FDA to approve pending NDAs or supplements to approved NDAs, or suspension or revocation of product license approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising, and promotion of products that are placed on the market. Drugs may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

Orphan Designation and Exclusivity

The FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the United States, or if it affects more than 200,000 individuals in the United States, there is no reasonable expectation that the cost of developing and making the drug for this type of disease or condition will be recovered from sales in the United States.

Orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical study costs, tax advantages, and user-fee waivers. In addition, if a product receives FDA approval for the indication for which it has orphan designation, the product is entitled to orphan drug exclusivity, which means the FDA may not approve any other application to market the same drug for the same indication for a period of seven years, except in limited circumstances, such as a showing of clinical superiority over the product with orphan exclusivity.

Pediatric Studies and Exclusivity

NDAs and BLAs must contain data (or a proposal for post-marketing activity) to assess the safety and effectiveness of an investigational new drug product for the claimed indications in all relevant pediatric populations in order to support dosing and administration for each pediatric subpopulation for which the drug is

safe and effective. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults or full or partial waivers if certain criteria are met. Discussions about pediatric development plans can be discussed with the FDA at any time, but usually occur any time between the end-of-Phase II meeting and submission of the NDA or BLA. The requirements for pediatric data do not apply to any drug for an indication for which orphan designation has been granted.

Pediatric exclusivity is another type of non-patent exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the five-year and three-year non-patent and orphan exclusivity. This six-month exclusivity may be granted if an NDA or BLA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical study is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of FDA-requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity or patent protection cover the product are extended by six months. This is not a patent term extension, but it effectively extends the regulatory period during which the FDA cannot accept or approve another application relying on the NDA or BLA sponsor's data.

Patent Term Restoration

Depending upon the timing, duration, and specifics of the FDA approval of the use of our product candidates, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of an NDA or BLA, plus the time between the submission date and the approval of that application. Only one patent applicable to an approved product is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The U.S. Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may apply for restoration of patent term for one of our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical studies and other factors involved in the filing of the relevant NDA or BLA.

Biosimilars and Exclusivity

The Patient Protection and Affordable Care Act, or Affordable Care Act, signed into law on March 23, 2010, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCI Act, which created an abbreviated approval pathway for biological products shown to be similar to, or interchangeable with, an FDA-licensed reference biological product. This amendment to the PHSA attempts to minimize duplicative testing. Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical study or studies. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product and, for products administered multiple times, the biologic and the reference biologic may be switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. However, complexities associated with the larger, and often more complex, structure of biological products, as well as the process by which such products are manufactured, pose significant hurdles to implementation that are still being worked out by the FDA.

A reference biologic is granted twelve years of exclusivity from the time of first licensure of the reference product. The first biologic product submitted under the abbreviated approval pathway that is determined to be

interchangeable with the reference product has exclusivity against other biologics submitting under the abbreviated approval pathway for the lesser of (i) one year after the first commercial marketing, (ii) eighteen months after approval if there is no legal challenge, (iii) eighteen months after the resolution in the applicant's favor of a lawsuit challenging the biologics' patents if an application has been submitted, or (iv) 42 months after the application has been approved if a lawsuit is ongoing within the 42-month period.

Abbreviated New Drug Applications for Generic Drugs

In 1984, with passage of the Hatch-Waxman Act, Congress authorized the FDA to approve generic drugs that are the same as drugs previously approved by the FDA under the NDA provisions of the statute. To obtain approval of a generic drug, an applicant must submit an abbreviated new drug application, or ANDA, to the agency. In support of such applications, a generic manufacturer may rely on the preclinical and clinical testing previously conducted for a drug product previously approved under an NDA, known as the reference listed drug, or RLD.

Specifically, in order for an ANDA to be approved, the FDA must find that the generic version is identical to the RLD with respect to the active ingredients, the route of administration, the dosage form, and the strength of the drug. At the same time, the FDA must also determine that the generic drug is "bioequivalent" to the innovator drug. Under the statute, a generic drug is bioequivalent to an RLD if "the rate and extent of absorption of the [generic] drug do not show a significant difference from the rate and extent of absorption of the listed drug. . . ."

Upon approval of an ANDA, the FDA indicates that the generic product is "therapeutically equivalent" to the RLD and it assigns a therapeutic equivalence rating to the approved generic drug in its publication "Approved Drug Products with Therapeutic Equivalence Evaluations," also referred to as the "Orange Book." Physicians and pharmacists consider an "AB" therapeutic equivalence rating to mean that a generic drug is fully substitutable for the RLD. In addition, by operation of certain state laws and numerous health insurance programs, the FDA's designation of an "AB" rating often results in substitution of the generic drug without the knowledge or consent of either the prescribing physician or patient.

The FDCA provides a period of five years of non-patent exclusivity for a new drug containing a new chemical entity. In cases where such exclusivity has been granted, an ANDA may not be filed with the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification, in which case the applicant may submit its application four years following the original product approval. The FDCA also provides for a period of three years of exclusivity if the NDA includes reports of one or more new clinical investigations, other than bioavailability or bioequivalence studies, that were conducted by or for the applicant and are essential to the approval of the application. This three-year exclusivity period often protects changes to a previously approved drug product, such as a new dosage form, route of administration, combination or indication.

Hatch-Waxman Patent Certification and the 30-Month Stay

Upon approval of an NDA or a supplement thereto, NDA sponsors are required to list with the FDA each patent with claims that cover the applicant's product or a method of using the product. Each of the patents listed by the NDA sponsor is published in the Orange Book. When an ANDA applicant files its application with the FDA, the applicant is required to certify to the FDA concerning any patents listed for the reference product in the Orange Book, except for patents covering methods of use for which the ANDA applicant is not seeking approval.

Specifically, the applicant must certify with respect to each patent that:

- the required patent information has not been filed;
- the listed patent has expired;
- the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or
- the listed patent is invalid, unenforceable or will not be infringed by the new product.

A certification that the new product will not infringe the already approved product's listed patents or that such patents are invalid or unenforceable is called a Paragraph IV certification. If the applicant does not challenge the listed patents or indicates that it is not seeking approval of a patented method of use, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired.

If the ANDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days after the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months after the receipt of the Paragraph IV notice, expiration of the patent, or a decision in the infringement case that is favorable to the ANDA applicant.

European Union/Rest of World Government Regulation

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical studies and any commercial sales and distribution of our products.

Whether or not we obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical studies or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical study application much like the IND prior to the commencement of human clinical studies. In the European Union, for example, a clinical study application, or CTA, must be submitted for each clinical protocol to each country's national health authority and an independent ethics committee, much like the FDA and IRB, respectively. Once the CTA is accepted in accordance with a country's requirements, the clinical study may proceed.

The requirements and process governing the conduct of clinical studies vary from country to country. In all cases, the clinical studies are conducted in accordance with cGCP, the applicable regulatory requirements, and the ethical principles that have their origin in the Declaration of Helsinki.

To obtain regulatory approval of an investigational medicinal product under European Union regulatory systems, we must submit a marketing authorization application. The content of the NDA or BLA filed in the United States is similar to that required in the European Union, with the exception of, among other things, country-specific document requirements.

For other countries outside of the European Union, such as countries in Eastern Europe, Latin America or Asia, the requirements governing product licensing, pricing, and reimbursement vary from country to country.

Countries that are part of the European Union, as well as countries outside of the European Union, have their own governing bodies, requirements, and processes with respect to the approval of pharmaceutical products. If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Authorization Procedures in the European Union

Medicines can be authorized in the European Union by using either the centralized authorization procedure or national authorization procedures.

- *Centralized procedure.* The EMA implemented the centralized procedure for the approval of human medicines to facilitate marketing authorizations that are valid throughout the European Economic Area, or EEA, which is comprised of the 28 member states of the European Union plus Norway, Iceland, and Lichtenstein. This procedure results in a single marketing authorization issued by the EMA that is valid across the EEA. The centralized procedure is compulsory for human medicines that are: derived from

biotechnology processes, such as genetic engineering, contain a new active substance indicated for the treatment of certain diseases, such as HIV/AIDS, cancer, diabetes, neurodegenerative disorders or autoimmune diseases and other immune dysfunctions, and officially designated orphan medicines.

- For medicines that do not fall within these categories, an applicant has the option of submitting an application for a centralized marketing authorization to the European Commission following a favorable opinion by the EMA, as long as the medicine concerned is a significant therapeutic, scientific or technical innovation, or if its authorization would be in the interest of public health.
- *National authorization procedures.* There are also two other possible routes to authorize medicinal products in several European Union countries, which are available for investigational medicinal products that fall outside the scope of the centralized procedure:
  - *Decentralized procedure*. Using the decentralized procedure, an applicant may apply for simultaneous authorization in more than one European Union country of medicinal products that have not yet been authorized in any European Union country and that do not fall within the mandatory scope of the centralized procedure.
  - *Mutual recognition procedure.* In the mutual recognition procedure, a medicine is first authorized in one European Union Member State, in accordance with the national procedures of that country. Following this, further marketing authorizations can be sought from other European Union countries in a procedure whereby the countries concerned agree to recognize the validity of the original, national marketing authorization.

In some cases, a Pediatric Investigation Plan, and/or a request for waiver or deferral, is required for submission prior to submitting a marketing authorization application. A PIP describes, among other things, proposed pediatric studies and their timing relative to clinical studies in adults.

New Chemical Entity Exclusivity

In the European Union, new chemical entities, sometimes referred to as new active substances, qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. This data exclusivity, if granted, prevents regulatory authorities in the European Union from referencing the innovator's data to assess a generic (abbreviated) application for eight years, after which generic marketing authorization can be submitted, and the innovator's data may be referenced, but not approved for two years. The overall ten-year period will be extended to a maximum of eleven years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies.

Orphan Designation and Exclusivity

In the European Union, the EMA's Committee for Orphan Medicinal Products, or COMP, grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than 5 in 10,000 persons in the European Union Community and for which no satisfactory method of diagnosis, prevention, or treatment has been authorized (or the product would be a significant benefit to those affected). Additionally, designation is granted for products intended for the diagnosis, prevention, or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the medicinal product.

In the European Union, orphan drug designation entitles a party to financial incentives such as reduction of fees or fee waivers and 10 years of market exclusivity is granted following medicinal product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity.

Orphan drug designation must be requested before submitting an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

Exceptional Circumstances/Conditional Approval

Orphan drugs or drugs with unmet medical needs may be eligible for EU approval under exceptional circumstances or with conditional approval. Approval under exceptional circumstances is applicable to orphan products and is used when an applicant is unable to provide comprehensive data on the efficacy and safety under normal conditions of use because the indication for which the product is intended is encountered so rarely that the applicant cannot reasonably be expected to provide comprehensive evidence, when the present state of scientific knowledge does not allow comprehensive information to be provided, or when it is medically unethical to collect such information. Conditional marketing authorization is applicable to orphan medicinal products, medicinal products for seriously debilitating or life-threatening diseases, or medicinal products to be used in emergency situations in response to recognized public threats. Conditional marketing authorization can be granted on the basis of less complete data than is normally required in order to meet unmet medical needs and in the interest of public health, provided the risk-benefit balance is positive, it is likely that the applicant will be able to provide the comprehensive clinical data, and unmet medical needs will be fulfilled. Conditional marketing authorization is subject to certain specific obligations to be reviewed annually.

Accelerated Review

Under the Centralized Procedure in the European Union, the maximum timeframe for the evaluation of a marketing authorization application is 210 days (excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the EMA's Committee for Medicinal Products for Human Use, or CHMP). Accelerated evaluation might be granted by the CHMP in exceptional cases, when a medicinal product is expected to be of a major public health interest, particularly from the point of view of therapeutic innovation. In this circumstance, EMA ensures that the opinion of the CHMP is given within 150 days, excluding clock stops.

Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any drug products for which we obtain regulatory approval. In the United States and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend in part on the availability of coverage and reimbursement from third-party payors. Third-party payors include government authorities, managed care providers, private health insurers and other organizations. The process for determining whether a payor will provide coverage for a drug product may be separate from the process for setting the reimbursement rate that the payor will pay for the drug product. Third-party payors may limit coverage to specific drug products on an approved list, or formulary, which might not include all of the FDA-approved drugs for a particular indication. Moreover, a payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Adequate third-party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

Third-party payors are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. In order to obtain coverage and reimbursement for any product that might be approved for sale, we may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain regulatory approvals. Our product candidates may not be considered medically necessary or cost-effective. If third-party payors do not consider a product to be cost-effective compared to other available therapies, they may not cover the product after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow a company to sell its products at a profit.

The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost containment programs to limit the growth of government-paid health care costs, including price controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. By way of example, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, collectively, the Healthcare Reform Law, contains provisions that may reduce the profitability of drug products, including, for example, increased rebates for drugs sold to Medicaid programs, extension of Medicaid rebates to Medicaid managed care plans, mandatory discounts for certain Medicare Part D beneficiaries and annual fees based on pharmaceutical companies' share of sales to federal health care programs. Adoption of government controls and measures, and tightening of restrictive policies in jurisdictions with existing controls and measures, could limit payments for pharmaceuticals.

In the European Community, governments influence the price of pharmaceutical products through their pricing and reimbursement rules and control of national health care systems that fund a large part of the cost of those products to consumers. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed to by the government. To obtain reimbursement or pricing approval, some of these countries may require the completion of clinical studies that compare the cost-effectiveness of a particular product candidate to currently available therapies. Other member states allow companies to fix their own prices for medicines, but monitor and control company profits. The downward pressure on health care costs in general, particularly prescription drugs, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert a commercial pressure on pricing within a country.

The marketability of any products for which we receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide adequate coverage and reimbursement. In addition, an increasing emphasis on cost containment measures in the United States and other countries has increased and we expect will continue to increase the pressure on pharmaceutical pricing. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

Other Healthcare Laws and Compliance Requirements

If we obtain regulatory approval for any of our product candidates, we may be subject to various federal and state laws targeting fraud and abuse in the healthcare industry. These laws may impact, among other things, our proposed sales, marketing and education programs. In addition, we may be subject to patient privacy regulation by both the federal government and the states in which we conduct our business. The laws that may affect our ability to operate include:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, to induce, or in return for, the purchase or recommendation of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs;
- federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid, or other third-party payers that are false or fraudulent;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal criminal statutes that prohibit executing a scheme to defraud any healthcare benefit program and making false statements relating to healthcare matters;
- the federal transparency laws, including the federal Physician Payment Sunshine Act, that requires drug manufacturers to disclose payments and other transfers of value provided to physicians and teaching hospitals;

- HIPAA, as amended by the Health Information Technology and Clinical Health Act, or HITECH, and its implementing regulations, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information; and
- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payer, including commercial insurers, and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

The Healthcare Reform Law broadened the reach of the fraud and abuse laws by, among other things, amending the intent requirement of the federal Anti-Kickback Statute and the applicable criminal healthcare fraud statutes contained within 42 U.S.C. § 1320a-7b, effective March 23, 2010. Pursuant to the statutory amendment, a person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation. In addition, the Healthcare Reform Law provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act (discussed below) or the civil monetary penalties statute. Many states have adopted laws similar to the federal Anti-Kickback Statute, some of which apply to the referral of patients for healthcare items or services reimbursed by any source, not only the Medicare and Medicaid programs.

We are also subject to the Foreign Corrupt Practices Act, or FCPA, which prohibits improper payments or offers of payments to foreign governments and their officials for the purpose of obtaining or retaining business. Safeguards we implement to discourage improper payments or offers of payments by our employees, consultants, and others may be ineffective, and violations of the FCPA and similar laws may result in severe criminal or civil sanctions, or other liabilities or proceedings against us, any of which would likely harm our reputation, business, financial condition and result of operations.

If our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, exclusion from participation in government healthcare programs, such as Medicare and Medicaid and imprisonment, damages, fines and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

Employees

As of September 30, 2013, we had 46 full-time employees. None of our employees is represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

Research and Development

We invested $4.7 million and $12.6 million in research and development in the years ended December 31, 2011 and 2012, respectively, and invested $19.6 million in research and development for the nine months ended September 30, 2013.

Facilities

Our offices are located at two leased facilities: a 19,916 square foot facility in Novato, California used primarily for corporate, clinical, regulatory, manufacturing, and quality functions; and a 910 square foot facility in Novato, California used for research laboratory space. These leases expire in July 2016 and September 2014, respectively.

Legal Proceedings

We are not currently a party to any material legal proceedings.

MANAGEMENT

Executive Officers, Directors, and Key Employees

The following table sets forth information regarding our executive officers, directors and nominee for director, and key employees as of November 1, 2013:

Name	Age	Position(s)
Executive Officers		
Emil D. Kakkis, M.D., Ph.D.	53	President and Chief Executive Officer, Director
Thomas Kassberg	53	Chief Business Officer and Senior Vice President
Shalini Sharp	38	Chief Financial Officer and Senior Vice President
Non-Employee Directors and Director Nominees		
Eran Nadav, Ph.D.	44	Chairman of the Board
William Aliski	66	Director
Benjamin Auspitz(1)	40	Director
Mårten Steen, M.D., Ph.D.	38	Director
Matthew K. Fust(2)	49	Director nominee
Key Employees		
John Ditton	49	Vice President, Commercial Planning
Steven Jungles	48	Senior Vice President, Technical Operations
Tony Koutsoukos, Ph.D.	53	Vice President, Biometrics
Cordelia Leonard, RAC	53	Vice President, Regulatory Affairs and Quality Assurance
Vimal Srivastava	48	Vice President, Program Development
Michael Vellard, Ph.D.	52	Vice President, Research
Spencer Guthrie	38	Senior Director, Clinical Operations
Alison Skrinar	43	Senior Director, Clinical Sciences

(1) Mr. Auspitz will resign from our board of directors upon the completion of this offering.
(2) We expect that Mr. Fust will become a member of our board of directors upon the completion of this offering.

Executive Officers

Emil D. Kakkis, M.D., Ph.D. is our founder and has served as our President and Chief Executive Officer and as a member of our board of directors since inception in April 2010. Prior to Ultragenyx, Dr. Kakkis served from September 1998 to February 2009 in various executive capacities, and ultimately as Chief Medical Officer, at BioMarin Pharmaceutical Inc., a biopharmaceutical company. Dr. Kakkis also serves as President and Founder of EveryLife Foundation for Rare Diseases, a non-profit organization he started in 2009 to accelerate biotechnology innovation for rare diseases. Dr. Kakkis is board certified in both Pediatrics and Medical Genetics. He holds a B.A. in Biology from Pomona College and received combined M.D. and Ph.D. degrees from the UCLA School of Medicine's Medical Scientist Training Program and received the Bogen prize for his research. We believe that Dr. Kakkis possesses specific expert knowledge of genetics and rare diseases that qualifies him to serve on our board of directors, including his leadership, management, and operational experience in the life sciences sector.

Thomas Kassberg has served as our Chief Business Officer and Senior Vice President since November 2011. Prior to Ultragenyx, Mr. Kassberg worked as Vice President of Business Development at Corium International, Inc., a biotechnology company, from July 2010 until October 2011. Prior to his work at Corium International, Inc., Mr. Kassberg worked as an independent consultant in Corporate Development and Business Strategy and consulted with a number of companies from March 2009 to June 2010, including Corium International, Inc. and Rib-X Pharmaceuticals, Inc., a pharmaceutical company focused on the development of novel antibiotics. Before becoming a consultant, Mr. Kassberg worked at Proteolix, Inc., a biotechnology company subsequently acquired by Onyx Pharmaceuticals, from January 2008 until February 2009, where he served as Senior Vice President of Corporate Development. Mr. Kassberg holds a B.A. in Business Administration from Gustavus Adolphus College and an M.B.A. from Northwestern University.

Shalini Sharp has served as our Chief Financial Officer and Senior Vice President since May 2012. Prior to Ultragenyx, Ms. Sharp served in various executive capacities, and ultimately as Chief Financial Officer, of Agenus Inc., a biotechnology company, from August 2003 until May 2012. Prior to Agenus, Ms. Sharp held strategic planning and corporate finance roles and ultimately served as chief of staff to the chairman of the board at Elan Pharmaceuticals, a biotechnology company, from August 1998 to August 1999 and September 2001 to August 2003. Prior to Elan, Ms. Sharp was a management consultant at McKinsey & Company and an investment banker at Goldman Sachs, specializing in pharmaceuticals and medical devices. Ms. Sharp has also served as a board member of Agenus since May 2012. Ms. Sharp holds a B.A. and an M.B.A. from Harvard University.

Non-Employee Directors

Eran Nadav, Ph.D. has served as a member of our board of directors since June 2011 and has served as our Chairman of the board since January 2012. Dr. Nadav is a Managing Director at TPG Biotech®, the life science venture investment arm of TPG, a global private investment firm. Dr. Nadav joined TPG in 2007 with a focus on global pharmaceuticals and biotechnology investments. Prior to TPG, Dr. Nadav served as Business Development Director at Eisai, a pharmaceutical company, from September 2003 to August 2007 and also as a manager at Johnson & Johnson Development Corporation, the venture capital arm of Johnson & Johnson, a healthcare company, from November 1999 until July 2002. Dr. Nadav served on the board of directors of Eden Springs Ltd., a European provider of drinking water solutions for the workplace, from July 2010 until August 2011. Since June 2013 he has been serving on the board of directors of MacroGenics, Inc., a biopharmaceutical company. Dr. Nadav received a B.Sc. magna cum laude in Life Sciences, an M.Sc. magna cum laude and Ph.D. in Biochemistry, as well as an M.B.A., from Tel Aviv University. We believe that Dr. Nadav is qualified to serve on our board of directors due to his experience in the venture capital industry and his years of analyzing development opportunities in the life sciences sector.

William Aliski has served as a member of our board of directors since January 2011. Mr. Aliski served as a commercial consultant for early stage orphan disease companies, including Enobia Pharma, from September 2011 until March 2012. Before that, Mr. Aliski served as Senior Vice President and Chief Commercial Officer of FoldRx Pharmaceuticals, a rare disease company that is now a wholly-owned subsidiary of Pfizer Inc., from June 2009 until March 2011, as Director of Simon Kucher Partners, a global consulting firm, from January 2008 until June 2009 and as General Manager of BioMarin Europe at BioMarin Pharmaceuticals Inc. from December 2005 until January 2008. Mr. Aliski received a B.S. in Economics and a Master of Social Planning from Boston College and an M.P.A. from the Kennedy School of Government at Harvard University. We believe that Mr. Aliski is qualified to serve on our board of directors due to his extensive experience in the life sciences industry, membership of various boards of directors, and his leadership and management experience.

Benjamin Auspitz has served as a member of our board of directors since June 2011. Mr. Auspitz has served as a Partner at Fidelity Biosciences, a venture capital firm, since November 2005. Mr. Auspitz received a B.A. in Philosophy from Harvard University. We believe that Mr. Auspitz is qualified to serve on our board of directors due to his experience in the venture capital industry, membership of various other boards of directors, and his leadership and management experience.

Mårten Steen, M.D., Ph.D. has served as a member of our board of directors since June 2011. Dr. Steen has served as a Partner at HealthCap, a private equity firm, since March 2010. Prior to HealthCap, Dr. Steen served as Associate Director at Merck Serono, a biopharmaceutical company, from February 2008 until March 2010. Dr. Steen received a B.Sc. in Business Administration, an M.D. and a Ph.D. in Clinical Chemistry from Lund University. We believe that Dr. Steen is qualified to serve on our board of directors due to his medical and scientific background as well as his experience in the venture capital industry.

Matthew K. Fust joined us as a board observer in November 2013 and is expected to become a member of our board of directors upon the completion of this offering. Mr. Fust currently serves as Executive Vice President of Onyx Pharmaceuticals, Inc., a biopharmaceutical company that was recently acquired by Amgen, Inc., and has served in this position since January 2009. From May 2003 to December 2008, Mr. Fust served as Chief

Financial Officer at Jazz Pharmaceuticals, Inc., a specialty pharmaceutical company. From 2002 to 2003, Mr. Fust served as Chief Financial Officer at Perlegen Sciences, a biopharmaceutical company. Previously, he was Senior Vice President and Chief Financial Officer at ALZA Corporation, a pharmaceutical company, where he was an executive from 1996 until 2002. From 1991 until 1996, Mr. Fust was a manager in the healthcare strategy practice at Andersen Consulting. Mr. Fust serves on the Board of Directors of Sunesis Pharmaceuticals, Inc., a biopharmaceutical company. Mr. Fust received a B.A. from the University of Minnesota and an M.B.A. from the Stanford Graduate School of Business. We believe that Mr. Fust is qualified to serve on our board of directors due to his extensive experience in the life sciences industry, his leadership and management experience, and his service as a director of another public biopharmaceutical company.

Key Employees

John Ditton has served as our Vice President, Commercial Planning since April 2011. Prior to Ultragenyx, Mr. Ditton was the Chief Operating Officer at EveryLife Foundation for Rare Diseases, from January 2009 to April 2011. Prior to working at the EveryLife Foundation, Mr. Ditton served as the Vice President of Marketing at Diamics, Inc., a maker of cancer diagnostics, from October 2006 to December 2008 and Director of Global Marketing at BioMarin Pharmaceutical Inc., a biopharmaceutical company, from March 2004 to March 2006. Mr. Ditton holds an M.B.A. from the University of Tasmania.

Steven Jungles has served as our Senior Vice President, Technical Operations since August 2011. Prior to Ultragenyx, Mr. Jungles worked as Vice President, Supply Chain at BioMarin Pharmaceutical Inc., a biopharmaceutical company, from June 1999 to July 2011, was Associate Director of Operations at Harvard Gene Therapy Initiative from June 1997 until June 1999, and worked at Somatix Therapy Corporation, a research and development company in the field of gene therapy that was acquired by Cell Genesys, Inc., from March 1993 to May 1997. Mr. Jungles holds a B.S. in Biology from the University of Iowa.

Tony Koutsoukos, Ph.D. has served as our Vice President of Biometrics since October 2013. Prior to Ultragenyx, Mr. Koutsoukos worked as Vice President of Biometrics at Allos Therapeutics, a biopharmaceutical company, from September 2007 to March 2013, which was acquired by Spectrum Pharmaceuticals. He was also Director of Biostatistics at Amgen Inc., a biotechnology company, from May 2002 to September 2007. Prior to Amgen, Mr. Koutsoukos spent three years at Quintiles, a contract research company, as a Director of Biostatistics. His experience also includes five years at the FDA, Center for Drugs Evaluation and Research (CDER) division and approximately four years at the National Cancer Institute, Biometric Research Branch, CTEP, DCT. Dr. Koutsoukos received his Ph.D. and M.A., both in Mathematical Statistics from the University of Maryland, College Park.

Cordelia Leonard has served as our Vice President, Regulatory Affairs and Quality Assurance since July 2011. Prior to Ultragenyx, Ms. Leonard was Senior Director, Regulatory Affairs at BioMarin Pharmaceutical Inc., a biopharmaceutical company, from October 2003 until July 2011. Prior to BioMarin, Ms. Leonard was the Manager, Regulatory Affairs at Cerus Corporation, a biomedical products company, from May 1999 until October 2003. Ms. Leonard received bachelor degrees in Chemistry and Biological Science from the University of California, Irvine and holds both U.S. and EU Regulatory Affairs Certifications.

Vimal Srivastava has served as our Vice President, Program Development since August 2011. Before joining Ultragenyx, Mr. Srivastava was Senior Director, Portfolio and Project Management at Elan/Janssen Alzheimer Immunotherapy, a biotechnology company, from January 2008 until August 2011. He was also Director, Global Program Manager, Diabetes at Amgen Inc., a biotechnology company, from September 2005 to January 2008 and Director, Program Management at BioMarin Pharmaceutical Inc., a biopharmaceutical company, from March 2003 to September 2005. Mr. Srivastava holds a B.S. in Pharmacy from Banaras Hindu University, an M.S. in Medicinal Chemistry from St. John’s University and an M.A.S. in Management from Johns Hopkins University.

Michael Vellard, Ph.D. has served as our Vice President, Research since May 2013. Prior to joining Ultragenyx, Dr. Vellard worked as Head of Lysosomal Biology at BioMarin Pharmaceutical Inc., a biopharmaceutical company, from October 1999 to May 2013. He was a postdoctoral fellow in the pediatric

department at UCLA Harbor Medical Center from September 1992 to June 1995. Dr. Vellard received his B.S. in Natural and Life Sciences and M.S. in Molecular and Cellular Genetics from the University of Lyon I, France. He obtained his Ph.D. in Virology from the Pasteur and Curie Institutes (Universities Paris VI, VII and XI), France.

Spencer Guthrie has served as our Senior Director, Clinical Operations since June 2012. Prior to Ultragenyx, Mr. Guthrie worked as Director of Clinical Operations and Project Team Leader at Elan Pharmaceuticals, a biotechnology company, and Janssen Alzheimer's Immunotherapy from September 2007 to June 2012. Prior to that, Mr. Guthrie spent nine years with increasing responsibilities at Genentech in Clinical Operations and Market Planning. At Genentech, he worked on several innovative clinical programs, IND and BLA filings with Rituxan, Avastin, and Lucentis, including work on orphan indications. Mr. Guthrie also spent two years at ICON Clinical Research and a year at NASA's space science lab. Mr. Guthrie received his B.A. in Neuroscience from Vanderbilt University, an M.B.A. from the University of California, Irvine and he is certified as a Project Management Professional.

Alison Skrinar, Ph.D. has served as our Senior Director, Clinical Sciences since March 2012. Prior to joining Ultragenyx, Dr. Skrinar worked as the Senior Director of Clinical Outcomes and Regulatory Affairs from February 2009 to February 2012 at Enobia Pharma, Inc., a private clinical stage orphan company focused on the development of an enzyme replacement therapy for hypophosphastasia, which was acquired by Alexion in 2012. Prior to Enobia Pharma, Dr. Skrinar was the Senior Director of Clinical Outcomes at Genzyme Corporation, a biotechnology company, from May 2001 until January 2009. In her nearly 15 years in the biotechnology industry, Dr. Skrinar has worked exclusively on the clinical development and regulatory approval of ultra-orphan drugs. Dr. Skrinar received a B.B.A. from Emory University and a Ph.D. and a Master of Public Health degree from the University of Alabama.

Board Composition

Director Independence

Our board of directors currently consists of five members. Our board of directors has determined that all of our directors, other than Dr. Kakkis, qualify as "independent" directors in accordance with the NASDAQ listing requirements. Dr. Kakkis is not considered independent because he is an employee of Ultragenyx. The NASDAQ independence definition includes a series of objective tests, such as that the director is not, and has not been for at least three years, one of our employees and that neither the director nor any of his family members has engaged in various types of business dealings with us. In addition, as required by NASDAQ rules, our board of directors has made a subjective determination as to each independent director that no relationships exist, which, in the opinion of our board of directors, would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. In making these determinations, our board of directors reviewed and discussed information provided by the directors and us with regard to each director's business and personal activities and relationships as they may relate to us and our management. There are no family relationships among any of our directors or executive officers.

Classified Board of Directors

We currently have five directors, all of whom were elected pursuant to the terms of a voting agreement by and among us and certain of our stockholders, which will terminate upon completion of this offering. Upon the termination of the voting agreement, we will not be bound by contractual obligations regarding the election of our directors.

Effective upon the closing of this offering, we will divide the terms of office of the directors into three classes:

- Class I, whose term will expire at the annual meeting of stockholders to be held in 2014;
- Class II, whose term will expire at the annual meeting of stockholders to be held in 2015; and
- Class III, whose term will expire at the annual meeting of stockholders to be held in 2016.

Upon the completion of this offering, Class I shall consist of Emil D. Kakkis and Mårten Steen, Class II shall consist of Eran Nadav and , and Class III is expected to consist of Matthew Fust and William Aliski.

Benjamin Auspitz, currently a member of our board of directors, has indicated to us his intention to resign from our board of directors upon the completion of this offering. At each annual meeting of stockholders after the initial classification, the successors to directors whose terms will then expire shall serve from the time of election and qualification until the third annual meeting following election and until their successors are duly elected and qualified. A resolution of the board of directors may change the authorized number of directors. Any additional directorships resulting from an increase in the number of directors will be distributed among the three classes so that, as nearly as possible, each class will consist of one-third of the directors. This classification of the board of directors may have the effect of delaying or preventing changes in control or management of our company.

Following the completion of this offering, our nominating and corporate governance committee and board of directors may consider a broad range of factors relating to the qualifications and background of nominees, which may include diversity and is not limited to race, gender, or national origin. We have no formal policy regarding board diversity. Our nominating and corporate governance committee's and board of directors' priority in selecting board members is identification of persons who will further the interests of our company through his or her established record of professional accomplishment, the ability to contribute positively to the collaborative culture among board members, and professional and personal experiences and expertise relevant to our growth strategy.

Board Committees

Effective upon the completion of this offering, our board of directors will have three standing committees: the audit committee, the compensation committee and the nominating and corporate governance committee.

Audit Committee

Effective upon the completion of this offering, our audit committee will consist of , and , with serving as chairman of the committee. Our board of directors has determined that each member of the audit committee meets the independence requirements of Rule 10A-3 under the Exchange Act and the applicable listing standards of NASDAQ. Our board of directors has determined that is an "audit committee financial expert" within the meaning of the SEC regulations and applicable listing standards of NASDAQ. The audit committee's responsibilities upon completion of this offering will include:

- appointing, approving the compensation of, reviewing the performance of, and assessing the independence of our independent registered public accounting firm;
- approving audit and permissible non-audit services, and the terms of such services, to be provided by our independent registered public accounting firm;
- reviewing the audit plan with the independent registered public accounting firm and members of management responsible for preparing our financial statements;
- reviewing and discussing with management and the independent registered public accounting firm our annual and quarterly financial statements and related disclosures as well as critical accounting policies and practices used by us;
- reviewing the adequacy of our internal control over financial reporting;
- establishing policies and procedures for the receipt and retention of accounting-related complaints and concerns;
- recommending, based upon its review and discussions with management and the independent registered public accounting firm, whether our audited financial statements shall be included in our Annual Report on Form 10-K;
- preparing the audit committee report required by the rules of the SEC to be included in our annual proxy statement;
- reviewing all related party transactions for potential conflict of interest situations and approving all such transactions;

- reviewing policies related to risk assessment and risk management; and
- establishing, maintaining and overseeing our Code of Business Conduct and Ethics.

Compensation Committee

Effective upon the completion of this offering, our compensation committee will consist of , and , with serving as chairman of the committee. Our board of directors has determined each member of the compensation committee is "independent" as defined under the applicable listing standards of NASDAQ. The compensation committee's responsibilities upon completion of this offering will include:

- annually reviewing and approving individual and corporate goals and objectives relevant to the compensation of our executive officers;
- evaluating the performance of our executive officers in light of such individual and corporate goals and objectives and determining the compensation of our executive officers;
- appointing, compensating and overseeing the work of any compensation consultant, legal counsel or other advisor retained by the compensation committee;
- conducting the independence assessment outlined in NASDAQ rules with respect to any compensation consultant, legal counsel, or other advisor retained by the compensation committee;
- annually reviewing and reassessing the adequacy of the committee charter in its compliance with the listing requirements of NASDAQ;
- overseeing and administering our compensation and similar plans;
- reviewing and approving our policies and procedures for the grant of equity-based awards;
- reviewing and making recommendations to the board of directors with respect to director compensation;
- reviewing and discussing with management the compensation discussion and analysis to be included in our annual proxy statement or Annual Report on Form 10-K;
- preparing the compensation committee report required by the rules of the SEC to be included in our annual proxy statement;
- reviewing and discussing with the board of directors corporate succession plans for the chief executive officer and other senior management positions; and
- periodically reviewing our policies, practices, and procedures relating to human resources matters.

Nominating and Corporate Governance Committee

Effective upon the completion of this offering, our nominating and corporate governance committee will consist of , and , with serving as chairman of the committee. Our board of directors has determined that each member of the nominating and corporate governance committee is "independent" as defined under the applicable listing standards of NASDAQ. The nominating and corporate governance committee's responsibilities upon completion of this offering will include:

- developing and recommending to the board of directors criteria for board and committee membership;
- establishing procedures for identifying and evaluating board of director candidates, including nominees recommended by stockholders;
- identifying individuals qualified to become members of the board of directors;
- recommending to the board of directors the persons to be nominated for election as directors and to each of the board's committees; and

- developing and recommending to the board of directors a set of corporate governance guidelines.

Our board of directors may establish other committees from time to time.

Leadership Structure and Risk Oversight

Our board of directors is currently chaired by Dr. Nadav. As a general policy, our board of directors believes that separation of the positions of chairman and chief executive officer reinforces the independence of the board of directors from management, creates an environment that encourages objective oversight of management's performance and enhances the effectiveness of the board of directors as a whole. As such, Dr. Kakkis serves as our president and chief executive officer while Dr. Nadav serves as our chairman of the board of directors but is not an officer.

Our board of directors oversees the management of risks inherent in the operation of our business and the implementation of our business strategies. Our board of directors performs this oversight role by using several different levels of review. In connection with its reviews of the operations and corporate functions of our company, our board of directors addresses the primary risks associated with those operations and corporate functions. In addition, our board of directors reviews the risks associated with our company's business strategies periodically throughout the year as part of its consideration of undertaking any such business strategies.

Each of our board committees also oversees the management of our company's risk that falls within the committee's areas of responsibility. In performing this function, each committee has full access to management, as well as the ability to engage advisors. Our chief financial officer reports to the audit committee and is responsible for identifying, evaluating, and implementing risk management controls and methodologies to address any identified risks. In connection with its risk management role, our audit committee meets privately with representatives from our independent registered public accounting firm. The audit committee oversees the operation of our risk management program, including the identification of the primary risks associated with our business and periodic updates to such risks, and reports to our board of directors regarding these activities.

Compensation Committee Interlocks and Insider Participation

None of the members of our compensation committee has at any time during the prior three years been one of our officers or employees. None of our executive officers currently serves, or in the past fiscal year has served, as a member of the board of directors or compensation committee of any entity that has one or more executive officers serving on our board of directors or compensation committee. For a description of transactions between us and members of our compensation committee and affiliates of such members, please see "Certain Relationships and Related Party Transactions."

Board Diversity

Effective upon the completion of this offering, our nominating and corporate governance committee will be responsible for reviewing with the board of directors, on an annual basis, the appropriate characteristics, skills and experience required for the board of directors as a whole and its individual members. Although we do not have a formal policy regarding board diversity, in evaluating the suitability of individual candidates (both new candidates and current members), the nominating and corporate governance committee, in recommending candidates for election, and the board of directors, in approving (and, in the case of vacancies, appointing) such candidates, will take into account many factors, including the following:

- personal and professional integrity;
- ethics and values;
- experience in corporate management, such as serving as an officer or former officer of a publicly held company;
- experience in the industries in which we compete;

- experience as a board member or executive officer of another publicly held company;
- diversity of expertise and experience in substantive matters pertaining to our business relative to other board members;
- conflicts of interest; and
- practical and mature business judgment.

Currently, our board of directors evaluates, and, following the completion of this offering will evaluate, each individual in the context of the board of directors as a whole, with the objective of assembling a group that can best maximize the success of the business and represent stockholder interests through the exercise of sound judgment using its diversity of experience in these various areas.

Code of Business Conduct and Ethics

We have adopted a code of business conduct and ethics that applies to all of our employees, officers, and directors, including those officers responsible for financial reporting. Upon the completion of this offering, our code of business conduct and ethics will be available on our website. We intend to disclose any amendments to the code, or any waivers of its requirements, on our website.

EXECUTIVE AND DIRECTOR COMPENSATION

The following is a summary of the compensation arrangements of our named executive officers. Actual compensation programs that we may adopt may differ materially from currently planned programs as summarized in this discussion. As an "emerging growth company" as defined in the JOBS Act, we are not required to include a Compensation Discussion and Analysis section and have elected to comply with the scaled disclosure requirements applicable to emerging growth companies.

2012 Summary Compensation Table

The following table sets forth the compensation earned during the year ended December 31, 2012 to our chief executive officer and our next two highest-paid executive officers as of December 31, 2012. We refer to these officers as our named executive officers.

Name and Principal Position	Year	Salary	Option Awards[1]	Nonequity Incentive Plan Compensation[2]	All Other Compensation	Total
Emil D. Kakkis, M.D., Ph.D. *President and Chief Executive Officer*	2012	$300,172	—	$ 92,125	$ 9,056[3]	$401,353
Thomas Kassberg *Chief Business Officer and Senior Vice President*	2012	$272,041	—	$ 81,813	$ 15,640[4]	$369,494
Shalini Sharp[5] *Chief Financial Officer and Senior Vice President*	2012	$148,669	$90,042	$ 51,560	$ 13,228[4]	$303,499

(1) The amounts reported in this column represent the grant date fair value of the stock options granted to our named executive officers during 2012 as computed in accordance with Accounting Standards Codification, or ASC, Topic 718, not including any estimates of forfeitures. The assumptions used in calculating the grant date fair value of the stock options reported in this column are set forth in Note 12 to our audited financial statements included elsewhere in this prospectus. Note that the amounts reported in this column reflect the accounting cost for these stock options, and do not correspond to the actual economic value that may be received by the named executive officers from the options.

(2) Amounts represent cash bonuses earned in 2012, and paid during 2013, based on achievement of performance goals and other factors deemed relevant by our board of directors.

(3) Amounts reported in this column consist of dental, vision and life/accidental death & dismemberment and key person life insurance premiums paid by us.

(4) Amounts reported in this column consist of medical, dental, vision and life/accidental death & dismemberment premiums paid by us.

(5) Ms. Sharp commenced employment with us in May 2012.

Narrative Disclosure to Summary Compensation Table

Employment Arrangements with Our Named Executive Officers

Emil D. Kakkis, M.D., Ph.D., President and Chief Executive Officer. We entered into an executive employment agreement with Dr. Kakkis in June 2011 for the position of President and Chief Executive Officer. Dr. Kakkis currently receives a base salary of $305,000, which is subject to adjustment at the discretion of the board of directors or the compensation committee. Dr. Kakkis is also eligible to participate in our employee benefit plans, subject to the terms of those plans. Pursuant to the terms of the executive employment agreement, the employment of Dr. Kakkis is at will; we may terminate his employment at any time, without advance notice,

for any reason or for no reason at all and Dr. Kakkis may terminate his employment at any time, upon four weeks' prior written notice, for any reason or for no reason at all.

Thomas Kassberg, Chief Business Officer and Senior Vice President. We entered into an offer letter in October 2011 with Thomas Kassberg for the position of Chief Business Officer and Senior Vice President. Mr. Kassberg currently receives a base salary of $288,750, which is subject to adjustment at the discretion of the board of directors or the compensation committee. Mr. Kassberg is also eligible for an annual performance bonus of up to 30% of his base salary, payable based on his individual performance evaluated against certain goals mutually agreed upon and our overall performance, as determined by the Chief Executive Officer in consultation with the board of directors. Additionally, pursuant to the terms of the offer letter, Mr. Kassberg received an option to purchase 635,000 shares of our common stock in connection with his hiring, as further detailed in the table entitled "Outstanding Equity Awards as of December 31, 2012". Mr. Kassberg is eligible to participate in our employee benefit plans, subject to the terms of those plans. Pursuant to the terms of the offer letter, Mr. Kassberg's employment is at will and may be terminated either by us or by him, with or without advance notice, for any reason or for no reason at all.

Shalini Sharp, Chief Financial Officer and Senior Vice President. We entered into an offer letter in March 2012 with Shalini Sharp for the position of Chief Financial Officer and Senior Vice President. Ms. Sharp currently receives a base salary of $278,299, which is subject to adjustment at the discretion of the board of directors or the compensation committee. Ms. Sharp is also eligible for an annual performance bonus of up to 30% of her base salary, payable based on her individual performance evaluated against certain goals mutually agreed upon and our overall performance, as determined by the Chief Executive Officer in consultation with the board of directors. Additionally, pursuant to the terms of the offer letter, Ms. Sharp received an option to purchase 600,000 shares of our common stock in connection with her hiring. Ms. Sharp is eligible to participate in our employee benefit plans, subject to the terms of those plans. Pursuant to the terms of the offer letter, Ms. Sharp's employment is at will and may be terminated either by us or by her, with or without advance notice, for any reason or for no reason at all.

Each of these employment arrangements also contain provisions that provide for certain payments and benefits in the event of an involuntary termination of employment. In addition, the named executive officers may be entitled to accelerated vesting of their outstanding and unvested awards in certain circumstances. The information below describes certain compensation that may become due and payable as a result of certain events.

Involuntary Termination of Employment

Pursuant to their employment arrangements, each named executive officer is eligible to receive certain payments and benefits in the event of certain qualifying terminations, including termination of his or her employment by us without "cause" (as defined below) or resignation of his or her employment with "good reason" or because of a "constructive termination" (each, as defined below). Upon the timely execution of a general release of claims, each named executive officer is eligible to receive the following payments and benefits:

- if Dr. Kakkis is terminated by us other than for cause or because of death or disability, he shall be entitled to receive six months of base salary continuation;
- if Dr. Kakkis resigns his employment with us for good reason following a "change in control" (as defined below) within six months of the event constituting good reason and after providing us with 20 days to cure the good reason, then he shall be entitled to receive 12 months of base salary continuation; and
- if Mr. Kassberg or Ms. Sharp is terminated by us without cause or resigns employment with us due to a constructive termination, each executive will be entitled to: (i) extend the exercise period applicable to any options then held such that the executive has 12 months from termination to exercise any of the vested shares, provided that in no event shall the exercise period be extended beyond the expiration date of any options then held; and (ii) six months of base salary continuation.

Deemed Liquidation Event

Pursuant to the offer letter with Mr. Kassberg, in addition to the severance benefits described above, in the event (i) we consummate a "deemed liquidation event" (as defined in our certificate of incorporation), which includes certain mergers or material asset sales, as well as any dissolution, liquidation, or winding down of the Company, (ii) Mr. Kassberg is employed by us on the date of the deemed liquidation event, and (iii) Mr. Kassberg is terminated by us without cause or resigns his employment with us due to a constructive termination within 12 months after the deemed liquidation event, the vesting of Mr. Kassberg's November 17, 2011 option to purchase 635,000 shares of our common stock shall accelerate with respect to 50% of the then-unvested shares subject to such option and any other equity held by Mr. Kassberg shall accelerate with respect to 100% of the then-unvested shares.

Pursuant to the offer letter with Ms. Sharp, in the event (i) we consummate a deemed liquidation event, (ii) Ms. Sharp is employed by us on the date of the deemed liquidation event, and (iii) Ms. Sharp is terminated without cause or resigns due to a constructive termination within 12 months of the deemed liquidation event, the vesting of all options held by Ms. Sharp as of the date of the deemed liquidation event shall accelerate with respect to 50% of the then-unvested shares.

Definitions

For purposes of Dr. Kakkis's employment agreement, "cause" means his:

- commission of a felony or any crime involving dishonesty, breach of trust, or physical harm to any person;
- willful engagement in conduct that is in bad faith and materially injurious to us, including but not limited to misappropriation of trade secrets, fraud, or embezzlement;
- material breach of his employment agreement that is not cured within 10 days after written notice to him from us; or
- willful refusal to implement or follow a lawful policy or directive of ours, which breach is not cured within 10 days after written notice to him from us.

For purposes of each of the offer letters with Mr. Kassberg and Ms. Sharp, "cause" means the named executive officer's:

- gross negligence in carrying out, or material failure to carry out, his or her duties for us (including, without limitation, failure to cooperate in any company investigation), after notice from the board of directors and a reasonable opportunity to cure (if deemed curable);
- breach of his or her fiduciary duties to us, after notice from the board of directors and a reasonable opportunity to cure (if deemed curable);
- conviction of, or plea of guilty or no contest to, any felony;
- any act of fraud or embezzlement with respect to his or her obligations to us or otherwise relating to our business;
- material violation of any of our policies;
- material breach of any agreement entered into with us; or
- unauthorized use or disclosure of confidential information or trade secrets of ours or of our affiliates.

For purposes of Dr. Kakkis's employment agreement, "good reason" means any of the following events if (i) we effect the event without the consent of Dr. Kakkis and (ii) such event occurs after a change in control:

- a change in his position with us that materially reduces his level of responsibility;

- a material reduction in his base salary, except for reductions that are comparable to reductions generally applicable to similarly situated executives of ours; or
- a relocation of his principal place of employment by more than 50 miles.

For purposes of Dr. Kakkis's employment agreement, "change in control" means a change in ownership or control of us effected through a merger, consolidation, or acquisition by any person or related group of persons (other than an acquisition by us or by an employee benefit plan sponsored by us or by a person or persons that directly or indirectly control, is controlled by, or is under common control with, us) of beneficial ownership of securities possessing more than 50% of the total combined voting power of our outstanding securities.

For purposes of each of the offer letters with Mr. Kassberg and Ms. Sharp, "constructive termination" means the occurrence of any of the following events without the named executive officer's consent if (i) the executive provides us with written objection (or notice) to the event or condition within 30 days following the occurrence of the event or condition, (ii) we do not reverse or otherwise cure the event within 30 days of receiving such written objection, and (iii) the executive resigns his or her employment with us within 30 days following the expiration of that cure period:

- a material reduction or change in the executive's job duties, responsibilities and requirements from the executive's job duties, responsibilities and requirements immediately prior to such reduction or change, taking into account the differences in job title and duties that are normally occasioned by reason of an acquisition of one company by another;
- a material reduction of the executive's base salary (other than an equal, across-the-board reduction in the compensation of all similarly-situated employees of ours or the surviving entity that is approved by the board of directors); or
- a requirement that the executive relocate to a principal office that increases his or her one-way commute by more than 50 miles relative to the executive's immediately preceding principal office.

Terms and Conditions of Annual Bonuses

Our board of directors has adopted a corporate bonus plan, or the bonus plan, which is effective as of the completion of this offering. The bonus plan provides for cash bonus payments based upon the attainment of performance targets established by our compensation committee. The payment targets will be related to corporate, financial, and operational measures or objectives, or corporate performance goals, as well as individual performance objectives.

Our compensation committee may select corporate performance goals from among the following: sales; revenue; assets; expenses; earnings from operations, earnings before or after deduction for all or any portion of interest, taxes, depreciation, amortization, incentives, service fees or extraordinary or special items, whether or not on a continuing operations or an aggregate or per share basis; net income or net income per common share (basic or diluted); return on equity, investment, capital or assets; one or more operating ratios; borrowing levels, leverage ratios or credit rating; market share; capital expenditures; cash flow, free cash flow, cash flow return on investment, or net cash provided by operations; stock price, dividends or total stockholder return; development of new technologies or products; sales of particular products or services; economic value created or added; operating margin or profit margin; customer acquisition or retention; raising or refinancing of capital; successful hiring of key individuals; resolution of significant litigation; acquisitions and divestitures (in whole or in part); joint ventures and strategic alliances; spin-offs, split-ups and the like; reorganizations; recapitalizations, restructurings, financings (issuance of debt or equity) or refinancings; or strategic business criteria, consisting of one or more objectives based on the following goals: meeting specified market penetration or value added, product development or introduction (including, without limitation, any clinical trial accomplishments, regulatory or other filings or approvals, or other product development milestones), geographic business expansion, cost targets, cost reductions or savings, customer satisfaction, operating efficiency, acquisition or retention, employee satisfaction, information technology, corporate development (including, without limitation, licenses, innovation, research or establishment of third party collaborations), manufacturing or process

development, legal compliance or risk reduction, patent application or issuance goals, or goals relating to acquisitions or divestitures (in whole or in part), joint ventures or strategic alliances, any of which may be measured in absolute terms, as compared to any incremental increase, in terms of growth, as compared to results of a peer group, against the market as a whole, compared to applicable market indices and/or measured on a pre-tax or post-tax basis.

Each executive officer who is selected to participate in the bonus plan will have a target bonus opportunity set for each performance period. The bonus formulas will be adopted in each performance period by the compensation committee and communicated to each executive. The corporate performance goals will be measured at the end of each performance period after our financial reports have been published or such other appropriate time as the compensation committee determines. If the corporate performance goals and individual performance objectives are met, payments will be made following the end of each performance period. Subject to the rights contained in any agreement between the executive officer and the company, an executive officer must be employed by the company on the bonus payment date to be eligible to receive a bonus payment. The bonus plan also permits the compensation committee to approve additional bonuses in its sole discretion.

Equity Compensation

Outstanding Equity Awards at December 31, 2012

The following table sets forth information concerning the outstanding equity awards held by each of the named executive officers as of December 31, 2012.

	Option awards			
Name	Number of securities underlying unexercised options (#) exercisable	Number of securities underlying unexercised options (#) unexercisable	Option exercise price ($)	Option expiration date
Emil D. Kakkis, M.D., Ph.D.	—	—	—	—
Thomas Kassberg[1]	13,229	463,021	0.10	11/16/2021
Shalini Sharp[2]	—	600,000	0.26	8/1/2022

(1) Represents an option to purchase 635,000 shares of our common stock granted on November 17, 2011. The shares underlying this option vest as follows: 25% vest on November 15, 2012, with the remainder of the shares vesting in equal monthly installments over the following three years through November 15, 2015, subject to the holder's continued service to us through each such vesting date. Vesting of 50% of the unvested shares shall accelerate in connection with a deemed liquidation event pursuant to the terms of Mr. Kassberg's offer letter dated October 31, 2011, as more fully described above under the section entitled "—Narrative Disclosure to Summary Compensation Table—Deemed Liquidation Event." Mr. Kassberg exercised 158,750 options on November 28, 2012.

(2) Represents an option to purchase 600,000 shares of our common stock granted on August 2, 2012. The shares underlying this option vest as follows: 25% vest on May 21, 2013, with the remainder of the shares vesting in equal monthly installments over the following three years through May 21, 2016, subject to the holder's continued service to us through each such vesting date. Vesting of 50% of the unvested shares shall accelerate in connection with a deemed liquidation event pursuant to the terms of Ms. Sharp's offer letter dated March 12, 2012, as more fully described above under the section entitled "—Narrative Disclosure to Summary Compensation Table—Deemed Liquidation Event."

Director Compensation

Dr. Kakkis, our president and chief executive officer, receives no compensation for his service as a director. None of our non-employee directors received compensation for their service on the board or otherwise during fiscal 2012.

Our board of directors has adopted a non-employee director compensation policy, effective as of the closing of this offering, that is designed to provide a total compensation package that enables us to attract and retain, on a long-term basis, high caliber non-employee directors. Under the policy, all non-employee directors will be paid cash compensation from and after the completion of this offering, as set forth below:

	Annual Retainer
Board of Directors:	
All non-employee members	$
Additional retainer for Non-Executive Chairman of the Board	$
Audit Committee:	
Chairman	$
Non-Chairman members	$
Compensation Committee:	
Chairman	$
Non-Chairman members	$
Nominating and Corporate Governance Committee:	
Chairman	$
Non-Chairman members	$

Under the non-employee director compensation policy, each person who is initially appointed or elected to the board of directors will be eligible for an option grant to purchase up to shares of our common stock under our stock option plan on the date he or she first becomes a non-employee director, which will vest annually over a -year period, subject to the holder's continued service to us through each such vesting date. In addition, on the date of the annual meeting of stockholders, each continuing non-employee director who has served on the board of directors for a minimum of will be eligible to receive an annual option grant to purchase up to shares of our common stock, which will vest in full upon the earlier of the first anniversary of the date of grant or the date of the following annual meeting of stockholders, subject to the holder's continued service to us through each such vesting date. All of the foregoing options will be granted at fair market value on the date of grant.

Compensation Risk Assessment

We believe that our executive compensation program does not encourage excessive or unnecessary risk taking. This is primarily due to the fact that our compensation programs are designed to encourage our executive officers and other employees to remain focused on both short-term and long-term strategic goals, in particular in connection with our pay-for-performance compensation philosophy. As a result, we do not believe that our compensation programs are reasonably likely to have a material adverse effect on us.

Equity Compensation Plans and Other Benefit Plans

2013 Incentive Plan

Prior to the completion of this offering, our board of directors intends to adopt the Ultragenyx Pharmaceutical Inc. 2013 Incentive Plan, or the 2013 Plan, and, following this offering, all equity-based awards will be granted under the 2013 Plan. As of the date of this prospectus, no awards have been made under the 2013 Plan. The following summary describes what we anticipate to be the material terms of the 2013 Plan. This summary of the 2013 Plan is not a complete description of all provisions of the 2013 Plan and is qualified in its entirety by reference to the 2013 Plan, which is filed as an exhibit to the registration statement of which this prospectus is a part.

Purpose. The purpose of the 2013 Plan is to advance the company's interests by providing for the grant to participants of equity and other incentive awards.

Plan Administration. The 2013 Plan is administered by our compensation committee. Our compensation committee has the authority to, among other things, interpret the 2013 Plan, determine eligibility for, grant and determine the terms of awards under the 2013 Plan, and to do all things necessary to carry out the purposes of the 2013 Plan. Our compensation committee's determinations under the 2013 Plan are conclusive and binding.

Authorized Shares. Subject to adjustment, the maximum number of shares of our common stock that may be delivered in satisfaction of awards under the 2013 Plan is , which includes the shares of our common stock that are available for grant under the 2011 Plan (defined and described below) on the date the 2013 Plan is adopted, and any shares that become available under the 2011 Plan as a result of the termination, cancellation or forfeiture of awards under the 2011 Plan. The number of shares of our common stock available for issuance under the 2013 Plan will be automatically increased on January 1 of each year, beginning January 1, 2015 through January 1, 2023, by an amount equal to the least of (i) shares of common stock, (ii) % of the number of shares of common stock outstanding on a fully diluted basis as of the close of business on the immediately preceding day (calculated by adding to the number of shares of common stock outstanding, all outstanding securities convertible into common stock on such date on an as converted basis), and (iii) a lesser amount determined by the compensation committee on or prior to January 1 of a given year.

Shares of our common stock to be issued under the 2013 Plan may be authorized but unissued shares of our common stock or previously issued shares acquired by us. Any shares of our common stock underlying awards that are settled in cash or otherwise expire, terminate, or are forfeited prior to the issuance of stock will again be available for issuance under the 2013 Plan.

Individual Limits. The maximum number of shares of our common stock subject to stock options and the maximum number of shares of our common stock subject to stock appreciation rights that may be granted to any person in any calendar year is each shares. The maximum number of shares of our common stock subject to other awards that may be granted to any person in any calendar year is shares. The maximum amount payable to any person in any twelve-month period under cash awards will be $.

Eligibility. Our compensation committee will select participants from among our key employees, directors, consultants and advisors and of our affiliates who are in a position to contribute significantly to the success of the company and its affiliates. Eligibility for options intended to be incentive stock options, or ISOs, is limited to employees of the company or certain affiliates.

Types of Awards. The 2013 Plan provides for grants of stock options, stock appreciation rights, restricted and unrestricted stock and stock units, performance awards, cash awards and other awards convertible into or otherwise based on shares of our common stock. Dividend equivalents may also be provided in connection with an award under the 2013 Plan.

- Stock options. A stock option is an award that entitles the participant to receive stock upon payment of the exercise price. The exercise price of an option may not be less than the fair market value (or, in the case of an ISO granted to a ten percent shareholder, 110% of the fair market value) of a share of our common stock on the date of grant. Our compensation committee will determine the time or times at which stock options become exercisable and the terms on which they remain exercisable.
- Stock appreciation rights. A stock appreciation right is an award that entitles the participant to receive stock or cash upon exercise equal to the excess of the value of the shares subject to the right over the base price. The base price of a stock appreciation right may not be less than the fair market value of a share of our common stock on the date of grant. Our compensation committee will determine the time or times at which stock appreciation rights become exercisable and the terms on which they remain exercisable.
- Restricted and unrestricted stock. A restricted stock award is an award of common stock subject to forfeiture restrictions, while an unrestricted stock award is not subject to restrictions.
- Stock units. A stock unit award is denominated in shares of our common stock and entitles the participant to receive stock or cash measured by the value of the shares in the future. The delivery of stock or cash under a stock unit may be subject to the satisfaction of performance conditions or other vesting conditions.

- Performance awards. A performance award is an award the vesting, settlement or exercisability of which is subject to specified performance criteria.
- Cash awards: An award that is settled in cash.

Vesting. Our compensation committee has the authority to determine the vesting schedule applicable to each award, and to accelerate the vesting or exercisability of any award.

Termination of Employment. Our compensation committee will determine the effect of termination of employment or service on an award. Unless otherwise provided by our compensation committee or in an award agreement, upon a termination of a participant's employment all unvested options then held by the participant and other awards requiring exercise will terminate and all other unvested awards will be forfeited. Unless otherwise provided for by our compensation committee, all vested stock options and stock appreciation rights then held by the participant will remain outstanding for three months, or one year in the case of death, or, in each case, until the applicable expiration date, if earlier. All stock options and stock appreciation rights held by a participant immediately prior to the participant's termination of employment will immediately terminate upon termination of employment if the termination is for cause as defined in the 2013 Plan or occurs in circumstances that would have constituted grounds for the participant's employment to be terminated for cause, in the determination of the Administrator.

Performance Criteria. The 2013 Plan provides for the grant of performance awards that are made based upon, and subject to achieving, "performance objectives." Performance objectives with respect to those awards that are intended to qualify as "performance-based compensation" for purposes of Section 162(m) of the Code, or Section 162(m) are limited to an objectively determinable measure or measures of performance relating to any or any combination of the following (measured either absolutely or by reference to an index or indices and determined either on a consolidated basis or, as the context permits, on a divisional, subsidiary, line of business, project or geographical basis or in combinations thereof): sales; revenue; assets; expenses; earnings from operations, earnings before or after deduction for all or any portion of interest, taxes, depreciation, amortization, incentives, service fees or extraordinary or special items, whether or not on a continuing operations or an aggregate or per share basis; net income or net income per common share (basic or diluted); return on equity, investment, capital or assets; one or more operating ratios; borrowing levels, leverage ratios or credit rating; market share; capital expenditures; cash flow, free cash flow, cash flow return on investment, or net cash provided by operations; stock price, dividends or total stockholder return; development of new technologies or products; sales of particular products or services; economic value created or added; operating margin or profit margin; customer acquisition or retention; raising or refinancing of capital; successful hiring of key individuals; resolution of significant litigation; acquisitions and divestitures (in whole or in part); joint ventures and strategic alliances; spin-offs, split-ups and the like; reorganizations; recapitalizations, restructurings, financings (issuance of debt or equity) or refinancings; or strategic business criteria, consisting of one or more objectives based on the following goals: meeting specified market penetration or value added, product development or introduction (including, without limitation, any clinical trial accomplishments, regulatory or other filings or approvals, or other product development milestones), geographic business expansion, cost targets, cost reductions or savings, customer satisfaction, operating efficiency, acquisition or retention, employee satisfaction, information technology, corporate development (including, without limitation, licenses, innovation, research or establishment of third party collaborations), manufacturing or process development, legal compliance or risk reduction, patent application or issuance goals, or goals relating to acquisitions or divestitures (in whole or in part), joint ventures or strategic alliances.

To the extent consistent with the requirements for satisfying the performance-based compensation exception under Section 162(m), our compensation committee may provide in the case of any award intended to qualify for such exception that one or more of the performance objectives applicable to an award will be adjusted in an objectively determinable manner to reflect events (for example, acquisitions or dispositions) occurring during the performance period of such award that affect the applicable performance objectives.

Transferability. Awards under the 2013 Plan may not be transferred except by will or by the laws of descent and distribution, unless (for awards other than ISOs) otherwise provided by our compensation committee.

Recovery of Compensation; Other Terms. Awards granted under the 2013 Plan are subject to forfeiture, termination and rescission, and a participant will be obligated to return to the company the value received with respect to awards, to the extent provided by our compensation committee in an award agreement, pursuant to Company policy relating to the recovery of erroneously-paid incentive compensation, or as otherwise required by law or applicable stock exchange listing standards.

Covered Transactions. In the event of a consolidation, merger or similar transaction, a sale or transfer of all or substantially all of our assets or our dissolution or liquidation, our compensation committee may, among other things, provide for continuation or assumption of outstanding awards, for new grants in substitution of outstanding awards, for the accelerated vesting or delivery of shares under awards or for a cash-out of outstanding awards, in each case on such terms and with such restrictions as it deems appropriate. Except as our compensation committee may otherwise determine, awards not assumed or continued will automatically terminate and in the case of outstanding shares of restricted stock, will automatically be forfeited upon the consummation of such covered transaction.

Adjustment. In the event of a stock dividend, stock split or combination of shares including a reverse stock split, recapitalization or other change in our capital structure that constitutes an equity restructuring within the meaning of the Financial Accounting Standards Board, Accounting Standards Codification Topic 718, *Compensation — Stock Compensation*, our compensation committee will make appropriate adjustments to the maximum number of shares that may be delivered under, and the individual share limits included in, the 2013 Plan, and will also make appropriate adjustments to the number and kind of shares of stock or securities subject to awards, the exercise prices of such awards or any other terms of awards affected by such change. Our compensation committee will also make the types of adjustments described above to take into account distributions and other events other than those listed above if it determines that such adjustments are appropriate to avoid distortion and preserve the value of awards.

Amendment and Termination. Our compensation committee will be able to amend the 2013 Plan or outstanding awards, or terminate the 2013 Plan as to future grants of awards, except that our compensation committee will not be able to alter the terms of an award if it would affect materially and adversely a participant's rights under the award without the participant's consent (unless expressly provided in the 2013 Plan or the right to alter the terms of an award was expressly reserved by our compensation committee at the time the award was granted). Stockholder approval will be required for any amendment to the 2013 Plan to the extent such approval is required by law, including the Code or applicable stock exchange requirements.

Employee Stock Purchase Plan

Prior to the completion of this offering, our board of directors intends to adopt the Ultragenyx Pharmaceutical Inc. 2013 Employee Stock Purchase Plan, or the 2013 ESPP, as a means of permitting our eligible employees, including our named executive officers, to acquire shares of our common stock. shares of our common stock will be available for issuance under the 2013 ESPP. The number of shares of our common stock available for issuance under the 2013 ESPP will be automatically increased on January 1 of each year, beginning January 1, 2015 through January 1, 2023, by an amount equal to the least of (i) shares of common stock, (ii) % of the number of shares of common stock outstanding on a fully diluted basis as of the close of business on the immediately preceding day (calculated by adding to the number of shares of common stock outstanding, all outstanding securities convertible into common stock on such date on an as converted basis), and (iii) a lesser amount determined by the compensation committee on or prior to January 1 of a given year. Under the 2013 ESPP, eligible employees of the company may purchase shares of our common stock during pre-specified purchase periods at a price equal to the lesser of 85% of the fair market value of a share of our common stock at the beginning of the purchase period or 85% of the fair market value of a share of our common stock at the end of the purchase period. As of the date of this prospectus, our board of directors has not determined the date on which the initial purchase period will commence under the 2013 ESPP, however the initial purchase period will not commence prior to the completion of this offering.

2011 Equity Incentive Plan, as amended

The Ultragenyx Pharmaceutical Inc. 2011 Equity Incentive Plan, which became effective June 14, 2011 and was amended on December 18, 2012 (as so amended, the "2011 Plan"), provides for the grant of equity-based awards to participants selected by our board of directors. The following summary of the 2011 Plan is not a complete description of all provisions of the 2011 Plan and is qualified in its entirety by reference to the 2011 Plan, which is filed as an exhibit to the registration statement of which this prospectus is a part. Following this offering, all equity-based awards will be granted under the company's 2013 Plan described above.

Purpose. The purpose of the 2011 Plan is to promote the success and enhance the value of the company by linking the personal interests of the members of the board of directors, employees and consultants of the company and our affiliates to those of the company stockholders and by providing such individuals with an incentive for performance to generate returns to stockholders. The 2011 Plan is also intended to provide the company flexibility in our ability to motivate, attract, and retain the services of such individuals.

Plan Administration. The 2011 Plan is administered by our board of directors, which has authority to determine eligibility for and grant awards and to determine the terms and conditions of all awards, including the time or times upon which awards vest or become exercisable and remain exercisable, and make all other decisions and determinations necessary or advisable to administer the 2011 Plan. The 2011 Plan administrator's determinations under the 2011 Plan are conclusive and binding.

Authorized Shares. Subject to adjustment, the aggregate number of shares of our common stock that may be delivered in satisfaction of awards under the 2011 Plan is 11,774,817 shares. As of September 30, 2013, options to purchase a total of 4,785,720 shares of common stock were issued and outstanding under the 2011 Plan, a total of 2,082,613 shares of common stock had been issued upon the exercise of options granted under the 2011 Plan and 4,906,484 shares remained available for future grants under the 2011 Plan. Shares of our common stock to be issued under the 2011 Plan may be authorized but unissued shares of our common stock, our treasury stock or, after the completion of this offering, our common stock purchased on the open market. The payment of dividend equivalents in cash will not be counted against the shares available for issuance under the 2011 Plan. Any shares of our common stock underlying awards that expire, terminate, are forfeited or repurchased by us prior to the issuance of stock, or that are withheld in payment of the exercise price of an award or in satisfaction of tax withholding will again be available for issuance under the 2011 Plan.

Eligibility. The 2011 Plan Administrator selects participants from among our key employees, directors, and consultants and of our affiliates. Eligibility for options intended to be incentive stock options, or ISOs, is limited to employees of the company or certain affiliates.

Types of Awards. The 2011 Plan provides for grants of stock options, restricted stock and stock units, stock appreciation rights and stock payments. Dividend equivalents may also be provided in connection with an award under the 2011 Plan. Under the 2011 Plan only stock options have been granted.

- Stock options. A stock option is an award that entitles the participant to receive stock upon payment of the exercise price. The exercise price of an option may not be less than the fair market value (or, in the case of an ISO granted to a ten percent shareholder, 110% of the fair market value) of a share of our common stock on the date of grant. The 2011 Plan administrator determines the time or times at which stock options become exercisable and the terms on which they remain exercisable. The 2011 Plan administrator may permit a participant to exercise an option prior to the full vesting of the award, provided that the shares acquired upon exercise of such option may be subject to forfeiture, transfer or other restrictions imposed by the 2011 Plan administrator.
- Restricted stock. A restricted stock award is an award of common stock subject to repurchase, forfeiture and transferability restrictions imposed by the 2011 Plan administrator. Unless the 2011 Plan administrator determines otherwise, upon the termination of a participant's service for any reason, restricted stock shall be forfeited or subject to repurchase by the company.

Transferability. Awards under the 2011 Plan may not be transferred except by will or by the laws of descent and distribution, unless (for awards other than ISOs) the 2011 Plan administrator permits awards to be transferred to, exercised by and paid to a permitted transferee as defined in the 2011 Plan, subject to certain terms and conditions as described in detail in the 2011 Plan.

Corporate Transactions. In the event of any combination or exchange of shares, merger, consolidation, distribution of assets to stockholders (other than normal cash dividends) or similar corporate transaction affecting out stock or the share price of our stock, the 2011 Plan administrator will make proportionate adjustments to the aggregate number and type of shares issuable under the 2011 Plan, the terms and conditions of outstanding, and the grant or exercise price per share of any outstanding awards. In addition, the 2011 Plan administrator may provide for the cash out of existing awards, the assumption or substitution of outstanding awards, an adjustment to the terms and conditions of outstanding awards including the number and type of shares subject to such awards, the acceleration of vesting conditions or the cancellation of outstanding awards.

Change in Control. Unless an award agreement provides otherwise, in the event of a change in control (as defined in the 2011 Plan) if awards are not continued, assumed, or replaced, each award will become fully exercisable and/or payable, as applicable, and all restrictions shall lapse immediately prior to such change in control. Any awards that are continued, assumed, or replaced in connection with the change of control shall become fully exercisable and/or payable, as applicable, and all restrictions shall lapse in the event the participant is terminated in connection with or within 12 months following the change in control, unless such termination is with cause or without good reason, each as defined in the 2011 Plan.

Adjustment. In the event of a stock dividend, stock split, spin-off, rights offering or recapitalization that affects the shares of stock or the share price of stock, the 2011 Plan administrator will make proportionate, nondiscretionary adjustments to the maximum number of shares that may be issued under the 2011 Plan, the number of shares of stock or securities subject to awards, the exercise prices of such awards or any other terms of awards affected by such change.

Amendment and Termination. Our Board may terminate, amend or modify the 2011 Plan; stockholder approval will be required for any amendment to the 2011 Plan to the extent necessary to comply with any applicable law, regulation or stock exchange rule. The 2011 Plan administrator has the authority to cancel any or all outstanding wards under the 2011 Plan with the consent of the affected award holders, and to grant substitute awards. No termination, amendment or modification of the 2011 Plan may adversely affect in any material way any outstanding awards without the prior written consent of the participant. Unless terminated sooner by the 2011 Plan administrator or extended with stockholder approval, the 2011 Plan will terminate on June 14, 2021. No awards may be granted under our 2011 Plan after it is terminated.

401(k) Plan

We maintain a 401(k) plan for employees. The 401(k) plan is intended to qualify under Section 401(k) of the Code, so that contributions to the 401(k) plan by employees or by us, and the investment earnings thereon, are not taxable to the employees until withdrawn from the 401(k) plan, and so that contributions by us, if any, will be deductible by us when made. Under the 401(k) plan, employees may elect to reduce their current compensation by up to the statutorily prescribed annual limit and to have the amount of such reduction contributed to the 401(k) plan. The 401(k) plan permits us to make contributions up to the limits allowed by law on behalf of all eligible employees. Historically, we have not made any matching contributions to the 401(k) plan.

CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS

The following is a description of transactions since January 1, 2010 to which we have been a party, in which the amount involved exceeds $120,000, and in which any of our directors, executive officers or holders of more than 5% of our capital stock, or an affiliate or immediate family member thereof, had or will have a direct or indirect material interest. We believe the terms obtained or consideration that we paid or received, as applicable, in connection with the transactions described below were comparable to terms available or the amounts that would be paid or received, as applicable, from unaffiliated third parties.

Sales and Purchases of Securities

Series A Convertible Preferred Stock Financing

On June 16, 2011, we sold an aggregate of 18,052,464 shares of our Series A convertible preferred stock to eight investors at a purchase price of $1.034 per share, for an aggregate purchase price of approximately $15.0 million in cash and $3.7 million in converted bridge notes. On July 16, 2012, we sold, pursuant to a second tranche closing, an aggregate of 14,604,895 shares of our Series A convertible preferred stock to six investors at a purchase price of $1.034 per share, for an aggregate purchase price of $15.1 million in cash. The table below sets forth the aggregate number of shares of Series A convertible preferred stock sold to our directors, executive officers or holders of more than 5% of our capital stock, or an immediate family member thereof, as applicable:

Name of Stockholder	Number of Shares of Series A Convertible Preferred Stock	Total Purchase Price
Emil D. Kakkis, M.D., Ph.D.	1,814,944	$ 1,876,471
William Aliski	247,049	$ 255,424

Convertible Notes and Series A Convertible Preferred Stock Warrants

On June 30, 2010, we entered into a Note and Warrant Purchase Agreement with Emil D. Kakkis, M.D., Ph.D. Pursuant to the Note and Warrant Purchase Agreement, we issued a convertible promissory note in the amount of $1.0 million to Dr. Kakkis and also issued him a warrant to purchase up to 241,803 shares of our Series A convertible preferred stock. Emil D. Kakkis, M.D., Ph.D. is our President and Chief Executive Officer and one of our directors.

On February 23, 2011, we entered into a Note and Warrant Purchase Agreement with William Aliski. Pursuant to the Note and Warrant Purchase Agreement, we issued a convertible promissory note in the amount of $250,000 to Mr. Aliski and also issued him a warrant to purchase up to 84,631 shares of our Series A convertible preferred stock. William Aliski is one of our directors.

On June 14, 2011, we entered into a Note and Warrant Purchase Agreement with Emil D. Kakkis, M.D., Ph.D. Pursuant to the Note and Warrant Purchase Agreement, we issued a convertible promissory note in the amount of $300,000 to Dr. Kakkis and also issued him a warrant to purchase up to 72,541 shares of our Series A convertible preferred stock.

On June 14, 2011, we entered into a second Note and Warrant Purchase Agreement with Emil D. Kakkis, M.D., Ph.D. Pursuant to the Note and Warrant Purchase Agreement, we issued a convertible promissory note in the amount of $500,000 to Dr. Kakkis and also issued him a warrant to purchase up to 120,901 shares of our Series A convertible preferred stock.

Series B Convertible Preferred Stock Financing

On December 18, 2012, we sold an aggregate of 27,081,680 shares of our Series B convertible preferred stock to 34 investors at a purchase price of $2.769 per share, for an aggregate purchase price of approximately $75 million in cash. The table below sets forth the aggregate number of shares of Series B convertible preferred stock sold to our directors, executive officers or holders of more than 5% of our capital stock, or an immediate family member thereof, as applicable:

Name of Stockholder	Number of Shares of Series B Convertible Preferred Stock	Total Purchase Price
TPG Biotechnology Partners III, L.P.	541,634	$ 1,500,001
Beacon Bioventures Fund II Limited Partnership	541,634	$ 1,500,001
HealthCap VI L.P.	481,452	$ 1,333,333
Entities affiliated with A.M. Pappas Life Science Ventures IV, L.P.	240,727	$ 666,669

Indemnification Agreements and Directors' and Officers' Liability Insurance

We intend to enter into indemnification agreements with each of our executive officers and directors prior to the completion of this offering. We also maintain an insurance policy that covers certain liabilities of directors and officers of our Company arising out of claims based on acts or omissions in their capacities as directors or officers.

Registration Rights Agreement

We and certain holders of our preferred stock have entered into an investor rights agreement pursuant to which these stockholders will have, among other things, registration rights under the Securities Act of 1933, as amended, with respect to common stock that they will hold following this offering. Upon the completion of this offering, all outstanding shares of our preferred stock will be converted into common stock. See "Description of Capital Stock — Registration Rights" for a further description of the terms of these agreements.

Procedures for Related Party Transactions

We have adopted a related person transaction approval policy that will govern the review of related person transactions following the closing of this offering. Pursuant to this policy, if we want to enter into a transaction with a related person or an affiliate of a related person, our chief financial officer will review the proposed transaction to determine, based on applicable NASDAQ and SEC rules, if such transaction qualifies as a related person transaction. If the chief financial officer determines that the proposed transaction is a related person transaction, then the proposed transaction shall be submitted to the audit committee for pre-approval at the next regular or special audit committee meeting; if the chief financial officer, in consultation with the chief executive officer, determines that it is not practicable to wait until the next meeting of the audit committee, then the chief financial officer may submit the proposed transaction to the chairperson of the audit committee. In the event that our chief executive officer or chief financial officer becomes aware of a related person transaction that has not been previously approved or previously ratified under our related person transaction approval policy, the transaction, if ongoing, will be promptly submitted to the audit committee or the chairperson of the audit committee for consideration. If the transaction is already completed, the audit committee or the chairperson of the audit committee shall evaluate the transaction to determine if rescission of the transaction and/or any disciplinary action is appropriate.

PRINCIPAL STOCKHOLDERS

The following table sets forth information relating to the beneficial ownership of our common stock as of November 1, 2013, by:

- each person, or group of affiliated persons, known by us to beneficially own more than 5% of our outstanding shares of common stock;
- each of our directors and our nominee for director;
- each of our named executive officers; and
- all directors and executive officers as a group.

The number of shares beneficially owned by each entity, person, director or executive officer is determined in accordance with the rules of the SEC, and the information is not necessarily indicative of beneficial ownership for any other purpose. Under such rules, beneficial ownership includes any shares over which the individual has sole or shared voting power or investment power as well as any shares that the individual has the right to acquire within 60 days of November 1, 2013 through the exercise of any stock option, warrants or other rights. Except as otherwise indicated, and subject to applicable community property laws, the persons named in the table have sole voting and investment power with respect to all shares of common stock held by that person.

The percentage of shares beneficially owned is computed on the basis of 73,167,287 shares of our common stock outstanding as of November 1, 2013, which reflects the assumed conversion of all of our outstanding shares of preferred stock into an aggregate of 61,431,574 shares of common stock. Shares of our common stock that a person has the right to acquire within 60 days of November 1, 2013 are deemed outstanding for purposes of computing the percentage ownership of the person holding such rights, but are not deemed outstanding for purposes of computing the percentage ownership of any other person, except with respect to the percentage ownership of all directors and executive officers as a group. Unless otherwise indicated below, the address for each beneficial owner listed is c/o Ultragenyx Pharmaceutical Inc., at 60 Leveroni Court, Novato, California 94949.

Name and Address of Beneficial Owner	Number of Shares Beneficially Owned	Percentage of Shares Beneficially Owned	
		Before Offering	After Offering
5% or Greater Stockholders:			
TPG Biotechnology Partners III, L.P.(1)	9,670,697	13.2%	
Beacon Bioventures Fund II Limited Partnership(2)	9,670,697	13.2%	
HealthCap VI, L.P.(3)	8,596,174	11.8%	
Adage Capital Partners, L.P.(4)	5,416,335	7.4%	
Funds managed by Capital Research Global Investors(5)	4,694,158	6.4%	
Entities affiliated with A.M. Pappas Life Science Ventures IV, L.P.(6)	4,298,087	5.9%	
Directors (including our Director Nominee) and Named Executive Officers:			
Eran Nadav, Ph.D.	—	—	
Benjamin Auspitz	—	—	
Mårten Steen, M.D., Ph.D.	—	—	
William Aliski(7)	184,631	*	
Matthew Fust(8)	—	—	
Emil D. Kakkis, M.D., Ph.D.(9)	10,391,931	14.1%	
Thomas Kassberg(10)	429,324	*	
Shalini Sharp(11)	237,500	*	
All executive officers and directors as a group(12) (7 persons)	11,243,386	15.2%	

* Indicates beneficial ownership of less than 1% of the total outstanding common stock.

(1) Consists of (a) 9,129,063 shares of common stock issuable upon conversion of shares of Series A convertible preferred stock and (b) 541,634 shares of common stock issuable upon conversion of shares of

Series B convertible preferred stock. TPG Biotechnology Partners III, L.P. is a Delaware limited partnership, whose general partner is TPG Biotechnology GenPar III, L.P., a Delaware limited partnership, whose general partner is TPG Biotechnology GenPar III Advisors, LLC, a Delaware limited liability company, whose sole member is TPG Holdings I, L.P., a Delaware limited partnership, whose general partner is TPG Holdings I-A, LLC, a Delaware limited liability company, whose sole member is TPG Group Holdings (SBS), L.P., a Delaware limited partnership, whose general partner is TPG Group Holdings (SBS) Advisors, Inc., or Group Advisors, a Delaware corporation. Messrs. David Bonderman and James G. Coulter are officers, directors and sole shareholders of Group Advisors and may therefore be deemed to be the beneficial owners of the shares held by TPG Biotechnology Partners III, L.P. The address for Messrs. Bonderman and Coulter is c/o TPG Capital, L.P., 301 Commerce Street, Suite 3300, Fort Worth, TX 76102.

(2) Consists of (a) 9,129,063 shares of common stock issuable upon conversion of shares of Series A convertible preferred stock and (b) 541,634 shares of common stock issuable upon conversion of shares of Series B convertible preferred stock. Beacon Bioventures Advisors Fund II Limited Partnership is the general partner of Beacon Bioventures Fund II Limited Partnership. Beacon Bioventures Advisors Fund II Limited Partnership is solely managed by Northern Neck Investors LLC, its general partner and investment manager. Northern Neck Investors LLC is owned by the shareholders and certain employees of FMR LLC. Northern Neck Investors LLC is managed on a day-to-day basis by its President, Paul L. Mucci, and as such Mr. Mucci may be deemed to share voting and dispositive power with respect to all shares held by Beacon Bioventures Fund II Limited Partnership. Each of the individuals and entities listed above expressly disclaims beneficial ownership of the securities listed above except to the extent of any pecuniary interest therein. The address for each of the individuals and entities listed above is 100 Summer Street R7B, Boston, Massachusetts 02110.

(3) Consists of (a) 8,114,722 shares of common stock issuable upon conversion of shares of Series A convertible preferred stock and (b) 481,452 shares of common stock issuable upon conversion of shares of Series B convertible preferred stock. HealthCap VI GP SA, L.L.C. (“HCSA”) is the sole general partner of HealthCap VI, L.P. HCSA has voting and dispositive power over the shares held by HealthCap VI, L.P. HCSA disclaims beneficial ownership of such shares, except to the extent of its pecuniary interest therein. Francois Kaiser, Dag Richter, and Daniel Schafer, the members of the board of HCSA, share voting and dispositive power over the shares held by HealthCap VI, L.P. and may be deemed to have indirect beneficial ownership of the shares held by such entities. The members of the board of HCSA disclaim beneficial ownership of shares held by HealthCap VI, L.P. except to the extent of any pecuniary interest therein. The address of HealthCap VI, L.P. is c/o HealthCap VI GP SA, 18, Avenue d Ouchy, 1006 Lausanne, Switzerland.

(4) Consists of 5,416,335 shares of common stock issuable upon conversion of shares of Series B convertible preferred stock. Adage Capital Partners, GP, LLC (“ACPGP”), serves as the general partner of Adage Capital Partners, L.P., a Delaware limited partnership (the “Fund”) and as such has discretion over the portfolio of securities beneficially owned by the Fund. Adage Capital Advisors, LLC, a Delaware limited liability company (“ACA”), is managing member of ACPGP and directs ACPGP’s operations. Robert Atchinson and Phillip Gross are the managing members of ACPGP and ACA and general partners of the Fund. Robert Atchinson and Phillip Gross disclaim beneficial ownership of the reported securities except to the extent of their pecuniary interest therein. The address of Adage Capital Partners, L.P. is 200 Clarendon Street, 52nd Floor, Boston, MA 02116.

(5) Consists of (a) 2,789,614 shares of common stock issuable upon conversion of shares of Series B convertible preferred stock held by SMALLCAP World Fund, Inc. (“SCWF”) and (b) 1,904,544 shares of common stock issuable upon conversion of shares of Series B convertible preferred stock held by American Funds Insurance Series — Global Small Capitalization Fund (“VISC”). SCWF and VISC are investment companies registered under the Investment Company Act of 1940, as amended. Capital Research and Management Company (“CRMC”), an investment adviser registered under the Investment Advisers Act of 1940, as amended, is the investment adviser to SCWF and VISC. CRMC provides investment advisory services to SCWF and VISC through its division Capital Research Global Investors (“CRGI”). In that capacity, CRGI may be deemed to be the beneficial owner of the shares of Series B convertible preferred

stock held by SCWF and VISC. CRGI, however, disclaims such beneficial ownership. CRMC has advised that Julian Abdey, Mark E. Denning, Brady L. Enright, J. Blair Frank, Bradford F. Freer, Claudia P. Huntington, Jonathan Knowles, Lawrence Kymisis, Harold H. La, Andraz Razan and Gregory Wendt, as portfolio counselors for SCWF, are primarily responsible for the portfolio management of SCWF, and, as such, have dispositive authority over the shares. CRMC has advised that Mark E. Denning, J. Blair Frank, Claudia P. Huntington, and Harold H. La, as portfolio counselors for VISC, are primarily responsible for the portfolio management of VISC, and, as such, have dispositive authority over the shares. The address for Capital Research and Management Company is 333 S. Hope Street, 55th Floor, Los Angeles, CA 90071.

(6) Consists of (a) 3,873,017 shares of common stock issuable upon conversion of shares of Series A convertible preferred stock held by A.M. Pappas Life Science Ventures IV, L.P., (b) 229,790 shares of common stock issuable upon conversion of shares of Series B convertible preferred stock held by A.M. Pappas Life Science Ventures IV, L.P., (c) 184,343 shares of common stock issuable upon conversion of shares of Series A convertible preferred stock held by PV IV CEO Fund, L.P. and (d) 10,937 shares of common stock issuable upon conversion of shares of Series B convertible preferred stock held by PV IV CEO Fund, L.P. AMP&A Management IV, LLC is the general partner of each of A.M. Pappas Life Science Ventures IV, L.P. and PV IV CEO Fund, L.P. (collectively, the “Pappas Funds”), and AMP&A Management IV, LLC has a management agreement with A.M. Pappas & Associates, LLC whereby A.M. Pappas & Associates, LLC provides management services for the Pappas Funds. As a result, A.M. Pappas & Associates, LLC’s investment committee exercises sole dispositive and voting power over the shares owned by the Pappas Funds. The address for each of A.M. Pappas Life Science Ventures IV, L.P. and PV IV CEO Fund, L.P. is c/o Pappas Ventures, 2520 Meridian Parkway, Suite 400, Durham, NC 27713.

(7) Consists of (a) 100,000 shares of common stock and (b) 84,631 shares of common stock issuable upon conversion of Series A convertible preferred stock that may be acquired pursuant to the exercise of a warrant held by Mr. Aliski.

(8) We expect that Mr. Fust will become a member of our board of directors upon the completion of this offering.

(9) Consists of (a) 8,000,000 shares of common stock held by the Emil Kakkis and Jenny Soriano Living Trust, dated June 18, 2009, (b) 1,956,686 shares of common stock issuable upon conversion of shares of Series A convertible preferred stock held by Dr. Kakkis and (c) 435,245 shares of common stock issuable upon conversion of Series A convertible preferred stock that may be acquired pursuant to the exercise of a warrant held by Dr. Kakkis. Dr. Kakkis shares voting and dispositive power over the 8,000,000 shares of common stock held by the Emil Kakkis and Jenny Soriano Living Trust, dated June 18, 2009; each of Dr. Kakkis and Ms. Soriano is a trustee of such trust. Dr. Kakkis has sole voting and dispositive power over the 1,956,686 shares of common stock issuable upon conversion of shares of Series A convertible preferred stock and the 435,245 shares of common stock issuable upon conversion of Series A convertible preferred stock that may be acquired pursuant to the exercise of a warrant held by Dr. Kakkis.

(10) Consists of (a) 291,040 shares of common stock, (b) 98,595 shares of common stock issuable upon conversion of Series A convertible preferred stock and (c) 39,689 shares of common stock issuable upon the exercise of stock options within 60 days of November 1, 2013.

(11) Consists of (a) 200,000 shares of common stock and (b) 37,500 shares of common stock issuable upon the exercise of stock options within 60 days of November 1, 2013.

(12) Consists of (a) 10,646,321 shares held by our directors and officers and entities affiliated with certain of our directors, (b) 519,876 shares of common stock issuable upon the conversion of shares of preferred stock that may be acquired pursuant to the exercise of warrants by our certain of our directors and officers, and (c) 77,189 shares of common stock issuable upon the exercise of stock options within 60 days of November 1, 2013 held by our officers.

DESCRIPTION OF CAPITAL STOCK

The following summary describes our capital stock and the material provisions of our amended and restated certificate of incorporation and our amended and restated bylaws, which will become effective immediately prior to the completion of this offering, warrants to purchase shares of our Series A convertible preferred stock, the amended and restated investor rights agreement to which we and certain of our stockholders are parties, and of the Delaware General Corporation Law. Because the following is only a summary, it does not contain all of the information that may be important to you. For a complete description, you should refer to our amended and restated certificate of incorporation, amended and restated bylaws, warrants to purchase shares of our Series A convertible preferred stock, and amended and restated investor rights agreement, copies of which have been filed as exhibits to the registration statement of which this prospectus is part.

General

Upon completion of this offering, our authorized capital stock will consist of shares of common stock, par value $0.001 per share, and shares of preferred stock, par value $0.001 per share. The following description of our capital stock is intended as a summary only and is qualified in its entirety by reference to our amended and restated certificate of incorporation, and amended and restated bylaws, each to be in effect at the closing of this offering, which are filed as exhibits to the registration statement, of which this prospectus forms a part, and to the applicable provisions of the Delaware General Corporation Law.

Common Stock

As of September 30, 2013, there were 73,038,747 shares of our common stock outstanding, held of record by 56 stockholders, which assumes the conversion of all outstanding shares of preferred stock for shares of our common stock. Based on shares outstanding as of September 30, 2013, upon completion of this offering, there will be shares of our common stock outstanding.

Holders of our common stock are entitled to one vote for each share of common stock held of record for the election of directors and on all matters submitted to a vote of stockholders. Holders of our common stock are entitled to receive dividends ratably, if any, as may be declared by our board of directors out of legally available funds, subject to any preferential dividend rights of any preferred stock then outstanding. Upon our dissolution, liquidation or winding up, holders of our common stock are entitled to share ratably in our net assets legally available after the payment of all our debts and other liabilities, subject to the preferential rights of any preferred stock then outstanding. Holders of our common stock have no preemptive, subscription, redemption or conversion rights. The rights, preferences and privileges of holders of common stock are subject to, and may be adversely affected by, the rights of the holders of shares of any series of preferred stock that we may designate and issue in the future. Except as described below in “Anti-Takeover Effects of Delaware Law, Our Certificate of Incorporation and Our By-laws,” the affirmative vote of a majority of our outstanding shares of capital stock is generally required to take action under our Certificate of Incorporation and By-laws.

Preferred Stock

Upon completion of this offering, our board of directors will be authorized, without action by the stockholders, to designate and issue up to an aggregate of shares of preferred stock in one or more series. The board of directors can fix the rights, preferences and privileges of the shares of each series and any of its qualifications, limitations or restrictions. Our board of directors may authorize the issuance of preferred stock with voting or conversion rights that could adversely affect the voting power or other rights of the holders of common stock. The issuance of preferred stock, while providing flexibility in connection with possible future financings and acquisitions and other corporate purposes could, under certain circumstances, have the effect of delaying or preventing a change in control of our company and might harm the market price of our common stock.

Our board of directors will make any determination to issue such shares based on its judgment as to our best interests and the best interests of our stockholders. We have no current plans to issue any shares of preferred stock.

Certain of our stockholders hold, as of the date of this prospectus, 34,349,894 shares of our Series A convertible preferred stock and 27,081,680 shares of our Series B convertible preferred stock. Upon completion of this offering, each share of Series A and Series B convertible preferred stock outstanding will be converted into shares of our common stock on a -for-1 basis. Holders of substantially all of the shares of our preferred stock are subject to lock-up agreements with the underwriters that restrict the sale of our securities for 180 days following the date of this prospectus. See "Underwriting" for a description of these lock-up agreements.

Warrants

As of September 30, 2013, warrants to purchase a total of 1,027,662 shares of our Series A convertible preferred stock were outstanding with an exercise price of $1.034 per share. These warrants to purchase 1,027,662 Series A convertible preferred stock, which will be converted into warrants to purchase shares of common stock with an exercise price of $ per share upon completion of this offering, are exercisable immediately and each expire on the first to occur of (i) the closing date of any reorganization, consolidation or merger of the Company, transfer of all or substantially all of the assets of the Company or any simultaneous sale of more than a majority of the then outstanding securities of the Company other than a mere reincorporation transaction, or (ii) the 10 year anniversary of the date of such warrant which in the case of the outstanding warrants of the Company is June 2020, February 2021 and June 2021, respectively.

Registration Rights

We are party to an amended and restated investors' rights agreement, dated as of December 18, 2012, with the holders of shares of our common stock issuable upon conversion of the shares of preferred stock. Following this offering, the holders of approximately 61.4 million shares, or 62.5 million including the shares underlying outstanding warrants, will have the right to require us to register their shares under the Securities Act of 1933, as amended. These shares will represent approximately % of our outstanding common stock after this offering, or % if the underwriters exercise their option to purchase additional shares in full. These shares also may be sold under Rule 144 under the Securities Act of 1933, depending on their holding period and subject to restrictions in the case of shares held by persons deemed to be our affiliates. The registration rights will terminate upon the later of (i) the fifth anniversary of the closing date of this offering and (ii) as to any holder of registrable securities, such earlier time after this offering at which such holder holds 1% or less of our common stock and all registrable securities held by such holder can be sold in any 90-day period without registration in compliance with Rule 144.

Demand Registration Rights

Under the amended and restated investors' rights agreement, beginning 180 days following the effectiveness of the registration statement of which this prospectus forms a part or 45 days following the effective date of any other Company-initiated registration statement, the holders of at least 50% of the registrable shares (or a lesser percentage if the anticipated aggregate offering price is not less than $10 million) then outstanding can, on not more than two occasions, demand that we file a registration statement or request that their shares be included on a registration statement that we are otherwise filing, in either case, registering the resale of their shares of common stock. We are required to use our best efforts to effect the registration and will pay all registration expenses, other than underwriting discounts and commissions, related to any demand registration. These registration rights are subject to conditions and limitations, including the right, in certain circumstances, of the underwriters of an offering to limit the number of shares included in such registration and our right, in certain circumstances, not to effect a requested registration.

Piggyback Registration Rights

If we propose to register any of our securities under the Securities Act for our own account or the account of any other holder, the "significant holders" (as defined in the amended and restated investors' rights agreement) are entitled to notice of such registration and to request that we include registrable shares for resale on such registration statement, subject to the right of any underwriter to limit the number of shares included in such registration.

We will pay all registration expenses, other than underwriting discounts and commissions, related to any piggyback registration. The amended and restated investors' rights agreement contains customary cross-indemnification provisions, pursuant to which we are obligated to indemnify the selling stockholders, in the event of misstatements or omissions in the registration statement attributable to us and they are obligated to indemnify us for misstatements or omissions attributable to them.

Form S-3 Registration Rights

The holders of at least 10% of the registrable securities outstanding can make a written request that we register their shares on Form S-3 if we are eligible to file a registration statement on Form S-3 and if the aggregate price to the public of the shares offered is at least $1 million. These stockholders may make an unlimited number of requests for registration on Form S-3. However, we will not be required to effect a registration on Form S-3 if we determine that such a registration would be seriously detrimental to us and our stockholders or if we have already effected three registration statements on Form S-3 in the 12-month period preceding the date of the request. We will pay all registration expenses, other than underwriting discounts and commissions, related to any S-3 registration.

Voting Agreement, Right of First Refusal and Co-Sale Agreement and Market Stand-Off Provision

We are party to an amended and restated voting agreement and an amended and restated right of first refusal and co-sale agreement, each dated as of December 18, 2012, with all holders of our preferred stock and certain holders of our common stock. These agreements provide for certain rights and obligations, such as rights to designate and covenants with regard to voting for board members and stock transfer restrictions. These agreements will terminate upon the completion of this offering. The market stand-off provision under our amended and restated investors' rights agreement shall survive the completion of this offering. See "Shares Eligible for Future Sales—Lock-Up Agreements."

Anti-Takeover Effects of Delaware Law, our Certificate of Incorporation and Our By-laws

Our certificate of incorporation and by-laws include a number of provisions that may have the effect of encouraging persons considering unsolicited tender offers or other unilateral takeover proposals to negotiate with our board of directors rather than pursue non-negotiated takeover attempts. These provisions include the items described below.

Board Composition and Filling Vacancies

In accordance with our certificate of incorporation, our board is divided into three classes serving three-year terms, with one class being elected each year. Our certificate of incorporation also provides that directors may be removed only for cause. Furthermore, any vacancy on our board of directors, however occurring, including a vacancy resulting from an increase in the size of our board, may only be filled by a resolution of the board.

No Written Consent of Stockholders

Our certificate of incorporation provides that all stockholder actions are required to be taken by a vote of the stockholders at an annual or special meeting, and that stockholders may not take any action by written consent in lieu of a meeting.

Meetings of Stockholders

Our certificate of incorporation and by-laws provide that, subject to any rights of holders of any series of preferred stock, only the board or the chairman of the board may call special meetings of stockholders and only those matters set forth in the notice of the special meeting may be considered or acted upon at a special meeting of stockholders. Our by-laws limit the business that may be conducted at an annual meeting of stockholders to those matters properly brought before the meeting.

Advance Notice Requirements

Our by-laws establish advance notice procedures with regard to stockholder proposals relating to the nomination of candidates for election as directors or new business to be brought before meetings of our stockholders. These procedures provide that notice of stockholder proposals must be timely given in writing to our corporate secretary prior to the meeting at which the action is to be taken. Generally, to be timely, notice must be received at our principal executive offices not less than 90 days or more than 120 days prior to the first anniversary date of the annual meeting for the preceding year. The notice must contain certain information specified in the by-laws. These provisions may have the effect of precluding the conduct of certain business at a meeting if the proper procedures are not followed. These provisions may also discourage or deter a potential acquirer from conducting a solicitation of proxies to elect the acquirer's own slate of directors or otherwise attempting to obtain control of our company.

Amendment to By-laws and Certificate of Incorporation

As required by the Delaware General Corporation Law, any amendment of our certificate of incorporation must first be approved by a majority of our board of directors and, if required by law or our certificate of incorporation, thereafter be approved by a majority of the outstanding shares entitled to vote on the amendment, and a majority of the outstanding shares of each class entitled to vote thereon as a class, except that the amendment of the provisions relating to directors, stockholders, the amendment of our by-laws and certificate of incorporation and exclusive jurisdiction of Delaware Courts must be approved by not less than 75% of the outstanding shares entitled to vote on the amendment, and not less than 75% of the outstanding shares of each class entitled to vote thereon as a class. Our by-laws may be amended by the affirmative vote of a majority of the directors then in office, subject to any limitations set forth in the by-laws, and may also be amended by the affirmative vote of at least 75% of the outstanding shares entitled to vote on the amendment.

Blank Check Preferred Stock

Our certificate of incorporation provides for authorized shares of preferred stock. The existence of authorized but unissued shares of preferred stock may enable our board of directors to render more difficult or to discourage an attempt to obtain control of us by means of a merger, tender offer, proxy contest or otherwise. For example, if in the due exercise of its fiduciary obligations, our board of directors were to determine that a takeover proposal is not in the best interests of us or our stockholders, our board of directors could cause shares of preferred stock to be issued without stockholder approval in one or more private offerings or other transactions that might dilute the voting or other rights of the proposed acquirer or insurgent stockholder or stockholder group. In this regard, our certificate of incorporation grants our board of directors broad power to establish the rights and preferences of authorized and unissued shares of preferred stock. The issuance of shares of preferred stock could decrease the amount of earnings and assets available for distribution to holders of shares of common stock. The issuance may also adversely affect the rights and powers, including voting rights, of these holders and may have the effect of delaying, deterring or preventing a change in control of us.

Section 203 of the Delaware General Corporation Law

Upon completion of this offering, we will be subject to the provisions of Section 203 of the Delaware General Corporation Law. In general, Section 203 prohibits a publicly-held Delaware corporation from engaging

in a "business combination" with an "interested stockholder" for a three-year period following the time that this stockholder becomes an interested stockholder, unless the business combination is approved in a prescribed manner. A "business combination" includes, among other things, a merger, asset or stock sale or other transaction resulting in a financial benefit to the interested stockholder. An "interested stockholder" is a person who, together with affiliates and associates, owns, or did own within three years prior to the determination of interested stockholder status, 15% or more of the corporation's voting stock.

Under Section 203, a business combination between a corporation and an interested stockholder is prohibited unless it satisfies one of the following conditions:

- before the stockholder became interested, the board of directors approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;
- upon consummation of the transaction which resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding, shares owned by persons who are directors and also officers, and employee stock plans, in some instances; or
- at or after the time the stockholder became interested, the business combination was approved by the board of directors of the corporation and authorized at an annual or special meeting of the stockholders by the affirmative vote of at least two-thirds of the outstanding voting stock which is not owned by the interested stockholder.

A Delaware corporation may "opt out" of these provisions with an express provision in its original certificate of incorporation or an express provision in its certificate of incorporation or by-laws resulting from a stockholders' amendment approved by at least a majority of the outstanding voting shares. We have not opted out of these provisions. As a result, mergers or other takeover or change in control attempts of us may be discouraged or prevented.

Exclusive Jurisdiction of Certain Actions

Our certificate of incorporation requires, to the fullest extent permitted by law, that derivative actions brought in our name, actions against our directors, officers and employees for breach of fiduciary duty and other similar actions may be brought only in the Court of Chancery in the State of Delaware, unless we otherwise consent. Although we believe this provision benefits us by providing increased consistency in the application of Delaware law in the types of lawsuits to which it applies, the provision may have the effect of discouraging lawsuits against our directors and officers. Although our certificate of incorporation contains the choice of forum provision described above, it is possible that a court could rule that such a provision is inapplicable for a particular claim or action or that such provision is unenforceable.

NASDAQ Global Market listing

We have applied for listing of our common stock on The NASDAQ Global Market under the symbol "RARE".

Transfer Agent and Registrar

The transfer agent and registrar for our common stock is American Stock Transfer & Trust Company, LLC. The transfer agent and registrar's address is 620 15th Avenue, Brooklyn, New York 11219.

SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for our common stock. Future sales of our common stock, including shares issued upon the exercise of outstanding options or warrants, in the public market after this offering, or the perception that those sales may occur, could cause the prevailing market price for our common stock to fall or impair our ability to raise equity capital in the future. As described below, only a limited number of shares of our common stock will be available for sale in the public market for a period of several months after completion of this offering due to contractual and legal restrictions on resale described below. Future sales of our common stock in the public market either before (to the extent permitted) or after restrictions lapse, or the perception that those sales may occur, could adversely affect the prevailing market price of our common stock at such time and our ability to raise equity capital at a time and price we deem appropriate.

Sale of Restricted Shares

As of September 30, 2013, based on the number of shares of our common stock then outstanding, upon the closing of this offering, and assuming (1) the conversion of our outstanding preferred stock into shares of our common stock, (2) no exercise of the underwriters' option to purchase additional shares of common stock and (3) no exercise of outstanding options or warrants, we would have had outstanding an aggregate of approximately shares of common stock. Of these shares, all of the shares of common stock to be sold in this offering, and any shares sold upon exercise of the underwriters' option to purchase additional shares will be freely tradable in the public market without restriction or further registration under the Securities Act of 1933, as amended, or the Securities Act, unless the shares are held by any of our "affiliates" as such term is defined in Rule 144 of the Securities Act. All remaining shares of common stock held by existing stockholders immediately prior to the completion of this offering will be "restricted securities" as such term is defined in Rule 144. These restricted securities were issued and sold by us in private transactions and are eligible for public sale only if registered under the Securities Act or if they qualify for an exemption from registration under the Securities Act, including the exemptions provided by Rule 144 or Rule 701, which rules are summarized below.

As a result of the lock-up agreements referred to below and the provisions of Rule 144 and Rule 701 under the Securities Act, the shares of our common stock (excluding the shares sold in this offering) that will be available for sale in the public market are as follows:

Approximate Number of Shares	First Date Available for Sale into Public Market
	180 days after the date of this prospectus upon expiration of the lock-up agreements referred to below, subject in some cases to applicable volume limitations under Rule 144

Lock-Up Agreements and Market Stand-Off Provision

In connection with this offering, we, our directors, our executive officers, substantially all of our stockholders, and substantially all of our option holders who are not also stockholders have agreed, subject to certain exceptions, with the underwriters not to dispose of or hedge any shares of our common stock or securities convertible into or exchangeable for shares of common stock during the period from the date of the lock-up agreement continuing through the date 180 days after the date of this prospectus, except with the prior written consent of J.P. Morgan Securities LLC and Morgan Stanley & Co. LLC, together the representatives of the underwriters. The representatives of the underwriters have advised us that they have no current intent or arrangement to release any of the shares subject to the lock-up agreements prior to the expiration of the lock-up period.

Following the lock-up periods set forth in the agreements described above, and assuming that the representatives of the underwriters do not release any parties from these agreements, all of the shares of our common stock that are restricted securities or are held by our affiliates as of the date of this prospectus will be eligible for sale in the public market in compliance with Rule 144 under the Securities Act.

In addition, pursuant to our amended and restated investors' rights agreement, the parties thereto have agreed under a market stand-off provision that, subject to certain conditions, they will not, directly or indirectly, without the prior written consent of the Company and the managing underwriter, during the same 180-day restricted period referred to above, (i) lend, offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of our common stock or any securities convertible into or exercisable or exchangeable for common stock or (ii) enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of common stock.

As of the date of this prospectus, holders of approximately million shares of common stock (including shares of our preferred stock that will be converted into shares of our common stock upon completion of this offering), or % of our outstanding shares of common stock on an as-converted basis, are, collectively subject to lock-up restrictions as parties to these agreements or lock-up agreements with the underwriters.

Rule 144

In general, under Rule 144, as currently in effect, once we have been subject to the public company reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act, for at least 90 days, a person (or persons whose shares are required to be aggregated) who is not deemed to have been one of our "affiliates" for purposes of Rule 144 at any time during the three months preceding a sale, and who has beneficially owned restricted securities within the meaning of Rule 144 for at least six months, including the holding period of any prior owner other than one of our "affiliates," is entitled to sell those shares in the public market (subject to the lock-up agreement referred to above, if applicable) without complying with the manner of sale, volume limitations or notice provisions of Rule 144, but subject to compliance with the public information requirements of Rule 144. If such a person has beneficially owned the shares proposed to be sold for at least one year, including the holding period of any prior owner other than "affiliates," then such person is entitled to sell such shares in the public market without complying with any of the requirements of Rule 144 (subject to the lock-up agreement referred to above, if applicable).

In general, under Rule 144, as currently in effect, once we have been subject to the public company reporting requirements of the Exchange Act for at least 90 days, our "affiliates," as defined in Rule 144, who have beneficially owned the shares proposed to be sold for at least six months are entitled to sell in the public market, upon expiration of any applicable lock-up agreements and within any three-month period, a number of those shares of our common stock that does not exceed the greater of:

- one percent of the number of common shares then outstanding, which will equal approximately million shares of common stock immediately after this offering (calculated on the basis of the number of shares of our common stock outstanding as of September 30, 2013, the assumptions described above and assuming no exercise of the underwriter's option to purchase additional shares and no exercise of outstanding options or warrants); or
- the average weekly trading volume of our common stock on The NASDAQ Global Market during the four calendar weeks preceding the filing of a notice on Form 144 with respect to such sale.

Such sales under Rule 144 by our "affiliates" or persons selling shares on behalf of our "affiliates" are also subject to certain manner of sale provisions, notice requirements and to the availability of current public information about us.

Notwithstanding the availability of Rule 144, the holders of substantially all of our restricted securities have entered into lock-up agreements as referenced above and their restricted securities will become eligible for sale (subject to the above limitations under Rule 144) upon the expiration of the restrictions set forth in those agreements.

Rule 701

In general, under Rule 701 as currently in effect, any of our employees, directors, officers, consultants or advisors who acquired common stock from us in connection with a written compensatory stock or option plan or other written agreement in compliance with Rule 701 under the Securities Act before the effective date of the registration statement of which this prospectus is a part (to the extent such common stock is not subject to a lock-up agreement) is entitled to rely on Rule 701 to resell such shares beginning 90 days after we become subject to the public company reporting requirements of the Exchange Act in reliance on Rule 144, but without compliance with the holding period requirements contained in Rule 144. Accordingly, subject to any applicable lock-up agreements, beginning 90 days after we become subject to the public company reporting requirements of the Exchange Act, under Rule 701 persons who are not our "affiliates," as defined in Rule 144, may resell those shares without complying with the minimum holding period or public information requirements of Rule 144, and persons who are our "affiliates" may resell those shares without compliance with Rule 144's minimum holding period requirements (subject to the terms of the lock-up agreement referred to below, if applicable).

Equity Incentive Plans and Employee Stock Purchase Plan

We intend to file with the SEC a registration statement under the Securities Act covering the shares of common stock that we may issue (i) upon exercise of outstanding options under the 2011 Equity Incentive Plan and reserved for issuance under the 2013 Incentive Plan, and (ii) pursuant to the 2013 Employee Stock Purchase Plan. Such registration statement is expected to be filed and become effective as soon as practicable after the completion of this offering. Accordingly, shares registered under such registration statement will be available for sale in the open market following its effective date, subject to Rule 144 volume limitations and the lock-up agreements described above, if applicable.

MATERIAL U.S. FEDERAL INCOME TAX CONSEQUENCES TO NON-U.S. HOLDERS

The following discussion is a summary of the material U.S. federal income tax consequences to non-U.S. holders (as defined below) of the purchase, ownership and disposition of our common stock issued pursuant to this offering, but does not purport to be a complete analysis of all potential tax effects. The effects of other U.S. federal tax laws, such as estate and gift tax laws, and any applicable state, local or foreign tax laws are not discussed. This discussion is based on the Internal Revenue Code of 1986, as amended, or the Code, Treasury Regulations promulgated under the Code, judicial decisions, and published rulings and administrative pronouncements of the U.S. Internal Revenue Service, or IRS, in effect as of the date of this offering. These authorities may change or be subject to differing interpretations. Any such change may be applied retroactively in a manner that could adversely affect a non-U.S. holder of our common stock. We have not sought and will not seek any rulings from the IRS regarding the matters discussed below. There can be no assurance the IRS or a court will not take a contrary position regarding the tax consequences of the purchase, ownership and disposition of our common stock.

This discussion is limited to non-U.S. holders that hold our common stock as a "capital asset" within the meaning of Section 1221 of the Code (property held for investment). This discussion does not address all U.S. federal income tax consequences relevant to a non-U.S. holder's particular circumstances. In addition, it does not address consequences relevant to non-U.S. holders subject to particular rules, including, without limitation:

- U.S. expatriates and certain former citizens or long-term residents of the United States;
- persons subject to the alternative minimum tax;
- persons holding our common stock as part of a hedge, straddle or other risk reduction strategy or as part of a conversion transaction or other integrated investment;
- banks, insurance companies and other financial institutions;
- real estate investment trusts or regulated investment companies;
- brokers, dealers or traders in securities;
- "controlled foreign corporations," "passive foreign investment companies" and corporations that accumulate earnings to avoid U.S. federal income tax;
- S corporations, partnerships or other entities or arrangements treated as partnerships for U.S. federal income tax purposes;
- tax-exempt organizations or governmental organizations;
- persons deemed to sell our common stock under the constructive sale provisions of the Code;
- persons who hold or receive our common stock pursuant to the exercise of any employee stock option or otherwise as compensation; and
- tax-qualified retirement plans.

If a partnership (or other entity treated as a partnership for U.S. federal income tax purposes) holds our common stock, the tax treatment of a partner in the partnership will depend on the status of the partner, the activities of the partnership and certain determinations made at the partner level. Accordingly, partnerships holding our common stock and the partners in such partnerships should consult their tax advisors regarding the U.S. federal income tax consequences to them.

THIS DISCUSSION IS FOR INFORMATION PURPOSES ONLY AND IS NOT INTENDED AS TAX ADVICE. INVESTORS SHOULD CONSULT THEIR TAX ADVISORS WITH RESPECT TO THE APPLICATION OF THE U.S. FEDERAL INCOME TAX LAWS TO THEIR PARTICULAR SITUATIONS AS WELL AS ANY TAX CONSEQUENCES OF THE PURCHASE, OWNERSHIP AND DISPOSITION OF OUR COMMON STOCK ARISING UNDER THE U.S. FEDERAL ESTATE OR GIFT TAX LAWS OR UNDER THE LAWS OF ANY STATE, LOCAL OR NON-U.S. TAXING JURISDICTION OR UNDER ANY APPLICABLE INCOME TAX TREATY.

Definition of a Non-U.S. Holder

For purposes of this discussion, a "non-U.S. holder" is any beneficial owner of our common stock that is neither a "U.S. person" nor a partnership for United States federal income tax purposes. A U.S. person is any of the following:

- an individual who is a citizen or resident of the United States;
- a corporation (or other entity taxable as a corporation for U.S. federal income tax purposes) created or organized under the laws of the United States, any state thereof, or the District of Columbia;
- an estate, the income of which is subject to U.S. federal income tax regardless of its source; or
- a trust that (1) is subject to the primary supervision of a U.S. court and the control of one or more United States persons (within the meaning of Section 7701(a)(30) of the Code), or (2) has a valid election in effect to be treated as a United States person for U.S. federal income tax purposes.

Distributions

As described in the section entitled "Dividend Policy," we do not anticipate declaring or paying dividends to holders of our common stock in the foreseeable future. However, if we do make distributions on our common stock, such distributions of cash or property on our common stock will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. Amounts not treated as dividends for U.S. federal income tax purposes will constitute a return of capital and first be applied against and reduce a non-U.S. holder's adjusted tax basis in its common stock, but not below zero. Any excess will be treated as capital gain and will be treated as described below in the section titled "— Sale or Other Taxable Disposition."

Dividends paid to a non-U.S. holder of our common stock generally will be subject to U.S. federal withholding tax at a rate of 30% of the gross amount of the dividends (or such lower rate specified by an applicable income tax treaty). Even if a non-U.S. holder is eligible for a lower treaty rate, dividend payments will generally be subject to withholding at a 30% rate (rather than the lower treaty rate) unless the non-U.S. holder provides a valid IRS Form W-8BEN (or applicable successor form) certifying such holder's qualification for the reduced rate.

Non-U.S. holders who do not timely provide us or our paying agent with the required certification, but who qualify for a reduced treaty rate, may obtain a refund of any excess amounts withheld by timely filing an appropriate claim for refund with the IRS. Non-U.S. holders should consult their tax advisors regarding their entitlement to benefits under a tax treaty.

Subject to the discussions below regarding backup withholding and foreign accounts, if dividends paid to a non-U.S. holder are effectively connected with the non-U.S. holder's conduct of a trade or business within the United States (and, if required by an applicable income tax treaty, the non-U.S. holder maintains a permanent establishment in the United States to which such dividends are attributable), the non-U.S. holder will be exempt from U.S. federal withholding tax. To claim the exemption, the non-U.S. holder must furnish to us or our paying agent a valid IRS Form W-8ECI (or applicable successor form), certifying that the dividends are effectively connected with the non-U.S. holder's conduct of a trade or business within the United States.

Any dividends paid on our common stock that are effectively connected with a non-U.S. holder's U.S. trade or business (and, if required by an applicable tax treaty, attributable to a permanent establishment maintained by the non-U.S. holder in the United States) generally will be subject to U.S. federal income tax on a net income basis in the same manner as if such holder were a U.S. person. A non-U.S. holder that is a corporation also may be subject to a branch profits tax at a rate of 30% (or such lower rate specified by an applicable tax treaty) of a portion of its effectively connected earnings and profits for the taxable year. Non-U.S. holders should consult their tax advisors regarding any applicable tax treaties that may provide for different rules.

Sale or Other Taxable Disposition

Subject to the discussions below regarding backup withholding and foreign accounts, a non-U.S. holder will not be subject to U.S. federal income tax on any gain realized upon the sale or other disposition of our common stock unless:

- the gain is effectively connected with the non-U.S. holder's conduct of a trade or business within the United States (and, if required by an applicable income tax treaty, the non-U.S. holder maintains a permanent establishment in the United States to which such gain is attributable);
- the non-U.S. holder is a nonresident alien individual present in the United States for 183 days or more during the taxable year of the disposition and certain other requirements are met; or
- our common stock constitutes a U.S. real property interest, or USRPI, by reason of our status as a U.S. real property holding corporation, or a USRPHC, for U.S. federal income tax purposes.

Gain described in the first bullet point above will generally be subject to U.S. federal income tax on a net income basis at the regular graduated U.S. federal income tax rates. A non-U.S. holder that is a foreign corporation also may be subject to a branch profits tax at a rate of 30% (or such lower rate specified by an applicable income tax treaty) on such effectively connected gain, as adjusted for certain items.

A non-U.S. holder described in the second bullet point above will be subject to U.S. federal income tax at a rate of 30% (or such lower rate specified by an applicable income tax treaty) on any gain derived from the disposition, which may be offset by certain U.S. source capital losses of the non-U.S. holder (even though the individual is not considered a resident of the United States) provided the non-U.S. holder has timely filed U.S. federal income tax returns with respect to such losses.

With respect to the third bullet point above, we believe we are not currently and do not anticipate becoming a USRPHC. However, because the determination of whether we are a USRPHC depends on the fair market value of our USRPIs relative to the fair market value of our other business assets and our non-U.S. real property interests, there can be no assurance we are not a USRPHC or will not become one in the future. Even if we are or were to become a USRPHC, gain arising from the sale or other taxable disposition by a non-U.S. holder of our common stock will not be subject to U.S. federal income tax if our common stock is "regularly traded," as defined by applicable Treasury Regulations, on an established securities market, and such non-U.S. holder owned, actually or constructively, 5% or less of our common stock throughout the shorter of the five-year period ending on the date of the sale or other disposition or the non-U.S. holder's holding period.

Non-U.S. holders should consult their tax advisors regarding potentially applicable income tax treaties that may provide for different rules.

Information Reporting and Backup Withholding

A non-U.S. holder will not be subject to backup withholding with respect to payments of dividends on our common stock we make to the non-U.S. holder, provided the holder certifies its non-U.S. status, such as by providing a valid IRS Form W-8BEN or W-8ECI, or otherwise establishes an exemption. However, information returns will be filed with the IRS in connection with any dividends on our common stock paid to the non-U.S. holder, regardless of whether any tax was actually withheld. Copies of these information returns may also be made available under the provisions of a specific treaty or agreement to the tax authorities of the country in which the non-U.S. holder resides or is established.

Information reporting and, depending on the circumstances, backup withholding will apply to the proceeds of a sale of our common stock within the United States or conducted through certain U.S.-related financial intermediaries, unless the beneficial owner certifies under penalty of perjury that it is a non-U.S. holder on Form W-8BEN or other applicable form or such owner otherwise establishes an exemption.

Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules may be allowed as a refund or a credit against a non-U.S. holder's U.S. federal income tax liability, provided the required information is timely furnished to the IRS.

Additional Withholding Tax on Payments Made to Foreign Accounts

Legislation incorporating provisions referred to as the Foreign Account Tax Compliance Act, or "FATCA," was enacted March 18, 2010. A 30% withholding tax may be imposed on dividends paid on, and the gross proceeds from the sale or other disposition of, our common stock paid to a "foreign financial institution" (as defined in the Code) or to a "non-financial foreign entity" (as defined in the Code) (whether such foreign financial institution or non-financial foreign entity is the beneficial owner or an intermediary), unless (1) the foreign financial institution undertakes certain diligence and reporting obligations, (2) the non-financial foreign entity either certifies it does not have any "substantial United States owners" (as defined in the Code) or furnishes identifying information regarding each substantial United States owner, or (3) the foreign financial institution or non-financial foreign entity otherwise qualifies for an exemption from these rules. If the payee is a foreign financial institution and is subject to the diligence and reporting requirements in (1) above, it must enter into an agreement with the U.S. Department of the Treasury requiring, among other things, that it undertake to identify accounts held by certain U.S. persons or U.S.-owned foreign entities (as defined in applicable Treasury Regulations), annually report certain information about such accounts, and withhold 30% on payments to non-compliant foreign financial institutions and certain other account holders. Foreign governments may enter into an agreement with the IRS to implement FATCA in a different manner. Under current IRS guidance, FATCA withholding will apply to payments of dividends on our common stock made on or after July 1, 2014, and to payments of gross proceeds from the sale or other disposition of such stock on or after January 1, 2017. Prospective investors should consult their tax advisors regarding these withholding provisions.

UNDERWRITING

We are offering the shares of common stock described in this prospectus through a number of underwriters. J.P. Morgan Securities LLC and Morgan Stanley & Co. LLC are acting as joint book-running managers of the offering and as representatives of the underwriters. We have entered into an underwriting agreement with the underwriters. Subject to the terms and conditions of the underwriting agreement, we have agreed to sell to the underwriters, and each underwriter has severally agreed to purchase, at the public offering price less the underwriting discounts and commissions set forth on the cover of this prospectus, the number of shares of common stock listed next to its name in the following table:

Name	Number of shares
J.P. Morgan Securities LLC	
Morgan Stanley & Co. LLC	
Cowen and Company, LLC	
Canaccord Genuity Inc.	
Total	

The underwriters are committed to purchase all the shares of common stock offered by us if they purchase any shares. The underwriting agreement also provides that if an underwriter defaults, the purchase commitments of non-defaulting underwriters may also be increased or the offering may be terminated.

The underwriters propose to offer the shares of common stock directly to the public at the initial public offering price set forth on the cover of this prospectus and to certain dealers at that price less a concession not in excess of $ per share. Any such dealers may resell shares to certain other brokers or dealers at a discount of up to $ per share from the initial public offering price. After the initial public offering of the shares, the offering price and other selling terms may be changed by the underwriters. Sales of shares made outside of the United States may be made by affiliates of the underwriters.

The underwriters have an option to buy up to additional shares of common stock from us to cover sales of shares by the underwriters which exceed the number of shares specified in the table above. The underwriters have 30 days from the date of this prospectus to exercise this option to purchase additional shares. If any shares are purchased with this option to purchase additional shares, the underwriters will purchase shares in approximately the same proportion as shown in the table above.

The underwriting fee is equal to the public offering price per share of common stock less the amount paid by the underwriters to us per share of common stock. The underwriting fee is $ per share. The following table shows the per share and total underwriting discounts and commissions to be paid to the underwriters assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

	Without exercise of option to purchase additional shares	With full exercise of option to purchase additional shares
Per share	$	$
Total	$	$

We estimate that the total expenses of this offering, including registration, filing and listing fees, printing fees and legal and accounting expenses, but excluding the underwriting discounts and commissions, will be approximately $ million. We have agreed to reimburse the underwriters for all expenses relating to the clearance of this offering with the Financial Industry Regulatory Authority (in an amount not to exceed $).

A prospectus in electronic format may be made available on the web sites maintained by one or more underwriters, or selling group members, if any, participating in the offering. The underwriters may agree to allocate a number of shares to underwriters and selling group members for sale to their online brokerage account

holders. Internet distributions will be allocated by the representatives to underwriters and selling group members that may make Internet distributions on the same basis as other allocations.

We have agreed that we will not (i) offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, or file with the Securities and Exchange Commission a registration statement under the Securities Act of 1933, as amended (the "Securities Act"), relating to, any shares of our common stock or any securities convertible into or exchangeable or exercisable for any shares of our common stock, or publicly disclose the intention to make any offer, sale, pledge, disposition or filing, or (ii) enter into any swap or other arrangement that transfers all or a portion of the economic consequences associated with the ownership of any shares of our common stock or any such other securities (regardless of whether any of these transactions are to be settled by the delivery of shares of our common stock or such other securities, in cash or otherwise), in each case without the prior written consent of J.P. Morgan Securities LLC and Morgan Stanley & Co. LLC for a period of 180 days after the date of this prospectus, other than the shares of our common stock to be sold hereunder and any shares of our common stock issued upon the exercise of options granted under our existing management incentive plans.

Our directors and executive officers, and substantially all of our stockholders have entered into lock-up agreements with the underwriters prior to the commencement of this offering pursuant to which each of these persons or entities, with limited exceptions, for a period of 180 days after the date of this prospectus, may not, without the prior written consent of J.P. Morgan Securities LLC and Morgan Stanley & Co. LLC, (1) offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of our common stock or any securities convertible into or exercisable or exchangeable for our common stock (including without limitation, common stock or such other securities which may be deemed to be beneficially owned by such directors, executive officers and shareholders in accordance with the rules and regulations of the Securities and Exchange Commission and securities which may be issued upon exercise of a stock option or warrant), or publicly disclose the intention to make any offer, sale, pledge or disposition, (2) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of our common stock or such other securities, whether any such transaction described in clause (1) or (2) above is to be settled by delivery of common stock or such other securities, in cash or otherwise or (3) make any demand for or exercise any right with respect to the registration of any shares of our common stock or any security convertible into or exercisable or exchangeable for our common stock. The restrictions described in the immediately preceding paragraph to do not apply to:

- transfers or dispositions of shares of common stock (or any security convertible into or exercisable or exchangeable for common stock):
  - as a bona fide gift;
  - to any trust for the direct or indirect benefit of the party subject to the lock-up restrictions or the immediate family of such person;
  - to any corporation, partnership, limited liability company, investment fund or other entity controlled or managed, or under common control or management by the party subject to the lock-up restrictions or the immediate family of such person;
  - by will, other testamentary document or intestate succession to the legal representative, heir, beneficiary or a member of the immediate family of the party subject to the lockup restrictions; and
  - as distributions to partners, members or stockholders of the party subject to the lock-up restrictions,

  provided that in the case of any transfer or distribution pursuant to the above five subclauses, (i) each donee or distributee shall sign and deliver a lock-up letter substantially in the form executed by the

party subject to the lock up restrictions and (ii) no filing under Section 16(a)of the Exchange Act, reporting a reduction in beneficial ownership of shares of common stock, shall be required or shall be voluntarily made during the restricted period (other than a filing on Form 5);

- the establishment of a trading plan pursuant to Rule 10b5-1 under the Exchange Act for the transfer of shares of common stock, *provided* that (i) such plan does not provide for the transfer of common stock during the restricted period and (ii) no filing under the Exchange Act or other public announcement shall be required or voluntarily made by or on behalf of the party subject to the lock-up restrictions regarding the establishment of such plan;
- the exercise of options to purchase shares of common stock granted under any stock incentive plan or stock purchase plan of the Company, *provided* that the underlying shares shall continue to be subject to the restrictions on transfer set forth in this agreement and *provided further* that no filing under Section 16(a) of the Exchange Act shall be required or shall be voluntarily made during the restricted period (other than a filing on Form 5);
- the exercise (whether for cash, cashless, or net exercise) of warrants to purchase shares of common stock (or any security convertible into or exercisable or exchangeable for common stock), *provided* that the underlying shares shall continue to be subject to the lock-up restrictions and *provided further* that, other than in respect of warrants that will expire or automatically exercise by their terms in connection with this offering, no filing under Section 16(a) of the Exchange Act shall be required or shall be voluntarily made during the restricted period (other than a filing on Form 5);
- the transfer of shares of common stock (or any security convertible into common stock) to the Company or sold in connection with a vesting event of the Company's securities or upon the exercise of options to purchase the Company's securities, on a "cashless" or "net exercise" basis or to cover tax withholding obligations of the party subject to the lock-up restrictions in connection with such vesting or exercise *provided* that no filing under Section 16(a) of the Exchange Act shall be required or shall be voluntarily made during the restricted period (other than a filing on Form 5);
- the transfer or disposition of the shares of common stock (or any security convertible into or exercisable or exchangeable for common stock) held by the party subject to the lock-up restrictions that occurs by operation of law, such as pursuant to a qualified domestic order or in connection with a divorce settlement *provided* that each transferee shall sign and deliver a lock-up letter substantially in the form executed by the party subject to the lock-up restrictions;
- the transfer of shares of common stock (or any security convertible into or exercisable or exchangeable for common stock) pursuant to a bona fide third party tender offer, merger, consolidation or other similar transaction made to all holders of the common stock involving a change of control of the Company, *provided* that in the event that the tender offer, merger, consolidation or other such transaction is not completed, the common stock owned by the party subject to the lock-up restrictions shall remain subject to such restrictions; or
- transactions by any person other than us relating to shares of common stock or other securities acquired in open market transactions after the completion of the offering of the shares, *provided* that no filing under Section 16(a) of the Exchange Act is required or voluntarily made in connection with subsequent sales of the common stock or other securities acquired in such open market transactions (other than a filing on Form 5).

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act of 1933, as amended.

We have applied to have our common stock approved for listing/quotation on The NASDAQ Global Market under the symbol "RARE."

In connection with this offering, the underwriters may engage in stabilizing transactions, which involves making bids for, purchasing and selling shares of common stock in the open market for the purpose of preventing

or retarding a decline in the market price of the common stock while this offering is in progress. These stabilizing transactions may include making short sales of the common stock, which involves the sale by the underwriters of a greater number of shares of common stock than they are required to purchase in this offering, and purchasing shares of common stock on the open market to cover positions created by short sales. Short sales may be "covered" shorts, which are short positions in an amount not greater than the underwriters' option to purchase additional shares referred to above, or may be "naked" shorts, which are short positions in excess of that amount. The underwriters may close out any covered short position either by exercising their option to purchase additional shares, in whole or in part, or by purchasing shares in the open market. In making this determination, the underwriters will consider, among other things, the price of shares available for purchase in the open market compared to the price at which the underwriters may purchase shares through the option to purchase additional shares. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market that could adversely affect investors who purchase in this offering. To the extent that the underwriters create a naked short position, they will purchase shares in the open market to cover the position.

The underwriters have advised us that, pursuant to Regulation M of the Securities Act, they may also engage in other activities that stabilize, maintain or otherwise affect the price of the common stock, including the imposition of penalty bids. This means that if the representatives of the underwriters purchase common stock in the open market in stabilizing transactions or to cover short sales, the representatives can require the underwriters that sold those shares as part of this offering to repay the underwriting discount received by them.

These activities may have the effect of raising or maintaining the market price of the common stock or preventing or retarding a decline in the market price of the common stock, and, as a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. If the underwriters commence these activities, they may discontinue them at any time. The underwriters may carry out these transactions on The NASDAQ Global Market, in the over-the-counter market or otherwise.

Prior to this offering, there has been no public market for our common stock. The initial public offering price will be determined by negotiations between us and the representatives of the underwriters. In determining the initial public offering price, we and the representatives of the underwriters expect to consider a number of factors including:

- the information set forth in this prospectus and otherwise available to the representatives;
- our prospects and the history and prospects for the industry in which we compete;
- an assessment of our management;
- our prospects for future earnings;
- the general condition of the securities markets at the time of this offering;
- the recent market prices of, and demand for, publicly traded common stock of generally comparable companies; and
- other factors deemed relevant by the underwriters and us.

Neither we nor the underwriters can assure investors that an active trading market will develop for our common shares, or that the shares will trade in the public market at or above the initial public offering price.

Selling restrictions

General

Other than in the United States, no action has been taken by us or the underwriters that would permit a public offering of the securities offered by this prospectus in any jurisdiction where action for that purpose is required. The securities offered by this prospectus may not be offered or sold, directly or indirectly, nor may this prospectus or any other offering material or advertisements in connection with the offer and sale of any such securities be

distributed or published in any jurisdiction, except under circumstances that will result in compliance with the applicable rules and regulations of that jurisdiction. Persons into whose possession this prospectus comes are advised to inform themselves about and to observe any restrictions relating to the offering and the distribution of this prospectus. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any securities offered by this prospectus in any jurisdiction in which such an offer or a solicitation is unlawful.

United Kingdom

Each underwriter has represented and agreed that:

(1) it has only communicated or caused to be communicated and will only communicate or cause to be communicated an invitation or inducement to engage in investment activity (within the meaning of Section 21 of the FSMA) received by it in connection with the issue or sale of our common shares in circumstances in which Section 21(1) of the FSMA does not apply to us; and

(2) it has complied and will comply with all applicable provisions of the FSMA with respect to anything done by it in relation to our common shares in, from or otherwise involving the United Kingdom.

European Economic Area

In relation to each Member State of the European Economic Area which has implemented the Prospectus Directive (each, a "Relevant Member State"), an offer to the public of any shares which are the subject of the offering contemplated by this prospectus (the "Shares") may not be made in that Relevant Member State, except that an offer to the public in that Relevant Member State of any Shares may be made at any time under the following exemptions under the Prospectus Directive, if they have been implemented in that Relevant Member State:

(1) to any legal entity which is a qualified investor as defined in the Prospectus Directive;

(2) to fewer than 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 150, natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the representatives for any such offer; or

(3) in any other circumstances falling within Article 3(2) of the Prospectus Directive, provided that no such offer of Shares shall result in a requirement for the publication by us or any underwriter of a prospectus pursuant to Article 3 of the Prospectus Directive.

For the purposes of this provision, the expression an "offer to the public" in relation to any Shares in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and any Shares to be offered so as to enable an investor to decide to purchase any Shares, as the same may be varied in that Member State by any measure implementing the Prospectus Directive in that Member State, the expression "Prospectus Directive" means Directive 2003/71/EC (and amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member State), and includes any relevant implementing measure in the Relevant Member State, and the expression "2010 PD Amending Directive" means Directive 2010/73/EU.

Switzerland

The shares may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange, or the SIX, or on any other stock exchange or regulated trading facility in Switzerland. This document has been prepared without regard to the disclosure standards for issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this prospectus nor any other offering or marketing material relating to the shares or the offering may be publicly distributed or otherwise made publicly available in Switzerland.

Neither this prospectus nor any other offering or marketing material relating to the offering, the Company, the shares have been or will be filed with or approved by any Swiss regulatory authority. In particular, this document will not be filed with, and the offer of shares will not be supervised by, the Swiss Financial Market Supervisory Authority FINMA, and the offer of shares has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes, or the CISA. The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of shares.

Hong Kong

The shares may not be offered or sold by means of any document other than (i) in circumstances which do not constitute an offer to the public within the meaning of the Companies Ordinance (Cap.32, Laws of Hong Kong), or (ii) to "professional investors" within the meaning of the Securities and Futures Ordinance (Cap.571, Laws of Hong Kong) and any rules made thereunder, or (iii) in other circumstances which do not result in the document being a "prospectus" within the meaning of the Companies Ordinance (Cap.32, Laws of Hong Kong), and no advertisement, invitation or document relating to the shares may be issued or may be in the possession of any person for the purpose of issue (in each case whether in Hong Kong or elsewhere), which is directed at, or the contents of which are likely to be accessed or read by, the public in Hong Kong (except if permitted to do so under the laws of Hong Kong) other than with respect to shares which are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" within the meaning of the Securities and Futures Ordinance (Cap. 571, Laws of Hong Kong) and any rules made thereunder.

Singapore

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares may not be circulated or distributed, nor may the shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor under Section 274 of the Securities and Futures Act, Chapter 289 of Singapore (the "SFA"), (ii) to a relevant person, or any person pursuant to Section 275(1A), and in accordance with the conditions, specified in Section 275 of the SFA or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA.

Where the shares are subscribed or purchased under Section 275 by a relevant person which is: (a) a corporation (which is not an accredited investor) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or (b) a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary is an accredited investor, shares, debentures and units of shares and debentures of that corporation or the beneficiaries' rights and interest in that trust shall not be transferable for 6 months after that corporation or that trust has acquired the shares under Section 275 except: (1) to an institutional investor under Section 274 of the SFA or to a relevant person, or any person pursuant to Section 275(1A), and in accordance with the conditions, specified in Section 275 of the SFA; (2) where no consideration is given for the transfer; or (3) by operation of law.

Japan

The securities have not been and will not be registered under the Financial Instruments and Exchange Law of Japan (the Financial Instruments and Exchange Law) and each underwriter has agreed that it will not offer or sell any securities, directly or indirectly, in Japan or to, or for the benefit of, any resident of Japan (which term as used herein means any person resident in Japan, including any corporation or other entity organized under the laws of Japan), or to others for re-offering or resale, directly or indirectly, in Japan or to a resident of Japan, except pursuant to an exemption from the registration requirements of, and otherwise in compliance with, the Financial Instruments and Exchange Law and any other applicable laws, regulations and ministerial guidelines of Japan.

LEGAL MATTERS

The validity of the issuance of our common stock offered in this prospectus will be passed upon for us by Ropes & Gray LLP, San Francisco, California. Certain legal matters in connection with this offering will be passed upon for the underwriters by Latham & Watkins LLP, Costa Mesa, California.

EXPERTS

Ernst & Young LLP, independent registered public accounting firm, has audited our financial statements at December 31, 2011 and 2012, and for each of the two years in the period ended December 31, 2012, and for the period from April 22, 2010 (Inception) to December 31, 2012, as set forth in their report. We have included our financial statements in the prospectus and elsewhere in the registration statement in reliance on Ernst & Young LLP's report, given on their authority as experts in accounting and auditing.

WHERE YOU CAN FIND MORE INFORMATION

We have filed with the SEC a registration statement on Form S-1, or the registration statement, under the Securities Act with respect to the shares of common stock offered hereby. This prospectus, which constitutes a part of the registration statement, does not contain all of the information set forth in the registration statement or the exhibits and schedules filed therewith. For further information with respect to Ultragenyx Pharmaceutical Inc. and the common stock offered hereby, reference is made to the registration statement and the exhibits and schedules filed therewith. Statements contained in this prospectus regarding the contents of any contract or any other document that is filed as an exhibit to the registration statement are not necessarily complete, and each such statement is qualified in all respects by reference to the full text of such contract or other document filed as an exhibit to the registration statement. A copy of the registration statement and the exhibits and schedules filed therewith may be inspected without charge at the public reference room maintained by the SEC, located at 100 F Street N.E., Washington, D.C. 20549, and copies of all or any part of the registration statement may be obtained from such offices upon the payment of the fees prescribed by the SEC. Please call the SEC at 1-800-SEC-0330 for further information about the public reference room. The SEC also maintains a website that contains reports, proxy and information statements and other information regarding registrants that file electronically with the SEC. The address is www.sec.gov.

Upon completion of this offering, we will become subject to the information and periodic reporting requirements of the Exchange Act and, in accordance therewith, will file periodic reports, proxy statements and other information with the SEC. Such periodic reports, proxy statements and other information will be available for inspection and copying at the public reference room and website of the SEC referred to above. We maintain a website at *www.ultragenyx.com*. The reference to our website address does not constitute incorporation by reference of the information contained on our website, and you should not consider the contents of our website in making an investment decision with respect to our common stock.

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)

Index to Financial Statements

Years Ended December 31, 2011 and 2012 and the Period From
April 22, 2010 (Inception) Through December 31, 2012

Report of Independent Registered Public Accounting Firm

To the Board of Directors and Stockholders of
Ultragenyx Pharmaceutical Inc.

We have audited the accompanying balance sheets of Ultragenyx Pharmaceutical Inc. (a development stage company) (the Company) as of December 31, 2011 and 2012, and the related statements of operations and comprehensive loss, convertible preferred stock and stockholders' deficit, and cash flows for each of the two years in the period ended December 31, 2012, and for the period from April 22, 2010 (Inception) through December 31, 2012. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. We were not engaged to perform an audit of the Company's internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of Ultragenyx Pharmaceutical Inc. at December 31, 2011 and 2012, and the results of its operations and comprehensive loss and its cash flows for each of the two years in the period ended December 31, 2012 and for the period from April 22, 2010 (Inception) through December 31, 2012, in conformity with U.S. generally accepted accounting principles.

/s/ Ernst & Young LLP

Redwood City, California
October 3, 2013

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)

BALANCE SHEETS
(In thousands, except share and per share amounts)

	December 31, 2011	December 31, 2012
Assets		
Current assets:		
Cash and cash equivalents	$10,645	$ 86,190
Receivables due from related party	103	—
Prepaid expenses and other current assets	205	255
Total current assets	10,953	86,445
Property and equipment, net	759	1,362
Restricted cash	376	476
Other assets	41	33
Total assets	$12,129	$ 88,316
Liabilities, Convertible Preferred Stock and Stockholders' Deficit		
Current liabilities:		
Accounts payable	$ 342	$ 1,200
Accrued liabilities	657	1,913
Deferred rent—current portion	—	75
Total current liabilities	999	3,188
Convertible preferred stock warrant liability	216	518
Other liabilities	271	270
Total liabilities	1,486	3,976
Commitments and contingencies (Note 6)		
Series A redeemable convertible preferred stock, par value of $0.001 per share—62,000,000 and 35,377,556 shares authorized as of December 31, 2011 and 2012; 18,052,464 and 34,349,894 shares issued and outstanding as of December 31, 2011 and 2012; redemption value of $47,159 as of December 31, 2012	18,604	37,458
Series B convertible preferred stock, par value of $0.001 per share—0 and 27,081,680 shares authorized as of December 31, 2011 and 2012; 0 and 27,081,680 shares issued and outstanding as of December 31, 2011 and 2012; aggregate liquidation preference of $75,060 as of December 31, 2012	—	73,929
Stockholders' deficit:		
Common stock, par value of $0.001 per share—85,000,000 shares authorized; 9,524,560 and 10,842,050 shares issued and outstanding as of December 31, 2011 and 2012	10	11
Additional paid-in capital	184	—
Deficit accumulated during the development stage	(8,155)	(27,058)
Total stockholders' deficit	(7,961)	(27,047)
Total liabilities, convertible preferred stock and stockholders' deficit	$12,129	$ 88,316

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)

STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(In thousands, except share and per share amounts)

	Year Ended December 31,		Period from April 22, 2010 (Inception) Through December 31,
	2011	2012	2012
Operating expenses:			
Research and development	$ 4,717	$ 12,641	$ 18,316
General and administrative	1,844	3,344	5,480
Total operating expenses	6,561	15,985	23,796
Loss from operations	(6,561)	(15,985)	(23,796)
Other income (expense), net:			
Interest income	4	1	5
Interest expense	(270)	—	(318)
Other expense, net	(22)	(350)	(380)
Total other income (expense), net	(288)	(349)	(693)
Net loss and comprehensive loss	$ (6,849)	$ (16,334)	$ (24,489)
Net loss attributable to common stockholders	$ (7,466)	$ (19,561)	
Net loss per share attributable to common stockholders, basic and diluted	$ (1.47)	$ (4.53)	
Shares used in computing net loss per share attributable to common stockholders, basic and diluted	5,069,694	4,316,868	
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)		$	
Shares used in computing pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)			

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)

STATEMENTS OF CONVERTIBLE PREFERRED STOCK AND STOCKHOLDERS' DEFICIT
(In thousands, except share and per share amounts)

	Convertible Preferred Stock				Stockholders' Deficit				
	Series A Redeemable Convertible Preferred Stock		Series B Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Deficit Accumulated During the Development Stage	Total Stockholders' Deficit
	Shares	Amount	Shares	Amount	Shares	Amount			
Balance as of April 22, 2010 (Inception)	—	$ —	—	$ —	—	$ —	$ —	$ —	$ —
Issuance of common stock for cash	—	—	—	—	8,240,000	8	(7)	—	1
Net loss and comprehensive loss	—	—	—	—	—	—	—	(1,306)	(1,306)
Balance as of December 31, 2010	—	—	—	—	8,240,000	8	(7)	(1,306)	(1,305)
Issuance of common stock for cash	—	—	—	—	1,284,560	2	(2)	—	—
Issuance of Series A redeemable convertible preferred stock for cash at $1.034 per share, net of $121 of issuance cost in June 2011	14,508,173	14,879	—	—	—	—	—	—	—
Issuance of Series A redeemable convertible preferred stock in exchange for conversion of promissory notes and accrued interest at $1.034 per share in June 2011	3,544,291	3,664	—	—	—	—	—	—	—
Stock-based compensation expense related to employee stock option grants	—	—	—	—	—	—	36	—	36
Stock-based compensation expense related to founder's stock	—	—	—	—	—	—	218	—	218
Accretion on convertible preferred stock	—	61	—	—	—	—	(61)	—	(61)
Net loss and comprehensive loss	—	—	—	—	—	—	—	(6,849)	(6,849)
Balance as of December 31, 2011	18,052,464	18,604	—	—	9,524,560	10	184	(8,155)	(7,961)
Issuance of common stock upon exercise of stock options	—	—	—	—	1,317,490	1	130	—	131
Issuance of Series A redeemable convertible preferred stock for cash at $1.034 per share, net of $20 of issuance cost in July 2012	14,604,895	15,080	—	—	—	—	—	—	—
Issuance of Series A redeemable convertible preferred stock in lieu of cash dividend	1,692,535	2,070	—	—	—	—	(1,205)	(865)	(2,070)
Issuance of Series B convertible preferred stock for cash at $2.77 per share, net of $1,071 of issuance cost in December 2012	—	—	27,081,680	73,929	—	—	—	—	—
Stock-based compensation expense related to employee stock option grants	—	—	—	—	—	—	178	—	178
Stock-based compensation expense related to founder's stock	—	—	—	—	—	—	713	—	713
Accretion on convertible preferred stock	—	1,704	—	—	—	—	—	(1,704)	(1,704)
Net loss and comprehensive loss	—	—	—	—	—	—	—	(16,334)	(16,334)
Balance as of December 31, 2012	34,349,894	$37,458	27,081,680	$73,929	10,842,050	$ 11	$ —	$ (27,058)	$ (27,047)

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)

STATEMENTS OF CASH FLOWS
(In thousands)

	Year Ended December 31,		Period from April 22, 2010 (Inception) Through December 31, 2012
	2011	2012	
Operating activities:			
Net loss	$ (6,849)	$ (16,334)	$ (24,489)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	34	313	353
Noncash interest expense	270	—	318
Stock-based compensation	254	891	1,145
Revaluation of convertible preferred stock warrant liability	13	302	315
Changes in operating assets and liabilities:			
Receivable—related party	(3)	3	—
Prepaid expenses and other current assets	(205)	(50)	(255)
Other assets	(41)	8	(33)
Accounts payable	252	858	1,200
Accrued expenses and other liabilities	450	1,505	2,257
Net cash used in operating activities	(5,825)	(12,504)	(19,189)
Investing activities:			
Purchase of property and equipment	(548)	(1,091)	(1,715)
Increase in restricted cash	(376)	(100)	(476)
Net cash used in investing activities	(924)	(1,191)	(2,191)
Financing activities:			
Net proceeds from issuance of convertible preferred stock	14,879	89,009	103,888
Net proceeds from issuance of common stock	—	131	132
Proceeds from issuance of promissory notes	2,450	100	3,550
Net cash provided by financing activities	17,329	89,240	107,570
Net increase in cash and cash equivalents	10,580	75,545	86,190
Cash and cash equivalents at beginning of period	65	10,645	—
Cash and cash equivalents at end of period	$ 10,645	$ 86,190	$ 86,190
Supplemental disclosures of non-cash investing and financing information:			
Issuance of convertible preferred stock warrants	$ 157	$ —	$ 202
Issuance of Series A redeemable convertible preferred stock in lieu of cash dividend	$ —	$ 2,070	$ 2,070
Conversion of promissory notes into Series A redeemable convertible preferred stock	$ 3,550	$ —	$ 3,550
Conversion of interest accrued on promissory notes into Series A redeemable convertible preferred stock	$ 114	$ —	$ 114

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)

Notes to Financial Statements

1. Organization and Basis of Presentation

Ultragenyx Pharmaceutical Inc. (the Company) is a development stage biotechnology company and was incorporated in California on April 22, 2010. The Company subsequently reincorporated in the state of Delaware in June 2011.

The Company is focused on the identification, acquisition, development, and commercialization of novel products for the treatment of rare and ultra-rare diseases, with an initial focus on serious, debilitating metabolic genetic diseases. The Company is currently conducting a Phase 2 clinical trial of sialic acid, extended release (SA-ER) in patients with hereditary inclusion body myopathy (HIBM), a progressive muscle-wasting disorder. The Company is in the development stage as of December 31, 2012, as defined by Financial Accounting Standards Board (FASB) Accounting Standards Codification (ASC) 915, *Development Stage Entities*, and since Inception has been engaged in developing its product candidates, raising capital and recruiting personnel. The Company operates in one reportable segment in the United States of America.

In the course of its development activities, the Company has sustained operating losses and expects such losses to continue over the next several years. The Company's ultimate success depends on the outcome of its research and development activities. From April 22, 2010 (Inception) through December 31, 2012, the Company has incurred cumulative net losses of $24.5 million. Management expects to incur additional losses in the future to conduct product research and development and recognizes the need to raise additional capital to fully implement its business plan. The Company intends to raise such capital through the issuance of additional equity, and potentially through borrowings, and strategic alliances with partner companies. However, if such financing is not available at adequate levels, the Company will need to reevaluate its operating plans.

2. Summary of Significant Accounting Policies

Use of Estimates

The accompanying financial statements have been prepared in accordance with U.S. generally accepted accounting principles ("GAAP"). The preparation of the financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent liabilities and the reported amounts of expenses in the financial statements and the accompanying notes. On an ongoing basis, management evaluates its estimates, including those related to clinical trial accruals, fair value of assets and liabilities, convertible preferred stock and related warrants, common stock, income taxes and stock-based compensation. Management bases its estimates on historical experience and on various other market-specific and relevant assumptions that management believes to be reasonable under the circumstances. Actual results could differ from those estimates.

Cash and Cash Equivalents

The Company considers all highly liquid investments with original maturities of three months or less from the date of purchase to be cash equivalents. Cash equivalents consist primarily of amounts invested in money market accounts.

Restricted Cash

Restricted cash consists of a money market account with one of the Company's financial institutions as collateral for its obligations under its facility lease of the Company's corporate headquarters in Novato, California. Additionally, restricted cash includes a savings account associated with a credit card agreement at one of the Company's financial institutions.

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)
Notes to Financial Statements (continued)

2. Summary of Significant Accounting Policies (continued)

Concentration of Credit Risk

Financial instruments that potentially subject the Company to a concentration of credit risk consist of cash and cash equivalents. The Company's cash and cash equivalents are held by two financial institutions in the United States. Such deposits may exceed federally insured limits. Management believes that these financial institutions are financially sound, and, accordingly, minimal credit risk exists with respect to those financial institutions. The Company is exposed to credit risk in the event of default by the financial institutions holding its cash and cash equivalents to the extent recorded in the balance sheets.

Fair Value Measurement

Fair value accounting is applied for all financial assets and liabilities and non-financial assets and liabilities that are recognized or disclosed at fair value in the financial statements on a recurring basis (at least annually).

Property and Equipment

Property and equipment are stated at cost, less accumulated depreciation and amortization. Depreciation is computed using the straight-line method over the estimated useful lives of the respective assets. Depreciation begins at the time the asset is placed in service. Maintenance and repairs are charged to operations as incurred. Upon sale or retirement of assets, the cost and related accumulated depreciation are removed from the balance sheet and the resulting gain or loss, if any, is reflected in operations.

The useful lives of the property and equipment are as follows:

Research and development equipment	5 years
Furniture and office equipment	5 years
Computer equipment	3 years
Software	3 years
Leasehold improvements	Shorter of lease term or estimated useful life

Impairment of Long-Lived Assets

The Company evaluates its long-lived assets, including property and equipment, for impairment whenever events or changes in circumstances indicate that the carrying value of these assets may not be recoverable. Recoverability of these assets is measured by comparison of the carrying amount of each asset to the future undiscounted cash flows expected to result from the use of the asset and its eventual disposition. If the asset is considered to be impaired, the amount of any impairment is measured as the difference between the carrying value and the fair value of the impaired asset. The Company has not recorded impairment of any long-lived assets since inception.

Accruals of Research and Development Costs

The Company records accruals for estimated costs of research, preclinical and clinical studies and manufacturing development. These costs are a significant component of the Company's research and development expenses. A substantial portion of the Company's ongoing research and development activities are conducted by third-party service providers, including contract research organizations. The Company accrues the costs incurred under its agreements with these third parties based on actual work completed in accordance with

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)
Notes to Financial Statements (continued)

2. Summary of Significant Accounting Policies (continued)

agreements established with these third parties. The Company determines the actual costs through discussions with internal personnel and external service providers as to the progress or stage of completion of the services and the agreed-upon fee to be paid for such services. The Company makes significant judgments and estimates in determining the accrual balance in each reporting period. As actual costs become known, the Company adjusts its accruals. The Company has not experienced any material deviations between accrued clinical trial expenses and actual clinical trial expenses. However, actual services performed, number of patients enrolled, and the rate of patient enrollment may vary from the Company's estimates, resulting in adjustments to clinical trial expense in future periods. Changes in these estimates that result in material changes to the Company's accruals could materially affect the Company's results of operations.

Leases

The Company enters into lease agreements for its office and laboratory facilities. These leases are classified as operating leases. Rent expense is recognized on a straight-line basis over the term of the lease and, accordingly, the Company records the difference between cash rent payments and the recognition of rent expense as a deferred rent liability. Incentives granted under the Company's facilities leases, including allowances to fund leasehold improvements, are deferred and are recognized as adjustments to rental expense on a straight-line basis over the term of the lease.

Convertible Preferred Stock

The Company initially records all shares of convertible preferred stock net of offering costs on the dates of issuance, which represents the carrying value. At any time after June 16, 2017, but within sixty (60) days after the receipt by the Company of a written request from the holders of not less than seventy-five percent (75%) of the then outstanding Series A convertible preferred stock, all shares of Series A convertible preferred stock can be redeemed. As only the passage of time is required for Series A convertible preferred stock to become redeemable, the Company is accreting the carrying value of Series A convertible preferred stock to its redemption value over the period from the date of issuance to June 16, 2017 (the earliest redemption date). In the event of a change of control of the Company, proceeds will be distributed in accordance with the liquidation preferences set forth in its Amended and Restated Certificate of Incorporation unless the holders of convertible preferred stock have converted their convertible preferred shares into common shares. Therefore, convertible preferred stock is classified outside of stockholders' deficit on the accompanying balance sheets, as Series A convertible preferred stock can be redeemed and as events triggering the liquidation preferences are not solely within the Company's control.

Convertible Preferred Stock Warrant Liability

Warrants for shares that are either puttable or that are contingently redeemable are classified as liabilities on the balance sheets. The warrants are subject to remeasurement at each balance sheet date, with changes in fair value recognized as a component of interest and other expense. The Company will continue to adjust the liability for changes in fair value until the earlier of the expiration of the warrants, exercise of the warrants, or conversion of the warrants upon the completion of a liquidation event, including the completion of an initial public offering to common stock warrants that will no longer be subject to remeasurement.

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)
Notes to Financial Statements (continued)

2. Summary of Significant Accounting Policies (continued)

Research and Development

Research and development costs are expensed as incurred and consist of salaries and benefits, stock-based compensation expense, lab supplies and facility costs, as well as fees paid to other nonemployees and entities that conduct certain research and development activities on the Company's behalf. Amounts incurred in connection with license agreements are also included in research and development expense. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or the services are performed.

Stock-Based Compensation

Stock-based awards issued to employees, including stock options, are recorded at fair value as of the grant date using the Black-Scholes option-pricing model and recognized as expense on a straight-line basis over the employee's requisite service period (generally the vesting period). Because noncash stock compensation expense is based on awards ultimately expected to vest, it is reduced by an estimate for future forfeitures. Forfeitures are estimated at the time of grant and revised, if necessary, in subsequent periods if actual forfeitures differ from estimates.

Income Taxes

The Company uses the liability method of accounting for income taxes. Under this method, deferred tax assets and liabilities are determined based on the differences between the financial reporting and the tax bases of assets and liabilities and are measured using the enacted tax rates and laws that will be in effect when the differences are expected to reverse. The Company must then assess the likelihood that the resulting deferred tax assets will be realized. A valuation allowance is provided when it is more likely than not that some portion or all of a deferred tax asset will not be realized. Due to the Company's lack of earnings history, the net deferred tax assets have been fully offset by a valuation allowance.

The Company recognizes benefits of uncertain tax positions if it is more likely than not that such positions will be sustained upon examination based solely on their technical merits, as the largest amount of benefit that is more likely than not to be realized upon the ultimate settlement. The Company's policy is to recognize interest and penalties related to the underpayment of income taxes as a component of income tax expense or benefit. To date, there have been no interest or penalties charged in relation to the unrecognized tax benefits.

Net Loss per Share Attributable to Common Stockholders

Basic net loss per share attributable to common stockholders is calculated by dividing the net loss attributable to common stockholders by the weighted average number of shares of common stock outstanding during the period, without consideration for common stock equivalents. The net loss attributable to common stockholders is calculated by adjusting the net loss of the Company for the accretion on the Series A convertible preferred stock and cumulative dividends on Series A and B convertible preferred stock. Diluted net loss per share attributable to common stockholders is the same as basic net loss per share attributable to common stockholders, since the effects of potentially dilutive securities are antidilutive.

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)
Notes to Financial Statements (continued)

2. Summary of Significant Accounting Policies (continued)

Unaudited Pro Forma Net Loss per Share Attributable to Common Stockholders

Pro forma basic and diluted net loss per share attributable to common stockholders has been computed to give effect to the conversion of the convertible preferred stock into common stock in connection with the Company's initial public offering. Also, the numerator in the pro forma basic and diluted net loss per share attributable to common stockholders calculation has been adjusted to remove gains and losses resulting from remeasurement of the convertible preferred stock warrant liability as these amounts will be reclassified to additional paid-in capital upon a qualifying initial public offering of the Company's common stock and has also been adjusted to reflect the payment of a dividend to the Company's convertible preferred stockholders concurrent with the conversion of the Company's convertible preferred stock to common stock immediately prior to the completion of this offering. The pro forma net loss per share attributable to common stockholders does not include the shares expected to be sold and related proceeds to be received from the initial public offering.

Recent Accounting Pronouncements

In February 2013, the FASB issued Accounting Standards Update (ASU) No. 2013-02, *Other Comprehensive Income (Topic 220): Reporting of Amounts Reclassified Out of Accumulated Other Comprehensive Income*. ASU No. 2013-02 supersedes the presentation requirements for reclassifications out of accumulated other comprehensive income in ASU 2011-05 and 2011-12 and requires an entity to provide additional information about reclassifications out of accumulated other comprehensive income. The amendment is effective in fiscal years beginning after December 15, 2012. The adoption of this amendment will not have a material impact on the Company's results of operations or financial position.

3. Fair Value Measurements

Financial assets and liabilities are recorded at fair value. The carrying amount of certain financial instruments, including cash and cash equivalents, accounts payable and accrued liabilities approximate fair value due to their relatively short maturities. Assets and liabilities recorded at fair value on a recurring basis in the balance sheets are categorized based upon the level of judgment associated with the inputs used to measure their fair values. Fair value is defined as the exchange price that would be received for an asset or an exit price that would be paid to transfer a liability in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. The authoritative guidance on fair value measurements establishes a three-tier fair value hierarchy for disclosure of fair value measurements as follows:

Level 1—Inputs are unadjusted, quoted prices in active markets for identical assets or liabilities at the measurement date;

Level 2—Inputs are observable, unadjusted quoted prices in active markets for similar assets or liabilities, unadjusted quoted prices for identical or similar assets or liabilities in markets that are not active, or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the related assets or liabilities; and

Level 3—Unobservable inputs that are significant to the measurement of the fair value of the assets or liabilities that are supported by little or no market data.

The Company's financial instruments consist of Level 1 assets and Level 3 liabilities. Where quoted prices are available in an active market, securities are classified as Level 1. Level 1 assets consist primarily of highly liquid money market funds that are included in restricted cash.

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)
Notes to Financial Statements (continued)

3. Fair Value Measurements (continued)

In certain cases where there is limited activity or less transparency around inputs to valuation, securities are classified as Level 3. Level 3 liabilities consist of the convertible preferred stock warrant liability. The following table sets forth the fair value of the Company's financial assets and liabilities remeasured on a recurring basis based on the three-tier fair value hierarchy (in thousands):

	December 31, 2011			
	Level 1	Level 2	Level 3	Total
Financial Assets:				
Money market funds	$ 376	$ —	$ —	$376
Total financial assets	$ 376	$ —	$ —	$376
Financial Liabilities:				
Convertible preferred stock warrant liability	$ —	$ —	$ 216	$216
Total financial liabilities	$ —	$ —	$ 216	$216

	December 31, 2012			
	Level 1	Level 2	Level 3	Total
Financial Assets:				
Money market funds	$ 376	$ —	$ —	$376
Total financial assets	$ 376	$ —	$ —	$376
Financial Liabilities:				
Convertible preferred stock warrant liability	$ —	$ —	$ 518	$518
Total financial liabilities	$ —	$ —	$ 518	$518

The following table sets forth a summary of the changes in the fair value of the Company's Level 3 financial liabilities (in thousands):

	Year Ended December 31,	
	2011	2012
Fair value, beginning of period	$ 46	$ 216
Issuance of preferred stock warrants	157	—
Change in fair value recorded as a loss in other expense, net	13	302
Fair value, end of period	$ 216	$ 518

The determination of the fair value of the convertible preferred stock warrants is discussed in Note 9. Generally, increases or decreases in the fair value of the underlying convertible preferred stock would result in a directionally similar impact in the fair value measurement of the warrant liability.

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)
Notes to Financial Statements (continued)

4. Balance Sheet Components

Property and Equipment, net

Property and equipment, net consists of the following (in thousands):

	December 31, 2011	December 31, 2012
Research and development equipment	$128	$ 225
Furniture and office equipment	80	266
Computer equipment	52	187
Software	10	34
Leasehold improvements	529	1,003
Property and equipment, gross	799	1,715
Less accumulated depreciation and amortization	(40)	(353)
Property and equipment, net	$759	$1,362

Depreciation and amortization expense for the years ended December 31, 2011 and 2012 and the period from April 22, 2010 (Inception) through December 31, 2012 was $34,000, $313,000 and $353,000, respectively.

Accrued Liabilities

Accrued liabilities consist of the following (in thousands):

	December 31, 2011	December 31, 2012
Research and clinical trial expenses	$147	$ 595
Payroll and related expenses	419	1,289
Other	91	29
Total accrued liabilities	$657	$1,913

5. Convertible Promissory Notes

In June 2010, the Company entered into a convertible promissory note and warrant purchase agreement with its Chief Executive Officer and Founder, Emil Kakkis, M.D., PhD, in which it could borrow up to $1,000,000 from Dr. Kakkis at an interest rate of 8% per annum. During 2010, the Company borrowed the entire amount of $1,000,000 available under the agreement. The note also contained a provision under which all outstanding principal and accrued interest would automatically be converted into preferred stock upon a financing event in which the Company issued newly authorized shares of preferred stock for aggregate proceeds of not less than $500,000. Under the terms of the agreement, the note automatically converted at the same terms and conditions of the financing. Pursuant to the agreement, the Company also issued a warrant to purchase shares of Series A convertible preferred stock (see Note 9). From July 2010 to June 2011, the Company entered into two additional convertible promissory note and warrant purchase agreements with Dr. Kakkis in which it borrowed an additional $800,000 under similar terms and issued additional warrants. In June 2011, the outstanding principal balance of $1,800,000 from all three notes plus accrued interest was converted into shares of Series A convertible preferred stock at the same issuance price per share of $1.034 as all other purchasers of Series A convertible preferred stock. The number of shares underlying the warrant to be issued was dependent on the aggregate amount owing at the time of the initial closing of the Series A convertible preferred stock financing and the price

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)
Notes to Financial Statements (continued)

5. Convertible Promissory Notes (continued)

per share of the Series A convertible preferred stock. Based on the formula specified in the Warrant Agreement, warrants to purchase an aggregate of 435,245 shares of Series A convertible preferred stock at an exercise price of $1.034 were issued to Dr. Kakkis upon the initial closing in June 2011.

In February 2011, the Company entered into two note and warrant purchase agreements with two related parties in which it borrowed a total of $1,750,000 at an interest rate of 8% per annum. The notes and all accrued interest were fully due and payable on the one-year anniversary date of each respective note agreement. The notes also contained a provision under which all outstanding principal and accrued interest would automatically be converted into preferred stock upon a financing event in which the Company issued newly authorized shares of preferred stock for aggregate proceeds of not less than $500,000. Under the terms of the agreement, the notes automatically converted at the same terms and conditions of the financing. In June 2011, the outstanding principal balance of $1,750,000 plus accrued interest from the two notes was converted into shares of Series A convertible preferred stock at the same issuance price per share of $1.034 paid by all other purchasers of Series A convertible preferred stock. Pursuant to the agreement, the Company issued a warrant to purchase shares of Series A convertible preferred stock (see Note 9). The number of shares underlying the warrant to be issued was dependent on the aggregate amount of principal outstanding at the time of the initial closing of the Series A convertible preferred stock financing, subsequently completed in June 2011, and the price per share of the Series A convertible preferred stock. Based on the formula specified in the Warrant Agreements, warrants to purchase an aggregate of 592,417 shares of Series A convertible preferred stock at an exercise price of $1.034 were issued to the two note holders upon the closing of the financing in June 2011.

6. Commitments and Contingencies

Facilities

From inception until March of 2012, the Company rented office space in a building under an informal monthly agreement. The Company's founder also rented space for one of his other business interests in that same building.

In July 2011, the Company entered into a lease agreement for office facilities in Novato, California, which provided for a tenant improvement allowance of up to $376,000. The lease commenced in March of 2012. This noncancelable operating lease expires five years after the commencement date. At the end of the lease term, the Company has the option to extend the lease for two additional consecutive terms of five years. The Company also signed an addendum to the lease agreement in July 2011 to add warehouse space to the arrangement.

In September 2010, the Company entered into a license and service agreement for a lab facility in Novato, California. The term commenced in September 2010. This agreement expires two years after the commencement date, and either party may terminate the agreement with one year's prior notice without cause and purely out of convenience of such party. In September 2012, the Company entered into an amendment to increase the size of the lab facility and extend the term. This amendment expires two years after the amendment date, and either party may terminate the agreement with one year's prior notice without cause.

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)
Notes to Financial Statements (continued)

6. Commitments and Contingencies (continued)

As of December 31, 2012, the aggregate future minimum lease payments under the noncancelable operating lease arrangements are as follows (in thousands):

Year Ending December 31:	
2013	$ 345
2014	285
2015	285
2016	285
2017	47
	$1,247

The Company recognizes rent expense on a straight-line basis over the noncancelable term of its operating lease. Rent expense was $84,000, $265,000 and $367,000 during the years ended December 31, 2011 and 2012 and the period from April 22, 2010 (Inception) through December 31, 2012, respectively.

Under the terms of the lease agreement of its Novato office facility, the Company provided the lessor with an irrevocable letter of credit in the amount of $376,000. The lessor shall be entitled to draw on the letter of credit in the event of any uncured default by the Company under the terms of the lease. Provided there has been no default on the lease, the Company may reduce the amount of the letter of credit by $75,000 on each anniversary date from the commencement date.

Other Commitments

The Company has various manufacturing, clinical, research, and other contracts with vendors in the conduct of the normal course of its business. All contracts are terminable, with varying provisions regarding termination. If a contract with a specific vendor were to be terminated, the Company would only be obligated for the products or services that the Company had received at the time the termination became effective.

Contingencies

While there are no legal proceedings the Company is aware of, the Company may become party to various claims and complaints arising in the ordinary course of business. Management does not believe that any ultimate liability resulting from any of these claims will have a material adverse effect on its results of operations, financial position, or liquidity. However, management cannot give any assurance regarding the ultimate outcome of these claims, and their resolution could be material to operating results for any particular period, depending upon the level of income for the period.

Guarantees and Indemnifications

The Company indemnifies each of its directors for certain events or occurrences, subject to certain limits, while the director is or was serving at the Company's request in such capacity, as permitted under Delaware law and in accordance with its certificate of incorporation and bylaws. The term of the indemnification period lasts as long as a director may be subject to any proceeding arising out of acts or omissions of such director in such capacity. The maximum amount of potential future indemnification is unlimited; however, the Company currently holds director liability insurance. This insurance allows the transfer of risk associated with the Company's exposure and may enable it to recover a portion of any future amounts paid. The Company believes that the fair value of these indemnification obligations is minimal. Accordingly, it has not recognized any liabilities relating to these obligations for any period presented.

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)
Notes to Financial Statements (continued)

7. License and Research Agreements

Nobelpharma License Agreement

In September 2010, the Company entered into a collaboration and license agreement with Nobelpharma Co., Ltd. (Nobelpharma). Under the terms of this collaboration and license agreement, each party granted the other party a worldwide exclusive license under certain of that party's intellectual property related to the compound identified as N-acetylneuraminic acid, also known as sialic acid, to develop, manufacture, and commercialize products. Nobelpharma's licensed territory includes Japan and certain other Asian countries, and the Company's licensed territory includes the rest of the world.

Under the collaboration and license agreement, the Company paid Nobelpharma $110,500 (10 million Yen) for the license, which was recorded as research and development expense in 2010, and also issued 240,000 shares of common stock to Nobelpharma with a minimal value. The Company is required to pay Nobelpharma annual royalties and earned royalties based on net sales upon product sales commencement. In addition, the Company is required to make certain payments to Nobelpharma based upon achievement of certain development and approval milestones. The Company paid $495,000 in development milestone payments from inception through December 31, 2012. The remaining total aggregate payments, if all milestones are achieved by Nobelpharma, would be 200 million Yen (approximately $2.3 million based on the exchange rate at December 31, 2012). The Company will pay a high single digit royalty on net sales in the Company's territory and will receive a mid-single digit royalty on net sales in the Nobelpharma territory, excluding Japan, if such product sales are ever achieved. Net sales, as defined in the collaboration and license agreement, represent the net sales of products whereby the licensed compound is the active ingredient. If the products include other active ingredients, the portion of the net sales allocated to the licensed compound would be used in determining the royalty payments.

Saint Louis University License Agreement

In November 2010, the Company entered into a license agreement with Saint Louis University (SLU). Under the terms of this license agreement, SLU granted the Company an exclusive worldwide license to make, have made, use, import, offer for sale, and sell therapeutics related to SLU's beta-glucuronidase product for use in the treatment of human diseases.

Under the license agreement, the Company paid SLU an up-front fee of $10,000, which was recorded as research and development expense in 2010. The Company will be required to make a milestone payment of $100,000 upon approval of a glucuronidase-based enzyme therapy for treatment of MPS 7. Additionally, upon reaching a certain level of cumulative worldwide sales of the product, the Company will be required to pay to SLU a low single-digit royalty on net sales of the licensed products in any country or region, if such product sales are ever achieved.

AAI Pharma License Agreement

In March 2011, the Company entered into a license agreement with AAI Pharma Services Corp. (AAI Pharma). Under the terms of this license agreement, AAI Pharma granted the Company a fully paid-up, royalty-free, exclusive, perpetual, and irrevocable license to research, develop, make, have made, use, import, offer for sale, and sell products incorporating AAI Pharma's controlled release matrix solid dose oral tablet. Under the license agreement, the Company will pay a mid-single digit percentage of any sublicense revenue received by Ultragenyx related to the sublicense of AAI Pharma technology that had been initially licensed by Ultragenyx.

ULTRAGENYX PHARMACEUTICAL INC.
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Notes to Financial Statements (continued)

7. License and Research Agreements (continued)

HIBM Research Group

License Agreement

In April 2012, the Company entered into an exclusive license agreement with HIBM Research Group (HRG). Under the terms of this license agreement, HRG granted the Company an exclusive worldwide license to certain intellectual property related to the treatment of HIBM.

Under the license agreement, the Company paid HRG an up-front fee of $25,000, which was recorded as research and development expense during the year ended December 31, 2012. The Company may make future payments that aggregate up to $300,000 and that are contingent upon attainment of various development and approval milestones. Additionally, the Company will pay to HRG a royalty of less than 1% of net sales of the licensed products in the licensed territories, if such product sales are ever achieved.

Research Agreement

In April 2012, the Company entered into a research agreement with HRG. Under the terms of this research agreement, the Company will engage HRG to perform certain nonclinical research activities related to the treatment for HIBM.

Under the research agreement, the Company was required to pay HRG an annual grant of $25,000 during the first five years from the effective date of the agreement. The agreement was terminated in 2013, and accordingly, no further amounts will be paid to HRG for research.

St. Jude Children's Research Hospital License Agreement

In September 2012, the Company entered into a license agreement with St. Jude Children's Research Hospital (St. Jude). Under the terms of this license agreement, St. Jude granted the Company an exclusive license under certain know-how to research, develop, make, use, offer to sell, import, and otherwise commercialize and exploit St. Jude's protective protein, cathepsin, a protein product to treat, prevent, and/or diagnose galactosialidosis and other monogenetic diseases.

Under the license agreement, the Company paid St. Jude an up-front fee of $10,000 which was recorded as research and development expense during the year ended December 31, 2012. Additionally, the Company will pay to St. Jude a royalty of less than 1% on net sales of the licensed products in the licensed territories, if such product sales are ever achieved.

Baylor Research Institute License Agreement

In September 2012, the Company entered into a license agreement with Baylor Research Institute (BRI). Under the terms of this license agreement, BRI exclusively licensed to the Company certain intellectual property related to triheptanoin for North America.

Under the license agreement, the Company paid BRI an up-front fee of $250,000 which was recorded as research and development expense during the year ended December 31, 2012. The Company may make future payments of up to $10.5 million contingent upon attainment of various development milestones and $7.5 million contingent upon attainment of various sales milestones. Additionally, the Company will pay to BRI a mid-single digit royalty on net sales of the licensed product in the licensed territories.

ULTRAGENYX PHARMACEUTICAL INC.
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Notes to Financial Statements (continued)

7. License and Research Agreements (continued)

The Company has an exclusive option to expand the licensed territory to a worldwide license if the previous holder of such territorial rights allows that option to lapse. The previous holder's option lapsed on December 31, 2012, and on June 26, 2013, the Company notified BRI that it was exercising its exclusive option to expand the licensed territory. The fee associated with this option exercise is $750,000 — See Note 15- Subsequent Events.

8. Related Party Transactions

Emil Kakkis, M.D., Ph.D., Founder, member of the Company's Board of Directors, President, and Chief Executive Officer, entered into a Note and Warrant Purchase Agreement with the Company whereby Dr. Kakkis loaned the Company a total of $1,800,000. In June 2011, the outstanding principal balance of $1,800,000 and all accrued interest was converted into Series A convertible preferred stock at the same issuance price of $1.034 paid by all other purchasers of Series A convertible preferred stock. As per the Note and Warrant Purchase Agreement, the Company issued Dr. Kakkis a warrant to purchase 435,245 shares of Series A convertible preferred stock with an exercise price of $1.034 per share.

William Aliski, a member of the Company's Board of Directors, entered into a Note and Warrant Purchase Agreement with the Company whereby Mr. Aliski loaned the Company $250,000 in February 2011. In June 2011, the outstanding principal balance of $250,000 and all accrued interest was converted into shares of Series A convertible preferred stock at the same issuance price of $1.034 paid by all other purchasers of Series A convertible preferred stock. As per the Note and Warrant Purchase Agreement, the Company issued Mr. Aliski a warrant to purchase 84,631 shares of Series A convertible preferred stock with an exercise price of $1.034 per share.

John Klock, a former member and current observer of the Company's Board of Directors, entered into a Note and Warrant Purchase Agreement with the Company whereby Mr. Klock loaned the Company $1,500,000 in February 2011. In June 2011, the outstanding principal balance of $1,500,000 and all accrued interest was converted into shares of Series A convertible preferred stock at the same issuance price of $1.034 paid by all other purchasers of Series A convertible preferred stock. As per the Note and Warrant Purchase Agreement, the Company issued Mr. Klock a warrant to purchase 507,786 shares of Series A convertible preferred stock with an exercise price of $1.034 per share.

From the inception of the Company to March 2012, the Company rented its office facility in Novato, California, from an entity affiliated with Dr. Kakkis. Rent expense under this arrangement was $92,000 for the period from April 22, 2010 (Inception) through December 31, 2012.

9. Convertible Preferred Stock Warrants

In connection with the terms of various promissory notes issued by the Company from June 2010 through February 2011, the Company issued warrants in which the number of shares and exercise price were subject to the per share price offered in the Series A convertible preferred stock sale. In June 2011, in connection with its closing of the first round of Series A convertible preferred stock financing, the Company determined that the warrants were convertible to 1,027,662 shares of Series A convertible preferred stock at an exercise price of $1.034 per share. The warrants expire on the earlier of (i) change of control of the Company or any simultaneous sale of more than a majority of the then-outstanding securities of the Company other than a mere reincorporation transaction or (ii) the ten year anniversary of their issue date. The Company determined the fair value of the warrants using an option-pricing method to allocate the equity value of the Company to the warrants based on the Company's capital structure. The equity value was estimated using the Back-Solve method, whereby the equity

ULTRAGENYX PHARMACEUTICAL INC.
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Notes to Financial Statements (continued)

9. Convertible Preferred Stock Warrants (continued)

value was derived from a recent transaction involving the Company's own securities. The fair value ascribed to these warrants upon their issuance was $203,000. The fair value of the warrants was recorded as debt issuance costs and was amortized to interest expense using the effective-interest-rate method over the loan term. In connection with the conversion of the promissory notes into shares of Series A convertible preferred stock in June 2011, the Company recognized all remaining unamortized debt issuance costs.

As of December 31, 2011 and 2012, outstanding warrants consisted of the following:

Convertible Preferred Stock Warrants:	Number of Warrants	Date Issued	Term	Exercise Price
Series A	241,803	June 2010	10 years	$ 1.034
Series A	592,417	February 2011	10 years	1.034
Series A	193,442	June 2011	10 years	1.034
Total convertible preferred stock warrants	1,027,662			

The fair value of the warrants was estimated to be $216,000 and $518,000 as of December 31, 2011 and 2012, respectively. The key assumptions in the option-pricing valuation method as of December 31, 2011 and 2012 are as follows:

	December 31,	
	2011	2012
Value of Company equity	$23.5 million	$145.8 million
Expected volatility	80.0%	75.0%
Expected time to liquidity event	3.0 years	2.0 years
Risk-free interest rate	0.36%	0.25%

The Company recorded $13,000, $302,000 and $315,000 to other expense for the years ended December 31, 2011 and 2012 and for the period from April 22, 2010 (Inception) through December 31, 2012, respectively, representing the change in fair value of the warrants between the issuance date and the end of the reporting period.

10. Common Stock

The Company has reserved sufficient shares of common stock for issuance upon conversion of convertible preferred stock, exercise of stock options, and exercise of warrants. Common stockholders are entitled to dividends if and when declared by the Board of Directors subject to the prior rights of the preferred stockholders. As of December 31, 2012, no common stock dividends had been declared by the Board of Directors.

The Company had reserved shares of common stock, on an as-converted basis, for future issuance as follows:

	December 31,	
	2011	2012
Convertible preferred stock	18,052,464	61,431,574
Convertible preferred stock warrants	1,027,662	1,027,662
2011 equity incentive plan	4,550,000	4,767,510
Shares available for future stock option grants	2,905,988	5,689,817
	26,536,114	72,916,563

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Notes to Financial Statements (continued)

11. Convertible Preferred Stock

Under the Company's Amended and Restated Certificate of Incorporation, as amended, the Company is authorized to issue two classes of shares: preferred and common stock. The preferred stock is issuable in series, and the Company's board of directors is authorized to determine the rights, preferences and terms of each series. As of December 31, 2011 and 2012, convertible preferred stock consisted of the following (in thousands, except share and per share amounts):

	As of December 31, 2011			
Convertible Preferred Stock:	Shares Authorized	Shares Issued and Outstanding	Proceeds Net of Issuance Costs	Aggregate Liquidation Preference
			(In thousands)	
Series A	62,000,000	18,052,464	$ 18,543	$ 19,272

	As of December 31, 2012			
Convertible Preferred Stock:	Shares Authorized	Shares Issued and Outstanding	Proceeds Net of Issuance Costs	Aggregate Liquidation Preference
			(In thousands)	
Series A	35,377,556	34,349,894	$ 33,623	$ 35,613
Series B	27,081,680	27,081,680	73,929	75,060
Total convertible preferred stock	62,459,236	61,431,574	$ 107,552	$ 110,673

The liquidation preference consists of the liquidation preference on the outstanding shares of the convertible preferred stock and the dividends in arrears on such shares in the amount of $607,000 and $159,000 as of December 31, 2011 and 2012.

Significant provisions of the convertible preferred stock are as follows:

Dividends—When and as declared by the Company's Board of Directors, the holders of the Series A convertible preferred stock (Series A) and the Series B convertible preferred stock (Series B), collectively referred to as "Preferred Stock," are entitled to receive dividends, out of any assets legally available therefor, prior and in preference to the declaration or payment of any dividend on the common stock or other securities and rights convertible of the Company, at the rate of $0.062 per share per annum, payable in the form of cash or property or, upon conversion of the preferred stock to common stock, payable in cash. Prior to the issuance of the Series B convertible preferred stock in December 2012, the Company was able to pay dividends in cash or additional shares of Series A convertible preferred stock. Such dividends shall accrue on each share from the date of issuance of such share, and shall accrue from day to day, whether or not earned or declared, and whether or not there are profits, surplus, shares, or other funds legally available for the payment of such dividends. Such dividends shall be cumulative so that, except as provided below, if such dividends in respect of any previous or current annual dividend period, at the annual rate specified above, shall not have been paid, the deficiency shall first be fully paid before any dividend or other distribution shall be paid on or declared and set apart for the common stock. Any accumulation of dividends on the Preferred Stock shall not bear interest. Cumulative dividends with respect to a share of Preferred Stock that are accrued, payable, and/or in arrears shall, upon conversion of such share to common stock, be paid to the extent assets are legally available therefor, and any amounts for which assets are not legally available shall be paid promptly as assets become legally available therefor. Any partial payment shall be made ratably among the holders of Preferred Stock in proportion to the payment each such holder would receive if the full amount of such dividends were paid. After dividends have been paid to the holders of the preferred stock, any additional dividends shall be paid among the holders of the preferred stock and

ULTRAGENYX PHARMACEUTICAL INC.
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Notes to Financial Statements (continued)

11. Convertible Preferred Stock (continued)

common stock then outstanding in proportion to the greatest whole number of shares of common stock held (assuming conversion of Preferred Stock).

During 2012, $2,070,000 of preferred stock dividends were declared and paid to holders of Series A convertible preferred stock in the form of additional shares of Series A convertible preferred stock. Dividends in arrears as of December 31, 2011 and 2012 totaled $607,000 and $159,000, respectively, for both series of preferred stock.

Liquidation—In the event of any liquidation, dissolution, or winding up of the Company, either voluntary or involuntary, the holders of Series A and Series B shall be entitled to receive, on a pari passu basis, prior and in preference to any distribution of any of the assets of this Corporation to the holders of common stock by reason of their ownership thereof, an amount per share equal to the sum of $1.034 (the Original Series A Issue Price) for each outstanding share of Series A convertible preferred stock (subject to adjustment for recapitalizations) and $2.769 (the Original Series B Issue Price) for each outstanding share of Series B convertible preferred stock (subject to adjustment for recapitalizations) and an amount equal to all declared or accrued but unpaid dividends on such shares. If available assets are insufficient to pay the full liquidation preference, the available assets will be distributed ratably among the holders of preferred stock. If there are excess assets to be allocated upon liquidation beyond what is described above, holders of preferred stock and common stock will share in such assets on an as-converted basis. However, the holders of Series A shall not be entitled to further participate in any distribution of the remaining assets of the corporation following the receipt of aggregate distributions equal to $3.102, plus any declared or accrued but unpaid dividends, and the holders of Series B shall not be entitled to further participate in any distribution of the remaining assets of the corporation following the receipt of aggregate distributions equal to $8.308, plus any declared or accrued but unpaid dividends.

Redemption—At any time after June 16, 2017, but within 60 days after the receipt by this Corporation of a written request from the holders of not less than 75% of the then outstanding shares of Series A convertible preferred stock that all shares of Series A be redeemed, the Company shall, to the extent it may lawfully do so, redeem (the payment date being referred to herein as a Series A Redemption Date) all of the then-outstanding shares of Series A by paying in cash in exchange for the shares of Series A to be redeemed a sum equal to the greater of (i) the original Series A issue price per share of Series A (subject to adjustment for any recapitalizations) plus all declared or accrued but unpaid dividends on such shares and (ii) the then-current fair market value per share of Series A plus all declared or accrued but unpaid dividends on such shares (but only if, and to the extent, such dividends are not reflected in the fair market value) as determined in good faith by the Board of Directors of the Company, and taking into account any independent third-party valuation reasonably requested by the holders of at least 75% of the Series A then outstanding; however, any holder of Series A may elect, by delivery of notice to the Company at least five days prior to the Series A redemption date, not to have such holder’s shares of Series A preferred stock redeemed. As only the passage of time is required for Series A to become redeemable, the Company is accreting the carrying value of Series A to its redemption value over the period from the date of issuance to June 16, 2017 (the earliest redemption date) using the interest method.

The Series B convertible preferred stock is not redeemable.

Voting—Each holder of shares of preferred stock shall be entitled to the number of votes equal to the number of shares of common stock into which such shares of Preferred Stock could be converted and shall have voting rights and powers equal to the voting rights and powers of the common stock, and shall vote

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Notes to Financial Statements (continued)

11. Convertible Preferred Stock (continued)

together with the common stock as a single class on an as- converted basis on all matters as to which holders of common stock have the right to vote. So long as at least 3,000,000 shares of Series A remain outstanding, the holders of Series A will be allowed to elect three directors of the Company, the holders of the common stock will be allowed to elect two directors of the Company, and any remaining directors will be elected by the holders of preferred stock and common stock, voting together as a single class.

Conversion—Each share of preferred stock, at the option of the holder, is convertible into common stock determined by dividing the Original Series A issue price or Original Series B issue price, as applicable, by the conversion price applicable to such share in effect on the date the certificate is surrendered for conversion, subject to certain provisions for adjustment of the conversion price. Conversion of each share of preferred stock is automatic upon the closing of a firm commitment underwritten public offering with aggregate proceeds of not less than $30.0 million (before deduction of underwriting discounts and commissions) or the agreement or written consent of holders of at least seventy-five percent (75%) of the then-outstanding shares of preferred stock.

12. Stock-Based Awards

2011 Equity Incentive Plan

In 2011, the Company adopted the 2011 Equity Incentive Plan (the Plan). The Plan provides for the granting of stock-based awards to employees, directors, and consultants under terms and provisions established by the Board of Directors. Under the terms of the Plan, options may be granted at an exercise price not less than fair market value. For employees holding more than 10% of the voting rights of all classes of stock, the exercise prices for incentive and nonstatutory stock options must be at least 110% of fair market of the common stock on the grant date, as determined by the Board of Directors. The terms of options granted under the Plan may not exceed ten years.

Options granted generally vest over a period of four years. Typically, the vesting schedule for option grants to newly hired employees provides that 1/4 of the grant vests upon the first anniversary of the employee’s date of hire, with the remainder of the shares vesting monthly thereafter at a rate of 1/48 of the total shares subject to the option. All other options typically vest in equal monthly installments over the four-year vesting schedule.

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Notes to Financial Statements (continued)

12. Stock-Based Awards (continued)

A summary of activity under the 2011 Plan and related information are as follows:

		Options Outstanding			
	Shares Available for Grant	Number of Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (Years)	Aggregate Intrinsic Value (In thousands)
Outstanding — December 31, 2010	—	—	—	—	
Shares reserved	7,455,988	—	—		
Options granted	(4,550,000)	4,550,000	$ 0.10		
Outstanding — December 31, 2011	2,905,988	4,550,000	0.10	9.88	
Shares reserved	4,318,829	—	—		
Options granted	(1,565,000)	1,565,000	0.26		
Options exercised	—	(1,317,490)	0.10		
Options cancelled	30,000	(30,000)	0.10		
Outstanding — December 31, 2012	5,689,817	4,767,510	$ 0.15	9.11	$ 2,038
Vested and exercisable — December 31, 2012		311,048	$ 0.10	8.88	$ 149
Vested and expected to vest — December 31, 2012		4,633,816	$ 0.15	9.11	$ 1,981

The aggregate intrinsic values of options outstanding, vested and exercisable, and vested and expected to vest were calculated as the difference between the exercise price of the options and the estimated fair value of the Company's common stock as determined by the Company's Board of Directors as of December 31, 2012. The total intrinsic value of options exercised during the years ended December 31, 2011 and 2012 and for the period from April 22, 2010 (Inception) through December 31, 2012 was $0, $211,000 and $211,000, respectively.

The options outstanding and exercisable by exercise price as of December 31, 2012, are as follows:

	Options Outstanding		Options Exercisable		
Exercise Price	Number Outstanding	Weighted-Average Remaining Contractual Life (in Years)	Number Exercisable	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Life (in Years)
$0.10	3,202,510	8.88	311,048	$ 0.10	8.88
$0.26	1,565,000	9.60	—	—	—
	4,767,510	9.11	311,048	$ 0.10	8.88

The weighted-average estimated fair value of stock options granted was $0.11, $0.15 and $0.12 per share of the Company's common stock during the years ended December 31, 2011 and 2012 and for the period from April 22, 2010 (Inception) through December 31, 2012, respectively. No options were granted in 2010.

The total estimated fair value of options vested during the years ended December 31, 2011 and 2012 and for the period from April 22, 2010 (Inception) through December 31, 2012 was $6,000, $172,000 and $178,000, respectively.

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Notes to Financial Statements (continued)

12. Stock-Based Awards (continued)

Founder's Stock

In connection with the Series A preferred stock financing, the Company entered into a stock repurchase agreement with the founder on June 16, 2011, where the 8,000,000 common shares previously owned by the founder are now subject to repurchase by the Company at the original issuance price in the event that the founder's employment is terminated either voluntarily or involuntarily. Such repurchase rights lapse over a period of two years from June 16, 2011. The Company calculated the fair value of these restricted shares at the time the restriction was added to the shares as $1,199,000 and is recording this amount as stock-based compensation ratably as the repurchase rights lapse. Stock-based compensation expense pertaining to the founder's stock was $218,000, $713,000 and $931,000 during the years ended December 31, 2011 and 2012 and for the period from April 22, 2010 (Inception) through December 31, 2012, respectively. As of December 31, 2012, 2,000,000 of these shares remained subject to repurchase and $268,000 of stock compensation remained unamortized.

Stock-Based Compensation Expense

Total stock-based compensation recognized was as follows (in thousands):

	Year Ended December 31,		Period from April 22, 2010 (Inception) through December 31, 2012
	2011	2012	
Research and development	$ 28	$ 130	$ 158
General and administrative	226	761	987
Total stock-based compensation expense	$ 254	$ 891	$ 1,145

As of December 31, 2012, the total unrecognized compensation expense related to unvested options, net of estimated forfeitures, was $495,000, which the Company expects to recognize over an estimated weighted average period of 2.8 years.

In determining the fair value of the stock-based awards, the Company uses the Black-Scholes option-pricing model and assumptions discussed below. Each of these inputs is subjective and generally requires significant judgment to determine.

Expected Term—The Company's expected term represents the period that the Company's stock-based awards are expected to be outstanding and is determined using the simplified method (based on the mid-point between the vesting date and the end of the contractual term).

Expected Volatility—Since the Company is privately held and does not have any trading history for its common stock, the expected volatility was estimated based on the average volatility for comparable publicly traded biopharmaceutical companies over a period equal to the expected term of the stock option grants. When selecting comparable publicly traded biopharmaceutical companies on which it has based its expected stock price volatility, the Company selected companies with comparable characteristics to it, including enterprise value, risk profiles, position within the industry, and with historical share price information sufficient to meet the expected life of the stock-based awards. The historical volatility data was computed using the daily closing prices for the selected companies' shares during the equivalent period of the calculated expected term of the stock-based awards. The Company will continue to apply this process until a sufficient amount of historical information regarding the volatility of its own stock price becomes available.

Risk-Free Interest Rate—The risk-free interest rate is based on the U.S. Treasury zero coupon issues in effect at the time of grant for periods corresponding with the expected term of option.

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Notes to Financial Statements (continued)

12. Stock-Based Awards (continued)

Expected Dividend—The Company has never paid dividends on its common stock and has no plans to pay dividends on its common stock. Therefore, the Company used an expected dividend yield of zero.

The fair value of stock option awards was estimated at the date of grant using a Black-Scholes option-pricing model with the following weighted average assumptions:

	Year Ended December 31,		Period from April 22, 2010 (Inception) through December 31, 2012
	2011	2012	
Expected term	6.25 years	6.25 years	6.25 years
Expected volatility	75%	67%	73%
Risk-free interest rate	1.15%	0.62%	1.01%
Dividend yield	—	—	—

13. Income Taxes

The Company did not record a provision or benefit for income taxes during the years ended December 31, 2011 and 2012. The Company has incurred net operating losses since inception. The Company has not reflected any benefit of such net operating loss carryforwards in the accompanying financial statements. The Company has established a full valuation allowance against its deferred tax assets due to the uncertainty surrounding the realization of such assets.

The effective tax rate of our provision for income taxes differs from the federal statutory rate as follows:

	Year Ended December 31,	
	2011	2012
Federal statutory income tax rate	34.0%	34.0%
State income taxes, net of federal benefit	5.3	6.0
Federal tax credits	5.5	8.6
Nondeductible permanent items	(1.5)	—
Stock compensation	(1.3)	(1.9)
Change in valuation allowance	(32.9)	(46.0)
Adjustment of loss carryforwards	(9.0)	—
Other	(0.1)	(0.7)
	0.0%	0.0%

The tax effect of temporary differences that give rise to significant portions of the deferred tax assets is presented below (in thousands):

	December 31,	
	2011	2012
Deferred tax assets:		
Net operating loss carryforwards	$ 1,812	$ 6,186
Tax credits	756	3,619
Other	(18)	272
Total deferred tax assets	2,550	10,077
Valuation allowance	(2,550)	(10,077)
Net deferred tax assets	$ —	$ —

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Notes to Financial Statements (continued)

13. Income Taxes (continued)

Additionally, the future utilization of the net operating loss carryforwards to offset future taxable income may be subject to an annual limitation, pursuant to Internal Revenue Code Section 382, as a result of ownership changes that may have occurred previously or that could occur in the future. A Section 382 analysis to determine the limitation of the net operating loss carryforwards has not been performed. Until this analysis has been performed, the deferred tax assets for net operating losses of $725,000 generated through December 31, 2012, have been removed from the deferred tax asset schedule and a corresponding decrease to the valuation allowance has been recorded. This represents the amount estimated to expire before utilization, assuming a change in ownership has occurred. The Company recorded unrecognized tax benefits for uncertainty in income taxes. Due to the existence of the valuation allowance, future changes in unrecognized tax benefits will not impact the effective tax rate. The valuation allowance increased by $7.5 million and $10.1 million during the year ended December 31, 2012 and for the period from April 22, 2010 (Inception) through December 31, 2012.

As of December 31, 2012, the Company had approximately $14.9 million and $19.3 million of federal and state NOL carryforwards available to reduce future taxable income that will begin to expire in 2030 for federal and 2030 for state tax purposes.

As of December 31, 2012, the Company also had research and development tax credit carryforwards of approximately $152,000 and $296,000 for federal and state purposes available to offset future regular and alternative minimum taxable income. If not utilized, the federal carryforwards will expire in various amounts beginning in 2030, and the state credits can be carried forward indefinitely.

On January 2, 2013, the American Taxpayer Relief Act of 2012 (the Act) was passed in to law. The Act included a retroactive extension of the U.S. research credit for 2012. Since the effects of the tax law changes are recognized in the first period the Company would recognize $109,000 of additional research credits as a discrete item during the first quarter of 2013. The tax effects of the research credits will be offset by valuation allowance and will not impact the financial statements.

As of December 31, 2012, the Company had an Orphan Drug Credit of approximately $4.8 million for federal tax purposes available to offset future regular and alternative minimum taxable income.

Uncertain Tax Positions

A reconciliation of the Company's unrecognized tax benefits for the years ended December 31, 2011 and 2012 is as follows (in thousands):

	December 31,	
	2011	2012
Balance at beginning of year	$ —	$ 324
Additions based on tax positions related to current year	324	1,227
Additions (reductions) for tax positions of prior years	—	—
Balance at end of year	$324	$1,551

The entire amount of the unrecognized tax benefits would not impact the Company's effective tax rate if recognized. The Company has elected to include interest and penalties as a component of tax expense. During the years ended December 31, 2011 and 2012, the Company did not recognize accrued interest and penalties related to unrecognized tax benefits. The Company does not anticipate that the amount of existing unrecognized tax benefits will significantly increase or decrease during the next 12 months.

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)
Notes to Financial Statements (continued)

13. Income Taxes (continued)

The Company files income tax returns in the U.S. federal jurisdiction and California tax jurisdictions. The federal and state income tax returns from inception to December 31, 2012 remain subject to examination.

14. Net Loss and Pro Forma Net Loss per Share Attributable to Common Stockholders

The following table sets forth the computation of the basic and diluted net loss per share attributable to common stockholders during the years ended December 31, 2011 and 2012 (in thousands, except share and per share data):

	Year Ended December 31,	
	2011	2012
Numerator:		
Net loss	$ (6,849)	$ (16,334)
Accretion and dividends on convertible preferred stock	(617)	(3,227)
Net loss attributable to common stockholders	$ (7,466)	$ (19,561)
Denominator:		
Weighted-average common shares outstanding	9,431,338	9,775,885
Less: weighted-average unvested common shares subject to repurchase	(4,361,644)	(5,459,017)
Weighted-average shares used to compute net loss per share attributable to common stockholders, basic and diluted	5,069,694	4,316,868
Net loss per share attributable to common stockholders, basic and diluted	$ (1.47)	$ (4.53)

The following weighted-average outstanding common stock equivalents were excluded from the computation of diluted net loss per share attributable to common stockholders for the periods presented because including them would have been antidilutive:

	December 31,	
	2011	2012
Convertible preferred stock	9,792,843	26,188,059
Stock options to purchase common stock	556,386	4,899,904
Common stock subject to repurchase	4,361,644	5,459,017
Convertible preferred stock warrants	921,931	1,027,662
	15,632,804	37,574,642

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)

Notes to Financial Statements (continued)

14. Net Loss and Pro Forma Net Loss per Share Attributable to Common Stockholders (continued)

The following table sets forth the computation of the Company's unaudited pro forma basic and diluted net loss per share attributable to common stockholders during the year ended December 31, 2012 (in thousands, except for share and per share amounts):

	Year Ended December 31, 2012 (Unaudited)
Net loss	$
Dividends to be paid on convertible preferred stock	
Change in fair value of convertible preferred stock warrant liabilities	
Net loss used in computing pro forma net loss per share attributable to common stockholders, basic and diluted	$
Shares used in computing net loss per share attributable to common stockholders, basic and diluted	
Pro forma adjustments to reflect assumed conversion of convertible preferred stock	
Shares used in computing pro forma net loss per share attributable to common stockholders, basic and diluted	
Pro forma net loss per share attributable to common stockholders, basic and diluted	$

15. Subsequent Events

On June 26, 2013, the Company notified BRI that it was exercising its option to license the rights to triheptanoin in all territories outside of the United States, Canada and Mexico. The option exercise fee associated with this is $750,000.

On August 29, 2013, the Company entered into a collaboration and license agreement with Kyowa Hakko Kirin Co. LTD. (KHK). Under the terms of this collaboration and license agreement, the Company and KHK will collaborate on the development and commercialization of certain products containing KRN23, an antibody directed towards FGF23, in the field of orphan diseases in the United States and Canada, or the profit share territory, and in the European Union, Switzerland, and Turkey, or the European territory, and the Company will have the right to develop and commercialize such products in the field of orphan diseases in Mexico and Central and South America, or Latin America. In the field of orphan diseases, and except for ongoing studies being conducted by KHK, the Company will be the lead party for development activities in the profit share territory and in the European territory until the applicable transition date. The Company will share the costs for development activities in the profit share territory and European territory conducted pursuant to the development plan before the applicable transition date equally with KHK. On the applicable transition date in the relevant territory, KHK will become the lead party and be responsible for these costs. However, the Company will continue to share the costs of the studies commenced prior to the applicable transition date equally with KHK. The Company has the primary responsibility for conducting certain research and development services. The Company is obligated to provide assistance in accordance with the agreed upon development plan as well as participate on various committees. If KRN23 is approved, the Company and KHK will share commercial responsibilities and profits in the profit share territory until the applicable transition date, KHK will commercialize KRN23 in the European territory and the Company will develop and commercialize KRN23 in Latin America. KHK will manufacture and supply KRN23 for clinical use globally and will manufacture and supply KRN23 for commercial use in the profit share territory and Latin America.

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Unaudited Interim Condensed Financial Statements

ULTRAGENYX PHARMACEUTICAL INC.
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CONDENSED BALANCE SHEETS
(In thousands, except share and per share amounts)

	December 31, 2012 (Note 1)	September 30, 2013 (Unaudited)	Pro Forma Stockholders' Equity as of September 30, 2013 (Unaudited)
Assets			
Current assets:			
Cash and cash equivalents	$ 86,190	$ 6,049	
Short term investments	—	57,608	
Accounts receivable	—	38	
Prepaid expenses and other current assets	255	2,980	
Total current assets	86,445	66,675	
Property and equipment, net	1,362	1,308	
Restricted cash	476	451	
Other assets	33	158	
TOTAL ASSETS	$ 88,316	$ 68,592	
Liabilities, Convertible Preferred Stock and Stockholders' (Deficit) Equity			
Current liabilities:			
Accounts payable	$ 1,200	$ 2,890	
Accrued liabilities	1,913	2,643	$ 6,058
Deferred rent — current portion	75	75	
Total current liabilities	3,188	5,608	
Convertible preferred stock warrant liability	518	1,596	—
Other liabilities	270	234	
Total liabilities	3,976	7,438	
Commitments and contingencies			
Series A redeemable convertible preferred stock, par value of $0.001 per share — 35,377,556 shares authorized as of December 31, 2012 and September 30, 2013 (unaudited); 34,349,894 shares issued and outstanding as of December 31, 2012 and September 30, 2013 (unaudited), no shares authorized, issued and outstanding, pro forma (unaudited); redemption value of $90,704 of September 30, 2013 (unaudited)	37,458	44,073	—
Series B convertible preferred stock, par value of $0.001 per share — 27,081,680 shares authorized as of December 31, 2012 and September 30, 2013 (unaudited); 27,081,680 shares issued and outstanding as of December 31, 2012 and September 30, 2013 (unaudited), no shares authorized, issued and outstanding, pro forma (unaudited); aggregate liquidation preference of $76,316 as of September 30, 2013 (unaudited)	73,929	73,929	—
Stockholders' (deficit) equity:			
Common stock, par value of $0.001 per share — 85,000,000 shares authorized as of December 31, 2012 and September 30, 2013 (unaudited); 10,842,050 and 11,607,173 shares issued and outstanding as of December 31, 2012 and September 30, 2013 (unaudited), shares authorized, 73,038,747 shares issued and outstanding, pro forma (unaudited)	11	12	73
Additional paid-in capital	—	—	116,122
Accumulated other comprehensive loss	—	(14)	(14)
Deficit accumulated during the development stage	(27,058)	(56,846)	(56,846)
Total stockholders' (deficit) equity	(27,047)	(56,848)	$ 59,335
Total liabilities, convertible preferred stock and stockholders' (deficit) equity	$ 88,316	$ 68,592	

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.
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CONDENSED STATEMENTS OF OPERATIONS
(Unaudited)
(In thousands, except share and per share amounts)

	Nine Months Ended September 30,		Period from April 22, 2010 (Inception) Through September 30, 2013
	2012	2013	
Operating expenses:			
Research and development	$ 8,866	$ 19,625	$ 37,941
General and administrative	2,441	3,130	8,610
Total operating expenses	11,307	22,755	46,551
Loss from operations	(11,307)	(22,755)	(46,551)
Other income (expense), net:			
Interest income	—	157	162
Interest expense	—	—	(318)
Other expense	(97)	(1,155)	(1,535)
Total other income (expense), net	(97)	(998)	(1,691)
Net loss	$ (11,404)	$ (23,753)	$ (48,242)
Net loss attributable to common stockholders	$ (12,749)	$ (31,624)	
Net loss per share attributable to common stockholders, basic and diluted	$ (3.94)	$ (3.09)	
Shares used in computing net loss per share attributable to common stockholders, basic and diluted	3,235,308	10,220,034	
Pro forma net loss per share attributable to common stockholders, basic and diluted		$	
Shares used in computing pro forma net loss per share attributable to common stockholders, basic and diluted			

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.
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CONDENSED STATEMENTS OF COMPREHENSIVE LOSS
(Unaudited)
(In thousands)

	Nine Months Ended September 30,		Period from April 22, 2010 (Inception) Through September 30,
	2012	2013	2013
Net loss	$(11,404)	$(23,753)	$ (48,242)
Other comprehensive loss:			
Unrealized loss on available-for-sale securities	—	(14)	(14)
Total comprehensive loss	$(11,404)	$(23,767)	$ (48,256)

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)
CONDENSED STATEMENTS OF CASH FLOWS
(Unaudited)
(In thousands)

	Nine Months Ended September 30,		Period from April 22, 2010 (Inception) Through September 30, 2013
	2012	2013	
Cash flows from operating activities:			
Net loss	$(11,404)	$(23,753)	$ (48,242)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation and amortization	221	332	685
Amortization of premium on securities	—	972	972
Noncash interest expense	—	—	318
Stock-based compensation	694	447	1,592
Revaluation of preferred stock warrant liability	67	1,078	1,393
Changes in operating assets and liabilities:			
Accounts receivable	—	(38)	(38)
Prepaid expenses and other current assets	(10)	(2,725)	(2,980)
Other assets	5	(125)	(158)
Accounts payable	817	1,690	2,890
Accrued expenses and other liabilities	820	695	2,952
Net cash used in operating activities	(8,790)	(21,427)	(40,616)
Cash flows from investing activities:			
Purchase of property and equipment	(967)	(278)	(1,993)
Decrease (increase) in restricted cash	(100)	25	(451)
Purchase of investments	—	(63,056)	(63,056)
Proceeds from maturities of investments	—	4,462	4,462
Net cash used in investing activities	(1,067)	(58,847)	(61,038)
Cash flows from financing activities:			
Net proceeds from issuance of convertible preferred stock, net of issuance costs	15,080	—	103,888
Net proceeds from issuance of common stock	48	133	265
Proceeds from issuance of promissory notes	100	—	3,550
Net cash provided by financing activities	15,228	133	107,703
Net increase (decrease) in cash and cash equivalents	5,371	(80,141)	6,049
Cash and cash equivalents — Beginning of period	10,645	86,190	—
Cash and cash equivalents — End of period	$ 16,016	$ 6,049	$ 6,049

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)

Notes to Unaudited Interim Condensed Financial Statements

1. Summary of Significant Accounting Policies

Unaudited Interim Condensed Financial Statements

The interim condensed balance sheet as of September 30, 2013 and the statements of operations, comprehensive loss and cash flows for the nine months ended September 30, 2012 and 2013 are unaudited. The unaudited interim financial statements have been prepared on the same basis as the annual financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary to present fairly the Company's financial position as of September 30, 2013 and its results of operations and cash flows for the nine months ended September 30, 2012 and 2013. The financial data and the other financial information disclosed in these notes to financial statements related to the nine month periods are also unaudited. The results of operations for the nine months ended September 30, 2013 are not necessarily indicative of the results to be expected for the year ending December 31, 2013 or for any other future annual or interim period. The condensed balance sheet as of December 31, 2012 included herein was derived from the audited financial statements as of that date. These financial statements should be read in conjunction with the Company's audited financial statements included elsewhere in this prospectus.

Unaudited Pro Forma Stockholders' Equity

The pro forma stockholders' equity as of September 30, 2013 presents the Company's stockholders' equity as though all of the Company's outstanding convertible preferred stock had automatically converted into shares of common stock upon the completion of an initial public offering (IPO) of the Company's common stock. In addition, the pro forma stockholders' equity assumes the reclassification of the convertible preferred stock warrant liability to additional paid-in capital upon completion of an IPO of the Company's common stock, as the warrants upon an IPO become common stock warrants that are not subject to remeasurement, and the payment in cash to the holders of the Company's preferred stock of a dividend concurrent with the conversion of the Company's convertible preferred stock. The pro forma stockholders' equity also assumes the payment of a dividend on the convertible preferred stock which is triggered upon the conversion of the convertible preferred stock to common stock. For the purpose of this pro forma presentation, the dividend payable has been calculated as if the conversion occurred as of November 8, 2013, the date of this filing.

Use of Estimates

The accompanying financial statements have been prepared in accordance with U.S. generally accepted accounting principles (GAAP). The preparation of the financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities and the reported amounts of expenses in the financial statements and the accompanying notes. On an ongoing basis, management evaluates its estimates, including those related to clinical trial accruals, fair value of assets and liabilities, convertible preferred stock and related warrants, common stock, income taxes and stock-based compensation. Management bases its estimates on historical experience and on various other market-specific and relevant assumptions that management believes to be reasonable under the circumstances. Actual results could differ from those estimates.

Additional Capital Requirements

The Company has incurred significant losses and negative cash flows from operations. At September 30, 2013, the Company had a total deficit accumulated during the development stage of $56.8 million and cash, cash equivalents and marketable securities of $63.7 million. Management believes that currently available resources

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)

Notes to Unaudited Interim Condensed Financial Statements (continued)

1. Summary of Significant Accounting Policies (continued)

will provide sufficient funds to enable us to meet the Company's obligations through at least December 31, 2013. However, if the Company's anticipated operating results are not achieved in future periods, planned expenditures may need to be reduced in order to extend the time period over which the then-available resources would be able to fund its operations. The Company will need to raise additional capital to fully implement its business plan.

Cash and Cash Equivalents

The Company considers all highly liquid investments with original maturities of three months or less at the time of purchase to be cash equivalents. Cash equivalents consist primarily of money market funds and are stated at fair value.

Marketable Securities

All investments have been classified as "available-for-sale" and are carried at estimated fair value as determined based upon quoted market prices or pricing models for similar securities. Management determines the appropriate classification of its investments in debt securities at the time of purchase and reevaluates such designation as of each balance sheet date. Unrealized gains and losses are excluded from earnings and were reported as a component of comprehensive loss. Realized gains and losses and declines in fair value judged to be other than temporary, if any, on available-for-sale securities are included in interest income and other expense, respectively. The cost of securities sold is based on the specific-identification method. Interest on marketable securities is included in interest income.

Concentration of Credit Risk and Other Risks and Uncertainties

Financial instruments that potentially subject the Company to a concentration of credit risk consist of cash, cash equivalents, and marketable securities. The Company's cash, cash equivalents, and marketable securities are held by financial institutions that management believes are of high credit quality. The Company's investment policy limits investments to fixed income securities denominated and payable in U.S. dollars such as U.S. government obligations, money market instruments and funds, corporate bonds, and asset-backed securities and places restrictions on maturities and concentrations by type and issuer. Such deposits may, at times, exceed federally insured limits. The Company has not experienced any losses on its deposits of cash and cash equivalents and its accounts are monitored by management to mitigate risk. The Company is exposed to credit risk in the event of default by the financial institutions holding its cash and cash equivalents and corporate bond issuers to the extent recorded in the balance sheets.

Deferred Offering Costs

Deferred offering costs, which primarily consist of direct incremental legal and accounting fees relating to the IPO, are capitalized. The deferred offering costs will be offset against IPO proceeds upon the consummation of the offering. In the event the offering is terminated, deferred offering costs will be expensed. As of September 30, 2013, $1.4 million of deferred offering costs were capitalized in prepaid and other current assets on the balance sheet. There were no such costs capitalized as of December 31, 2012.

Net Loss per Share Attributable to Common Stockholders

Basic net loss per share attributable to common stockholders is calculated by dividing the net loss attributable to common stockholders by the weighted average number of shares of common stock outstanding

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)
Notes to Unaudited Interim Condensed Financial Statements (continued)

1. Summary of Significant Accounting Policies (continued)

during the period without consideration of common stock equivalents. The net loss attributable to common stockholders is calculated by adjusting the net loss of the Company for the accretion on the Series A convertible preferred stock and cumulative dividends on Series A and B convertible preferred stock. Diluted net loss per share attributable to common stockholders is the same as basic net loss per share attributable to common stockholders, since the effects of potentially dilutive securities are antidilutive.

Unaudited Pro Forma Net Loss per Share Attributable to Common Stockholders

Pro forma basic and diluted net loss per share attributable to common stockholders has been computed to give effect to the conversion of the convertible preferred stock into common stock. Also, the numerator in the pro forma basic and diluted net loss per share attributable to common stockholders calculation has been adjusted to remove gains and losses resulting from remeasurement of the convertible preferred stock warrant liability as these amounts will be reclassified to additional paid-in capital upon a qualifying initial public offering of the Company's common stock, and has also been adjusted to reflect the payment of a dividend to the holders of the Company's preferred stock concurrent with the conversion of the Company's convertible preferred stock to common stock immediately prior to the completion of this offering. The pro forma net loss per share attributable to common stockholders does not include the shares expected to be sold and related proceeds to be received from an initial public offering.

Recently Issued Accounting Pronouncements

In February 2013, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2013-02, *Other Comprehensive Income (Topic 220): Reporting of Amounts Reclassified Out of Accumulated Other Comprehensive Income*. ASU No. 2013-02 supersedes the presentation requirements for reclassifications out of accumulated other comprehensive income in ASU 2011-05 and 2011-12 and requires an entity to provide additional information about reclassifications out of accumulated other comprehensive income. The Company adopted this guidance on January 1, 2013 on a prospective basis. The adoption of this amendment did not have a material impact on the Company's results of operations or financial position.

2. Fair Value Measurements

Financial assets and liabilities are recorded at fair value. The carrying amount of certain financial instruments, including cash and cash equivalents, marketable securities, accounts payable and accrued liabilities approximate fair value due to their relatively short maturities. Assets and liabilities recorded at fair value on a recurring basis in the balance sheets are categorized based upon the level of judgment associated with the inputs used to measure their fair values. Fair value is defined as the exchange price that would be received for an asset or an exit price that would be paid to transfer a liability in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. The authoritative guidance on fair value measurements establishes a three-tier fair value hierarchy for disclosure of fair value measurements as follows:

Level 1—Inputs are unadjusted, quoted prices in active markets for identical assets or liabilities at the measurement date;

Level 2—Inputs are observable, unadjusted quoted prices in active markets for similar assets or liabilities, unadjusted quoted prices for identical or similar assets or liabilities in markets that are not active, or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the related assets or liabilities; and

ULTRAGENYX PHARMACEUTICAL INC.
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Notes to Unaudited Interim Condensed Financial Statements (continued)

2. Fair Value Measurements (continued)

Level 3—Unobservable inputs that are significant to the measurement of the fair value of the assets or liabilities that are supported by little or no market data.

The Company's financial instruments consist of Level 1 and 2 assets and Level 3 liabilities. Where quoted prices are available in an active market, securities are classified as Level 1. Level 1 assets consist primarily of highly liquid money market funds that are included in cash and cash equivalents and restricted cash. The Company's Level 2 investments include corporate bonds. Level 2 inputs are based upon quoted prices for similar instruments in active markets, quoted prices for identical or similar instruments in markets that are not active and model-based valuation techniques for which all significant inputs are observable in the market or can be corroborated by observable market data for substantially the full term of the assets. Where applicable, these models project future cash flows and discount the future amounts to a present value using market-based observable inputs obtained from various third-party data providers, including but not limited to, benchmark yields, interest rate curves, reported trades, broker/dealer quotes and market reference data. Level 3 liabilities consist of the convertible preferred stock warrant liability. The determination of the fair value of the convertible preferred stock warrants is discussed in Note 4. Generally, increases or decreases in the fair value of the underlying convertible preferred stock would result in a directionally similar impact in the fair value measurement of the warrant liability.

The following table sets forth the fair value of the Company's financial assets and liabilities measured at fair value on a recurring basis based on the three-tier fair value hierarchy (in thousands):

	December 31, 2012			
	Level 1	Level 2	Level 3	Total
Financial Assets:				
Money market funds	$ 376	$ —	$ —	$ 376
Total financial assets	$ 376	$ —	$ —	$ 376
Financial Liabilities:				
Convertible preferred stock warrant liability	$ —	$ —	$ 518	$ 518
Total financial liabilities	$ —	$ —	$ 518	$ 518

	September 30, 2013			
	Level 1	Level 2	Level 3	Total
Financial Assets:				
Money market funds	$ 4,835	$ —	$ —	$ 4,835
Commercial paper	—	999	—	999
Corporate bonds	—	56,765	—	56,765
Total financial assets	$ 4,835	$57,764	$ —	$62,599
Financial Liabilities:				
Convertible preferred stock warrant liability	$ —	$ —	$ 1,596	$ 1,596
Total financial liabilities	$ —	$ —	$ 1,596	$ 1,596

ULTRAGENYX PHARMACEUTICAL INC.
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Notes to Unaudited Interim Condensed Financial Statements (continued)

2. Fair Value Measurements (continued)

The following table sets forth a summary of the changes in the fair value of the Company's Level 3 financial liabilities (in thousands):

Balance as of December 31, 2012	$ 518
Change in fair value recorded in other expense	1,078
Balance as of September 30, 2013	$1,596

3. Balance Sheet Components

Cash Equivalents, Restricted Cash and Marketable Securities

The fair values of cash equivalents, restricted cash and marketable securities classified as available-for-sale securities, consisted of the following:

	December 31, 2012			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Money market funds	$ 376	$ —	$ —	$ 376
Total	$ 376	$ —	$ —	$ 376

	September 30, 2013			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
Money market funds	$ 4,835	$ —	$ —	$ 4,835
Commercial paper	999	—	—	999
Corporate bonds	56,779	7	(21)	56,765
Total	$ 62,613	$ 7	$ (21)	$ 62,599

The available-for-sale securities held as of September 30, 2013 had contractual maturities of less than two years.

Property and Equipment, net

Property and equipment, net consists of the following (in thousands):

	December 31, 2012	September 30, 2013
Research and development equipment	$ 225	$ 264
Office furniture and equipment	266	289
Computer equipment	187	235
Software	34	58
Leasehold improvements	1,003	1,147
Property and equipment, gross	1,715	1,993
Less accumulated depreciation and amortization	(353)	(685)
Property and equipment, net	$ 1,362	$ 1,308

ULTRAGENYX PHARMACEUTICAL INC.
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Notes to Unaudited Interim Condensed Financial Statements (continued)

3. Balance Sheet Components (continued)

Depreciation and amortization expense for the nine months ended September 30, 2012 and 2013 and the period from April 22, 2010 (Inception) through September 30, 2013 was $221,000, $332,000 and $685,000, respectively.

Accrued Liabilities

Accrued liabilities consist of the following (in thousands):

	December 31, 2012	September 30, 2013
Research and clinical trial expenses	$ 595	$ 733
Payroll and related expenses	1,302	1,370
Accrued professional services	—	440
Other	16	100
Total accrued liabilities	$ 1,913	$ 2,643

4. Convertible Preferred Stock Warrants

As of December 31, 2012 and September 30, 2013, outstanding warrants consisted of the following:

Convertible Preferred Stock Warrants:	Number of Warrants	Date Issued	Term	Exercise Price
Series A convertible preferred stock	241,803	June 2010	10 years	$ 1.034
Series A convertible preferred stock	592,417	February 2011	10 years	1.034
Series A convertible preferred stock	193,442	June 2011	10 years	1.034
Total convertible preferred stock warrants	1,027,662			

The fair value of the warrants was estimated to be $518,000 and $1.6 million as of December 31, 2012 and September 30, 2013, respectively. The key assumptions in the option-pricing valuation method as of December 31, 2012 and September 30, 2013 are summarized in the table below.

	December 31, 2012	September 30, 2013
Value of Company equity	$145.8 million	$240.3 million
Expected volatility	75.0%	75.0%
Expected time to liquidity event	2.0 years	0.5 – 1.5 years
Risk-free interest rate	0.25%	0.04 – 0.22%

The Company recorded $67,000, $1.1 million and $1.4 million to other expense for the nine months ended September 30, 2012 and 2013 and for the period from April 22, 2010 (Inception) through September 30, 2013, respectively, representing the change in fair value of the warrants between the issuance date and the end of the reporting period.

5. Convertible Preferred Stock

The holders of the Series A and Series B convertible preferred stock are entitled to receive dividends at the rate of $0.062 per share per annum, payable in the form of cash. Dividends accrue from day to day, whether or not declared, but will be paid only when, as, and if declared by the Board of Directors. After dividends have been

ULTRAGENYX PHARMACEUTICAL INC.
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Notes to Unaudited Interim Condensed Financial Statements (continued)

5. Convertible Preferred Stock (continued)

paid to the holders of the preferred stock, any additional dividends shall be paid among the holders of the preferred stock and common stock then outstanding in proportion to the greatest whole number of shares of common stock held (assuming conversion of Preferred Stock). During 2012, $2.1 million of dividends were declared and paid to holders of Series A convertible preferred stock in the form of additional Series A convertible preferred stock. Dividends in arrears as of December 31, 2012 and September 30, 2013, totaled $159,000 and $3.0 million, respectively, for both series of preferred stock.

The Company initially recorded the Series A and Series B convertible preferred stock at their issuance price, which represents the carrying value. The Series A convertible preferred stock is redeemable at any time after June 16, 2017 once a written request to redeem such stock is received by the Company from holders of not less than seventy-five percent of the then outstanding Series A convertible preferred stock. As only the passage of time is required for the Series A convertible preferred stock to become redeemable, the difference in the initial carrying value of the Series A convertible preferred stock and their total redemption value is being accreted from the issuance date through the first redemption date of June 16, 2017. The Company recorded accretion of $116,000 and $6.6 million for the nine months ended September 30, 2012 and 2013, respectively.

6. License Agreements

Baylor Research Institute

In September 2012, the Company entered into a license agreement with Baylor Research Institute (BRI). Under the terms of this license agreement, BRI exclusively licensed to the Company certain intellectual property related to triheptanoin for North America. Under the license agreement, the Company paid BRI an up-front fee of $250,000 which was recorded as research and development expense during the year ended December 31, 2012.

The Company has an exclusive option to expand the licensed territory to a worldwide license if the previous holder of such territorial rights allows that option to lapse. The previous holder’s option lapsed on December 31, 2012, and on June 26, 2013, the Company notified BRI that it was exercising its exclusive option to expand the licensed territory. The fee associated with this option exercise was $750,000, which was recorded as research and development expense during the nine months ended September 30, 2013.

Kyowa Hakko Kirin Co. LTD.

On August 29, 2013, the Company entered into a collaboration and license agreement with Kyowa Hakko Kirin Co. Ltd. (KHK). Under the terms of this collaboration and license agreement, the Company and KHK will collaborate on the development and commercialization of certain products containing KRN23, an antibody directed towards FGF23, in the field of orphan diseases in the United States and Canada, or the profit share territory, and in the European Union, Switzerland, and Turkey, or the European territory, and the Company will have the right to develop and commercialize such products in the field of orphan diseases in Mexico and Central and South America, or Latin America. In the field of orphan diseases, and except for ongoing studies being conducted by KHK, the Company will be the lead party for development activities in the profit share territory and in the European territory until, with respect to the profit share territory, the fifth anniversary of the first commercial sale in the United States in the first indication and, with respect to the European territory, the date on which marketing approval for a licensed product for the first indication is obtained in the European territory on a country-by-country basis; each such date is referred to herein as the applicable transition date. The Company will share the costs for development activities in the profit share territory and European territory conducted pursuant to the development plan before the applicable transition date equally with KHK. On the applicable transition date in the relevant territory, KHK will become the lead party and be responsible for these costs. However, the

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)

Notes to Unaudited Interim Condensed Financial Statements (continued)

6. License Agreements (continued)

Company will continue to share the costs of the studies commenced prior to the applicable transition date equally with KHK. The Company has the primary responsibility for conducting certain research and development services. The Company is obligated to provide assistance in accordance with the agreed upon development plan as well as participate on various committees. If KRN23 is approved, the Company and KHK will share commercial responsibilities and profits in the profit share territory until the applicable transition date, KHK will commercialize KRN23 in the European territory and the Company will develop and commercialize KRN23 in Latin America. KHK will manufacture and supply KRN23 for clinical use globally and will manufacture and supply KRN23 for commercial use in the profit share territory and Latin America.

The Company has incurred net development costs of $42,000 in connection with this agreement during the nine months ended September 30, 2013 and such costs have been recognized as research and development expenses.

7. Stock-Based Awards

2011 Equity Incentive Plan

The following table summarizes option activity under the 2011 Plan and related information during the nine months ended September 30, 2013:

	Shares Available for Grant	Options Outstanding	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (Years)	Aggregate Intrinsic Value (in thousands)
Outstanding — December 31, 2012	5,689,817	4,767,510	$ 0.15	9.11	
Options granted	(1,575,000)	1,575,000	0.66		
Options exercised	—	(765,123)	0.17		
Options cancelled	791,667	(791,667)	0.40		
Outstanding — September 30, 2013	4,906,484	4,785,720	$ 0.27	8.65	$ 9,170
Vested and exercisable — September 30, 2013		806,241	$ 0.13	8.26	$ 1,661
Vested and expected to vest — September 30, 2013		4,666,336	$ 0.27	8.65	$ 8,945

The aggregate intrinsic values of options outstanding, vested and exercisable, and vested and expected to vest were calculated as the difference between the exercise price of the options and the estimated fair value of the Company's common stock as determined by the Company's Board of Directors as of September 30, 2013. The total intrinsic value of options exercised during the nine months ended September 30, 2012 and 2013 was $77,000 and $622,000, respectively.

The total estimated fair value of options vested during the nine months ended September 30, 2012 and 2013 was $83,000 and $158,000, respectively.

Founder's Stock

On June 16, 2011, 8,000,000 common shares were issued to the Company's founder, which are subject to repurchase by the Company at the original issuance price in the event that the founder's employment is

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)

Notes to Unaudited Interim Condensed Financial Statements (continued)

7. Stock-Based Awards (continued)

terminated either voluntarily or involuntarily. Such repurchase rights lapse over a period of two years from June 16, 2011. The Company calculated the fair value of these restricted shares at the time the restriction was added to the shares as $1.2 million and was recording this amount as stock-based compensation ratably as the repurchase rights lapse. As of September 30, 2013, all of the founder's shares have vested and thus are no longer subject to repurchase.

Stock Compensation Expense

Total stock-based compensation recognized for stock-based awards was as follows (in thousands):

	Nine Months Ended September 30,		Period from April 22, 2010 (Inception) through September 30, 2013
	2012	2013	
Research and development	$ 98	$ 139	$ 297
General and administrative	596	308	1,295
Total stock-based compensation expense	$ 694	$ 447	$ 1,592

As of September 30, 2013, there was $799,000 of total unrecognized compensation costs, net of estimated forfeitures, related to nonvested stock option awards that will be recognized on a straight-line basis over the weighted average remaining period of 2.4 years.

The weighted-average estimated fair value of stock options granted was $0.15 and $0.44 per share of the Company's common stock during the nine months ended September 30, 2012 and 2013, respectively. The estimated grant date fair value of the Company's equity-based awards issued to employees was calculated using the Black-Scholes option-pricing model, based on the following assumptions:

	Nine Months Ended September 30,	
	2012	2013
Expected term	6.25 years	6.25 years
Expected volatility	67%	75%
Risk-free interest rate	0.61%	0.92%
Dividend yield	—	—

ULTRAGENYX PHARMACEUTICAL INC.
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Notes to Unaudited Interim Condensed Financial Statements (continued)

8. Net Loss and Pro Forma Net Loss per Share Attributable to Common Stockholders

The following table sets forth the computation of the basic and diluted net loss per share attributable to common stockholders during the nine months ended September 30, 2012 and 2013 (in thousands, except share and per share data):

	Nine Months Ended September 30,	
	2012	2013
Numerator:		
Net loss	$ (11,404)	$ (23,753)
Accretion and dividends on convertible preferred stock	(1,345)	(7,871)
Net loss attributable to common stockholders	$ (12,749)	$ (31,624)
Denominator:		
Weighted-average common shares outstanding	9,592,972	11,062,525
Less: weighted-average unvested common shares subject to repurchase	(6,357,664)	(842,491)
Weighted average shares used to compute net loss per share attributable to common stockholders, basic and diluted	3,235,308	10,220,034
Net loss per share attributable to common stockholders, basic and diluted	$ (3.94)	$ (3.09)

The following weighted-average outstanding common stock equivalents were excluded from the computation of diluted net loss per share attributable to common stockholders for the periods presented because including them would have been antidilutive:

	September 30,	
	2012	2013
Convertible preferred stock	30,700,953	61,431,574
Stock options to purchase common stock	4,775,452	5,014,758
Common stock subject to repurchase	6,357,664	842,491
Convertible preferred stock warrants	1,027,662	1,027,662
	42,861,731	68,316,485

ULTRAGENYX PHARMACEUTICAL INC.
(A Development Stage Company)

Notes to Unaudited Interim Condensed Financial Statements (continued)

8. Net Loss and Pro Forma Net Loss per Share Attributable to Common Stockholders (continued)

The following table sets forth the computation of the Company's unaudited pro forma basic and diluted net loss per share attributable to common stockholders during the nine months ended September 30, 2013 (in thousands, except for share and per share amounts):

	Nine Months Ended September 30, 2013
Net loss	$
Dividends to be paid on convertible preferred stock	
Change in fair value of convertible preferred stock warrant liabilities	
Net loss used in computing pro forma net loss per share attributable to common stockholders, basic and diluted	$
Shares used in computing net loss per share attributable to common stockholders, basic and diluted	
Pro forma adjustments to reflect assumed conversion of convertible preferred stock	
Shares used in computing pro forma net loss per share attributable to common stockholders, basic and diluted	
Pro forma net loss per share attributable to common stockholders, basic and diluted	$

9. Defined Contribution Plan

In March 2013, the Company began to sponsor a 401(k) retirement plan, in which substantially all of its full-time employees are eligible to participate. Participants may contribute a percentage of their annual compensation to this plan, subject to statutory limitations. The Company did not provide any contributions during the nine months ended September 30, 2013.

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shares

Common stock

Prospectus

J.P. Morgan **Morgan Stanley**

Cowen and Company

Canaccord Genuity

, 201

Until , 201 , all dealers that buy, sell or trade in our common stock, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to the dealers' obligation to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.

PART II
Information Not Required in Prospectus

Item 13. Other Expenses of Issuance and Distribution.

The following table sets forth the costs and expenses, other than the underwriting discounts and commissions, payable by the registrant in connection with the sale of common stock being registered. All amounts are estimates except for the Securities and Exchange Commission, or SEC, registration fee, the FINRA filing fee and The NASDAQ Global Market listing fee.

Item	Amount to be paid
SEC registration fee	$11,109
FINRA filing fee	13,438
The NASDAQ Global Market Listing fee	*
Printing and engraving expenses	*
Legal fees and expenses	*
Accounting fees and expenses	*
Blue Sky, qualification fees and expenses	*
Transfer Agent fees and expenses	*
Miscellaneous expenses	*
Total	$ *

* To be provided by amendment

Item 14. Indemnification of Directors and Officers.

Section 145(a) of the Delaware General Corporation Law provides, in general, that a corporation may indemnify any person who was or is a party or is threatened to be made a party to any threatened, pending or completed action, suit or proceeding, whether civil, criminal, administrative or investigative (other than an action by or in the right of the corporation), because he or she is or was a director, officer, employee or agent of the corporation, or is or was serving at the request of the corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise, against expenses (including attorneys' fees), judgments, fines and amounts paid in settlement actually and reasonably incurred by the person in connection with such action, suit or proceeding, if he or she acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the corporation and, with respect to any criminal action or proceeding, had no reasonable cause to believe his or her conduct was unlawful.

Section 145(b) of the Delaware General Corporation Law provides, in general, that a corporation may indemnify any person who was or is a party or is threatened to be made a party to any threatened, pending or completed action or suit by or in the right of the corporation to procure a judgment in its favor because the person is or was a director, officer, employee or agent of the corporation, or is or was serving at the request of the corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise, against expenses (including attorneys' fees) actually and reasonably incurred by the person in connection with the defense or settlement of such action or suit if he or she acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the corporation, except that no indemnification shall be made with respect to any claim, issue or matter as to which he or she shall have been adjudged to be liable to the corporation unless and only to the extent that the Court of Chancery or other adjudicating court determines that, despite the adjudication of liability but in view of all of the circumstances of the case, he or she is fairly and reasonably entitled to indemnity for such expenses which the Court of Chancery or other adjudicating court shall deem proper.

Section 145(g) of the Delaware General Corporation Law provides, in general, that a corporation may purchase and maintain insurance on behalf of any person who is or was a director, officer, employee or agent of

the corporation, or is or was serving at the request of the corporation as a director, officer, employee or agent of another corporation, partnership, joint venture, trust or other enterprise against any liability asserted against such person and incurred by such person in any such capacity, or arising out of his or her status as such, whether or not the corporation would have the power to indemnify the person against such liability under Section 145 of the Delaware General Corporation Law.

Article VI of our amended and restated certificate of incorporation (the "Charter"), provides that no director of our company shall be personally liable to us or our stockholders for monetary damages for any breach of fiduciary duty as a director, except for liability (1) for any breach of the director's duty of loyalty to us or our stockholders, (2) for acts or omissions not in good faith or which involve intentional misconduct or a knowing violation of law, (3) in respect of unlawful dividend payments or stock redemptions or repurchases, or (4) for any transaction from which the director derived an improper personal benefit. In addition, our Charter provides that if the Delaware General Corporation Law is amended to authorize the further elimination or limitation of the liability of directors, then the liability of a director of our company shall be eliminated or limited to the fullest extent permitted by the Delaware General Corporation Law, as so amended.

Article VI of the Charter further provides that any repeal or modification of such article by our stockholders or amendment to the Delaware General Corporation Law will not adversely affect any right or protection existing at the time of such repeal or modification with respect to any acts or omissions occurring before such repeal or modification of a director serving at the time of such repeal or modification.

In connection with the sale of common stock being registered hereby, we have entered into indemnification agreements with each of our directors and our executive officers. These agreements will provide that we will indemnify each of our directors and such officers to the fullest extent permitted by law and the Charter.

We also maintain an insurance policy that covers certain liabilities of directors and officers of our company arising out of claims based on acts or omissions in their capacities as directors or officers.

In any underwriting agreement we enter into in connection with the sale of common stock being registered hereby, attached hereto as exhibit 1.1, the underwriters will agree to indemnify, under certain conditions, us, our directors, our officers and persons who control us within the meaning of the Securities Act against certain liabilities.

Item 15. Recent Sales of Unregistered Securities.

In the three years preceding the filing of this registration statement, we or our California corporation predecessor have issued the following securities that were not registered under the Securities Act. Issuances made prior to June 2011 were made by Ultragenyx Pharmaceutical, Inc., a California corporation. In connection with our reincorporation into Delaware in June 2011, each outstanding share of common stock was converted into 80 shares of the new Delaware corporation.

Common Stock Purchase Agreements

On April 27, 2010, we issued 100,000 shares of common stock for consideration of $0.01 per share, for an aggregate purchase price of $1,000.00 to Emil D. Kakkis. Emil D. Kakkis is our current President and Chief Executive Officer and one of our directors.

On November 19, 2010, we issued 3,000 shares of common stock for consideration of $0.01 per share, for an aggregate purchase price of $30.00 to Nobelpharma Co. Ltd.

On February 11, 2011, we issued 1,030 shares of common stock for consideration of $0.01 per share, for an aggregate purchase price of $10.30 to William E. Aliski. Mr. Aliski is one of our directors.

On February 28, 2011, we issued 1,030 shares of common stock for consideration of $0.01 per share, for an aggregate purchase price of $10.30 to Jonathan K. Wright.

On March 11, 2011, we issued 1,667 shares of common stock for consideration of $0.01 per share, for an aggregate purchase price of $16.67 to William E. Aliski. Mr. Aliski is one of our directors.

On March 28, 2011, we issued 1,030 shares of common stock for consideration of $0.10 per share, for an aggregate purchase price of $103.00 to Steven Jungles. Mr. Jungles is our Senior Vice President, Technical Operations.

On April 6, 2011, we issued 11,300 shares of common stock for consideration of $0.01 per share, for an aggregate purchase price of $113.00 to John Klock.

We claimed exemption from registration under the Securities Act for the sale and issuance of these shares of common stock by virtue of Section 4(a)(2) and/or Regulation D promulgated thereunder as transactions not involving any public offering. All of the purchasers of the shares of common stock for which we relied on Section 4(a)(2) and/or Regulation D represented that they were accredited investors as defined under the Securities Act. We claimed such exemption on the basis that (a) the purchasers in each case represented that they intended to acquire the securities for investment only and not with a view to the distribution thereof and that they either received adequate information about the registrant or had access, through employment or other relationships, to such information and (b) appropriate legends were affixed to the stock certificates issued in such transactions.

Convertible Notes and Series A Convertible Preferred Stock Warrants

On June 30, 2010, we entered into a Note and Warrant Purchase Agreement with Emil D. Kakkis, our President and Chief Executive Officer and one of our directors. Pursuant to the Note and Warrant Purchase Agreement, we issued a convertible promissory note in the amount of $1.0 million to Dr. Kakkis and also issued him a warrant to purchase up to 241,803 shares of our Series A convertible preferred stock.

On February 15, 2011, we entered into a Note and Warrant Purchase Agreement with the John and Cynthia Klock Trust. Pursuant to the Note and Warrant Purchase Agreement, we issued a convertible promissory note in the amount of $1.5 million to the John and Cynthia Klock Trust and also issued to the trust a warrant to purchase up to 507,786 shares of our Series A convertible preferred stock.

On February 23, 2011, we entered into a Note and Warrant Purchase Agreement with William Aliski, one of our directors. Pursuant to the Note and Warrant Purchase Agreement, we issued a convertible promissory note in the amount of $250,000 to Mr. Aliski and also issued him a warrant to purchase up to 84,631 shares of our Series A convertible preferred stock.

On June 14, 2011, we entered into a Note and Warrant Purchase Agreement with Emil D. Kakkis, our President and Chief Executive Officer and one of our directors. Pursuant to the Note and Warrant Purchase Agreement, we issued a convertible promissory note in the amount of $300,000 to Dr. Kakkis and also issued him a warrant to purchase up to 72,541 shares of our Series A convertible preferred stock.

On June 14, 2011, we entered into a second Note and Warrant Purchase Agreement with Emil D. Kakkis, our President and Chief Executive Officer and one of our directors. Pursuant to the Note and Warrant Purchase Agreement, we issued a convertible promissory note in the amount of $500,000 to Dr. Kakkis and also issued him a warrant to purchase up to 120,901 shares of our Series A convertible preferred stock.

We claimed exemption from registration under the Securities Act for the sale and issuance of these securities by virtue of Section 4(a)(2) and/or Regulation D promulgated thereunder as transactions not involving any public offering. All of the purchasers of unregistered securities for which we relied on Section 4(a)(2) and/or Regulation D represented that they were accredited investors as defined under the Securities Act. We claimed such exemption on the basis that (a) the purchasers in each case represented that they intended to acquire the securities for investment only and not with a view to the distribution thereof and that they either received adequate information about the registrant or had access, through employment or other relationships, to such information and (b) appropriate legends were affixed to the stock certificates issued in such transactions.

Series A Convertible Preferred Stock Financing

On June 16, 2011, we sold an aggregate of 18,052,464 shares of our Series A convertible preferred stock to eight investors at a purchase price of $1.034 per share, for an aggregate purchase price of approximately $15.0 million in cash and $3.7 million in converted bridge notes. On July 16, 2012, we sold, pursuant to a second tranche closing, an aggregate of 14,604,895 shares of our Series A convertible preferred stock to six investors at a purchase price of $1.034 per share, for an aggregate purchase price of $15.1 million in cash. We claimed exemption from registration under the Securities Act for the sale and issuance of these securities by virtue of Section 4(a)(2) and/or Regulation D promulgated thereunder as transactions not involving any public offering. All of the purchasers of unregistered securities for which we relied on Section 4(a)(2) and/or Regulation D represented that they were accredited investors as defined under the Securities Act. We claimed such exemption on the basis that (a) the purchasers in each case represented that they intended to acquire the securities for investment only and not with a view to the distribution thereof and that they either received adequate information about the registrant or had access, through employment or other relationships, to such information and (b) appropriate legends were affixed to the stock certificates issued in such transactions.

Series A Paid-in-Kind Dividends

On August 16, 2012, we issued an aggregate of 1,193,088 shares of our Series A convertible preferred stock to holders of our Series A convertible preferred stock in accordance with Article IV.B.1.(a) of our Certificate of Incorporation that was filed with the Secretary of State of the State of Delaware on June 13, 2011.We claimed exemption from registration under the Securities Act for the sale and issuance of these securities by virtue of Section 4(a)(2) and/or Regulation D promulgated thereunder as transactions not involving any public offering.

On December 14, 2012, we issued an aggregate of 499,447 shares of our Series A convertible preferred stock to holders of our Series A convertible preferred stock in accordance with Article IV.B.1.(a) of our Certificate of Incorporation that was filed with the Secretary of State of the State of Delaware on June 13, 2011.We claimed exemption from registration under the Securities Act for the sale and issuance of these securities by virtue of Section 4(a)(2) and/or Regulation D promulgated thereunder as transactions not involving any public offering.

Series B Convertible Preferred Stock Financing

On December 18, 2012, we sold an aggregate of 27,081,680 shares of our Series B convertible preferred stock to 34 investors at a purchase price of $2.7694 per share, for an aggregate purchase price of approximately $75 million in cash. We claimed exemption from registration under the Securities Act for the sale and issuance of these securities by virtue of Section 4(a)(2) and/or Regulation D promulgated thereunder as transactions not involving any public offering. All of the purchasers of unregistered securities for which we relied on Section 4(a)(2) and/or Regulation D represented that they were accredited investors as defined under the Securities Act. We claimed such exemption on the basis that (a) the purchasers in each case represented that they intended to acquire the securities for investment only and not with a view to the distribution thereof and that they either received adequate information about the registrant or had access, through employment or other relationships, to such information and (b) appropriate legends were affixed to the stock certificates issued in such transactions.

Stock Options

From November 17, 2011 through November 1, 2013, we granted stock options to employees under our 2011 Equity Incentive Plan, as amended, covering an aggregate of 9,319,000 shares of common stock, at a weighted-average average exercise price of $0.59 per share. Of these, options covering an aggregate of 821,667 shares were cancelled without being exercised and we sold an aggregate of 2,211,153 shares of common stock to employees for cash consideration in the aggregate amount of $0.3 million upon the exercise of stock options.

We claimed exemption from registration under the Securities Act for these sales and issuances under Section 4(a)(2) of the Securities Act in that such sales and issuances did not involve a public offering or under

Rule 701 promulgated under the Securities Act, in that they were offered and sold either pursuant to written compensatory plans or pursuant to a written contract relating to compensation, as provided by Rule 701.

Item 16. Exhibits and Financial Statement Schedules.

(a) Exhibits.

See the Exhibit Index attached to this Registration Statement, which is incorporated by reference herein.

(b) Financial Statement Schedules.

Schedules not listed above have been omitted because the information required to be set forth therein is not applicable or is shown in the financial statements or notes thereto.

Item 17. Undertakings.

The undersigned Registrant hereby undertakes to provide to the underwriters at the closing specified in the underwriting agreement certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the Registrant pursuant to the foregoing provisions, or otherwise, the Registrant has been advised that in the opinion of the SEC such indemnification is against public policy as expressed in the Securities Act, and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the Registrant of expenses incurred or paid by a director, officer, or controlling person of the Registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the Registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question of whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.

The undersigned Registrant hereby undertakes that:

1. For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this Registration Statement in reliance upon Rule 430A and contained in a form of prospectus filed by the Registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this Registration Statement as of the time it was declared effective.
2. For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

The undersigned Registrant hereby undertakes to provide to the underwriters at the closing specified in the underwriting agreement certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

Signatures

Pursuant to the requirements of the Securities Act of 1933, as amended, the Registrant has duly caused this Registration Statement on Form S-1 to be signed on its behalf by the undersigned, thereunto duly authorized, in Novato, California, on November 8, 2013.

ULTRAGENYX PHARMACEUTICAL INC.

By: /s/ EMIL D. KAKKIS
Emil D. Kakkis, M.D., Ph.D.
President and Chief Executive Officer

Power of Attorney

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below hereby constitutes and appoints Emil D. Kakkis and Shalini Sharp, and each of them acting individually, as his or her true and lawful attorneys-in-fact and agents, each with full power of substitution, for him or her in any and all capacities, to sign any and all amendments to this Registration Statement, including post-effective amendments or any abbreviated registration statement and any amendments thereto filed pursuant to Rule 462(b) increasing the number of securities for which registration is sought, and to file the same, with all exhibits thereto and other documents in connection therewith, with the SEC, granting unto said attorneys-in-fact and agents, with full power of each to act alone, full power and authority to do and perform each and every act and thing requisite and necessary to be done in connection therewith, as fully for all intents and purposes as he might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents, or his or their substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Act, this Registration Statement has been signed by the following persons in the capacities and on the dates indicated.

Signature	Title	Date
/S/ EMIL D. KAKKIS Emil D. Kakkis, M.D., Ph.D.	Director, President and Chief Executive Officer *(Principal Executive Officer)*	November 8, 2013
/S/ SHALINI SHARP Shalini Sharp	Senior Vice President, Chief Financial Officer *(Principal Financial and Accounting Officer)*	November 8, 2013
/S/ ERAN NADAV Eran Nadav, Ph.D.	Chairman of the Board	November 8, 2013
/S/ BENJAMIN AUSPITZ Benjamin Auspitz	Director	November 8, 2013
/S/ MÅRTEN STEEN Mårten Steen, M.D., Ph.D.	Director	November 8, 2013
/S/ WILLIAM ALISKI William Aliski	Director	November 8, 2013

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Exhibit Index

Exhibit Number	Exhibit Description	Incorporated by Reference			Filed Herewith
		Form	Date	Number	
1.1*	Form of Underwriting Agreement				
3.1	Amended and Restated Certificate of Incorporation, as currently in effect				X
3.2	Bylaws, as currently in effect				X
3.3*	Amended and Restated Certificate of Incorporation, to be in effect immediately prior to the completion of this offering				
3.4*	Amended and Restated Bylaws, to be in effect immediately prior to the completion of this offering				
4.1	Reference is made to Exhibits 3.1 through 3.4				
4.2	Form of Common Stock Certificate				X
4.3	Warrant to purchase Series A Preferred Stock of the Registrant, dated as of June 30, 2010, issued to Emil D. Kakkis, M.D., Ph.D.				X
4.4	Warrant to purchase Series A Preferred Stock of the Registrant, dated as of February 15, 2011, issued to the John and Cynthia Klock Charitable Trust				X
4.5	Warrant to purchase Series A Preferred Stock of the Registrant, dated as of February 23, 2011, issued to William E. Aliski				X
4.6	Warrant to purchase Series A Preferred Stock of the Registrant, dated as of June 14, 2011, issued to Emil D. Kakkis, M.D., Ph.D.				X
4.7	Warrant to purchase Series A Preferred Stock of the Registrant, dated as of June 14, 2011, issued to Emil D. Kakkis, M.D., Ph.D.				X
4.8	Amended and Restated Investors' Rights Agreement, dated December 18, 2012, among the Registrant and the investors named therein				X
5.1*	Opinion of Ropes & Gray LLP				
10.1†	Collaboration and License Agreement, dated as of August 29, 2013, between the Registrant and Kyowa Hakko Kirin Co., Ltd.				X
10.2†	License Agreement, dated as of March 1, 2011, between the Registrant and AAIPharma Services Corp.				X
10.3†	License Agreement, dated as of September 20, 2012, between the Registrant and Baylor Research Institute				X
10.4†	Amendment to the License Agreement, dated as of March 22, 2013, between the Registrant and Baylor Research Institute				X
10.5†	Exclusive License Agreement, dated as of April 23, 2012, between the Registrant and HIBM Research Group				X
10.6†	Collaboration and License Agreement, dated as of September 30, 2010, between the Registrant and Nobelpharma Co., Ltd.				X
10.7†	License Agreement, dated as of September 1, 2012, between the Registrant and St. Jude Children's Research Hospital				X

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Exhibit Number	Exhibit Description	Incorporated by Reference			Filed Herewith
		Form	Date	Number	
10.8†	Exclusive License Agreement, dated as of November 22, 2010, between the Registrant and Saint Louis University				X
10.9†	Supply Agreement, dated as of November 19, 2012, between the Registrant and CREMER OLEO GmbH & Co KG				X
10.10†	Development and Clinical Supply Agreement, dated as of August 31, 2012, between the Registrant and Rentschler Biotechnologie GmbH				X
10.11#	2011 Equity Incentive Plan (including forms of Stock Option Grant Notice and Stock Option Agreement thereunder)				X
10.12#	Amendment to the 2011 Equity Incentive Plan				X
10.13#*	2013 Incentive Plan				
10.14#*	Form of Incentive Stock Option Award Agreement				
10.15#*	Form of Non-Qualified Stock Option Award Agreement				
10.16#*	Form of Restricted Stock Unit Award Agreement				
10.17#*	2013 Employee Stock Purchase Plan				
10.18#	Executive Employment Agreement, dated as of June 15, 2011, between the Registrant and Emil D. Kakkis, M.D., Ph.D.				X
10.19#	Offer Letter, dated as of October 31, 2011, between the Registrant and Thomas Kassberg				X
10.20#	Offer Letter, dated March 12, 2012, between the Registrant and Shalini Sharp				X
10.21#*	Form of Indemnification Agreement				
10.22	Standard Lease, dated as of July 5, 2011, between the Registrant and Condiotti Enterprises, Inc.				X
10.23	License and Services Agreement, dated as of September 24, 2010, between the Registrant and The Buck Institute for Age Research				X
10.24	Amendment No. 1 to License and Services Agreement, dated as of September 4, 2012, between the Registrant and The Buck Institute for Research on Aging				X
10.25#*	Corporate Bonus Plan				
23.1	Consent of independent registered public accounting firm				X
23.2*	Consent of Ropes & Gray LLP (included in Exhibit 5.1)				
24.1	Power of Attorney (included on the signature page to the Registration Statement)				X
99.1	Consent of Matthew Fust				X

* To be filed by amendment.

† Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment and this exhibit has been filed separately with the SEC.

# Indicates management contract or compensatory plan.

AMENDED AND RESTATED CERTIFICATE OF INCORPORATION
OF
ULTRAGENYX PHARMACEUTICAL INC.

Ultragenyx Pharmaceutical Inc. (the "Corporation"), a corporation organized and existing under the laws of the State of Delaware, hereby certifies as follows:

A. The name of the Corporation is Ultragenyx Pharmaceutical Inc.

B. The original Certificate of Incorporation of the Corporation was filed with the Secretary of State of the State of Delaware on June 13, 2011.

C. Pursuant to Sections 242 and 245 of the General Corporation Law of the State of Delaware, the text of the Corporation's Certificate of Incorporation is hereby amended and restated by this Amended and Restated Certificate of Incorporation (hereafter, the "Certificate of Incorporation") in its entirety to read as follows:

Article I.

The name of this corporation is Ultragenyx Pharmaceutical Inc.

Article II.

The address of the registered office of this Corporation in the State of Delaware is 1209 Orange Street, City of Wilmington, County of New Castle, 19801. The name of its registered agent at such address is The Corporation Trust Company.

Article III.

The nature of the business of the Corporation and the objects or purposes to be transacted, promoted or carried on by it are to engage in any lawful act or activity for which corporations may be organized under the General Corporation Law of the State of Delaware (the "Delaware General Corporation Law").

Article IV.

A. Classes of Stock. This Corporation is authorized to issue two classes of stock to be designated, respectively, "Common Stock" and "Preferred Stock." The total number of shares that this Corporation is authorized to issue is One Hundred Forty-Seven Million, Four Hundred Fifty-Nine Thousand, Two Hundred Thirty-Six (147,459,236) shares. Eighty-Five Million (85,000,000) shares shall be Common Stock, each with a par value of $0.001 per share and Sixty-Two Million, Four Hundred Fifty-Nine Thousand, Two Hundred Thirty-Six (62,459,236) shares shall be Preferred Stock, each with a par value of $0.001 per share.

B. Rights, Preferences and Restrictions of Preferred Stock. Thirty-Five Million, Three Hundred Seventy-Seven Thousand, Five Hundred Fifty-Six (35,377,556) shares are hereby designated "Series A Preferred Stock," and Twenty-Seven Million, Eighty-One Thousand Six Hundred Eighty (27,081,680) shares are hereby designated "Series B Preferred Stock" (together with the Series A Preferred Stock, the "Preferred Stock"). The rights, preferences, privileges, and restrictions granted to and imposed on the Preferred Stock are as set forth below in this Article IV.B.

1. Dividend Provisions.

(a) When and as declared by the Corporation's Board of Directors, the holders of shares of Series A Preferred Stock and Series B Preferred Stock shall be entitled to receive, on a pari passu basis, dividends out of any assets legally available therefor, prior and in preference to any declaration or payment of any dividend (payable other than in Common Stock or other securities and rights convertible into or entitling the holder thereof to receive, directly or indirectly, additional shares of Common Stock of this Corporation) on the Common Stock of this Corporation, at the rate of $0.0620 per share per annum (as adjusted for any stock splits, stock dividends, combinations, recapitalizations or the like (collectively, "Recapitalizations")), payable in the form of cash or property. Such dividends shall accrue on each share from the date of issuance of such share, and shall accrue from day to day, whether or not earned or declared, and whether or not there are profits, surplus, shares or other funds legally available for the payment of such dividends. Such dividends shall be cumulative so that, except as provided below, if such dividends in respect of any previous or current annual dividend period, at the annual rate specified above, shall not have been paid the deficiency shall first be fully paid before any dividend or other distribution shall be paid on or declared and set apart for the Common Stock. Any accumulation of dividends on the Preferred Stock shall not bear interest. Cumulative dividends with respect to a share of Preferred Stock which are accrued, payable and/or in arrears shall, upon conversion of such share to Common Stock, be paid to the extent assets are legally available therefor and any amounts for which assets are not legally available shall be paid promptly as assets become legally available therefor. Any partial payment shall be made ratably among the holders of Series A Preferred Stock and Series B Preferred Stock on a pari passu basis and in proportion to the payment each such holder would receive if the full amount of such dividends were paid.

(b) After payment of the full amount of any dividends pursuant to Article IV.B.1(a), any additional dividends shall be distributed among all holders of Common Stock and all holders of Preferred Stock in proportion to the number of shares of Common Stock which would be held by each such holder if all shares of such series of Preferred Stock were converted to Common Stock at the then effective conversion rate for each such series of Preferred Stock. Whenever any Distribution provided for in this Section 1 shall be payable in property other than cash, the value of such Distribution shall be deemed to be the fair market value of such property as determined by the Board of Directors.

(c) As authorized by Section 402.5(c) of the California Corporations Code, if Section 503 of the California General Corporation Law ("CGCL") is applicable to a payment made by the Corporation then such applicable section or sections shall not apply with respect to payments made by this Corporation in connection with (i) repurchases of Common Stock issued to or held by employees, officers, directors or consultants of this Corporation or its subsidiaries upon termination of their employment or services pursuant to agreements providing

for the right of said repurchase and (ii) repurchases of Common Stock issued to or held by employees, officers, directors or consultants of this Corporation or its subsidiaries pursuant to rights of first refusal contained in bylaw provisions or agreements providing for such rights.

2. Liquidation Preference.

(a) In the event of any liquidation, dissolution or winding up of this Corporation, either voluntary or involuntary, the holders of Series A Preferred Stock and Series B Preferred Stock shall be entitled to receive, on a pari passu basis, prior and in preference to any distribution of any of the assets of this Corporation to the holders of Common Stock by reason of their ownership thereof, an amount per share equal to the sum of (A) $1.0339 (the "Original Series A Issue Price") for each outstanding share of Series A Preferred Stock (subject to adjustment for Recapitalizations) and $2.7694 (the "Original Series B Issue Price") for each outstanding share of Series B Preferred Stock (subject to adjustment for Recapitalizations) and (B) an amount equal to all declared or accrued but unpaid dividends on such shares. If upon the occurrence of such event, the assets and funds thus distributed among the holders of the Preferred Stock shall be insufficient to permit the payment to such holders of the full aforesaid preferential amounts, then the entire assets and funds of this Corporation legally available for distribution to stockholders shall be distributed ratably, on a pari passu basis, among the holders of the Preferred Stock in proportion to the full preferential amount each such holder is otherwise entitled to receive under this Article IV.B.2(a).

(b) Upon completion of the distributions of the full amount required by Article IV.B.2(a), all of the remaining assets of this Corporation available for distribution to stockholders shall be distributed, on a pari passu basis, ratably among the holders of Preferred Stock and Common Stock based on the number of shares of Common Stock held by each (treating the shares of Preferred Stock for this purpose as if they had been converted to shares of Common Stock at the then-effective Conversion Price for such shares); provided, however, that (i) the holders of Series A Preferred Stock shall not be entitled to further participate in any distribution of the remaining assets of the Corporation pursuant to this Article IV.B.2(b) following receipt by such holders of Series A Preferred Stock of aggregate distributions pursuant to this Article IV.B.2 (including amounts distributed pursuant to Article IV.B.2(a)) equal to $3.1017 (subject to adjustment for Recapitalizations), plus any declared or accrued but unpaid dividends and (ii) the holders of Series B Preferred Stock shall not be entitled to further participate in any distribution of the remaining assets of the Corporation pursuant to this Article IV.B.2(b) following receipt by such holders of Series B Preferred Stock of aggregate distributions pursuant to this Article IV.B.2 (including amounts distributed pursuant to Article IV.B.2(a)) equal to $8.3082 (subject to adjustment for Recapitalizations), plus any declared or accrued but unpaid dividends.

(c)

(i) For purposes of this Article IV.B.2, a liquidation, dissolution or winding up of this Corporation shall be deemed to be occasioned by, or to include (unless the holders of at least 66.67% of the outstanding shares of Preferred Stock (treating the shares of Preferred Stock for this purpose as if they had been converted to shares of Common Stock at the then-effective Conversion Price for such shares) shall determine otherwise), (A) the

acquisition of this Corporation by another entity by means of any reorganization, merger or consolidation (but excluding any reorganization, merger or consolidation effected exclusively for the purpose of changing the name or domicile of the Corporation), or any transaction or series of related transactions in which the Corporation's stockholders of record as constituted immediately prior to such transaction or series of related transactions will, immediately after such transaction or series of related transactions (by virtue of securities issued in such transaction or series of related transactions) fail to hold at least 50% of the voting power of the resulting or surviving corporation following such transaction or series of related transactions; provided, however, that any transaction or series of transactions principally for bona fide equity financing purposes in which cash is received by the Corporation or indebtedness of the Corporation is cancelled or converted (or a combination thereof) shall not be deemed to be a liquidation, dissolution or winding up of this Corporation; or (B) a sale or disposition of all or substantially all of the assets of this Corporation or an exclusive, worldwide license of all or substantially all of the intellectual property rights of this Corporation.

(ii) In any of such events, if the consideration received by this Corporation is other than cash, its value will be deemed its fair market value as determined by the Board of Directors of this Corporation. Any securities shall be valued as follows:

(A) The value of securities not subject to investment letter or other similar restrictions on free marketability (other than restrictions arising solely by virtue of a stockholder's status as an affiliate or former affiliate) shall be:

(1) if traded on a securities exchange, the value shall be deemed to be the average of the closing prices of the securities on such exchange or system over the period of time set forth for such purpose in the definitive agreements for such transaction, or, if none, as determined by the Board of Directors of this Corporation in good faith;

(2) if actively traded over-the-counter, the value shall be deemed to be the average of the closing bid or sale prices (whichever is applicable) over the period of time set forth for such purpose in the definitive agreements for such transaction, or, if none, as determined by the Board of Directors of this Corporation in good faith; and

(3) if there is no active public market, the value shall be the fair market value thereof, as determined by the Board of Directors of this Corporation in good faith.

(B) The method of valuation of securities subject to investment letter or other restrictions on free marketability (other than restrictions arising solely by virtue of a stockholder's status as an affiliate or former affiliate) shall be to make an appropriate discount from the value determined as above in Article IV.B.2(c)(ii)(A) to reflect the approximate fair market value thereof, as determined by the Board of Directors of this Corporation in good faith.

(iii) This Corporation shall give each holder of Preferred Stock written notice of such impending transaction not later than ten (10) days prior to the stockholders' meeting called to approve such transaction, or ten (10) days prior to the closing of such transaction, whichever is earlier, and shall also notify such holders in writing of the final approval of such transaction. The first of such notices shall describe the material terms and conditions of the impending transaction, and this Corporation shall thereafter give such holders prompt notice of any material changes. The transaction shall in no event take place sooner than ten (10) days after this Corporation has given the first notice provided for herein or sooner than ten (10) days after this Corporation has given notice of any material changes provided for herein; provided, however, that such periods may be shortened upon the written consent of the holders of at least 66.67% of the outstanding shares of Preferred Stock (treating the shares of Preferred Stock for this purpose as if they had been converted to shares of Common Stock at the then-effective Conversion Price for such shares).

(iv) In the event of a liquidation, dissolution or winding up of this Corporation pursuant to Articles IV.B.2(c)(i) (any such occurrence, a "Deemed Liquidation Event"), if any portion of the consideration payable to the stockholders of the Corporation is payable to the stockholders of the Corporation subject to contingencies (including without limitation escrow, earn-out provisions and the like), the agreement or plan of merger or consolidation for such transaction shall provide that (a) the portion of such consideration that is not subject to any such contingencies (the "Initial Consideration") shall be allocated among the holders of capital stock of the Corporation in accordance with paragraphs (a) and (b) of this Article IV.B.2 as if the Initial Consideration were the only consideration payable in connection with such Deemed Liquidation Event and (b) any additional consideration which becomes payable to the stockholders of the Corporation upon satisfaction of such contingencies shall be allocated among the holders of capital stock of the Corporation in accordance with paragraphs (a) and (b) of this Article IV.B.2 after taking into account the previous payment of the Initial Consideration as part of the same transaction.

(v) Notwithstanding the foregoing, upon any voluntary or involuntary liquidation, dissolution or winding up of the Corporation or other Deemed Liquidation Event (a "Liquidation Event"), each holder of Preferred Stock shall be entitled to receive, for each share of Preferred Stock then held, out of the proceeds available for distribution, the greater of (i) the amount of cash, securities or other property to which such holder would be entitled to receive with respect to such shares in a Liquidation Event pursuant to paragraphs (a) and (b) of this Article IV.B.2 (without giving effect to this Article IV.B.2(c)(v)) or (ii) the amount of cash, securities or other property to which such holder would be entitled to receive in a Liquidation Event with respect to such shares if such shares had been converted to Common Stock immediately prior to such Liquidation Event, giving effect to this Article IV.B.2(c)(v).

3. Redemption. Subject to the provisions of Article IV.B.6.(c)(iii):

(a) At any time after June 16, 2017, but within sixty (60) days after the receipt by this Corporation of a written request from the holders of not less than seventy-five percent (75%) of the then outstanding Series A Preferred Stock that all shares of Series A Preferred Stock be redeemed, and concurrently with surrender by such holders of the certificates representing such shares, this Corporation shall, to the extent it may lawfully do so, redeem (the payment date being referred to herein as a "Series A Redemption Date") all of the then

outstanding shares of Series A Preferred Stock by paying in cash in exchange for the shares of Series A Preferred Stock to be redeemed a sum equal to the greater of (i) the Original Series A Issue Price per share of Series A Preferred Stock (subject to adjustment for any Recapitalizations) plus all declared or accrued but unpaid dividends on such shares and (ii) the then-current fair market value per share of Series A Preferred Stock plus all declared or accrued but unpaid dividends on such shares (but only if, and to the extent, such dividends are not reflected in the fair market value) as determined in good faith by the Board of Directors of this Corporation, and taking into account any independent third-party valuation reasonably requested by the holders of at least seventy-five percent (75%) of the Series A Preferred Stock then outstanding, the expense of which shall be borne by this Corporation; provided, however, that any holder of Series A Preferred Stock may elect, by delivery of notice to this Corporation at least five (5) days prior to the Series A Redemption Date, not to have such holder's shares of Series A Preferred Stock redeemed pursuant to this Article IV.B.3(a).

(b) At least fifteen (15) but no more than thirty (30) days prior to the Series A Redemption Date, written notice shall be mailed, first class postage prepaid, to each holder of record (at the close of business on the business day next preceding the day on which notice is given) of Series A Preferred Stock to be redeemed, at the address last shown on the records of this Corporation for such holder, (i) notifying such holder of the redemption to be effected on the Series A Redemption Date, specifying the number of shares of Series A Preferred Stock to be redeemed from such holder, the Series A Redemption Date, the Series A Redemption Price, the place at which payment may be obtained and calling upon such holder to surrender to this Corporation, in the manner and at the place designated, his, her or its certificate or certificates representing the shares to be redeemed and (ii) notifying any holder not already participating in such redemption of its rights, subject to the requirements of this Article IV.B.3, as applicable, to elect to participate or not participate in such redemption (the "Series A Redemption Notice"). Except as provided in Article IV.B.3(c), on or after the Series A Redemption Date, each holder of Series A Preferred Stock to be redeemed on such Series A Redemption Date shall surrender to this Corporation the certificate or certificates representing such shares, in the manner and at the place designated in the Series A Redemption Notice, and thereupon the Series A Redemption Price of such shares shall be payable to the order of the person whose name appears on such certificate or certificates as the owner thereof and each surrendered certificate shall be cancelled. No transfers of Series A Preferred Stock shall be permitted during the five (5) day period prior to and including the Series A Redemption Date, and this Corporation shall not recognize any such prohibited transfer on its books and records.

(c) From and after the Series A Redemption Date, unless there shall have been a default in payment of the Series A Redemption Price, all rights of the holders of shares of Series A Preferred Stock designated for redemption on such Series A Redemption Date in the Series A Redemption Notice (except the right to receive the Series A Redemption Price, without interest upon surrender of their certificate or certificates) shall cease with respect to such shares, and such shares shall not thereafter be transferred on the books of this Corporation or be deemed to be outstanding for any purpose whatsoever. If the funds of this Corporation legally available for redemption of shares of Series A Preferred Stock on the Series A Redemption Date are insufficient to redeem the total number of such shares of Series A Preferred Stock to be redeemed on such date, those funds that are legally available will be used to redeem the maximum possible number of such shares, ratably among the holders of such shares in

proportion to the percentage of the total redemption payments to be made on the Series A Redemption Date that each such holder would be entitled to received were sufficient funds legally available. The shares of Series A Preferred Stock not redeemed shall remain outstanding and entitled to all the rights and preferences provided herein. At any time thereafter when additional funds of this Corporation are legally available for the redemption of shares of Series A Preferred Stock, such funds will immediately be used to redeem the balance of the shares that this Corporation has become obligated to redeem on the Series A Redemption Date but that it has not redeemed.

4. Conversion. The holders of the Preferred Stock shall have conversion rights as follows (the "Conversion Rights"):

(a) Right to Convert. Each share of Preferred Stock shall be convertible, at the option of the holder thereof, at any time after the date of issuance of such share and, with respect to the Series A Preferred Stock only, on or prior to five (5) business days prior to the Series A Redemption Date, if any, as may have been fixed in any Series A Redemption Notice with respect to such shares of Series A Preferred Stock, at the office of this Corporation or any transfer agent for such stock, into such number of fully paid and nonassessable shares of Common Stock as is determined by dividing the Original Series A Issue Price or Original Series B Issue Price, as applicable, by the Conversion Price applicable to such share, determined as hereafter provided, in effect on the date the certificate is surrendered for conversion. The initial Conversion Price per share for shares of Series A Preferred Stock shall be the Original Series A Issue Price (as the same may be adjusted in accordance with the terms hereof, the "Series A Conversion Price") and the initial Conversion Price per share for shares of Series B Preferred Stock shall be the Original Series B Issue Price (as the same may be adjusted in accordance with the terms hereof, the "Series B Conversion Price"); provided, however, that the Conversion Prices for the Series A Preferred Stock and Series B Preferred Stock shall be subject to adjustment as set forth in Article IV.B.4(d). The "Conversion Price" shall mean the Series A Conversion Price, in the case of the Series A Preferred Stock, and the Series B Conversion Price, in the case of the Series B Preferred Stock.

(b) Automatic Conversion. Each share of Preferred Stock shall automatically be converted into shares of Common Stock at the Conversion Price at the time in effect for such Preferred Stock immediately upon the earlier of (i) except as provided in Article IV.B.4(c), the Corporation's sale of its Common Stock in a firm commitment underwritten public offering pursuant to a registration statement under the Securities Act of 1933, as amended (the "Act"), which raises gross proceeds of at least $30,000,000 in the aggregate (before deduction of underwriting discounts and commissions) or (ii) the date specified by the vote, written consent or written election of the holders of at least seventy-five percent (75%) of the then outstanding shares of Preferred Stock, voting or consenting together as a single class on an as converted to Common Stock basis.

(c) Mechanics of Conversion. Before any holder of Preferred Stock shall be entitled to convert the same into shares of Common Stock, he, she or it shall surrender the certificate or certificates therefor, duly endorsed, at the office of this Corporation or of any transfer agent for the Preferred Stock, and shall give written notice to this Corporation at its principal corporate office, of the election to convert the same and shall state therein the name or

names in which the certificate or certificates for shares of Common Stock are to be issued. This Corporation shall, as soon as practicable thereafter, issue and deliver at such office to such holder of Preferred Stock, or to the nominee or nominees of such holder, a certificate or certificates for the number of shares of Common Stock to which such holder shall be entitled as aforesaid and a certificate for the number (if any) of shares of Preferred Stock represented by the surrendered certificate that were not converted into Common Stock, together with (i) cash in lieu of any fraction of a share of Common Stock otherwise issuable upon such conversion and (ii) cash equal to all accrued but unpaid dividends on the shares of Preferred Stock converted. Such conversion shall be deemed to have been made immediately prior to the close of business on the date of such surrender of the shares of Preferred Stock to be converted, and the person or persons entitled to receive the shares of Common Stock issuable upon such conversion shall be treated for all purposes as the record holder or holders of such shares of Common Stock as of such date. If the conversion is in connection with an underwritten offering of securities registered pursuant to the Act, the conversion may, at the option of any holder tendering Preferred Stock for conversion, be conditioned upon the closing with the underwriters of the sale of securities pursuant to such offering, in which event the persons entitled to receive the Common Stock upon conversion of the Preferred Stock shall not be deemed to have converted such Preferred Stock until immediately prior to the closing of such sale of securities.

(d) Conversion Price Adjustments of Preferred Stock. The Conversion Prices of the Preferred Stock shall be subject to adjustment from time to time as follows:

(i) (A) If this Corporation shall issue, after the date upon which any shares of Series B Preferred Stock were first issued (the "Series B Purchase Date"), any Additional Stock (as defined below) without consideration or for a consideration per share less than the Conversion Price for any series of Preferred Stock in effect immediately prior to the issuance of such Additional Stock, the Conversion Price for the affected series of Preferred Stock in effect immediately prior to each such issuance shall (except as otherwise provided in this Article IV.B.4(d)(i)) be adjusted concurrently with such issuance to a price determined by multiplying the relevant Conversion Price by a fraction, the numerator of which shall be the number of shares of Common Stock outstanding and deemed issued pursuant to Article IV.B.4(d)(i)(D) immediately prior to such issuance plus the number of shares of Common Stock that the aggregate consideration received by this Corporation for such issuance would purchase at such Conversion Price; and the denominator of which shall be the number of shares of Common Stock outstanding and deemed issued pursuant to Article IV.B.4(d)(i)(D) immediately prior to such issuance plus the number of shares of such Additional Stock.

(A) No adjustment of the Conversion Price for any series of Preferred Stock shall be made in an amount less than one cent per share, provided that any adjustments that are not required to be made by reason of this sentence shall be carried forward and shall be either taken into account in any subsequent adjustment made prior to three (3) years from the date of the event giving rise to the adjustment being carried forward, or shall be made at the end of three (3) years from the date of the event giving rise to the adjustment being carried forward. Except to the limited extent provided for in Article IV.B.4(d)(i)(D)(3) and Article IV.B.4(d)(i)(D)(4), no adjustment of such Conversion Price pursuant to this Article IV.B.4(d)(i) shall have the effect of increasing the Conversion Price above the Conversion Price in effect immediately prior to such adjustment.

(B) In the case of the issuance of Additional Stock for cash, the consideration shall be deemed to be the amount of cash paid therefor before deducting any reasonable discounts, commissions or other expenses allowed, paid or incurred by this Corporation for any underwriting or otherwise in connection with the issuance and sale thereof.

(C) In the case of the issuance of the Additional Stock for a consideration in whole or in part other than cash, the consideration other than cash shall be deemed to be the fair value thereof as determined by the Board of Directors irrespective of any accounting treatment.

(D) In the case of the issuance (whether before, on or after the Series B Purchase Date) of options to purchase or rights to subscribe for Common Stock, securities by their terms convertible into or exchangeable for Common Stock or options to purchase or rights to subscribe for such convertible or exchangeable securities, the following provisions shall apply for all purposes of this Article IV.B.4(d)(i) and Article IV.B.4(d)(ii):

(1) The aggregate maximum number of shares of Common Stock deliverable upon exercise (assuming the satisfaction of any conditions to exercisability, including, without limitation, the passage of time) of such options to purchase or rights to subscribe for Common Stock shall be deemed to have been issued at the time such options or rights were issued and for a consideration equal to the consideration (determined in the manner provided in Article IV.B.4(d)(i)(B) and Article IV.B.4(d)(i)(C)), if any, received by this Corporation upon the issuance of such options or rights plus the minimum exercise price provided in such options or rights for the Common Stock covered thereby.

(2) The aggregate maximum number of shares of Common Stock deliverable upon conversion of, or in exchange (assuming the satisfaction of any conditions to convertibility or exchangeability, including, without limitation, the passage of time) for any such convertible or exchangeable securities or upon the exercise of options to purchase or rights to subscribe for such convertible or exchangeable securities and subsequent conversion or exchange thereof shall be deemed to have been issued at the time such securities were issued or such options or rights were issued and for a consideration equal to the consideration, if any, received by this Corporation for any such securities and related options or rights (excluding any cash received on account of accrued interest or accrued dividends), plus the minimum additional consideration, if any, to be received by this Corporation upon the conversion or exchange of such securities or the exercise of any related options or rights (the consideration in each case to be determined in the manner provided in Article IV.B.4(d)(i)(B) and Article IV.B.4(d)(i)(C)).

(3) In the event of any change in the number of shares of Common Stock deliverable or in the consideration payable to this Corporation upon exercise of such options or rights or upon conversion of or in exchange for such convertible or exchangeable securities, including, but not limited to, a change resulting from the antidilution provisions thereof, the Conversion Price of each series of Preferred Stock, to the extent in any way affected by or computed using such options, rights or securities, shall be recomputed to reflect such change, but no further adjustment shall be made for the actual issuance of Common Stock or any payment of such consideration upon the exercise of any such options or rights or the conversion or exchange of such securities.

(4) Upon the expiration of any such options or rights, the termination of any such rights to convert or exchange or the expiration of any options or rights related to such convertible or exchangeable securities, the Conversion Price of each series of Preferred Stock, to the extent in any way affected by or computed using such options, rights or securities or options or rights related to such securities, shall be recomputed to reflect the issuance of only the number of shares of Common Stock (and convertible or exchangeable securities that remain in effect) actually issued upon the exercise of such options or rights, upon the conversion or exchange of such securities or upon the exercise of the options or rights related to such securities.

(5) The number of shares of Common Stock deemed issued and the consideration deemed paid therefor pursuant to Article IV.B.4(d)(i)(D)(1) and Article IV.B.4(d)(i)(D)(2) shall be appropriately adjusted to reflect any change, termination or expiration of the type described in either Article IV.B.4(d)(i)(D)(3) or Article IV.B.4(d)(i)(D)(4).

(ii) "Additional Stock" shall mean any shares of Common Stock issued (or deemed to have been issued pursuant to Article IV.B.4(d)(i)(D)) by this Corporation after the Series B Purchase Date other than:

(A) shares of Common Stock issued pursuant to a transaction described in Article IV.B.4(d)(iii) hereof;

(B) up to Ten Million, Four Hundred Sixty Nine Thousand, Eight Hundred Twenty-Seven (10,469,827) shares of Common Stock issued or deemed issued to employees, consultants, officers, directors or vendors of this Corporation pursuant to a stock option plan or restricted stock purchase plan approved by the stockholders and Board of Directors of this Corporation (such number being inclusive of options to purchase Common Stock outstanding as of the Series B Purchase Date);

(C) shares of Common Stock issued or issuable (I) in a bona fide, firmly underwritten public offering under the Act before which or in connection with which all outstanding shares of Preferred Stock will be automatically converted to Common Stock, or (II) upon exercise of warrants or rights granted to underwriters in connection with such a public offering;

(D) shares of Common Stock issued upon conversion of any shares of the Corporation's Preferred Stock;

(E) shares of Common Stock issued pursuant to the conversion or exercise of convertible or exercisable securities outstanding as of the Series B Purchase Date or subsequently issued after the Series B Purchase Date in accordance with this Article IV.B.4(d)(ii);

(F) shares of Common Stock issued or issuable in connection with a bona fide business acquisition of or by this Corporation, whether by merger, consolidation, sale of assets, sale or exchange of stock or otherwise, each as approved by the Board of Directors of this Corporation;

(G) shares of Common Stock issued or issuable to financial institutions or lessors in connection with commercial credit arrangements, equipment financings, commercial property lease transactions or similar transactions, provided such issuances are approved by the Board of Directors and for other than primarily equity financing purposes;

(H) shares of Common Stock issued or issuable to persons or entities with which this Corporation has business relationships, including, without limitation, pursuant to sponsored research, collaboration, technology license, development, marketing or similar agreements or strategic partnerships, provided such issuances are approved by the Board of Directors and for other than primarily equity financing purposes;

(I) shares of Series B Preferred Stock issued pursuant to the Series B Preferred Stock Purchase Agreement (the "Series B Purchase Agreement") dated on or about December 18, 2012 by and among the Company and the Investors signatory thereto;

(J) shares of Common Stock issued or issuable in connection with any transaction where such securities so issued are excepted from the definition of "Additional Stock" by the affirmative vote of (i) at least seventy-five percent (75%) of the then outstanding shares of Series B Preferred Stock voting separately as a single class and (ii) the affirmative vote of at least seventy-five percent (75%) of the then outstanding shares of Series A Preferred Stock, voting separately as a single class;

(iii) In the event this Corporation should at any time or from time to time after the Series B Purchase Date fix a record date for the effectuation of a split or subdivision of the outstanding shares of Common Stock or the determination of holders of Common Stock entitled to receive a dividend or other distribution payable in additional shares of Common Stock or other securities or rights convertible into, or entitling the holder thereof to receive directly or indirectly, additional shares of Common Stock (hereinafter referred to as "Common Stock Equivalents") without payment of any consideration by such holder for the additional shares of Common Stock or the Common Stock Equivalents (including the additional shares of Common Stock issuable upon conversion or exercise thereof), then, as of such record date (or the date of such dividend distribution, split or subdivision if no record date is fixed), the Conversion Prices of each series of Preferred Stock shall be appropriately decreased so that the number of shares of Common Stock issuable on conversion of each share of such series shall be increased in proportion to such increase in the aggregate number of shares of Common Stock outstanding and those issuable with respect to such Common Stock Equivalents.

(iv) If the number of shares of Common Stock outstanding at any time after the Series B Purchase Date is decreased by a combination of the outstanding shares of Common Stock, then, following the record date of such combination, the Conversion Prices for each series of Preferred Stock shall be appropriately increased so that the number of shares of Common Stock issuable on conversion of each share of such series shall be decreased in proportion to such decrease in outstanding shares.

(e) Other Distributions. In the event this Corporation shall declare a distribution payable in securities of other persons, evidences of indebtedness issued by this Corporation or other persons, assets (excluding cash dividends) or options or rights not referred to in Article IV.B.4(d)(iii), then, in each such case for the purpose of this Article IV.B.4(e), the holders of each series of Preferred Stock shall be entitled to a proportionate share of any such distribution as though they were the holders of the number of shares of Common Stock of this Corporation into which their shares of such series of Preferred Stock are convertible as of the record date fixed for the determination of the holders of Common Stock of this Corporation entitled to receive such distribution.

(f) Recapitalizations. If at any time or from time to time there shall be a recapitalization of the Common Stock (including, without limitation, pursuant to a merger, consolidation, reorganization, reclassification or similar event) (other than a subdivision, combination or merger or sale of assets transaction provided for elsewhere in Article IV.B.2 or this Article IV.B.4) provision shall be made so that the holders of each series of the Preferred Stock shall thereafter be entitled to receive upon conversion of such series of Preferred Stock the number of shares of stock or other securities or property of this Corporation or otherwise, to which a holder of the number of shares of Common Stock deliverable upon conversion of the Preferred Stock held by such holder would have been entitled on such recapitalization. In any such case, appropriate adjustment shall be made in the application of the provisions of this Article IV.B.4 with respect to the rights of the holders of each series of Preferred Stock after the recapitalization to the end that the provisions of this Article IV.B.4 (including adjustment of the Conversion Price then in effect and the number of shares purchasable upon conversion of each such series of Preferred Stock) shall be applicable after that event as nearly equivalent as may be practicable.

(g) No Impairment. This Corporation will not, by amendment of this Certificate of Incorporation or through any reorganization, recapitalization, transfer of assets, consolidation, merger, dissolution, issue or sale of securities or any other voluntary action, avoid or seek to avoid the observance or performance of any of the terms to be observed or performed hereunder by this Corporation, but will at all times in good faith assist in the carrying out of all the provisions of this Article IV.B.4 and in the taking of all such action as may be necessary or appropriate in order to protect the Conversion Rights of the holders of Preferred Stock against impairment.

(h) No Fractional Shares and Certificate as to Adjustments,

(i) No fractional shares shall be issued upon the conversion of any share or shares of Preferred Stock. In lieu of any fractional shares to which the holder would otherwise be entitled, the Corporation shall pay cash equal to such fraction multiplied by the then fair market value of a share of Common Stock as determined in good faith by the Board of Directors. The number of shares of Common Stock to be issued upon such conversion shall be determined on the basis of the total number of shares of Preferred Stock the holder is at the time converting into Common Stock and the number of shares of Common Stock issuable upon such aggregate conversion.

(ii) Upon the occurrence of each adjustment or readjustment of the Conversion Price of any series of Preferred Stock pursuant to this Article IV.B.4, this Corporation, at its expense, shall promptly compute such adjustment or readjustment in accordance with the terms hereof and prepare and furnish to each holder of such series of Preferred Stock a certificate setting forth such adjustment or readjustment and showing in detail the facts upon which such adjustment or readjustment is based. This Corporation shall, upon the written request at any time of any holder of Preferred Stock, furnish or cause to be furnished to such holder a like certificate setting forth (A) such adjustment and readjustment, (B) the Conversion Price for such series of Preferred Stock at the time in effect, and (C) the number of shares of Common Stock and the amount, if any, of other property that at the time would be received upon the conversion of a share of such series of Preferred Stock.

(i) Notices of Record Date. In the event of any taking by this Corporation of a record of the holders of any class of securities for the purpose of determining the holders thereof who are entitled to receive any dividend (other than a cash dividend) or other distribution, any right to subscribe for, purchase or otherwise acquire any shares of stock of any class or any other securities or property, or to receive any other right, this Corporation shall mail to each holder of Preferred Stock, at least twenty (20) days prior to the date specified therein, a notice specifying the date on which any such record is to be taken for the purpose of such dividend, distribution or right, and the amount and character of such dividend, distribution or right.

(j) Reservation of Stock Issuable Upon Conversion. This Corporation shall at all times reserve and keep available out of its authorized but unissued shares of Common Stock, solely for the purpose of effecting the conversion of the shares of Preferred Stock, such number of its shares of Common Stock as shall from time to time be sufficient to effect the conversion of all outstanding shares of Preferred Stock; and if at any time the number of authorized but unissued shares of Common Stock shall not be sufficient to effect the conversion of all then outstanding shares of Preferred Stock, in addition to such other remedies as shall be available to the holder of such Preferred Stock, this Corporation will take such corporate action as may, in the opinion of its counsel, be necessary to increase its authorized but unissued shares of Common Stock to such number of shares as shall be sufficient for such purposes, including, without limitation, engaging in best efforts to obtain the requisite stockholder approval of any necessary amendment to this Certificate of Incorporation.

(k) Notices. Any notice required by the provisions of this Article IV.B.4 to be given to the holders of shares of Preferred Stock shall be deemed given if deposited in the United States mail, postage prepaid, and addressed to each holder of record at his address appearing on the books of this Corporation.

(l) Waiver of Adjustment to Conversion Prices.

(i) Notwithstanding anything herein to the contrary, any downward adjustment of the Conversion Price of the Series B Preferred Stock may be waived, either prospectively or retroactively and either generally or in a particular instance by the vote or written consent of the holders of at least seventy-five percent (75%) of the outstanding shares of Series B Preferred Stock. Any such waiver shall be binding upon all current and future holders of shares of Series B Preferred Stock.

(ii) Notwithstanding anything herein to the contrary, any downward adjustment of the Conversion Price of the Series A Preferred Stock may be waived, either prospectively or retroactively and either generally or in a particular instance by the vote or written consent of the holders of at least seventy-five percent (75%) of the outstanding shares of Series A Preferred Stock. Any such waiver shall be binding upon all current and future holders of shares of Series A Preferred Stock.

5. Voting Rights.

(a) General. The holder of each share of Preferred Stock shall have the right to one vote for each share of Common Stock into which such share of Preferred Stock could then be converted. With respect to such vote and except as otherwise expressly provided herein or as required by applicable law, such holder shall have full voting rights and powers equal to the voting rights and powers of the holders of Common Stock, and shall be entitled, notwithstanding any provision hereof, to notice of any stockholders' meeting in accordance with the Bylaws of this Corporation, and shall be entitled to vote, together with holders of Common Stock as a single class, with respect to any matter upon which holders of Common Stock have the right to vote. Fractional votes shall not, however, be permitted and any fractional voting rights available on an as-converted basis (after aggregating all shares into which shares of Preferred Stock held by each holder could be converted) shall be rounded to the nearest whole number (with one-half being rounded upward).

(b) Election of Directors. So long as at least 3,000,000 shares of Series A Preferred Stock remain outstanding,

(i) the holders of shares of Series A Preferred Stock shall be entitled, voting separately as a single class, to elect three (3) directors (the "Series A Directors") of the Corporation at or pursuant to each meeting or consent of the Corporation's stockholders for the election of directors, to remove from office such directors, to fill any vacancy caused by the resignation or death of such directors and to fill any vacancy (by written consent or by majority vote) caused by the removal of such directors, provided, however, that if less than 3,000,000 shares (as adjusted for any Recapitalizations) remain outstanding, then the holders of shares of Series A Preferred Stock and Common Stock voting together as a single class on an as-converted basis shall be entitled to elect the directors which the holders of shares of Series A Preferred Stock would otherwise be entitled to elect pursuant to this Article IV.B.5(b)(i), to remove from office such director, to fill any vacancy caused by the resignation or death of such director and to fill any vacancy (by unanimous consent if done in writing, or by majority vote otherwise) caused by the removal of such director,

(ii) the holders of shares of Common Stock shall be entitled, voting separately as a single class, to elect two (2) directors of the Corporation at or pursuant to each meeting or consent of the Corporation's stockholders for the election of directors, and to remove from office such directors, to fill any vacancy caused by the resignation or death of such directors and to fill any vacancy (by written consent or by majority vote) caused by the removal of any such directors, and

(iii) the holders of shares of Common Stock and Preferred Stock shall be entitled, voting together in accordance with Article IV.B.5(a) hereof, to elect the remaining directors of the Corporation at or pursuant to each meeting or consent of the Corporation's stockholders for the election of directors, to remove from office such directors, to fill any vacancy caused by the resignation or death of such directors and to fill any vacancy (by written consent or by majority vote) caused by the removal of any such directors.

(iv) No person entitled to vote at an election for directors may cumulate votes to which such person is entitled, unless, at the time of such election, Section 2115 of the CGCL purports to apply to the Corporation. During such time or times that Section 2115(b) of the CGCL purports to apply to the Corporation, every stockholder entitled to vote at an election for directors may cumulate such stockholder's votes and give one candidate a number of votes equal to the number of directors to be elected multiplied by the number of votes to which such stockholder's shares are otherwise entitled, or distribute the stockholder's votes on the same principle among as many candidates as such stockholder desires. No stockholder, however, shall be entitled to so cumulate such stockholder's votes unless (i) the names of such candidate or candidates have been placed in nomination prior to the voting and (ii) the stockholder has given notice at the meeting, prior to the voting, of such stockholder's intention to cumulate such stockholder's votes. If any stockholder has given proper notice to cumulate votes, all stockholders may cumulate their votes for any candidates who have been properly placed in nomination. Under cumulative voting, the candidates receiving the highest number of votes, up to the number of directors to be elected, are elected.

(v) During such time or times that Section 2115(b) of the CGCL purports to apply to the Corporation, one or more directors may be removed from office at any time without cause by the affirmative vote of the holders of a majority of the outstanding shares entitled to vote for that director as provided above; provided, however, that unless the entire Board is removed, no individual director may be removed when the votes cast against such director's removal, or not consenting in writing to such removal, would be sufficient to elect that director if voted cumulatively at an election at which the same total number of votes were cast (or, if such action is taken by written consent, all shares entitled to vote were voted) and the entire number of directors authorized at the time of such director's most recent election were then being elected.

6. Protective Provisions.

(a) So long as any shares of Preferred Stock are outstanding, this Corporation shall not, whether by merger, consolidation or otherwise, without first obtaining the approval (by vote or written consent, as provided by law) of the holders of at least 66.67% of the then outstanding shares of Preferred Stock voting separately as a single class on an as-converted to Common Stock basis:

(i) effect any transaction described in Article IV.B.2(c)(i); provided, however, that prior to June 16, 2014, this Corporation shall not effect any such transaction without first also obtaining the approval (by vote or written consent, as provided by law) of the holders of at least ninety percent (90%) of the then outstanding shares of Series A Preferred Stock voting separately as a single class;

(ii) authorize or issue, or obligate itself to issue any equity security, including any other security convertible into or exercisable for any equity security, having any rights, preferences or privileges over, or being on a parity with, the Series B Preferred Stock;

(iii) enter into any agreement that materially restricts the business of this Corporation (for example without limitation, by a non-compete provision or exclusivity provision); or

(iv) amend or waive any provision of this Certificate of Incorporation or Bylaws of the Corporation.

(b) So long as any shares of Preferred Stock are outstanding, this Corporation shall not, whether by merger, consolidation or otherwise, without first obtaining the approval (by vote or written consent, as provided by law) of the holders of at least a majority of the then outstanding shares of Preferred Stock voting separately as a single class:

(i) authorize or issue, or obligate itself to issue any equity security (other than shares of Series B Preferred Stock to be issued pursuant to the Series B Purchase Agreement); or

(ii) incur aggregate indebtedness in excess of $200,000 (excluding purchase-money financing for equipment and equipment leases);

(iii) increase or decrease the authorized number of directors of the Corporation;

(iv) declare or pay dividends or make other distributions on the capital stock of the Corporation;

(v) redeem, purchase or otherwise acquire (or pay into or set aside for a sinking fund for such purpose) any share or shares of Common Stock; provided, however, that this restriction shall not apply to the repurchase of shares of Common Stock from employees, officers, directors, consultants or other persons performing services for this Corporation or any subsidiary pursuant to agreements under which this Corporation has the option to repurchase such shares at cost upon the occurrence of certain events, such as the termination of employment or other provision of services to the Corporation and this restriction shall not apply to the redemptions contemplated by Article IV.B.3;

(vi) consummate a public offering pursuant to a registration statement under the Act or otherwise become subject to the periodic reporting requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, as amended;

(vii) enter into any material sponsored research, collaboration or business development, technology license, development, marketing or other similar agreement or strategic partnership, unless otherwise approved by the Board of Directors of this Corporation, including the affirmative vote of all of the Series A Directors;

(viii) enter into any agreement with payments, obligations or liabilities of this Corporation in excess of $200,000 or commitments for the same, unless otherwise approved by the Board of Directors of this Corporation, including the affirmative vote of all of the Series A Directors;

(ix) hire, engage, elect, appoint or remove the president, chief executive officer, chief financial officer, chief operating officer or any other member of senior management (vice president level or above), unless otherwise approved by the Board of Directors of this Corporation, including the affirmative vote of all of the Series A Directors;

(x) cause or permit any entity over which the Corporation has, directly or indirectly, a majority of the voting power to take any of the actions set forth in this Article IV.B.6;

(xi) increase the number of shares of Common Stock reserved for issuance under any stock option or equity incentive plan or the like (each a "Plan"), or create any such Plans except for the Corporation's 2011 Stock Incentive Plan, unless otherwise approved by the Board of Directors of this Corporation, including the affirmative vote of all of the Series A Directors; or

(xii) increase or decrease the total number of authorized shares of the Corporation's Common Stock.

(c) So long as any shares of Series B Preferred Stock are outstanding, this Corporation shall not, whether by merger, consolidation or otherwise, without first obtaining the approval (by vote or written consent, as provided by law) of the holders of at least seventy-five percent (75%) of the then outstanding shares of Series B Preferred Stock voting separately as a single class:

(i) alter or change the rights, preferences or privileges of the shares of Series B Preferred Stock (for the avoidance of doubt, the Corporation authorizing or issuing, or obligating itself to issue any equity security, including any other security convertible into or exercisable for any equity security, having any rights, preferences or privileges over, or being on a parity with, the Series B Preferred Stock, shall not be deemed to be an alteration or change to the rights, preferences or privileges of the shares of Series B Preferred Stock for purposes of this Article IV.B.6(c));

(ii) increase or decrease the total number of authorized shares of Series B Preferred Stock; or

(iii) redeem, including without limitation a redemption pursuant to Article IV.B.3, or pay a dividend on, any capital stock of the Company prior to the Series B Preferred Stock; provided, however, that this restriction shall not apply to the repurchase of

shares of Common Stock from employees, officers, directors, consultants or other persons performing services for this Corporation or any subsidiary pursuant to agreements under which this Corporation has the option to repurchase such shares at cost upon the occurrence of certain events, such as the termination of employment or other provision of services to the Corporation.

(d) So long as any shares of Series A Preferred Stock are outstanding, this Corporation shall not, whether by merger, consolidation or otherwise, without first obtaining the approval (by vote or written consent, as provided by law) of the holders of at least seventy-five percent (75%) of the then outstanding shares of Series A Preferred Stock voting separately as a single class:

(i) alter or change the rights, preferences or privileges of the shares of Series A Preferred Stock (for the avoidance of doubt, the Corporation authorizing or issuing, or obligating itself to issue any equity security, including any other security convertible into or exercisable for any equity security, having any rights, preferences or privileges over, or being on a parity with, the Series A Preferred Stock, shall not be deemed to be an alteration or change to the rights, preferences or privileges of the shares of Series A Preferred Stock for purposes of this Article Article IV.B.6(d));

(ii) increase or decrease the total number of authorized shares of Series A Preferred Stock; or

(iii) redeem, or pay a dividend on, the Common Stock of the Company prior to the Series A Preferred Stock; provided, however, that this restriction shall not apply to the repurchase of shares of Common Stock from employees, officers, directors, consultants or other persons performing services for this Corporation or any subsidiary pursuant to agreements under which this Corporation has the option to repurchase such shares at cost upon the occurrence of certain events, such as the termination of employment or other provision of services to the Corporation and this restriction shall not apply to the redemptions contemplated by Article IV.B.3.

7. Status of Redeemed or Converted Stock. In the event any shares of Preferred Stock shall be redeemed or converted pursuant to Article IV.B.3 or Article IV.B.4, the shares so redeemed or converted shall be cancelled and shall not be issuable by this Corporation. This Certificate of Incorporation shall be appropriately amended to effect the corresponding reduction in this Corporation's authorized capital stock.

C. Common Stock. The rights, preferences, privileges and restrictions granted to and imposed on the Common Stock are as set forth below in this Article IV.C.

1. Dividend Rights. Subject to the prior rights of holders of all classes of stock at the time outstanding having prior rights as to dividends, the holders of the Common Stock shall be entitled to receive, when and as declared by the Board of Directors, out of any assets of this Corporation legally available therefor, such dividends as may be declared from time to time by the Board of Directors.

2. Liquidation Rights. Upon the liquidation, dissolution or winding of this Corporation, the assets of this Corporation shall be distributed as provided in Article IV.B.2.

3. Redemption. Except as may otherwise be provided in a written agreement between the Corporation and a holder of Common Stock or the Bylaws of this Corporation, neither the Corporation nor the holders of Common Stock shall have the unilateral right to call or redeem or cause to have called or redeemed any shares of Common Stock.

4. Voting Rights. The holder of each share of Common Stock shall have the right to one vote for each such share, and shall be entitled to notice of any stockholders' meeting in accordance with the Bylaws of this Corporation, and shall be entitled to vote upon such matters and in such manner as may be provided by law; provided, however, that except as otherwise required by law, the holders of Common Stock, as such, shall not be entitled to vote on any amendment to this Certificate of Incorporation (including any Certificate of Designation relating to any series of Preferred Stock) that relates solely to the terms of one or more outstanding series of Preferred Stock if the holders of such affected series are entitled, either separately or together with the holders of one or more other such series, to vote thereon pursuant to this Certificate of Incorporation (including any Certificate of Designation relating to any series of Preferred Stock) or pursuant to the Delaware General Corporation Law. The number of authorized shares of Common Stock may be increased or decreased (but not below the number of shares thereof then outstanding) by the affirmative vote of the holders of shares of stock of the Corporation representing a majority of the votes represented by all of the outstanding shares of stock of the Corporation entitled to vote, irrespective of the provisions of Section 242(b)(2) of the Delaware General Corporation Law.

Article V.

Except as otherwise provided in this Certificate of Incorporation, in furtherance and not in limitation of the powers conferred by statute, the Board of Directors is expressly authorized to make, alter or repeal the Bylaws of the Corporation.

Article VI.

For the management of the business and for the conduct of the affairs of the Corporation, and in further definition, limitation, and regulation of the powers of the Corporation and of its directors and of its stockholders or any class thereof, as the case may be, it is further provided:

1. The management of the business and the conduct of the affairs of the Corporation shall be vested in its Board of Directors. The number of directors which shall constitute the whole Board of Directors shall be fixed by, or in the manner provided in, the Bylaws. The phrase "whole Board" and the phrase "total number of directors" shall be deemed to have the same meaning, to wit, the total number of directors which the Corporation would have if there were no vacancies. Elections of directors need not be by written ballot unless the Bylaws of the Corporation shall so provide.

2. After the original or other Bylaws of the Corporation have been adopted, amended, or repealed, as the case may be, in accordance with the provisions of Section 109 of the Delaware General Corporation Law, and, after the Corporation has received any payment for any of its stock, the power to adopt, amend, or repeal the Bylaws of the Corporation may be exercised by the Board of Directors of the Corporation.

Article VII.

Whenever a compromise or arrangement is proposed between this Corporation and its creditors or any class of them and/or between this Corporation and its stockholders or any class of them, any court of equitable jurisdiction within the State of Delaware may, on the application in a summary way of this Corporation or of any creditor or stockholder thereof, or on the application of any receiver or receivers appointed for this Corporation under the provisions of Section 291 of the Delaware General Corporation Law or on the application of trustees in dissolution or of any receiver or receivers appointed for this Corporation under the provisions of Section 279 of the Delaware General Corporation Law order a meeting of the creditors or class of creditors, and/or of the stockholders or class of stockholders of this Corporation, as the case may be, to be summoned in such manner as the said court directs. If a majority in number representing three-fourths in value of the creditors or class of creditors, and/or of the stockholders or class of stockholders of this Corporation, as the case may be, agree to any compromise or arrangement and to any reorganization of this Corporation as a consequence of such compromise or arrangement, the said compromise or arrangement and the said reorganization shall, if sanctioned by the court to which the said application has been made, be binding on all the creditors or class of creditors, and/or on all the stockholders or class of stockholders, of this Corporation, as the case may be, and also on this Corporation.

Article VIII.

A director of this Corporation shall, to the fullest extent permitted by the Delaware General Corporation Law as it now exists or as it may hereafter be amended, not be personally liable to this Corporation or its stockholders for monetary damages for breach of fiduciary duty as a director, except for liability (i) for any breach of the director's duty of loyalty to this Corporation or its stockholders, (ii) for acts or omissions not in good faith or that involve intentional misconduct or a knowing violation of law, (iii) under Section 174 of the Delaware General Corporation Law, or (iv) for any transaction from which the director derived any improper personal benefit. If the Delaware General Corporation Law is amended, after approval by the stockholders of this Article VIII, to authorize any action by the Corporation which further eliminates or limits the personal liability of directors, then the liability of a director of this Corporation shall be eliminated or limited to the fullest extent permitted by the Delaware General Corporation Law, as so amended.

Any amendment, repeal or modification of this Article VIII, or the adoption of any provision of this Certificate of Incorporation inconsistent with this Article VIII, shall not adversely affect any right or protection of a director of this Corporation existing at the time of such amendment, repeal, modification or adoption.

Article IX.

The Corporation shall, to the fullest extent permitted by the provisions of Section 145 of the Delaware General Corporation Law, as the same may be amended and supplemented, indemnify any and all persons whom it shall have power to indemnify under said section from and against any and all of the expenses, liabilities, or other matters referred to in or covered by said section, and the indemnification provided for herein shall not be deemed exclusive of any other rights to which those indemnified may be entitled under any Bylaw, agreement, vote of stockholders or disinterested directors or otherwise, both as to action in his or her official capacity and as to action in another capacity while holding such office, and shall continue as to a person who has ceased to be a director, officer, employee, or agent and shall inure to the benefit of the heirs, executors, and administrators of such person.

Any amendment, repeal or modification of this Article IX, or the adoption of any provision of this Certificate of Incorporation inconsistent with this Article IX, shall not adversely affect any right or protection existing at the time of such amendment, repeal, modification or adoption.

The Corporation renounces any interest or expectancy of the Corporation in, or in being offered an opportunity to participate in, any Excluded Opportunity. An “Excluded Opportunity” is any matter, transaction or interest that is presented to, or acquired, created or developed by, or which otherwise comes into the possession of, any director of the Corporation who is not an employee of the Corporation or any of its subsidiaries, (collectively, “Covered Persons”), unless in either case such matter, transaction or interest is presented to, or acquired, created or developed by, or otherwise comes into the possession of, a Covered Person expressly and solely in such Covered Person’s capacity as a director of the Corporation.

Article X.

Meetings of stockholders may be held within or without the State of Delaware, as the Bylaws may provide. The books of this Corporation may be kept (subject to any provision contained in the statutes) outside the State of Delaware at such place or places as may be designated from time to time by the Board of Directors or in the Bylaws of this Corporation.

* * *

IN WITNESS WHEREOF, the Corporation has caused this Certificate to be signed by its Chief Financial Officer, Senior Vice President, Finance and Secretary, this 18th day of December, 2012.

ULTRAGENYX PHARMACEUTICAL INC.

/s/ Shalini Sharp
Shalini Sharp
Chief Financial Officer, Senior Vice President, Finance and Secretary

BYLAWS

OF

ULTRAGENYX PHARMACEUTICAL INC.

a Delaware corporation

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BYLAWS

OF

ULTRAGENYX PHARMACEUTICAL INC.

ARTICLE 1

OFFICES

Section 1.1 Registered Office.

The registered office of the corporation in the State of Delaware shall be in the City of Wilmington, County of New Castle.

Section 1.2 Other Offices.

The corporation shall also have and maintain an office or principal place of business within or without the State of Delaware as the Board of Directors may from time to time determine or the business of the corporation may require.

ARTICLE 2

STOCKHOLDERS' MEETINGS

Section 2.1 Place of Meetings.

(a) Meetings of stockholders may be held at such place, either within or without this State, as may be designated by or in the manner provided in these Bylaws or, if not so designated, as determined by the Board of Directors. The Board of Directors may, in its sole discretion, determine that the meeting shall not be held at any place, but may instead be held solely by means of remote communication as authorized by paragraph (b) of this Section 2.1.

(b) If authorized by the Board of Directors in its sole discretion, and subject to such guidelines and procedures as the Board of Directors may adopt, stockholders and proxyholders not physically present at a meeting of stockholders may, by means of remote communication:

(1) Participate in a meeting of stockholders; and

(2) Be deemed present in person and vote at a meeting of stockholders whether such meeting is to be held at a designated place or solely by means of remote communication, provided that (A) the corporation shall implement reasonable measures to verify that each person deemed present and permitted to vote at the meeting by means of remote communication is a stockholder or proxyholder, (B) the corporation shall implement reasonable measures to provide such stockholders and proxyholders a reasonable opportunity to participate

in the meeting and to vote on matters submitted to the stockholders, including an opportunity to read or hear the proceedings of the meeting substantially concurrently with such proceedings, and (C) if any stockholder or proxyholder votes or takes other action at the meeting by means of remote communication, a record of such vote or other action shall be maintained by the corporation.

(c) For purposes of this Section 2.1, “remote communication” shall include (1) telephone or other voice communications and (2) electronic mail or other form of written or visual electronic communications satisfying the requirements of Section 2.11(b).

Section 2.2 Annual Meetings.

The annual meetings of the stockholders of the corporation, for the purpose of election of directors and for such other business as may lawfully come before it, shall be held on such date and at such time as may be designated from time to time by the Board of Directors.

Section 2.3 Special Meetings.

Special Meetings of the stockholders of the corporation may be called, for any purpose or purposes, by the Chairman of the Board or the President or the Board of Directors at any time. Upon written request of any stockholder or stockholders holding in the aggregate ten percent (10%) of the voting power of all stockholders delivered in person or sent by registered mail to the Chairman of the Board, President or Secretary of the Corporation, the Secretary shall call a special meeting of stockholders to be held as provided in Section 2.1 at such time as the Secretary may fix, such meeting to be held not less than 10 nor more than 60 days after the receipt of such request, and if the Secretary shall neglect or refuse to call such meeting within seven days after the receipt of such request, the stockholder making such request may do so.

Section 2.4 Notice of Meetings.

(a) Except as otherwise provided by law or the Certificate of Incorporation, written notice of each meeting of stockholders, specifying the place, if any, date and hour and purpose or purposes of the meeting, and the means of remote communication, if any, by which stockholders and proxyholders may be deemed to be present in person and vote at such meeting, shall be given not less than 10 nor more than 60 days before the date of the meeting to each stockholder entitled to vote thereat, directed to his address as it appears upon the books of the corporation; except that where the matter to be acted on is a merger or consolidation of the Corporation or a sale, lease or exchange of all or substantially all of its assets, such notice shall be given not less than 20 nor more than 60 days prior to such meeting.

(b) If at any meeting action is proposed to be taken which, if taken, would entitle shareholders fulfilling the requirements of section 262(d) of the Delaware General Corporation Law to an appraisal of the fair value of their shares, the notice of such meeting shall contain a statement of that purpose and to that effect and shall be accompanied by a copy of that statutory section.

(c) When a meeting is adjourned to another time or place, notice need not be given of the adjourned meeting if the time, place, if any, thereof, and the means of remote communication, if any, by which stockholders and proxyholders may be deemed to be present in person and vote at such adjourned meeting, are announced at the meeting at which the adjournment is taken unless the adjournment is for more than thirty days, or unless after the adjournment a new record date is fixed for the adjourned meeting, in which event a notice of the adjourned meeting shall be given to each stockholder of record entitled to vote at the meeting.

(d) Notice of the time, place and purpose of any meeting of stockholders may be waived in writing, either before or after such meeting and, to the extent permitted by law, will be waived by any stockholder by his attendance thereat, in person or by proxy. Any stockholder so waiving notice of such meeting shall be bound by the proceedings of any such meeting in all respects as if due notice thereof had been given.

(e) Without limiting the manner by which notice otherwise may be given effectively to stockholders, any notice to stockholders given by the corporation under any provision of Delaware General Corporation Law, the certificate of incorporation, or these Bylaws shall be effective if given by a form of electronic transmission consented to by the stockholder to whom the notice is given. Any such consent shall be revocable by the stockholder by written notice to the corporation. Any such consent shall be deemed revoked if (i) the corporation is unable to deliver by electronic transmission two consecutive notices given by the corporation in accordance with such consent, and (ii) such inability becomes known to the secretary or an assistant secretary of the corporation or to the transfer agent or other person responsible for the giving of notice; provided, however, the inadvertent failure to treat such inability as a revocation shall not invalidate any meeting or other action. Notice given pursuant to this subparagraph (e) shall be deemed given: (1) if by facsimile telecommunication, when directed to a number at which the stockholder has consented to receive notice; (2) if by electronic mail, when directed to an electronic mail address at which the stockholder has consented to receive notice; (3) if by a posting on an electronic network together with separate notice to the stockholder of such specific posting, upon the later of (A) such posting and (B) the giving of such separate notice; and (4) if by any other form of electronic transmission, when directed to the stockholder. An affidavit of the secretary or an assistant secretary or of the transfer agent or other agent of the corporation that the notice has been given by a form of electronic transmission shall, in the absence of fraud, be prima facie evidence of the facts stated therein. For purposes of these Bylaws, “electronic transmission” means any form of communication, not directly involving the physical transmission of paper, that creates a record that may be retained, retrieved and reviewed by a recipient thereof, and that may be directly reproduced in paper form by such a recipient through an automated process.

Section 2.5 Quorum and Voting.

(a) At all meetings of stockholders except where otherwise provided by law, the Certificate of Incorporation or these Bylaws, the presence, in person or by proxy duly authorized, of the holders of a majority of the outstanding shares of stock entitled to vote shall constitute a quorum for the transaction of business. Shares, the voting of which at said meeting have been enjoined, or which for any reason cannot be lawfully voted at such meeting, shall not be counted to determine a quorum at said meeting. In the absence of a quorum, any meeting of stockholders may be adjourned, from time to time, by vote of the holders of a majority of the shares represented thereat, but no other business shall be transacted at such meeting. At such adjourned

meeting at which a quorum is present or represented, any business may be transacted which might have been transacted at the original meeting. The stockholders present at a duly called or convened meeting at which a quorum is present may continue to transact business until adjournment, notwithstanding the withdrawal of enough stockholders to leave less than a quorum.

(b) Except as otherwise provided by law, the Certificate of Incorporation or these Bylaws, all action taken by the holders of a majority of the voting power represented at any meeting at which a quorum is present shall be valid and binding upon the corporation.

(c) Where a separate vote by a class or classes is required, a majority of the outstanding shares of such class or classes present in person or represented by proxy shall constitute a quorum entitled to take action with respect to that vote on that matter, and the affirmative vote of the majority of shares of such class or classes present in person or represented by proxy at the meeting shall be the act of such class.

Section 2.6 Voting Rights.

(a) Except as otherwise provided by law, only persons in whose names shares entitled to vote stand on the stock records of the corporation on the record date for determining the stockholders entitled to vote at said meeting shall be entitled to vote at such meeting. Shares standing in the names of two or more persons shall be voted or represented in accordance with the determination of the majority of such persons, or, if only one of such persons is present in person or represented by proxy, such person shall have the right to vote such shares and such shares shall be deemed to be represented for the purpose of determining a quorum.

(b) Every person entitled to vote or to execute consents shall have the right to do so either in person or by an agent or agents authorized by a written proxy executed by such person or his duly authorized agent, which proxy shall be filed with the Secretary of the corporation at or before the meeting at which it is to be used. Said proxy so appointed need not be a stockholder. No proxy shall be voted on after three (3) years from its date unless the proxy provides for a longer period. Unless and until voted, every proxy shall be revocable at the pleasure of the person who executed it or of his legal representatives or assigns, except in those cases where an irrevocable proxy permitted by statute has been given.

(c) Without limiting the manner in which a stockholder may authorize another person or persons to act for him as proxy pursuant to subsection (b) of this section, the following shall constitute a valid means by which a stockholder may grant such authority:

(1) A stockholder may execute a writing authorizing another person or persons to act for him as proxy. Execution may be accomplished by the stockholder or his authorized officer, director, employee or agent signing such writing or causing his or her signature to be affixed to such writing by any reasonable means including, but not limited to, by facsimile signature.

(2) A stockholder may authorize another person or persons to act for him as proxy by transmitting or authorizing the transmission of a telephone, telegram, cablegram or other means of electronic transmission to the person who will be the holder of the proxy or to a

proxy solicitation firm, proxy support service organization or like agent duly authorized by the person who will be the holder of the proxy to receive such transmission, provided that any such telephone, telegram, cablegram or other means of electronic transmission must either set forth or be submitted with information from which it can be determined that the telephone, telegram, cablegram or other electronic transmission was authorized by the stockholder. Such authorization can be established by the signature of the stockholder on the proxy, either in writing or by a signature stamp or facsimile signature, or by a number or symbol from which the identity of the stockholder can be determined, or by any other procedure deemed appropriate by the inspectors or other persons making the determination as to due authorization.

If it is determined that such telegrams, cablegrams or other electronic transmissions are valid, the inspectors or, if there are no inspectors, such other persons making that determination shall specify the information upon which they relied.

(d) Any copy, facsimile telecommunication or other reliable reproduction of the writing or transmission created pursuant to subsection (c) of this section may be substituted or used in lieu of the original writing or transmission for any and all purposes for which the original writing or transmission could be used, provided that such copy, facsimile telecommunication or other reproduction shall be a complete reproduction of the entire original writing or transmission.

Section 2.7 Voting Procedures and Inspectors of Elections.

(a) The corporation shall, in advance of any meeting of stockholders, appoint one or more inspectors to act at the meeting and make a written report thereof. The corporation may designate one or more persons as alternate inspectors to replace any inspector who fails to act. If no inspector or alternate is able to act at a meeting of stockholders, the person presiding at the meeting shall appoint one or more inspectors to act at the meeting. Each inspector, before entering upon the discharge of his duties, shall take and sign an oath faithfully to execute the duties of inspector with strict impartiality and according to the best of his ability.

(b) The inspectors shall (i) ascertain the number of shares outstanding and the voting power of each, (ii) determine the shares represented at a meeting and the validity of proxies and ballots, (iii) count all votes and ballots, (iv) determine and retain for a reasonable period a record of the disposition of any challenges made to any determination by the inspectors, and (v) certify their determination of the number of shares represented at the meeting and their count of all votes and ballots. The inspectors may appoint or retain other persons or entities to assist the inspectors in the performance of the duties of the inspectors.

(c) The date and time of the opening and the closing of the polls for each matter upon which the stockholders will vote at a meeting shall be announced at the meeting. No ballot, proxies or votes, nor any revocations thereof or changes thereto, shall be accepted by the inspectors after the closing of the polls unless the Court of Chancery upon application by a stockholder shall determine otherwise.

(d) In determining the validity and counting of proxies and ballots, the inspectors shall be limited to an examination of the proxies, any envelopes submitted with those proxies, any information provided in accordance with Sections 211(e) or 212(c)(2) of the Delaware

General Corporation Law, or any information provided pursuant to Section 21 I(a)(2)(B)(i) or (iii) thereof, ballots and the regular books and records of the corporation, except that the inspectors may consider other reliable information for the limited purpose of reconciling proxies and ballots submitted by or on behalf of banks, brokers, their nominees or similar persons which represent more votes than the holder of a proxy is authorized by the record owner to cast or more votes than the stockholder holds of record. If the inspectors consider other reliable information for the limited purpose permitted herein, the inspectors at the time they make their certification pursuant to subsection (b)(v) of this section shall specify the precise information considered by them including the person or persons from whom they obtained the information, when the information was obtained, the means by which the information was obtained and the basis for the inspectors' belief that such information is accurate and reliable.

Section 2.8 List of Stockholders.

The officer who has charge of the stock ledger of the corporation shall prepare and make, at least ten days before every meeting of stockholders, a complete list of the stockholders entitled to vote at said meeting, arranged in alphabetical order, showing the address of and the number of shares registered in the name of each stockholder. The corporation need not include electronic mail addresses or other electronic contact information on such list. Such list shall be open to the examination of any stockholder for any purpose germane to the meeting for a period of at least 10 days prior to the meeting: (i) on a reasonably accessible electronic network, provided that the information required to gain access to such list is provided with the notice of the meeting, or (ii) during ordinary business hours at the principal place of business of the corporation. In the event that the corporation determines to make the list available on an electronic network, the corporation may take reasonable steps to ensure that such information is available only to stockholders of the corporation. If the meeting is to be held at a place, then the list shall be produced and kept at the time and place of the meeting during the whole time thereof, and may be inspected by any stockholder who is present. If the meeting is to be held solely by means of remote communication, then the list shall also be open to the examination of any stockholder during the whole time of the meeting on a reasonably accessible electronic network, and the information required to access such list shall be provided with the notice of the meeting.

Section 2.9 Stockholder Proposals at Annual Meetings.

At an annual meeting of the stockholders, only such business shall be conducted as shall have been properly brought before the meeting. To be properly brought before an annual meeting, business must be specified in the notice of meeting (or any supplement thereto) given by or at the direction of the Board of Directors, otherwise properly brought before the meeting by or at the direction of the Board of Directors, or otherwise properly brought before the meeting by a stockholder. In addition to any other applicable requirements for business to be properly brought before an annual meeting by a stockholder, the stockholder must have given timely notice thereof in writing to the Secretary of the corporation. To be timely a stockholder's notice must be delivered to or mailed and received at the principal executive offices of the corporation not less than 45 days nor more than 75 days prior to the date on which the corporation first mailed its proxy materials for the previous year's annual meeting of stockholders (or the date on which the corporation mails its proxy materials for the current year if during the prior year the corporation did not hold an annual meeting or if the date of the annual meeting was changed

more than 30 days from the prior year). A stockholder's notice to the Secretary shall set forth as to each matter the stockholder proposes to bring before the annual meeting (i) a brief description of the business desired to be brought before the annual meeting and the reasons for conducting such business at the annual meeting, (ii) the name and record address of the stockholder proposing such business, (iii) the class and number of shares of the corporation which are beneficially owned by the stockholder, and (iv) any material interest of the stockholder in such business.

Notwithstanding anything in the Bylaws to the contrary; no business shall be conducted at the annual meeting except in accordance with the procedures set forth in Section 2.1 and this Section 2.9, provided, however, that nothing in this Section 2.9 shall be deemed to preclude discussion by any stockholder of any business properly brought before the annual meeting in accordance with said procedure.

The Chairman of an annual meeting shall, if the facts warrant, determine and declare to the meeting that business was not properly brought before the meeting in accordance with the provisions of Section 2.1 and this Section 2.9, and if he should so determine he shall so declare to the meeting, and any such business not properly brought before the meeting shall not be transacted.

Nothing in this Section 2.9 shall affect the right of a stockholder to request inclusion of a proposal in the corporation's proxy statement to the extent that such right is provided by an applicable rule of the Securities and Exchange Commission.

Section 2.10 Nominations of Persons for Election to the Board of Directors.

In addition to any other applicable requirements, only persons who are nominated in accordance with the following procedures shall be eligible for election as directors. Nominations of persons for election to the Board of Directors of the corporation may be made at a meeting of stockholders by or at the direction of the Board of Directors, by any nominating committee or person appointed by the Board of Directors or by any stockholder of the corporation entitled to vote for the election of directors at the meeting who complies with the notice procedures set forth in this Section 2.10. Such nominations, other than those made by or at the direction of the Board of Directors, shall be made pursuant to timely notice in writing to the Secretary of the corporation. To be timely, a stockholder's notice must be delivered to or mailed and received at the principal executive offices of the corporation, not less than 45 days nor more than 75 days prior to the date on which the corporation first mailed its proxy materials for the previous year's annual meeting of shareholders (or the date on which the corporation mails its proxy materials for the current year if during the prior year the corporation did not hold an annual meeting or if the date of the annual meeting was changed more than 30 days from the prior year). Such stockholder's notice shall set forth (a) as to each person whom the stockholder proposes to nominate for election or re-election as a director, (i) the name, age, business address and residence address of the person, (ii) the principal occupation or employment of the person, (iii) the class and number of shares of the corporation which are beneficially owned by the person, and (iv) any other information relating to the person that is required to be disclosed in solicitations for proxies for election of directors pursuant to Rule 14a under the Securities Exchange Act of 1934; and (b) as to the stockholder giving the notice, (i) the name and record

address of the stockholder, and (ii) the class and number of shares of the corporation which are beneficially owned by the stockholder. The corporation may require any proposed nominee to furnish such other information as may reasonably be required by the corporation to determine the eligibility of such proposed nominee to serve as a director of the corporation. No person shall be eligible for election as a director of the corporation unless nominated in accordance with the procedures set forth herein. These provisions shall not apply to nomination of any persons entitled to be separately elected by holders of preferred stock.

The Chairman of the meeting shall, if the facts warrant, determine and declare to the meeting that a nomination was not made in accordance with the foregoing procedure, and if he should so determine, he shall so declare to the meeting and the defective nomination shall be disregarded.

Section 2.11 Action Without Meeting.

(a) Unless otherwise provided in the Certificate of Incorporation, any action required by statute to be taken at any annual or special meeting of stockholders of the corporation, or any action which may be taken at any annual or special meeting of such stockholders, may be taken without a meeting, without prior notice and without a vote, if a consent or consents in writing setting forth the action so taken are signed by the holders of outstanding stock having not less than the minimum number of votes that would be necessary to authorize or take such action at a meeting at which all shares entitled to vote thereon were present and voted. To be effective, a written consent must be delivered to the corporation by delivery to its registered office in Delaware, its principal place of business, or an officer or agent of the corporation having custody of the book in which proceedings of meetings of stockholders are recorded. Delivery made to a corporation's registered office shall be by hand or by certified or registered mail, return receipt requested. Every written consent shall bear the date of signature of each stockholder who signs the consent, and no written consent shall be effective to take the corporate action referred to therein unless, within 60 days of the earliest dated consent delivered in the manner required by this Section to the corporation, written consents signed by a sufficient number of holders to take action are delivered to the corporation in accordance with this Section. Prompt notice of the taking of the corporate action without a meeting by less than unanimous written consent shall be given to those stockholders who have not consented in writing.

(b) A telegram, cablegram or other electronic transmission consent to an action to be taken and transmitted by a stockholder or proxyholder, or by a person or persons authorized to act for a stockholder or proxyholder, shall be deemed to be written, signed and dated for the purposes of this section, provided that any such telegram, cablegram or other electronic transmission sets forth or is delivered with information from which the corporation can determine (i) that the telegram, cablegram or other electronic transmission was transmitted by the stockholder or proxyholder or by a person or persons authorized to act for the stockholder or proxyholder, and (ii) the date on which such stockholder or proxyholder or authorized person or persons transmitted such telegram, cablegram or electronic transmission. The date on which such telegram, cablegram or electronic transmission is transmitted shall be deemed to be the date on which such consent was signed. No consent given by telegram, cablegram or other electronic transmission shall be deemed to have been delivered until such consent is reproduced in paper form and until such paper form shall be delivered to the corporation by delivery to its registered

office in this State, its principal place of business or an officer or agent of the corporation having custody of the book in which proceedings of meetings of stockholders are recorded. Delivery made to a corporation's registered office shall be made by hand or by certified or registered mail, return receipt requested. Notwithstanding the foregoing limitations on delivery, consents given by telegram, cablegram or other electronic transmission may be otherwise delivered to the principal place of business of the corporation or to an officer or agent of the corporation having custody of the book in which proceedings of meetings of stockholders are recorded if to, the extent and in the manner provided by resolution of the Board of Directors of the corporation.

(c) Any copy, facsimile or other reliable reproduction of a consent in writing may be substituted or used in lieu of the original writing for any and all purposes for which the original writing could be used, provided that such copy, facsimile or other reproduction shall be a complete reproduction of the entire original writing.

ARTICLE 3

DIRECTORS

Section 3.1 Number and Term of Office.

The number of directors shall be determined from time to time by resolution of the Board of Directors, provided that the Board of Directors shall consist of at least one member. With the exception of the first Board of Directors, which shall be elected by the incorporators, and except as provided in Section 3.3 of this Article III, the directors shall be elected by a plurality vote of the shares represented in person or by proxy at the stockholders annual meeting in each year and entitled to vote on the election of directors. Elected directors shall hold office until the next annual meeting and until their successors shall be duly elected and qualified. Directors need not be stockholders. If, for any cause, the Board of Directors shall not have been elected at an annual meeting, they may be elected as soon thereafter as convenient at a special meeting of the stockholders called for that purpose in the manner provided in these Bylaws.

Section 3.2 Powers.

The powers of the corporation shall be exercised, its business conducted and its property controlled by or under the direction of the Board of Directors.

Section 3.3 Vacancies.

Vacancies and newly created directorships resulting from any increase in the authorized number of directors may be filled by a majority of the directors then in office, although less than a quorum, or by a sole remaining director, and each director so elected shall hold office for the unexpired portion of the term of the director whose place shall be vacant and until his successor shall have been duly elected and qualified. A vacancy in the Board of Directors shall be deemed to exist under this section in the case of the death, removal or resignation of any director, or if the stockholders fail at any meeting of stockholders at which directors are to be elected (including any meeting referred to in Section 3.4 below) to elect the number of directors then constituting the whole Board.

Section 3.4 Resignations and Removals.

(a) Any director may resign at any time by delivering his resignation to the Secretary in writing or by electronic transmission, such resignation to specify whether it will be effective at a particular time, upon receipt by the Secretary or at the pleasure of the Board of Directors. If no such specification is made it shall be deemed effective at the pleasure of the Board of Directors. When one or more directors shall resign from the Board effective at a future date, a majority of the directors then in office, including those who have so resigned, shall have power to fill such vacancy or vacancies, the vote thereon to take effect when such resignation or resignations shall become effective, and each director so chosen shall hold office for the unexpired portion of the term of the director whose place shall be vacated and until his successor shall have been duly elected and qualified.

(b) At a special meeting of stockholders called for the purpose in the manner hereinabove provided, the Board of Directors or any individual director may be removed from office, with or without cause, and a new director or directors elected by a vote of stockholders holding a majority of the outstanding shares entitled to vote at an election of directors.

Section 3.5 Meetings.

(a) The annual meeting of the Board of Directors shall be held immediately after the annual stockholders' meeting and at the place where such meeting is held or at the place announced by the Chairman at such meeting. No notice of an annual meeting of the Board of Directors shall be necessary, and such meeting shall be held for the purpose of electing officers and transacting such other business as may lawfully come before it.

(b) Except as hereinafter otherwise provided; regular meetings of the Board of Directors shall be held in the office of the corporation required to be maintained pursuant to Section 1.2 of Article I hereof. Regular meetings of the Board of Directors may also be held at any place, within or without the State of Delaware, which has been designated by resolutions of the Board of Directors or the written consent of all directors.

(c) Special meetings of the Board of Directors may be held at any time and place within or without the State of Delaware whenever called by the Chairman of the Board or, if there is no Chairman of the Board, by the President, or by any of the directors.

(d) Written notice of the time and place of all regular and special meetings of the Board of Directors shall be delivered personally to each director or sent by telegram or facsimile transmission or other form of electronic transmission at least 48 hours before the start of the meeting, or sent by first class mail at least 120 hours before the start of the meeting. Notice of any meeting may be waived in writing at any time before or after the meeting and will be waived by any director by attendance thereat.

Section 3.6 Quorum and Voting.

(a) A quorum of the Board of Directors shall consist of a majority of the exact number of directors fixed from time to time in accordance with Section 3.1 of Article III of these Bylaws, but not less than one; provided, however, at any meeting whether a quorum be present or otherwise, a majority of the directors present may adjourn from time to time until the time fixed for the next regular meeting of the Board of Directors, without notice other than by announcement at the meeting.

(b) At each meeting of the Board at which a quorum is present, all questions and business shall be determined by a vote of a majority of the directors present, unless a different vote be required by law, the Certificate of Incorporation, or these Bylaws.

(c) Any member of the Board of Directors, or of any committee thereof, may participate in a meeting by means of conference telephone or other communication equipment by means of which all persons participating in the meeting can hear each other, and participation in a meeting by such means shall constitute presence in person at such meeting.

(d) The transactions of any meeting of the Board of Directors, or any committee thereof, however called or noticed, or wherever held, shall be as valid as though had at a meeting duly held after regular call and notice if a quorum be present and if, either before or after the meeting, each of the directors not present shall sign a written waiver of notice, or a consent to holding such meeting or an approval of the minutes thereof. All such waivers, consents or approvals shall be filed with the corporate records or made a part of the minutes of the meeting.

Section 3.7 Action Without Meeting.

Unless otherwise restricted by the Certificate of Incorporation or these Bylaws, any action required or permitted to be taken at any meeting of the Board of Directors or of any committee thereof may be taken without a meeting, if all members of the Board or of such committee, as the case may be, consent thereto in writing or by electronic transmission, and such writing or writings or electronic transmission or transmissions are filed with the minutes of proceedings of the Board or committee. Such filing shall be in paper form if the minutes are maintained in paper form and shall be in electronic form if the minutes are maintained in electronic form.

Section 3.8 Fees and Compensation.

Directors and members of committees may receive such compensation, if any, for their services, and such reimbursement for expenses, as may be fixed or determined by resolution of the Board of Directors.

Section 3.9 Committees.

(a) **Executive Committee**: The Board of Directors may appoint an Executive Committee of not less than one member, each of whom shall be a director. The Executive Committee, to the extent permitted by law, shall have and may exercise when the Board of Directors is not in session all powers of the Board in the management of the business and affairs of the corporation, except such committee shall not have the power or authority to amend these Bylaws or to approve or recommend to the stockholders any action which must be submitted to stockholders for approval under the General Corporation Law.

(b) **Other Committees:** The Board of Directors may from time to time appoint such other committees as may be permitted by law. Such other committees appointed by the Board of Directors shall have such powers and perform such duties as may be prescribed by the resolution or resolutions creating such committee, but in no event shall any such committee have the powers denied to the Executive Committee in these Bylaws.

(c) **Term:** The terms of members of all committees of the Board of Directors shall expire on the date of the next annual meeting of the Board of Directors following their appointment; provided that they shall continue in office until their successors are appointed. The Board, subject to the provisions of subsections (a) or (b) of this Section 3.9, may at any time increase or decrease the number of members of a committee or terminate the existence of a committee; provided that no committee shall consist of less than one member. The membership of a committee member shall terminate on the date of his death or voluntary resignation, but the Board may at any time for any reason remove any individual committee member and the Board may fill any committee vacancy created by death, resignation, removal or increase in the number of members of the committee. The Board of Directors may designate one or more directors as alternate members of any committee, who may replace any absent or disqualified member at any meeting of the committee, and, in addition, in the absence or disqualification of any member of a committee, the member or members thereof present at any meeting and not disqualified from voting, whether or not he or they constitute a quorum, may unanimously appoint another member of the Board of Directors to act at the meeting in the place of any such absent or disqualified member.

(d) **Meetings:** Unless the Board of Directors shall otherwise provide, regular meetings of the Executive Committee or any other committee appointed pursuant to this Section 3.9 shall be held at such times and places as are determined by the Board of Directors, or by any such committee, and when notice thereof has been given to each member of such committee, no further notice of such regular meetings need be given thereafter; special meetings of any such committee may be held at the principal office of the corporation required to be maintained pursuant to Section 1.2 of Article I hereof; or at any place which has been designated from time to time by resolution of such committee or by written consent of all members thereof, and may be called by any director who is a member of such committee upon written notice to the members of such committee of the time and place of such special meeting given in the manner provided for the giving of written notice to members of the Board of Directors of the time and place of special meetings of the Board of Directors. Notice of any special meeting of any committee may be waived in writing at any time after the meeting and will be waived by any director by attendance thereat. A majority of the authorized number of members of any such committee shall constitute a quorum for the transaction of business, and the act of a majority of those present at any meeting at which a quorum is present shall be the act of such committee.

ARTICLE 4

OFFICERS

Section 4.1 Officers Designated.

The officers of the corporation shall be a President, a Secretary and a Treasurer. The Board of Directors or the President may also appoint a Chairman of the Board, one or more Vice-Presidents, assistant secretaries, assistant treasurers, and such other officers and agents with such powers and duties as it or he shall deem necessary. The order of the seniority of the Vice-Presidents shall be in the order of their nomination unless otherwise determined by the Board of Directors. The Board of Directors may assign such additional titles to one or more of the officers as they shall deem appropriate. Any one person may hold any number of offices of the corporation at any one time unless specifically prohibited therefrom by law. The salaries and other compensation of the officers of the corporation shall be fixed by or in the manner designated by the Board of Directors.

Section 4.2 Tenure and Duties of Officers.

(a) **General:** All officers shall hold office at the pleasure of the Board of Directors and until their successors shall have been duly elected and qualified, unless sooner removed. Any officer elected or appointed by the Board of Directors may be removed at any time by the Board of Directors. If the office of any officer becomes vacant for any reason, the vacancy may be filled by the Board of Directors. Nothing in these Bylaws shall be construed as creating any kind of contractual right to employment with the corporation.

(b) **Duties of the Chairman of the Board of Directors:** The Chairman of the Board of Directors (if there be such an officer appointed) when present shall preside at all meetings of the stockholders and the Board of Directors. The Chairman of the Board of Directors shall perform such other duties and have such other powers as the Board of Directors shall designate from time to time.

(c) **Duties of President:** The President shall be the chief executive officer of the corporation and shall preside at all meetings of the stockholders and at all meetings of the Board of Directors, unless the Chairman of the Board of Directors has been appointed and is present. The President shall perform such other duties and have such other powers as the Board of Directors shall designate from time to time.

(d) **Duties of Vice-Presidents:** The Vice-Presidents, in the order of their seniority, may assume and perform the duties of the President in the absence or disability of the President or whenever the office of the President is vacant. The Vice-President shall perform such other duties and have such other powers as the Board of Directors or the President shall designate from time to time.

(e) **Duties of Secretary:** The Secretary shall attend all meetings of the stockholders and of the Board of Directors and any committee thereof, and shall record all acts and proceedings thereof in the minute book of the corporation, which may be maintained in either paper or electronic form. The Secretary shall give notice, in conformity with these Bylaws, of all

meetings of the stockholders and of all meetings of the Board of Directors and any Committee thereof requiring notice. The Secretary shall perform such other duties and have such other powers as the Board of Directors shall designate from time to time. The President may direct any assistant secretary to assume and perform the duties of the Secretary in the absence or disability of the Secretary, and each assistant secretary shall perform such other duties and have such other powers as the Board of Directors or the President shall designate from time to time.

(f) **Duties of Treasurer:** The Treasurer shall keep or cause to be kept the books of account of the corporation in a thorough and proper manner, and shall render statements of the financial affairs of the corporation in such form and as often as required by the Board of Directors or the President. The Treasurer, subject to the order of the Board of Directors, shall have the custody of all funds and securities of the corporation. The Treasurer shall perform all other duties commonly incident to his office and shall perform such other duties and have such other powers as the Board of Directors or the President shall designate from time to time. The President may direct any assistant treasurer to assume and perform the duties of the Treasurer in the absence or disability of the Treasurer, and each assistant treasurer shall perform such other duties and have such other powers as the Board of Directors or the President shall designate from time to time.

ARTICLE 5

EXECUTION OF CORPORATE INSTRUMENTS, AND VOTING OF SECURITIES OWNED BY THE CORPORATION

Section 5.1 Execution of Corporate Instruments.

(a) The Board of Directors may in its discretion determine the method and designate the signatory officer or officers, or other person or persons, to execute any corporate instrument or document, or to sign the corporate name without limitation, except where otherwise provided by law, and such execution or signature shall be binding upon the corporation.

(b) Unless otherwise specifically determined by the Board of Directors or otherwise required by law, formal contracts of the corporation, promissory notes, deeds of trust, mortgages and other evidences of indebtedness of the corporation, and other corporate instruments or documents requiring the corporate seal, and certificates of shares of stock owned by the corporation, shall be executed, signed or endorsed by the Chairman of the Board (if there be such an officer appointed) or by the President; such documents may also be executed by any Vice-President and by the Secretary or Treasurer or any assistant secretary or assistant treasurer. All other instruments and documents requiring the corporate signature but not requiring the corporate seal may be executed as aforesaid or in such other manner as may be directed by the Board of Directors.

(c) All checks and drafts drawn on banks or other depositaries on funds to the credit of the corporation or in special accounts of the corporation shall be signed by such person or persons as the Board of Directors shall authorize so to do.

(d) Execution of any corporate instrument may be effected in such form, either manual, facsimile or electronic signature, as may be authorized by the Board of Directors.

Section 5.2 Voting of Securities Owned by Corporation.

All stock and other securities of other corporations owned or held by the corporation for itself or for other parties in any capacity shall be voted, and all proxies with respect thereto shall be executed, by the person authorized so to do by resolution of the Board of Directors or, in the absence of such authorization, by the Chairman of the Board (if there be such an officer appointed), or by the President, or by any Vice-President.

ARTICLE 6

SHARES OF STOCK

Section 6.1 Form and Execution of Certificates.

The shares of the corporation shall be represented by certificates, provided that the Board of Directors may provide by resolution or resolutions that some or all of any or all classes or series of its stock shall be uncertificated shares. Any such resolution shall not apply to shares represented by a certificate until such certificate is surrendered to the corporation. Certificates for the shares of stock of the corporation shall be in such form as is consistent with the Certificate of Incorporation and applicable law. Every holder of stock in the corporation shall be entitled to have a certificate signed by, or in the name of the corporation by, the Chairman of the Board (if there be such an officer appointed), or by the President or any Vice-President and by the Treasurer or assistant treasurer or the Secretary or assistant secretary, certifying the number of shares owned by him in the corporation. Any or all of the signatures on the certificate may be a facsimile. In case any officer, transfer agent, or registrar who has signed or whose facsimile signature has been placed upon a certificate shall have ceased to be such officer, transfer agent, or registrar before such certificate is issued, it may be issued with the same effect as if he were such officer, transfer agent, or registrar at the date of issue. If the corporation shall be authorized to issue more than one class of stock or more than one series of any class, the powers, designations, preferences and relative, participating, optional or other special rights of each class of stock or series thereof and the qualifications, limitations or restrictions of such preferences and/or rights shall be set forth in full or summarized on the face or back of the certificate which the corporation shall issue to represent such class or series of stock, provided that, except as otherwise provided in section 202 of the Delaware General Corporation Law, in lieu of the foregoing requirements, there may be set forth on the face or back of the certificate which the corporation shall issue to represent such class or series of stock, a statement that the corporation will furnish without charge to each stockholder who so requests the powers, designations, preferences and relative, participating, optional or other special rights of each class of stock or series thereof and the qualifications, limitations or restrictions of such preferences and/or rights.

Section 6.2 Lost Certificates.

The Board of Directors may direct a new certificate or certificates (or uncertificated shares in lieu of a new certificate) to be issued in place of any certificate or certificates theretofore issued by the corporation alleged to have been lost or destroyed, upon the making of an affidavit of that fact by the person claiming the certificate of stock to be lost or destroyed. When authorizing such issue of a new certificate or certificates (or uncertificated shares in lieu of a new certificate), the Board of Directors may, in its discretion and as a condition precedent to the issuance thereof, require the owner of such lost or destroyed certificate or certificates, or his legal representative, to indemnify the corporation in such manner as it shall require and/or to give the corporation a surety bond in such form and amount as it may direct as indemnity against any claim that may be made against the corporation with respect to the certificate alleged to have been lost or destroyed.

Section 6.3 Transfers.

Transfers of record of shares of stock of the corporation shall be made only upon its books by the holders thereof, in person or by attorney duly authorized, who shall furnish proper evidence of authority to transfer, and in the case of stock represented by a certificate, upon the surrender of a certificate or certificates for a like number of shares, properly endorsed.

Section 6.4 Fixing Record Dates.

(a) In order that the corporation may determine the stockholders entitled to notice of or to vote at any meeting of stockholders or any adjournment thereof, the Board of Directors may fix a record date, which record date shall not precede the date upon which the resolution fixing the record date is adopted by the Board of Directors, and which record date shall not be more than 60 nor less than 10 days before the date of such meeting. If no record date is fixed by the Board of Directors, the record date for determining stockholders entitled to notice of or to vote at a meeting of stockholders shall be at the close of business on the day next preceding the day on which notice is given, or, if notice is waived, at the close of business on the day next preceding the date on which the meeting is held. A determination of stockholders of record entitled notice of or to vote at a meeting of stockholders shall apply to any adjournment of the meeting; provided, however, that the Board of Directors may fix a new record date for the adjourned meeting.

(b) In order that the corporation may determine the stockholders entitled to consent to corporate action in writing or by electronic transmission without a meeting, the Board of Directors may fix a record date, which record date shall not precede the date upon which the resolution fixing the record date is adopted by the Board of Directors, and which date shall not be more than 10 days after the date upon which the resolution fixing the record date is adopted by the Board of Directors. If no record date has been fixed by the Board of Directors, the record date for determining stockholders entitled to consent to corporate action in writing or by electronic transmission without a meeting, when no prior action by the Board of Directors is required by the Delaware General Corporation Law, shall be the first date on which a signed written consent or electronic transmission setting forth the action taken or proposed to be taken is delivered to the corporation by delivery to its registered office in Delaware, its principal place of business, or an officer or agent of the corporation having custody of the book in which proceedings of meetings of stockholders are recorded; provided that any such electronic transmission shall satisfy the requirements of Section 2.11(b) and, unless the Board of Directors otherwise provides by resolution, no such consent by electronic transmission shall be deemed to

have been delivered until such consent is reproduced in paper form and until such paper form shall be delivered to the corporation by delivery to its registered office in Delaware, its principal place of business or an officer or agent of the corporation having custody of the book in which proceedings of meetings of stockholders are recorded. Delivery made to a corporation's registered office shall be by hand or by certified or registered mail, return receipt requested. If no record date has been fixed by the Board of Directors and prior action by the Board of Directors is required by law, the record date for determining stockholders entitled to consent to corporate action in writing or by electronic transmission without a meeting shall be at the close of business on the day on which the Board of Directors adopts the resolution taking such prior action.

(c) In order that the corporation may determine the stockholders entitled to receive payment of any dividend or other distribution or allotment of any rights or the stockholders entitled to exercise any rights in respect of any change, conversion or exchange of stock, or for the purpose of any other lawful action, the Board of Directors may fix a record date, which record date shall not precede the date upon which the resolution fixing the record date is adopted, and which record date shall be not more than 60 days prior to such action. If no record date is fixed, the record date for determining stockholders for any such purpose shall be at the close of business on the day on which the Board of Directors adopts the resolution relating thereto.

Section 6.5 Registered Stockholders.

The corporation shall be entitled to recognize the exclusive right of a person registered on its books as the owner of shares to receive dividends and to vote as such owner, and shall not be bound to recognize any equitable or other claim to or interest in such share or shares on the part of any other person, whether or not it shall have express or other notice thereof, except as otherwise provided by the laws of Delaware.

ARTICLE 7

OTHER SECURITIES OF THE CORPORATION

All bonds, debentures and other corporate securities of the corporation, other than stock certificates, may be signed by the Chairman of the Board (if there be such an officer appointed), or the President or any Vice-President or such other person as may be authorized by the Board of Directors and the corporate seal impressed thereon or a facsimile of such seal imprinted thereon and attested by the signature of the Secretary or an assistant secretary, or the Treasurer or an assistant treasurer; provided, however, that where any such bond, debenture or other corporate security shall be authenticated by the manual signature of a trustee under an indenture pursuant to which such bond, debenture or other corporate security shall be issued, the signature of the persons signing and attesting the corporate seal on such bond, debenture or other corporate security may be the imprinted facsimile of the signatures of such persons. Interest coupons appertaining to any such bond, debenture or other corporate security, authenticated by a trustee as aforesaid, shall be signed by the Treasurer or an assistant treasurer of the corporation, or such other person as may be authorized by the Board of Directors, or bear imprinted thereon the facsimile signature of such person. In case any officer who shall have signed or attested any bond, debenture or other corporate security, or whose facsimile signature shall appear thereon

has ceased to be an officer of the corporation before the bond, debenture or other corporate security so signed or attested shall have been delivered, such bond, debenture or other corporate security nevertheless may be adopted by the corporation and issued and delivered as though the person who signed the same or whose facsimile signature shall have been used thereon had not ceased to be such officer of the corporation.

ARTICLE 8

CORPORATE SEAL

The corporate seal shall consist of a die bearing the name of the corporation and the state and date of its incorporation. Said seal may be used by causing it or a facsimile thereof to be impressed or affixed or reproduced or otherwise.

ARTICLE 9

INDEMNIFICATION OF OFFICERS, DIRECTORS, EMPLOYEES AND AGENTS

Section 9.1 Right to Indemnification.

Each person who was or is a party or is threatened to be made a party to or is involved (as a party, witness, or otherwise); in any threatened, pending, or completed action, suit, or proceeding, whether civil, criminal, administrative, or investigative (hereinafter a "Proceeding"), by reason of the fact that he, or a person of whom he is the legal representative, is or was a director, officer, employee, or agent of the corporation or is or was serving at the request of the corporation as a director, officer, employee, or agent of another corporation or of a partnership, joint venture, trust, or other enterprise, including service with respect to employee benefit plans, whether the basis of the Proceeding is alleged action in an official capacity as a director, officer, employee, or agent or in any other capacity while serving as a director, officer, employee, or agent (hereafter an "Agent"), shall be indemnified and held harmless by the corporation to the fullest extent authorized by the Delaware General Corporation Law, as the same exists or may hereafter be amended or interpreted (but, in the case of any such amendment or interpretation, only to the extent that such amendment or interpretation permits the corporation to provide broader indemnification rights than were permitted prior thereto) against all expenses, liability, and loss (including attorneys' fees, judgments, fines, ERISA excise taxes or penalties, and amounts paid or to be paid in settlement, and any interest, assessments, or other charges imposed thereon, and any federal, state, local, or foreign taxes imposed on any Agent as a result of the actual or deemed receipt of any payments under this Article) reasonably incurred or suffered by such person in connection with investigating, defending, being a witness in, or participating in (including on appeal), or preparing for any of the foregoing in, any Proceeding (hereinafter "Expenses"), *provided, however*, that except as to actions to enforce indemnification rights pursuant to Section 9.3 of this Article, the corporation shall indemnify any Agent seeking indemnification in connection with a Proceeding (or part thereof) initiated by such person only if the Proceeding (or part thereof) was authorized by the Board of Directors of the corporation. The right to indemnification conferred in this Article shall be a contract right.

Section 9.2 Authority to Advance Expenses.

Expenses incurred by an officer or director (acting in his capacity as such) in defending a Proceeding shall be paid by the corporation in advance of the final disposition of such Proceeding, provided, however, that if required by the Delaware General Corporation Law, as amended, such Expenses shall be advanced only upon delivery to the corporation of an undertaking by or on behalf of such director or officer to repay such amount if it shall ultimately be determined that he is not entitled to be indemnified by the corporation as authorized in this Article or otherwise. Expenses incurred by other Agents of the corporation (or by the directors or officers not acting in their capacity as such, including service with respect to employee benefit plans) may be advanced upon such terms and conditions as the Board of Directors deems appropriate. Any obligation to reimburse the corporation for Expense advances shall be unsecured and no interest shall be charged thereon.

Section 9.3 Right of Claimant to Bring Suit.

If a claim under Section 9.1 or 9.2 of this Article is not paid in full by the corporation within 30 days after a written claim has been received by the corporation, the claimant may at any time thereafter bring suit against the corporation to recover the unpaid amount of the claim and, if successful in whole or in part, the claimant shall be entitled to be paid also the expense (including attorneys' fees) of prosecuting such claim. It shall be a defense to any such action (other than an action brought to enforce a claim for expenses incurred in defending a Proceeding in advance of its final disposition where the required undertaking has been tendered to the corporation) that the claimant has not met the standards of conduct that make it permissible under the Delaware General Corporation Law for the corporation to indemnify the claimant for the amount claimed. The burden of proving such a defense shall be on the corporation. Neither the failure of the corporation (including its Board of Directors, independent legal counsel, or its stockholders) to have made a determination prior to the commencement of such action that indemnification of the claimant is proper under the circumstances because he has met the applicable standard of conduct set forth in the Delaware General Corporation Law, nor an actual determination by the corporation (including its Board of Directors, independent legal counsel, or its stockholders) that the claimant had not met such applicable standard of conduct, shall be a defense to the action or create a presumption that claimant has not met the applicable standard of conduct.

Section 9.4 Provisions Nonexclusive.

The rights conferred on any person by this Article shall not be exclusive of any other rights that such person may have or hereafter acquire under any statute, provision of the Certificate of Incorporation, agreement, vote of stockholders or disinterested directors, or otherwise, both as to action in an official capacity and as to action in another capacity while holding such office. To the extent that any provision of the Certificate, agreement, or vote of the stockholders or disinterested directors is inconsistent with these Bylaws, the provision, agreement, or vote shall take precedence.

Section 9.5 Authority to Insure.

The corporation may purchase and maintain insurance to protect itself and any Agent against any Expense, whether or not the corporation would have the power to indemnify the Agent against such Expense under applicable law or the provisions of this Article.

Section 9.6 Enforcement of Rights.

Without the necessity of entering into an express contract, all rights provided under this Article shall be deemed to be contractual rights and be effective to the same extent and as if provided for in a contract between the corporation and such Agent. Any rights granted by this Article to an Agent shall be enforceable by or on behalf of the person holding such right in any court of competent jurisdiction.

Section 9.7 Survival of Rights.

The rights provided by this Article shall continue as to a person who has ceased to be an Agent and shall inure to the benefit of the heirs, executors, and administrators of such a person.

Section 9.8 Settlement of Claims.

The corporation shall not be liable to indemnify any Agent under this Article (a) for any amounts paid in settlement of any action or claim effected without the corporation's written consent, which consent shall not be unreasonably withheld; or (b) for any judicial award if the corporation was not given a reasonable and timely opportunity, at its expense, to participate in the defense of such action.

Section 9.9 Effect of Amendment.

Any amendment, repeal, or modification of this Article that adversely affects any rights provided in this Article to an Agent shall only be effective upon the prior written consent of such Agent.

Section 9.10 Primacy of Indemnification.

Notwithstanding that an Agent may have certain rights to indemnification, advancement of expenses and/or insurance provided by other persons (collectively, the "Other Indemnitors"), the corporation: (i) shall be the indemnitor of first resort (i.e., its obligations to an Agent are primary and any obligation of the Other Indemnitors to advance expenses or to provide indemnification for the same expenses or liabilities incurred by such Agent are secondary); and (ii) shall be required to advance the full amount of expenses incurred by an Agent and shall be liable for the full amount of all Expenses, without regard to any rights such Agent may have against any of the Other Indemnitors. No advancement or payment by the Other Indemnitors on behalf of an Agent with respect to any claim for which such Agent has sought indemnification from the corporation shall affect the immediately preceding sentence, and the Other Indemnitors shall have a right of contribution and/or be subrogated to the extent of such advancement or payment to all of the rights of recovery of such Agent against the corporation.

Section 9.11 Subrogation.

In the event of payment under this Article, the corporation shall be subrogated to the extent of such payment to all of the rights of recovery of the Agent, who shall execute all papers required and shall do everything that may be necessary to secure such rights, including the execution of such documents necessary to enable the corporation effectively to bring suit to enforce such rights.

Section 9.12 No Duplication of Payments.

The corporation shall not be liable under this Article to make any payment in connection with any claim made against the Agent to the extent the Agent has otherwise actually received payment (under any insurance policy, agreement, vote, or otherwise) of the amounts otherwise indemnifiable hereunder.

Section 9.13 Saving Clause.

If this Article or any portion hereof shall be invalidated on any ground by any court of competent jurisdiction, then the corporation shall nevertheless indemnify each Agent to the fullest extent not prohibited by any applicable portion of this Article that shall not have been invalidated, or by any other applicable law.

ARTICLE 10

NOTICES

Whenever, under any provisions of these Bylaws, notice is required to be given to any stockholder, the same shall be given either (1) in writing, timely and duly deposited in the United States Mail, postage prepaid, and addressed to his last known post office address as shown by the stock record of the corporation or its transfer agent, or (2) by a means of electronic transmission that satisfies the requirements of Section 2.4(e) of these Bylaws, and has been consented to by the stockholder to whom the notice is given. Any notice required to be given to any director may be given by either of the methods hereinabove stated, except that such notice other than one which is delivered personally, shall be sent to such address or (in the case of electronic communication) such e-mail address; facsimile telephone number or other form of electronic address as such director shall have filed in writing or by electronic communication with the Secretary of the corporation, or, in the absence of such filing, to the last known post office address of such director. If no address of a stockholder or director be known, such notice may be sent to the office of the corporation required to be maintained pursuant to Section 1.2 of Article 1 hereof. An affidavit of mailing, executed by a duly authorized and competent employee of the corporation or its transfer agent appointed with respect to the class of stock affected, specifying the name and address or the names and addresses of the stockholder or stockholders, director or directors, to whom any such notice or notices was or were given, and the time and method of giving the same, shall be conclusive evidence of the statements therein contained. All notices given by mail, as above provided, shall be deemed to have been given as at the time of mailing and all notices given by means of electronic transmission shall be deemed to have been given as at the sending time recorded by the electronic transmission equipment operator transmitting the

same. It shall not be necessary that the same method of giving notice be employed in respect of all directors, but one permissible method may be employed in respect of any one or more, and any other permissible method or methods may be employed in respect of any other or others. The period or limitation of time within which any stockholder may exercise any option or right, or enjoy any privilege or benefit, or be required to act, or within which any director may exercise any power or right, or enjoy any privilege, pursuant to any notice sent him in the manner above provided, shall not be affected or extended in any manner by the failure of such a stockholder or such director to receive such notice. Whenever any notice is required to be given under the provisions of the statutes or of the Certificate of Incorporation, or of these Bylaws, a waiver thereof in writing signed by the person or persons entitled to said notice, or a waiver by electronic transmission by the person entitled to notice, whether before or after the time stated therein, shall be deemed equivalent thereto. Whenever notice is required to be given, under any provision of law or of the Certificate of Incorporation or Bylaws of the corporation, to any person with whom communication is unlawful, the giving of such notice to such person shall not be required and there shall be no duty to apply to any governmental authority or agency for a license or permit to give such notice to such person. Any action or meeting which shall be taken or held without notice to any such person with whom communication is unlawful shall have the same force and effect as if such notice had been duly given. In the event that the action taken by the corporation is such as to require the filing of a certificate under any provision of the Delaware General Corporation Law, the certificate shall state, if such is the fact and if notice is required, that notice was given to all persons entitled to receive notice except such persons with whom communication is unlawful.

ARTICLE 11

AMENDMENTS

These Bylaws may be repealed, altered or amended or new Bylaws adopted by written consent of stockholders in the manner authorized by Section 2.11 of Article II, or at any meeting of the stockholders, either annual or special, by the affirmative vote of a majority of the stock entitled to vote at such meeting, unless a larger vote is required by these Bylaws or the Certificate of Incorporation. The Board of Directors shall also have the authority to repeal, alter or amend these Bylaws or adopt new Bylaws (including, without limitation, the amendment of any Bylaws setting forth the number of directors who shall constitute the whole Board of Directors) by unanimous written consent or at any annual, regular, or special meeting by the affirmative vote of a majority of the whole number of directors. Subject to the power of the stockholders to change or repeal such Bylaws and provided that the Board of Directors shall not make or alter any Bylaws fixing the qualifications, classifications, or term of office of directors.

ARTICLE 12

ANNUAL AND OTHER REPORTS

Section 12.1 Reports to Stockholders.

The Board of Directors of the corporation shall cause an annual report to be sent to the stockholders not later than 120 days after the close of the fiscal year, and at least fifteen (15) days (or, if sent by third-class mail, thirty-five (35) days) prior to the annual meeting of stockholders to be held during the next fiscal year. If approved by the Board of Directors, the report and any accompanying material may be sent by electronic transmission by the corporation (as defined in Section 2.4 hereof). This report shall contain a balance sheet as of the end of that fiscal year and an income statement and statement of changes in financial position for that fiscal year, accompanied by any report thereon of independent accountants or, if there is no such report, the certificate of an authorized officer of the corporation that the statements were prepared without audit from the books and records of the corporation. This report shall also contain such other matters as required by Section 1501(b) of the California General Corporation Law, unless the corporation is subject to the reporting requirements of Section 13 of the Securities Exchange Act of 1934, and is not exempted therefrom under Section 12(g)(2) thereof As long as the corporation has less than 100 holders of record of its shares (determined as provided in Section 605 of the California General Corporation Law), the foregoing requirement of an annual report is hereby waived.

If no annual report for the last fiscal year has been sent to stockholders, the corporation shall, upon the written request of any stockholder made more than 120 days after the close of such fiscal year, deliver (including by electronic transmission by the corporation (as defined in Section 2.4 hereof) or mail to the person making the request within thirty (30) days thereafter the financial statements for such year as required by Section 1501(a) of the California General Corporation Law. A stockholder or stockholders holding at least five percent (5%) of the outstanding shares of any class of the corporation may make a written request to the corporation for an income statement of the corporation for the three-month, six-month or nine-month period of the current fiscal year ended more than thirty (30) days prior to the date of the request and a balance sheet of the corporation as of the end of such period and, in addition, if no annual report for the last fiscal year has been sent to stockholders, the annual report for the last fiscal year, unless such report has been waived under these Bylaws. The statements shall be delivered (including by electronic transmission by the corporation (as defined in Section 2.4 hereof) if such transmission is permitted to such stockholder pursuant to such definition) or mailed to the person making the request within thirty (30) days thereafter. A copy of any such statements shall be kept on .file in the principal executive office of the corporation for twelve (12) months, and they shall be exhibited at all reasonable times to any stockholder demanding an examination of the statements, or a copy shall be mailed to the stockholder.

The quarterly income statements and balance sheets referred to in this section shall be accompanied by the report thereon, if any, of any independent accountants engaged by the corporation or the certificate of an authorized officer of the corporation that the financial statements were prepared without audit from the books and records of the corporation.

Section 12.2 Reports to the Secretary of State.

(a) Except as otherwise required by the Secretary of State of the State of California, every year, during the applicable filing period, the corporation shall file a certified statement with the Secretary of State of the State of California on the prescribed form, setting forth the names and complete business or residence addresses of all incumbent directors; the number of vacancies on the Board of Directors, if any; the names and complete business or residence addresses of the chief executive officer, the secretary, and the chief financial officer; the street

address of the corporation's principal executive office or principal business office in California; a statement of the general type of business constituting the principal business activity of the corporation; and a designation of the agent of the corporation for the purpose of service of process, all in compliance with Section 2117 of the California General Corporation Law.

(b) Notwithstanding the provisions of paragraph (a) of this section, if there has been no change in the information contained in the corporation's last annual statement on file in the Secretary of State of the State of California's office, the corporation may in lieu of filing the annual statement described in paragraph (a) of this section, advise the Secretary of State of the State of California; on the appropriate form, that no changes in the required information have occurred during the applicable period, as permitted by Section 2117 of the California General Corporation Law.

(c) In addition to the statement required pursuant to paragraph (a) of this section, except as otherwise required by the Secretary of State of the State of California, if and as long as the corporation is a publicly traded corporation, within 150 days after the end of its fiscal year, each year it shall file a certified statement on the appropriate form setting forth (i) the name of the independent auditor that prepared the most recent auditor's report on the corporation's annual financial statements; (ii) a description of other services, if any, performed by the independent auditor, its parent, subsidiary or affiliate corporation, during the two most recent fiscal years; (iii) the name of the independent auditor employed by the corporation on the date of the statement; (iv) the compensation paid for the most recent fiscal year to each member of the Board of Directors, to each of the five most highly compensated executive officers of the corporation who are not members of the Board of Directors and to the chief executive officer if not otherwise disclosed, including equity-based compensation; (v) a description of any loan and the terms thereof made to any member of the Board of Directors by the corporation during the corporation's two most recent fiscal years at an interest rate lower than that available from unaffiliated commercial lenders to a similarly-situated borrower; (vi) a statement indicating whether an order of relief has within the preceding ten-year period been entered in a bankruptcy case with respect to the corporation, its executive officers or members of the Board of Directors; (vii) a statement indicating whether any member of the Board of Directors or executive officer of the corporation was convicted of fraud during the preceding ten-year period, as long as the conviction has not been overturned or expunged; and (viii) a description of any material pending legal proceedings, other than routine litigation incidental to the business, to which the corporation or any of its subsidiaries is a party, and a description of any material legal proceeding within the preceding five-year period resulting in a final judgment or final order where the corporation was found liable.

Section 12.3 Effectiveness of Article 12.

If at any time following the adoption of these Bylaws the corporation is no longer subject to Section 2115 of the California General Corporation Law, this Article 12 shall cease to apply to the corporation and it shall have no further obligation to deliver any of the reports to its stockholders or to the Secretary of State of California as herein described.

ARTICLE 13

RIGHT OF FIRST REFUSAL

Section 13.1 Right of First Refusal.

No stockholder shall sell, assign, pledge, or in any manner transfer any of the shares of common stock of the corporation or any right or interest therein, whether voluntarily or by operation of law, or by gift or otherwise, except by a transfer which meets the requirements hereinafter set forth in this bylaw:

(a) (i) In the event a stockholder receives from anyone a bona fide offer acceptable to the stockholder to purchase any of his shares of common stock or (ii) in the event of a restricted transfer (as defined below) by a stockholder, such stockholder shall give written notice thereof to the corporation. The notice shall name the proposed transferee and state the number of shares, right or interest to be transferred, the price per share and all other terms and conditions of the offer or restricted transfer, as applicable. As used herein, "restricted transfer" shall mean: (v) the filing of a petition in bankruptcy by or against a stockholder; (w) an adjudication that a stockholder is an insane or incompetent person; (x) any assignment by a stockholder for the benefit of his, her or its creditors; (y) any transfer, award, or confirmation of any common stock to a stockholder's spouse pursuant to a decree of divorce, dissolution, or separate maintenance, or pursuant to a property settlement or separation agreement; and (z) any testamentary or other similar disposition of any interest in any common stock upon a stockholder's death to any person other than an immediate family member (as defined in Section 13.1(e)(1) below).

(b) For thirty (30) days following receipt of such notice, the corporation or its assigns shall have the option to purchase all or any lesser part of the shares specified in the notice at the price and upon the terms set forth in such bona fide offer; provided, however, that in the event of a restricted transfer, the purchase price per share shall equal the net book value per share of the common stock of the corporation determined on a fully diluted, fully converted basis as of the last day of the preceding fiscal year, as determined by the independent accountants of the corporation (or, in the event that the corporation has not engaged an independent accountant, the Board of Directors of the corporation) based on their review, but not necessarily an audit, of the corporation's financial statements. Net book value shall be calculated using the historical cost of the corporation's assets as reflected on its financial statements decreased by any depreciation, amortization or other cost recover method consistently applied for financial accounting purposes. Net book value shall not include any unrealized gain or loss on the corporation's assets or the value, if any, of the corporation's goodwill or other assets that are not reflected on the corporation's financial statements.

(c) In the event the corporation elects to purchase all or any part of the shares, the Secretary of the corporation shall give written notice to the selling stockholder of such election and the corporation shall; within thirty (30) days after the Secretary of the corporation mails such notice, deliver to the selling stockholder the consideration set forth in the selling stockholder's notice of sale.

(d) In the event that all of the shares are not purchased by the corporation, the selling stockholder may, within the sixty (60) day period following the expiration of the option rights granted to the corporation, sell elsewhere the shares specified in said selling stockholder's notice which were not acquired by the corporation in accordance with the provisions of paragraph (e) of this bylaw, provided that said sale shall not be on terms and conditions more favorable to the purchaser than those contained in the bona fide offer set forth in said selling stockholder's notice. All shares so sold by said selling stockholder shall continue to be subject to the provisions of this bylaw in the same manner as before said transfer.

(e) Anything to the contrary contained herein notwithstanding, the following transactions shall be exempt from the provisions of this bylaw:

(1) A stockholder's transfer of any or all shares held either during such stockholder's lifetime or on death by will or intestacy to such stockholder's immediate family or a trust for the sole benefit of such stockholder and/or his or her immediate family. "Immediate family" as used herein shall mean spouse (subject to limitations in the event of a restricted transfer), lineal descendent, father, mother, brother, or sister of the stockholder making such transfer.

(2) A stockholder's bona fide pledge or mortgage of any shares of common stock with a commercial lending institution, provided that any subsequent transfer of said shares by said institution shall be conducted in the manner set forth in this bylaw.

(3) A stockholder's transfer of any or all of such stockholder's shares of common stock to any other stockholder of the corporation.

(4) A stockholder's transfer of any or all of such stockholders shares of common stock to a person who, at the time of such transfer, is an officer or director of the corporation.

(5) A corporate stockholder's transfer of any or all of its shares of common stock pursuant to and in accordance with the terms of any merger, consolidation, reclassification of shares or capital reorganization of the corporate stockholder, or pursuant to a sale of all or substantially all of the stock or assets of a corporate stockholder.

(6) A corporate stockholder's transfer of any or all of its shares of common stock to any or all of its stockholders.

(7) A transfer by a stockholder which is a limited or general partnership to any or all of its partners.

(8) A stockholder's transfer to any affiliate of the stockholder or to any person who or which, directly or indirectly, controls, is controlled by, or is under common control with such stockholder, including, without limitation, any general partner, officer, director or manager of such stockholder and any venture capital fund now or hereafter existing that is controlled by one or more general partners or managing members of, or is under common investment management with, such stockholder.

In any such case, the transferee, assignee, or other recipient shall receive and hold such stock subject to the provisions of this bylaw, and there shall be no further transfer of such stock except in accord with this bylaw.

(f) The provisions of this bylaw may be waived with respect to any transfer either by the corporation, upon duly authorized action of its Board of Directors, or by the stockholders, upon the express written consent of the owners of a majority of the voting power of the corporation (excluding the votes represented by those shares to be sold by the selling stockholder). This bylaw may be amended or repealed either by a duly authorized action of the Board of Directors or by the stockholders, upon the express written consent of the owners of a majority of the voting power of the corporation.

(g) Any sale or transfer or purported sale or transfer, of securities of the corporation by stockholders shall be null and void unless the terms, conditions, and provisions of this bylaw are strictly observed and followed.

(h) The foregoing right of first refusal shall terminate upon the date securities of the corporation are first offered to the public pursuant to a registration statement filed with, and declared effective by, the Securities and Exchange Commission under the Securities Act of 1933, as amended.

(i) The certificates representing shares of common stock of the corporation shall bear on their face the following legend so long as the foregoing right of first refusal remains in effect:

“THE SHARES REPRESENTED BY THIS CERTIFICATE ARE SUBJECT TO A RIGHT OF FIRST REFUSAL OPTION, AS PROVIDED IN THE BYLAWS OF THE CORPORATION.”

(j) Whenever the corporation shall have the right to purchase common stock under this right of first refusal, the corporation may designate and assign to one or more employees, officers, directors or stockholders of the corporation or other persons or organizations, to exercise all or a part of the corporation’s right of first refusal.

Exhibit 4.2

Number «Cert» **Ultragenyx Pharmaceutical Inc.** ***«Shares»* Shares**
Common Stock

State of Delaware

*THIS CERTIFIES THAT *«Name»* is the record holder of *«NumShares» («Shares»)* shares of Common Stock of Ultragenyx Pharmaceutical Inc., transferable only on the share register of said corporation, in person or by such holder's duly authorized attorney, upon surrender of this certificate properly endorsed or assigned.*

This certificate and the shares represented hereby shall be held subject to all of the provisions of the Certificate of Incorporation and the Bylaws of said Corporation and any amendments thereto, to all of which the holder of this Certificate, by acceptance hereof, assents.

A statement of the number of shares constituting each class or series of stock and the designation thereof and a statement of all of the rights, preferences, privileges, and restrictions granted to or imposed upon the respective classes and/or series of shares of stock of the corporation and upon the holders thereof may be obtained by any stockholder upon request and without charge, at the principal office of the corporation, and the corporation will furnish any stockholder, upon request and without charge, a copy of each statement.

WITNESS the signatures of the corporation's duly authorized officers this «Day» day of «Month_Year».

President

Secretary

Exhibit 4.3

THIS WARRANT AND THE SHARES PURCHASABLE HEREUNDER HAVE BEEN ACQUIRED FOR INVESTMENT AND HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED, OR QUALIFIED UNDER ANY STATE SECURITIES LAWS. SUCH SECURITIES MAY NOT BE SOLD OR TRANSFERRED IN THE ABSENCE OF SUCH REGISTRATION OR QUALIFICATION OR AN EXEMPTION THEREFROM UNDER SAID ACT AND ANY APPLICABLE STATE SECURITIES LAWS.

THIS WARRANT AND THE SHARES PURCHASABLE HEREUNDER ARE SUBJECT TO RESTRICTIONS ON TRANSFER CONTAINED IN THAT CERTAIN NOTE AND WARRANT PURCHASE AGREEMENT, DATED JUNE 30, 2010, WHICH RESTRICTIONS ON TRANSFER ARE INCORPORATED HEREIN BY REFERENCE.

Dated: June 30, 2010

WARRANT TO PURCHASE

SERIES A PREFERRED STOCK OF

ULTRAGENYX PHARMACEUTICAL INC.

This certifies that Emil D. Kakkis, M.D., Ph.D., or assigns (collectively, the "Holder"), for value received, is entitled to purchase, at the Stock Purchase Price (as defined below), from Ultragenyx Pharmaceutical Inc., a California corporation (the "Company"), up to that number of fully paid and nonassessable shares of the Company's Series A Preferred Stock (the "Series A Preferred Stock"), equal to the quotient obtained in accordance with the following calculation:

Maximum number of shares of Series A Preferred Stock (the "Warrant Shares") issuable upon exercise of this Warrant	=	**(aggregate Principal Amount of the Note issued by the Company to Holder) x (0.25) the Stock Purchase Price**

This Warrant shall be exercisable at any time from time to time from and after the closing of the Series A Preferred Financing (as defined below) (such date being referred to herein as the "Initial Exercise Date") up to and including 5:00 p.m. (Pacific Time) on the first to occur of (i) the closing date of any reorganization, consolidation or merger of the Company, transfer of all or substantially all of the assets of the Company or any simultaneous sale of more than a majority of the then outstanding securities of the Company other than a mere reincorporation transaction, or (ii) the ten (10) year anniversary of the date hereof (such earlier date being referred to herein as the "Expiration Date"), upon surrender to the Company at its principal office (or at such other location as the Company may advise the Holder in writing) of this Warrant properly endorsed with (i) the Form of Subscription attached hereto duly completed and executed, (ii) payment pursuant to Section 2 of the aggregate Stock Purchase Price for the number of shares for which this Warrant is being exercised determined in accordance with the provisions hereof, and (iii) any documents reasonably requested by the Company to be executed by the Holder as if Holder were an investor in the Series A Preferred Stock Financing, including without limitation a stock purchase agreement, an investors' rights agreement, a right of first refusal and co-sale agreement, and a voting agreement, thereby agreeing to be bound by all obligations and receive all rights thereunder. The Stock Purchase Price and the number of shares purchasable hereunder are subject to adjustment as provided in Section 4 of this Warrant.

For purposes of this Warrant, (a) the term "Purchase Agreement" shall mean that certain Note and Warrant Purchase Agreement, dated as of June __, 2010 by and among the Company and the investors listed therein and (b) the terms "Note," "Principal Amount," "Series A Preferred Stock Financing" and "Stock Purchase Price" shall have the meanings given them in the Purchase Agreement.

1. Exercise, Issuance of Certificates; Acknowledgement. This Warrant is exercisable at the option of the holder of record hereof, at any time or from time to time from or after the Initial Exercise Date up to the Expiration Date for all or any part of the Warrant Shares (but not for a fraction of a share) which may be purchased hereunder. The Company agrees that the shares of Series A Preferred Stock purchased under this Warrant shall be and are deemed to be issued to the Holder hereof as the record owner of such shares as of the close of business on the date on which this Warrant shall have been surrendered, properly endorsed, the completed, executed Form of Subscription delivered and payment made for such shares. Certificates for the shares of the Series A Preferred Stock so purchased, together with any other securities or property to which the Holder hereof is entitled upon such exercise, shall be delivered to the Holder hereof by the Company at the Company's expense within a reasonable time after the rights represented by this Warrant have been so exercised. Each certificate so delivered shall be in such denominations of the Warrant Shares as may be requested by the Holder hereof and shall be registered in the name of such Holder. In case of a purchase of less than all the Warrant Shares, the Company shall execute and deliver to Holder within a reasonable time an Acknowledgement in the form attached hereto indicating the number of Warrant Shares which remain subject to this Warrant, if any.

2. Payment for Shares. The aggregate purchase price for Warrant Shares being purchased hereunder may be paid either (i) by cash or wire transfer of immediately available funds, (ii) if the fair market value of one (1) share of the Warrant Shares on the date of exercise is greater than the Stock Purchase Price, by surrender of a number of Warrant Shares which have a fair market value equal to the aggregate purchase price of the Warrant Shares being purchased ("Net Issuance") as determined herein, or (iii) any combination of the foregoing. If the Holder elects the Net Issuance method of payment, the Company shall issue to Holder upon exercise a number of shares of Warrant Shares determined in accordance with the following formula:

$$X = \frac{Y(A-B)}{A}$$

where: X = the number of Warrant Shares to be issued to the Holder;

Y = the number of Warrant Shares with respect to which the Holder is exercising its purchase rights under this Warrant;

A = the fair market value of one (1) share of the Warrant Shares on the date of exercise; and

B = the Stock Purchase Price.

No fractional shares arising out of the above formula for determining the number of shares to be issued to the Holder shall be issued, and the Company shall in lieu thereof make payment to the Holder of cash in the amount of such fraction multiplied by the fair market value of one (1) share of the Warrant Shares on the date of exercise. For purposes of the above calculation, the fair market value of one (1) share of the Warrant Shares shall mean (a) if the date of exercise is after the commencement of trading of the Common Stock on a securities exchange or over-the-counter but prior to the closing of the IPO, the price per share to the public set forth on the final prospectus relating to the IPO, multiplied by the number of shares of Common Stock into which each share of the Warrant Shares is then convertible, (b) if the Common Stock is then traded on a securities exchange, the average of the closing prices of such Common Stock on such exchange over the thirty (30) calendar day period (or portion thereof) ending three (3) days prior to the date of exercise, multiplied by the number of shares of Common Stock into which each share of the Warrant Shares is then convertible, (c) if the Common Stock is then regularly traded over-the-counter, the average of the closing sale prices or secondarily the closing bid of such Common Stock over the thirty (30) calendar day period (or portion thereof) ending three (3) days prior to the date of exercise, multiplied by the number of shares of Common Stock into which each share of the Warrant Shares is then convertible, or (d) if there is no active public market for the Common Stock, the fair market value of one share of the Warrant Shares as determined in good faith by the Board of Directors of the Company.

3. Shares to be Fully Paid; Reservation of Shares. The Company covenants and agrees that all shares of Series A Preferred Stock which may be issued upon the exercise of the rights represented by this Warrant (together with all shares of Common Stock issuable upon conversion of such Series A Preferred Stock) will, upon issuance, be duly authorized, validly issued, fully paid and nonassessable and free from all preemptive rights of any shareholder and free of all taxes, liens and charges with respect to the issue thereof. The Company further covenants and agrees that during the period within which the rights represented by this Warrant may be exercised, the Company will at all times have authorized and reserved, for the purpose of issue or transfer upon exercise of the subscription rights evidenced by this Warrant, a sufficient number of shares of authorized but unissued shares of Series A Preferred Stock (together with the number of shares of Common Stock issuable upon conversion of such Series A Preferred Stock), or other securities and property, when and as required to provide for the exercise of the rights represented by this Warrant.

4. Adjustment of Stock Purchase Price and Number of Shares. The Stock Purchase Price and the number of shares purchasable upon the exercise of this Warrant shall be subject to adjustment from time to time upon the occurrence of certain events described in this Section 4. Upon each adjustment of the Stock Purchase Price, the Holder of this Warrant shall thereafter be entitled to purchase, at the Stock Purchase Price resulting from such adjustment, the number of shares obtained by multiplying the Stock Purchase Price in effect immediately prior to such adjustment by the number of shares purchasable pursuant hereto immediately prior to such adjustment, and dividing the product thereof by the Stock Purchase Price resulting from such adjustment.

4.1 Conversion of Preferred Stock. If all of the outstanding Preferred Stock of the Company is converted into shares of Common Stock, then this Warrant shall automatically become exercisable for that number of shares of Common Stock equal to the number of shares of

Common Stock that would have been received if this Warrant had been exercised in full and the shares of Preferred Stock received thereupon had been simultaneously converted into shares of Common Stock immediately prior to such event, and the Stock Purchase Price shall be automatically adjusted to equal the number obtained by dividing (i) the aggregate Stock Purchase Price of the shares of Preferred Stock for which this Warrant was exercisable immediately prior to such conversion, by (ii) the number of shares of Common Stock for which this Warrant is exercisable immediately after such conversion

4.2 Subdivisions, Combinations and Dividends. In case the Company shall at any time subdivide its outstanding shares of Series A Preferred Stock into a greater number of shares or pay a dividend in Series A Preferred Stock in respect of outstanding shares of Series A Preferred Stock, the Stock Purchase Price in effect immediately prior to such subdivision or at the record date of such dividend shall be proportionately reduced, and conversely, in case the outstanding shares of the Series A Preferred Stock of the Company shall be combined into a smaller number of shares, the Stock Purchase Price in effect immediately prior to such combination shall be proportionately increased.

4.3 Reclassification. If any reclassification of the capital stock of the Company shall be effected in such a way that holders of Series A Preferred Stock shall be entitled to receive stock, securities, or other assets or property, then, as a condition of such reclassification, lawful and adequate provisions shall be made whereby the Holder hereof shall thereafter have the right to purchase and receive (in lieu of the shares of the Series A Preferred Stock immediately theretofore purchasable and receivable upon the exercise of the rights represented hereby) such shares of stock, securities or other assets or property as may be issued or payable with respect to or in exchange for a number of outstanding shares of such Series A Preferred Stock equal to the number of shares of such Series A Preferred Stock immediately theretofore purchasable and receivable upon the exercise of the rights represented hereby. In any reclassification described above, appropriate provision shall be made with respect to the rights and interests of the Holder of this Warrant to the end that the provisions hereof (including, without limitation, provisions for adjustments of the Stock Purchase Price and of the number of shares purchasable and receivable upon the exercise of this Warrant) shall thereafter be applicable, as nearly as may be, in relation to any shares of stock, securities or assets thereafter deliverable upon the exercise hereof.

4.4 Notice of Adjustment. Upon any adjustment of the Stock Purchase Price or any increase or decrease in the number of shares purchasable upon the exercise of this Warrant, the Company shall give written notice thereof, by first class mail postage prepaid, addressed to the registered Holder of this Warrant at the address of such Holder as shown on the books of the Company. The notice shall be signed by the Company's chief financial officer and shall state the Stock Purchase Price resulting from such adjustment and the increase or decrease, if any, in the number of shares purchasable at such price upon the exercise of this Warrant, setting forth in reasonable detail the method of calculation and the facts upon which such calculation is based. For the avoidance of doubt, the Company acknowledges that the Holder of this Warrant shall be entitled to the benefit of all adjustments in the number of shares of Common Stock of the Company issuable upon conversion of the Series A Preferred Stock of the Company which occur prior to the exercise of this Warrant, including without limitation, any increase in the number of shares of Common Stock issuable upon conversion as a result of a dilutive issuance of capital stock.

4.5 Other Notices. If at any time:

(1) the Company shall declare any cash dividend upon its Series A Preferred Stock (or Common Stock issuable upon conversion thereof);

(2) there shall be any capital reorganization or reclassification of the capital stock of the Company; or consolidation or merger of the Company with, or sale of all or substantially all of its assets to, another Person;

(3) there shall be a voluntary or involuntary dissolution, liquidation or winding-up of the Company; or

(4) there shall be an IPO;

then, in any one or more of said cases, the Company shall give, by first class mail, postage prepaid, addressed to the Holder of this Warrant at the address of such Holder as shown on the books of the Company, (a) at least ten (10) days prior written notice of the date on which the books of the Company shall close or a record shall be taken for such dividend or for determining rights to vote in respect of any such reorganization, reclassification, consolidation, merger, sale, dissolution, liquidation or winding-up, and (b) in the case of any such reorganization, reclassification, consolidation, merger, sale, dissolution, liquidation, winding-up or public offering, at least ten (10) days prior written notice of the date when the same shall take place; provided, however, that the Holder shall make a best efforts attempt to respond to such notice as early as possible after the receipt thereof. Any notice given in accordance with the foregoing clause (a) shall also specify, in the case of any such dividend, the date on which the holders of Series A Preferred Stock (or Common Stock issuable upon conversion thereof) shall be entitled thereto. Any notice given in accordance with the foregoing clause (b) shall also specify the date on which the holders of Series A Preferred Stock (or Common Stock issuable upon conversion thereof) shall be entitled to exchange their Series A Preferred Stock (or Common Stock issuable upon conversion thereof) for securities or other property deliverable upon such reorganization, reclassification, consolidation, merger, sale, dissolution, liquidation, winding-up, conversion or public offering, as the case may be.

5. No Voting or Dividend Rights. Nothing contained in this Warrant shall be construed as conferring upon the Holder hereof the right to vote or to consent to receive notice as a shareholder of the Company or any other matters or any rights whatsoever as a shareholder of the Company. No dividends or interest shall be payable or accrued in respect of this Warrant or the interest represented hereby or the shares purchasable hereunder until, and only to the extent that, this Warrant shall have been exercised.

6. Warrants Transferable. Subject to compliance with applicable federal and state securities laws and the transfer restrictions set forth in the Purchase Agreement, under which this Warrant was issued, this Warrant and all rights hereunder may be transferred, in whole or in part, without charge to the holder hereof (except for transfer taxes), upon the prior written consent of the Company and, thereafter, upon surrender of this Warrant properly endorsed and in

compliance with the provisions of the Purchase Agreement. Each taker and holder of this Warrant, by taking or holding the same, consents and agrees that this Warrant, when endorsed in blank, shall be deemed negotiable, and that the holder hereof, when this Warrant shall have been so endorsed, may be treated by the Company, at the Company's option, and all other persons dealing with this Warrant as the absolute owner hereof for any purpose and as the person entitled to exercise the rights represented by this Warrant, or to the transfer hereof on the books of the Company and notice to the contrary notwithstanding; but until such transfer on such books, the Company may treat the registered owner hereof as the owner for all purposes.

7. Lost Warrants. Upon receipt of evidence reasonably satisfactory to the Company of the loss, theft, destruction, or mutilation of this Warrant and, in the case of any such loss, theft or destruction, upon receipt of an indemnity reasonably satisfactory to the Company, or in the case of any such mutilation upon surrender and cancellation of such Warrant, the Company, at its expense, will make and deliver a new Warrant, of like tenor, in lieu of the lost, stolen, destroyed or mutilated Warrant.

8. Modification and Waiver. Any term of this Warrant and all Warrants issued pursuant to the Purchase Agreement may be amended and the observance of any term of this Warrant and all Warrants issued pursuant to the Purchase Agreement may be waived (either generally or in a particular instance and either retroactively or prospectively) by a writing signed by the Company and the holders of Warrants representing at least a majority of the aggregate number of Warrant Shares issuable upon exercise of all outstanding Warrants issued pursuant to the Purchase Agreement. Any amendment or waiver effected in accordance with this Section shall be binding upon the Company, the Holder and the holders of all Warrants issued pursuant to the Purchase Agreement.

9. Notices. Except as may be otherwise provided herein, all notices, requests, waivers and other communications made pursuant to this Warrant shall be made in accordance with Section 5.6 of the Purchase Agreement.

10. Governing Law. This Warrant is to be construed in accordance with and governed by the laws of the State of California.

IN WITNESS WHEREOF, the Company has caused this Warrant to be duly executed by its officers, thereunto duly authorized as of the date first above written.

ULTRAGENYX PHARMACEUTICAL INC.

By: /s/ John Ditton
John Ditton
Secretary

FORM OF SUBSCRIPTION

(To be signed only upon exercise of Warrant)

To:_____

The undersigned, the holder of a right to purchase shares of Series A Preferred Stock of Ultragenyx Pharmaceutical Inc., a California corporation (the "Company"), pursuant to that certain Warrant to Purchase Series A Preferred Stock of Ultragenyx Pharmaceutical Inc. (the "Warrant"), dated as of June __, 2010, hereby irrevocably elects to exercise the purchase right represented by such Warrant for, and to purchase thereunder, _____ (_____) shares of Series A Preferred Stock of the Company and herewith makes payment of _____ Dollars ($_____) therefor by the following method:

(Check one of the following):

_____ (check if applicable) The undersigned hereby elects to make payment of _____ Dollars ($_____) therefor in cash.

_____ (check if applicable) The undersigned hereby elects to make payment for the aggregate exercise price of this exercise using the Net Issuance method pursuant to Section 2 of the Warrant.

The undersigned represents that it is acquiring such securities for its own account for investment and not with a view to or for sale in connection with any distribution thereof and in order to induce the issuance of such securities makes to the Company, as of the date hereof, the representations and warranties set forth in Section 3 of the Note and Warrant Purchase Agreement, dated as of June __, 2010, by and among the Company and the investors listed on Exhibit A thereto.

DATED: _____

EMIL D. KAKKIS, M.D., Ph.D.

By: _____

ACKNOWLEDGMENT

To: Emil D. Kakkis, M.D., Ph.D.

The undersigned hereby acknowledges that as of the date hereof, _____ (_____) shares of Series A Preferred Stock remain subject to the right of purchase in favor of Emil D. Kakkis, M.D., Ph.D. pursuant to that certain Warrant to Purchase Series A Preferred Stock of Ultragenyx Pharmaceutical Inc., dated as of June __, 2010.

DATED: _____

ULTRAGENYX PHARMACEUTICAL INC.

By: _____

Name: _____

Title: _____

IN WITNESS WHEREOF, the Company has caused this Warrant to be duly executed by its officers, thereunto duly authorized as of the date first above written.

ULTRAGENYX PHARMACEUTICAL INC.

By: /s/ John Ditton
John Ditton
Secretary

Exhibit 4.4

THIS WARRANT AND THE SHARES PURCHASABLE HEREUNDER ARE SUBJECT TO RESTRICTIONS ON TRANSFER CONTAINED IN THAT CERTAIN NOTE AND WARRANT PURCHASE AGREEMENT, DATED JANUARY 2011, WHICH RESTRICTIONS ON TRANSFER ARE INCORPORA TED HEREIN BY REFERENCE.

THIS WARRANT AND THE SHARES PURCHASABLE HEREUNDER HAVE BEEN ACQUIRED FOR INVESTMENT AND HA VE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED, OR QUALIFIED UNDER ANY STATE SECURITIES LAWS SUCH SECURITIES MAY NOT BE SOLD OR TRANSFERRED IN THE ABSENCE OF SUCH REGISTRATION OR QUALIFICATION OR ANEXEMPTION THEREFROM UNDER SAID ACT AND ANY APPLICABLE STATE SECURITIES LAWS.

Dated: February 15, 2011

WARRANT TO PURCHASE

SERIES A PREFERRED STOCK OF

ULTRAGENYX PHARMACEUTICAL INC.

This certifies that the John And Cynthia Klock Charitable Trust, or assigns (collectively, the "Holder"), for value received, is entitled to purchase, at the Stock Purchase Price (as defined below), from Ultragenyx Pharmaceutical Inc., a California corporation (the "Company"), up to that number of fully paid and nonassessable shares of the Company's Series A Preferred Stock (the "Series A Preferred Stock"), equal to the quotient obtained in accordance with the following calculation:

Maximum number of shares of Series A Preferred Stock (the "Warrant Shares") issuable upon exercise of this Warrant	=	**(aggregate Principal Amount of the Note issued by the Company to Holder) x (0.35) the Stock Purchase Price**

This Warrant shall be exercisable at any time from time to time from and after the closing of the Series A Preferred Financing (as defined below) (such date being referred to herein as the "Initial Exercise Date") up to and including 5:00 p.m. (Pacific Time) on the first to occur of (i) the closing date of any reorganization, consolidation or merger of the Company, transfer of all or substantially all of the assets of the Company or any simultaneous sale of more than a majority of the then outstanding securities of the Company other than a mere reincorporation transaction, or (ii) the ten (10) year anniversary of the date hereof (such earlier date being referred to herein as the "Expiration Date"), upon surrender to the Company at its principal office (or at such other location as the Company may advise the Holder in writing) of this Warrant properly endorsed with (i) the Form of Subscription attached hereto duly completed and executed, (ii) payment pursuant to Section 2 of the aggregate Stock Purchase Price for the number of shares for which this Warrant is being exercised determined in accordance with the provisions hereof, and (iii) any documents reasonably requested by the Company to be executed by the Holder as if Holder were an investor in the Series A Preferred Stock Financing, including without limitation a stock purchase agreement, an investors' rights agreement, a right of first refusal and co-sale agreement, and a voting agreement, thereby agreeing to be bound by all obligations and receive all rights thereunder. The Stock Purchase Price and the number of shares purchasable hereunder are subject to adjustment as provided in Section 4 of this Warrant.

For purposes of this Warrant, (a) the term "Purchase Agreement" shall mean that certain Note and Warrant Purchase Agreement, dated as of January _, 2011 by and among the Company and the investors listed therein and (b) the terms "Note," "Principal Amount," "Series A Preferred Stock Financing" and "Stock Purchase Price" shall have the meanings given them in the Purchase Agreement.

1. Exercise, Issuance of Certificates; Acknowledgement. This Warrant is exercisable at the option of the holder of record hereof, at any time or from time to time from or after the Initial Exercise Date up to the Expiration Date for all or any part of the Warrant Shares (but not for a fraction of a share) which may be purchased hereunder. The Company agrees that the shares of Series A Preferred Stock purchased under this Warrant shall be and are deemed to be issued to the Holder hereof as the record owner of such shares as of the close of business on the date on which this Warrant shall have been surrendered, properly endorsed, the completed, executed Form of Subscription delivered and payment made for such shares. Certificates for the shares of the Series A Preferred Stock so purchased, together with any other securities or property to which the Holder hereof is entitled upon such exercise, shall be delivered to the Holder hereof by the Company at the Company's expense within a reasonable time after the rights represented by this Warrant have been so exercised. Each certificate so delivered shall be in such denominations of the Warrant Shares as may be requested by the Holder hereof and shall be registered in the name of such Holder. In case of a purchase of less than all the Warrant Shares, the Company shall execute and deliver to Holder within a reasonable time an Acknowledgement in the form attached hereto indicating the number of Warrant Shares which remain subject to this Warrant, if any.

2. Payment for Shares. The aggregate purchase price for Warrant Shares being purchased hereunder may be paid either (i) by cash or wire transfer of immediately available funds, (ii) if the fair market value of one (1) share of the Warrant Shares on the date of exercise is greater than the Stock Purchase Price, by surrender of a number of Warrant Shares which have a fair market value equal to the aggregate purchase price of the Warrant Shares being purchased ("Net Issuance") as determined herein, or (iii) any combination of the foregoing. If the Holder elects the Net Issuance method of payment, the Company shall issue to Holder upon exercise a number of shares of Warrant Shares determined in accordance with the following formula:

$$X = \frac{Y(A-B)}{A}$$

Where: X = The number of Warrant Shares to be issued to Holder.

Y = The number of Warrant Shares purchasable under this Warrant (at the date of such calculation).

A = The fair market value of one share of Warrant Shares on the date of exercise; and

B = The Stock Purchase Price.

No fractional shares arising out of the above formula for determining the number of shares to be issued to the Holder shall be issued, and the Company shall in lieu thereof make payment to the Holder of cash in the amount of such fraction multiplied by the fair market value of one (1) share of the Warrant Shares on the date of exercise. For purposes of the above calculation, the fair market value of one (1) share of the Warrant Shares shall mean (a) if the date of exercise is after the commencement of trading of the Common Stock on a securities exchange or over-the-counter but prior to the closing of the IPO, the price per share to the public set forth on the final prospectus relating to the IPO, multiplied by the number of shares of Common Stock into which each share of the Warrant Shares is then convertible, (b) if the Common Stock is then traded on a securities exchange, the average of the closing prices of such Common Stock on such exchange over the thirty (30) calendar day period (or portion thereof) ending three (3) days prior to the date of exercise, multiplied by the number of shares of Common Stock into which each share of the Warrant Shares is then convertible, (c) if the Common Stock is then regularly traded over-the-counter, the average of the closing sale prices or secondarily the closing bid of such Common Stock over the thirty (30) calendar day period (or portion thereof) ending three (3) days prior to the date of exercise, multiplied by the number of shares of Common Stock into which each share of the Warrant Shares is then convertible, or (d) if there is no active public market for the Common Stock, the fair market value of one share of the Warrant Shares as determined in good faith by the Board of Directors of the Company.

2. Shares to be Fully Paid; Reservation of Shares. The Company covenants and agrees that all shares of Series A Preferred Stock which may be issued upon the exercise of the rights represented by this Warrant (together with all shares of Common Stock issuable upon conversion of such Series A Preferred Stock) will, upon issuance, be duly authorized, validly issued, fully paid and nonassessable and free from all preemptive rights of any shareholder and free of all taxes, liens and charges with respect to the issue thereof The Company further covenants and agrees that during the period within which the rights represented by this Warrant may be exercised, the Company will at all times have authorized and reserved, for the purpose of issue or transfer upon exercise of the subscription rights evidenced by this Warrant, a sufficient number of shares of authorized but unissued shares of Series A Preferred Stock (together with the number of shares of Common Stock issuable upon conversion of such Series A Preferred Stock), or other securities and property, when and as required to provide for the exercise of the rights represented by this Warrant.

4. Adjustment of Stock Purchase Price and Number of Shares. The Stock Purchase Price and the number of shares purchasable upon the exercise of this Warrant shall be subject to adjustment from time to time upon the occurrence of certain events described in this Section 4. Upon each adjustment of the Stock Purchase Price, the Holder of this Warrant shall thereafter be entitled to purchase, at the Stock Purchase Price resulting from such adjustment, the number of shares obtained by multiplying the Stock Purchase Price in effect immediately prior to such adjustment by the number of shares purchasable pursuant hereto immediately prior to such adjustment, and dividing the product thereof by the Stock Purchase Price resulting from such adjustment.

4.1 Conversion of Preferred Stock. If all of the outstanding Preferred Stock of the Company is converted into shares of Common Stock, then this Warrant shall automatically become exercisable for that number of shares of Common Stock equal to the number of shares of

Common Stock that would have been received if this Warrant had been exercised in full and the shares of Preferred Stock received thereupon had been simultaneously converted into shares of Common Stock immediately prior to such event, and the Stock Purchase Price shall be automatically adjusted to equal the number obtained by dividing (i) the aggregate Stock Purchase Price of the shares of Preferred Stock for which this Warrant was exercisable immediately prior to such conversion, by (ii) the number of shares of Common Stock for which this Warrant is exercisable immediately after such conversion

4.2 Subdivisions, Combinations and Dividends. In case the Company shall at any time subdivide its outstanding shares of Series A Preferred Stock into a greater number of shares or pay a dividend in Series A Preferred Stock in respect of outstanding shares of Series A Preferred Stock, the Stock Purchase Price in effect immediately prior to such subdivision or at the record date of such dividend shall be proportionately reduced, and conversely, in case the outstanding shares of the Series A Preferred Stock of the Company shall be combined into a smaller number of shares, the Stock Purchase Price in effect immediately prior to such combination shall be proportionately increased.

4.3 Reclassification. If any reclassification of the capital stock of the Company shall be effected in such a way that holders of Series A Preferred Stock shall be entitled to receive stock, securities, or other assets or property, then, as a condition of such reclassification, lawful and adequate provisions shall be made whereby the Holder hereof shall thereafter have the right to purchase and receive (in lieu of the shares of the Series A Preferred Stock immediately theretofore purchasable and receivable upon the exercise of the rights represented hereby) such shares of stock, securities or other assets or property as may be issued or payable with respect to or in exchange for a number of outstanding shares of such Series A Preferred Stock equal to the number of shares of such Series A Preferred Stock immediately theretofore purchasable and receivable upon the exercise of the rights represented hereby. In any reclassification described above, appropriate provision shall be made with respect to the rights and interests of the Holder of this Warrant to the end that the provisions hereof (including, without limitation, provisions for adjustments of the Stock Purchase Price and of the number of shares purchasable and receivable upon the exercise of this Warrant) shall thereafter be applicable, as nearly as may be, in relation to any shares of stock, securities or assets thereafter deliverable upon the exercise hereof.

4.4 Notice of Adjustment. Upon any adjustment of the Stock Purchase Price or any increase or decrease in the number of shares purchasable upon the exercise of this Warrant, the Company shall give written notice thereof, by first class mail postage prepaid, addressed to the registered Holder of this Warrant at the address of such Holder as shown on the books of the Company. The notice shall be signed by the Company's chief financial officer and shall state the Stock Purchase Price resulting from such adjustment and the increase or decrease, if any, in the number of shares purchasable at such price upon the exercise of this Warrant, setting forth in reasonable detail the method of calculation and the facts upon which such calculation is based. For the avoidance of doubt, the Company acknowledges that the Holder of this Warrant shall be entitled to the benefit of all adjustments in the number of shares of Common Stock of the Company issuable upon conversion of the Series A Preferred Stock of the Company which occur prior to the exercise of this Warrant, including without limitation, any increase in the number of shares of Common Stock issuable upon conversion as a result of a dilutive issuance of capital stock.

4.5 Other Notices. If at any time:

(1) the Company shall declare any cash dividend upon its Series A Preferred Stock (or Common Stock issuable upon conversion thereof);

(2) there shall be any capital reorganization or reclassification of the capital stock of the Company; or consolidation or merger of the Company with, or sale of all or substantially all of its assets to, another Person;

(3) there shall be a voluntary or involuntary dissolution, liquidation or winding-up of the Company; or

(4) there shall be an IPO;

then, in any one or more of said cases, the Company shall give, by first class mail, postage prepaid, addressed to the Holder of this Warrant at the address of such Holder as shown on the books of the Company, (a) at least ten (10) days prior written notice 0 f the date on which the books of the Company shall close or a record shall be taken for such dividend or for determining rights to vote in respect of any such reorganization, reclassification, consolidation, merger, sale, dissolution, liquidation or winding-up, and (b) in the case of any such reorganization, reclassification, consolidation, merger, sale, dissolution, liquidation, winding-up or public offering, at least ten (10) days prior written notice of the date when the same shall take place; provided, however, that the Holder shall make a best efforts attempt to respond to such notice as early as possible after the receipt thereof Any notice given in accordance with the foregoing clause (a) shall also specify, in the case of any such dividend, the date on which the holders of Series A Preferred Stock (or Common Stock issuable upon conversion thereof) shall be entitled thereto. Any notice given in accordance with the foregoing clause (b) shall also specify the date on which the holders of Series A Preferred Stock (or Common Stock issuable upon conversion thereof) shall be entitled to exchange their Series A Preferred Stock (or Common Stock issuable upon conversion thereof) for securities or other property deliverable upon such reorganization, reclassification, consolidation, merger, sale, dissolution, liquidation, winding-up, conversion or public offering, as the case may be.

5. No Voting; or Dividend Rights. Nothing contained in this Warrant shall be construed as conferring upon the Holder hereof the right to vote or to consent to receive notice as a shareholder of the Company or any other matters or any rights whatsoever as a shareholder of the Company. No dividends or interest shall be payable or accrued in respect of this Warrant or the interest represented hereby or the shares purchasable hereunder until, and only to the extent that, this Warrant shall have been exercised.

6. Warrants Transferable. Subject to compliance with applicable federal and state securities laws and the transfer restrictions set forth in the Purchase Agreement, under which this Warrant was issued, this Warrant and all rights hereunder may be transferred, in whole or in part, without charge to the holder hereof (except for transfer taxes), upon the prior written consent of the Company and, thereafter, upon surrender of this Warrant properly endorsed and in

compliance with the provisions of the Purchase Agreement. Each taker and holder of this Warrant, by taking or holding the same, consents and agrees that this Warrant, when endorsed in blank, shall be deemed negotiable, and that the holder hereof, when this Warrant shall have been so endorsed, may be treated by the Company, at the Company's option, and all other persons dealing with this Warrant as the absolute owner hereof for any purpose and as the person entitled to exercise the rights represented by this Warrant, or to the transfer hereof on the books of the Company and notice to the contrary notwithstanding; but until such transfer on such books, the Company may treat the registered owner hereof as the owner for all purposes.

7. Lost Warrants. Upon receipt of evidence reasonably satisfactory to the Company of the loss, theft, destruction, or mutilation of this Warrant and, in the case of any such loss, theft or destruction, upon receipt of an indemnity reasonably satisfactory to the Company, or in the case of any such mutilation upon surrender and cancellation of such Warrant, the Company, at its expense, will make and deliver a new Warrant, of like tenor, in lieu of the lost, stolen, destroyed or mutilated Warrant.

8. Modification and Waiver. Any term of this Warrant and all Warrants issued pursuant to the Purchase Agreement may be amended and the observance of any term of this Warrant and all Warrants issued pursuant to the Purchase Agreement may be waived (either generally or in a particular instance and either retroactively or prospectively) by a writing signed by the Company and the holders of Warrants representing at least a majority of the aggregate number of Warrant Shares issuable upon exercise of all outstanding Warrants issued pursuant to the Purchase Agreement. Any amendment or waiver effected in accordance with this Section shall be binding upon the Company, the Holder and the holders of all Warrants issued pursuant to the Purchase Agreement.

9. Notices. Except as may be otherwise provided herein, all notices, requests, waivers and other communications made pursuant to this Warrant shall be made in accordance with Section 5.6 of the Purchase Agreement.

10. Governing Law. This Warrant is to be construed in accordance with and governed by the laws of the State of California.

IN WITNESS WHEREOF, the Company has caused this Warrant to be duly executed by its officers, thereunto duly authorized as of the date first above written.

ULTRAGENYX PHARMACEUTICAL INC.

By: /s/ Emil Kakkis
Emil Kakkis, CEO

FORM OF SUBSCRIPTION
(To be signed only upon exercise of Warrant)

To: Ultragenyx Pharmaceutical Inc.

The undersigned, the holder of a right to purchase shares of Series A Preferred Stock of Ultragenyx Pharmaceutical Inc., a California corporation (the "Company"), pursuant to that certain Warrant to Purchase Series A Preferred Stock of Ultragenyx Pharmaceutical Inc. (the "Warrant"), dated as of January __, 2011, hereby irrevocably elects to exercise the purchase right represented by such Warrant for, and to purchase thereunder, _____ (_____) shares of Series A Preferred Stock of the Company and herewith makes payment of _____ Dollars ($_____) therefor by the following method:

Method of Exercise *(Please initial the blank):*

_____ The undersigned elects to exercise the attached Warrant by means of a cash payment, and tenders herewith payment in full for the purchase price of the shares being purchased, together with all applicable transfer taxes, if any.

_____ The undersigned hereby elects to make payment for the aggregate exercise price of this exercise using the Net Issuance method pursuant to Section 2 of such Warrant.

3. Please issue a certificate or certificates representing said Warrant Shares in the name of the undersigned or in such other name as is specified below:

_____
(Name)

_____

_____
(Address)

4. The undersigned hereby represents and warrants that the aforesaid Warrant Shares are being acquired for the account of the undersigned for investment and not with a view to, or for resale, in connection with the distribution thereof, and that the undersigned has no present intention of distributing or reselling such shares and makes to the Company, as of the date hereof, the representations and warranties set forth in Section 3 of the Note and Warrant Purchase Agreement, dated as of January __, 2011, by and among the Company and the investors listed on Exhibit A thereto.

_____
(Signature)

_____
(Name)

_____ _____
(Date) (Title)

ACKNOWLEDGMENT

To: The John And Cynthia Klock Charitable Trust

The undersigned hereby acknowledges that as of the date hereof, _____ (_____) shares of Series A Preferred Stock remain subject to the right of purchase in favor of the John And Cynthia Klock Charitable Trust pursuant to that certain Warrant to Purchase Series A Preferred Stock of Ultragenyx Pharmaceutical Inc., dated as of January __, 2011.

DATED:

ULTRAGENYX PHARMACEUTICAL INC.

By: _____

Name: _____

Title: _____

Exhibit 4.5

THIS WARRANT AND THE SHARES PURCHASABLE HEREUNDER ARE SUBJECT TO RESTRICTIONS ON TRANSFER CONTAINED IN THAT CERTAIN NOTE AND WARRANT PURCHASE AGREEMENT, DATED FEBRUARY 2011, WHICH RESTRICTIONS ON TRANSFER ARE INCORPORATED HEREIN BY REFERENCE.

THIS WARRANT AND THE SHARES PURCHASABLE HEREUNDER HAVE BEEN ACQUIRED FOR INVESTMENT AND HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED, OR QUALIFIED UNDER ANY STATE SECURITIES LAWS SUCH SECURITIES MAY NOT BE SOLD OR TRANSFERRED IN THE ABSENCE OF SUCH REGISTRATION OR QUALIFICATION OR AN EXEMPTION THEREFROM UNDER SAID ACT AND ANY APPLICABLE STATE SECURITIES LAWS.

Dated: February 23, 2011

WARRANT TO PURCHASE

SERIES A PREFERRED STOCK OF

ULTRAGENYX PHARMACEUTICAL INC.

This certifies that the William E. Aliski, or assigns (collectively, the "Holder"), for value received, is entitled to purchase, at the Stock Purchase Price (as defined below), from Ultragenyx Pharmaceutical Inc., a California corporation (the "Company"), up to that number of fully paid and nonassessable shares of the Company's Series A Preferred Stock (the "Series A Preferred Stock"), equal to the quotient obtained in accordance with the following calculation:

Maximum number of shares of Series A Preferred Stock (the "Warrant Shares") issuable upon exercise of this Warrant	=	**(aggregate Principal Amount of the Note issued by the Company to Holder) x (0.35) the Stock Purchase Price**

This Warrant shall be exercisable at any time from time to time from and after the closing of the Series A Preferred Financing (as defined below) (such date being referred to herein as the "Initial Exercise Date") up to and including 5:00 p.m. (Pacific Time) on the first to occur of (i) the closing date of any reorganization, consolidation or merger of the Company, transfer of all or substantially all of the assets of the Company or any simultaneous sale of more than a majority of the then outstanding securities of the Company other than a mere reincorporation transaction, or (ii) the ten (10) year anniversary of the date hereof (such earlier date being referred to herein as the "Expiration Date"), upon surrender to the Company at its principal office (or at such other location as the Company may advise the Holder in writing) of this Warrant properly endorsed with (i) the Form of Subscription attached hereto duly completed and executed, (ii) payment pursuant to Section 2 of the aggregate Stock Purchase Price for the number of shares for which this Warrant is being exercised determined in accordance with the provisions hereof, and (iii) any documents reasonably requested by the Company to be executed by the Holder as if Holder were an investor in the Series A Preferred Stock Financing, including without limitation a stock purchase agreement, an investors' rights agreement, a right of first refusal and co-sale agreement, and a voting agreement, thereby agreeing to be bound by all obligations and receive all rights thereunder. The Stock Purchase Price and the number of shares purchasable hereunder are subject to adjustment as provided in Section 4 of this Warrant.

For purposes of this Warrant, (a) the term "Purchase Agreement" shall mean that certain Note and Warrant Purchase Agreement, dated as of February _, 2011 by and among the Company and the investors listed therein and (b) the terms "Note," "Principal Amount," "Series A Preferred Stock Financing" and "Stock Purchase Price" shall have the meanings given them in the Purchase Agreement.

1. Exercise, Issuance of Certificates; Acknowledgement. This Warrant is exercisable at the option of the holder of record hereof, at any time or from time to time from or after the Initial Exercise Date up to the Expiration Date for all or any part of the Warrant Shares (but not for a fraction of a share) which may be purchased hereunder. The Company agrees that the shares of Series A Preferred Stock purchased under this Warrant shall be and are deemed to be issued to the Holder hereof as the record owner of such shares as of the close of business on the date on which this Warrant shall have been surrendered, properly endorsed, the completed, executed Form of Subscription delivered and payment made for such shares. Certificates for the shares of the Series A Preferred Stock so purchased, together with any other securities or property to which the Holder hereof is entitled upon such exercise, shall be delivered to the Holder hereof by the Company at the Company's expense within a reasonable time after the rights represented by this Warrant have been so exercised. Each certificate so delivered shall be in such denominations of the Warrant Shares as may be requested by the Holder hereof and shall be registered in the name of such Holder. In case of a purchase of less than all the Warrant Shares, the Company shall execute and deliver to Holder within a reasonable time an Acknowledgement in the form attached hereto indicating the number of Warrant Shares which remain subject to this Warrant, if any.

2. Payment for Shares. The aggregate purchase price for Warrant Shares being purchased hereunder may be paid either (i) by cash or wire transfer of immediately available funds, (ii) if the fair market value of one (1) share of the Warrant Shares on the date of exercise is greater than the Stock Purchase Price, by surrender of a number of Warrant Shares which have a fair market value equal to the aggregate purchase price of the Warrant Shares being purchased ("Net Issuance") as determined herein, or (iii) any combination of the foregoing. If the Holder elects the Net Issuance method of payment, the Company shall issue to Holder upon exercise a number of shares of Warrant Shares determined in accordance with the following formula:

$$X = \frac{Y(A-B)}{A}$$

Where: X = The number of Warrant Shares to be issued to Holder.

Y = The number of Warrant Shares purchasable under this Warrant (at the date of such calculation).

A = The fair market value of one share of Warrant Shares on the date of exercise; and

B = The Stock Purchase Price.

No fractional shares arising out of the above formula for determining the number of shares to be issued to the Holder shall be issued, and the Company shall in lieu thereof make payment to the Holder of cash in the amount of such fraction multiplied by the fair market value of one (1) share of the Warrant Shares on the date of exercise. For purposes of the above calculation, the fair market value of one (1) share of the Warrant Shares shall mean (a) if the date of exercise is after the commencement of trading of the Common Stock on a securities exchange or over-the-counter but prior to the closing of the IPO, the price per share to the public set forth on the final prospectus relating to the IPO, multiplied by the number of shares of Common Stock into which each share of the Warrant Shares is then convertible, (b) if the Common Stock is then traded on a securities exchange, the average of the closing prices of such Common Stock on such exchange over the thirty (30) calendar day period (or portion thereof) ending three (3) days prior to the date of exercise, multiplied by the number of shares of Common Stock into which each share of the Warrant Shares is then convertible, (c) if the Common Stock is then regularly traded over-the-counter, the average of the closing sale prices or secondarily the closing bid of such Common Stock over the thirty (30) calendar day period (or portion thereof) ending three (3) days prior to the date of exercise, multiplied by the number of shares of Common Stock into which each share of the Warrant Shares is then convertible, or (d) if there is no active public market for the Common Stock, the fair market value of one share of the Warrant Shares as determined in good faith by the Board of Directors of the Company.

2. Shares to be Fully Paid; Reservation of Shares. The Company covenants and agrees that all shares of Series A Preferred Stock which may be issued upon the exercise of the rights represented by this Warrant (together with all shares of Common Stock issuable upon conversion of such Series A Preferred Stock) will, upon issuance, be duly authorized, validly issued, fully paid and nonassessable and free from all preemptive rights of any shareholder and free of all taxes, liens and charges with respect to the issue thereof The Company further covenants and agrees that during the period within which the rights represented by this Warrant may be exercised, the Company will at all times have authorized and reserved, for the purpose of issue or transfer upon exercise of the subscription rights evidenced by this Warrant, a sufficient number of shares of authorized but unissued shares of Series A Preferred Stock (together with the number of shares of Common Stock issuable upon conversion of such Series A Preferred Stock), or other securities and property, when and as required to provide for the exercise of the rights represented by this Warrant.

4. Adjustment of Stock Purchase Price and Number of Shares. The Stock Purchase Price and the number of shares purchasable upon the exercise of this Warrant shall be subject to adjustment from time to time upon the occurrence of certain events described in this Section 4. Upon each adjustment of the Stock Purchase Price, the Holder of this Warrant shall thereafter be entitled to purchase, at the Stock Purchase Price resulting from such adjustment, the number of shares obtained by multiplying the Stock Purchase Price in effect immediately prior to such adjustment by the number of shares purchasable pursuant hereto immediately prior to such adjustment, and dividing the product thereof by the Stock Purchase Price resulting from such adjustment.

4.1 Conversion of Preferred Stock. If all of the outstanding Preferred Stock of the Company is converted into shares of Common Stock, then this Warrant shall automatically become exercisable for that number of shares of Common Stock equal to the number of shares of

Common Stock that would have been received if this Warrant had been exercised in full and the shares of Preferred Stock received thereupon had been simultaneously converted into shares of Common Stock immediately prior to such event, and the Stock Purchase Price shall be automatically adjusted to equal the number obtained by dividing (i) the aggregate Stock Purchase Price of the shares of Preferred Stock for which this Warrant was exercisable immediately prior to such conversion, by (ii) the number of shares of Common Stock for which this Warrant is exercisable immediately after such conversion

4.2 Subdivisions, Combinations and Dividends. In case the Company shall at any time subdivide its outstanding shares of Series A Preferred Stock into a greater number of shares or pay a dividend in Series A Preferred Stock in respect of outstanding shares of Series A Preferred Stock, the Stock Purchase Price in effect immediately prior to such subdivision or at the record date of such dividend shall be proportionately reduced, and conversely, in case the outstanding shares of the Series A Preferred Stock of the Company shall be combined into a smaller number of shares, the Stock Purchase Price in effect immediately prior to such combination shall be proportionately increased.

4.3 Reclassification. If any reclassification of the capital stock of the Company shall be effected in such a way that holders of Series A Preferred Stock shall be entitled to receive stock, securities, or other assets or property, then, as a condition of such reclassification, lawful and adequate provisions shall be made whereby the Holder hereof shall thereafter have the right to purchase and receive (in lieu of the shares of the Series A Preferred Stock immediately theretofore purchasable and receivable upon the exercise of the rights represented hereby) such shares of stock, securities or other assets or property as may be issued or payable with respect to or in exchange for a number of outstanding shares of such Series A Preferred Stock equal to the number of shares of such Series A Preferred Stock immediately theretofore purchasable and receivable upon the exercise of the rights represented hereby. In any reclassification described above, appropriate provision shall be made with respect to the rights and interests of the Holder of this Warrant to the end that the provisions hereof (including, without limitation, provisions for adjustments of the Stock Purchase Price and of the number of shares purchasable and receivable upon the exercise of this Warrant) shall thereafter be applicable, as nearly as may be, in relation to any shares of stock, securities or assets thereafter deliverable upon the exercise hereof.

4.4 Notice of Adjustment. Upon any adjustment of the Stock Purchase Price or any increase or decrease in the number of shares purchasable upon the exercise of this Warrant, the Company shall give written notice thereof, by first class mail postage prepaid, addressed to the registered Holder of this Warrant at the address of such Holder as shown on the books of the Company. The notice shall be signed by the Company's chief financial officer and shall state the Stock Purchase Price resulting from such adjustment and the increase or decrease, if any, in the number of shares purchasable at such price upon the exercise of this Warrant, setting forth in reasonable detail the method of calculation and the facts upon which such calculation is based. For the avoidance of doubt, the Company acknowledges that the Holder of this Warrant shall be entitled to the benefit of all adjustments in the number of shares of Common Stock of the Company issuable upon conversion of the Series A Preferred Stock of the Company which occur prior to the exercise of this Warrant, including without limitation, any increase in the number of shares of Common Stock issuable upon conversion as a result of a dilutive issuance of capital stock.

4.5 Other Notices. If at any time:

(1) the Company shall declare any cash dividend upon its Series A Preferred Stock (or Common Stock issuable upon conversion thereof);

(2) there shall be any capital reorganization or reclassification of the capital stock of the Company; or consolidation or merger of the Company with, or sale of all or substantially all of its assets to, another Person;

(3) there shall be a voluntary or involuntary dissolution, liquidation or winding-up of the Company; or

(4) there shall be an IPO;

then, in any one or more of said cases, the Company shall give, by first class mail, postage prepaid, addressed to the Holder of this Warrant at the address of such Holder as shown on the books of the Company, (a) at least ten (10) days prior written notice 0 f the date on which the books of the Company shall close or a record shall be taken for such dividend or for determining rights to vote in respect of any such reorganization, reclassification, consolidation, merger, sale, dissolution, liquidation or winding-up, and (b) in the case of any such reorganization, reclassification, consolidation, merger, sale, dissolution, liquidation, winding-up or public offering, at least ten (10) days prior written notice of the date when the same shall take place; provided, however, that the Holder shall make a best efforts attempt to respond to such notice as early as possible after the receipt thereof Any notice given in accordance with the foregoing clause (a) shall also specify, in the case of any such dividend, the date on which the holders of Series A Preferred Stock (or Common Stock issuable upon conversion thereof) shall be entitled thereto. Any notice given in accordance with the foregoing clause (b) shall also specify the date on which the holders of Series A Preferred Stock (or Common Stock issuable upon conversion thereof) shall be entitled to exchange their Series A Preferred Stock (or Common Stock issuable upon conversion thereof) for securities or other property deliverable upon such reorganization, reclassification, consolidation, merger, sale, dissolution, liquidation, winding-up, conversion or public offering, as the case may be.

5. No Voting or Dividend Rights. Nothing contained in this Warrant shall be construed as conferring upon the Holder hereof the right to vote or to consent to receive notice as a shareholder of the Company or any other matters or any rights whatsoever as a shareholder of the Company. No dividends or interest shall be payable or accrued in respect of this Warrant or the interest represented hereby or the shares purchasable hereunder until, and only to the extent that, this Warrant shall have been exercised.

6. Warrants Transferable. Subject to compliance with applicable federal and state securities laws and the transfer restrictions set forth in the Purchase Agreement, under which this Warrant was issued, this Warrant and all rights hereunder may be transferred, in whole or in part, without charge to the holder hereof (except for transfer taxes), upon the prior written consent of the Company and, thereafter, upon surrender of this Warrant properly endorsed and in

compliance with the provisions of the Purchase Agreement. Each taker and holder of this Warrant, by taking or holding the same, consents and agrees that this Warrant, when endorsed in blank, shall be deemed negotiable, and that the holder hereof, when this Warrant shall have been so endorsed, may be treated by the Company, at the Company's option, and all other persons dealing with this Warrant as the absolute owner hereof for any purpose and as the person entitled to exercise the rights represented by this Warrant, or to the transfer hereof on the books of the Company and notice to the contrary notwithstanding; but until such transfer on such books, the Company may treat the registered owner hereof as the owner for all purposes.

7. Lost Warrants. Upon receipt of evidence reasonably satisfactory to the Company of the loss, theft, destruction, or mutilation of this Warrant and, in the case of any such loss, theft or destruction, upon receipt of an indemnity reasonably satisfactory to the Company, or in the case of any such mutilation upon surrender and cancellation of such Warrant, the Company, at its expense, will make and deliver a new Warrant, of like tenor, in lieu of the lost, stolen, destroyed or mutilated Warrant.

8. Modification and Waiver. Any term of this Warrant and all Warrants issued pursuant to the Purchase Agreement may be amended and the observance of any term of this Warrant and all Warrants issued pursuant to the Purchase Agreement may be waived (either generally or in a particular instance and either retroactively or prospectively) by a writing signed by the Company and the holders of Warrants representing at least a majority of the aggregate number of Warrant Shares issuable upon exercise of all outstanding Warrants issued pursuant to the Purchase Agreement. Any amendment or waiver effected in accordance with this Section shall be binding upon the Company, the Holder and the holders of all Warrants issued pursuant to the Purchase Agreement.

9. Notices. Except as may be otherwise provided herein, all notices, requests, waivers and other communications made pursuant to this Warrant shall be made in accordance with Section 5.6 of the Purchase Agreement.

10. Governing Law. This Warrant is to be construed in accordance with and governed by the laws of the State of California.

IN WITNESS WHEREOF, the Company has caused this Warrant to be duly executed by its officers, thereunto duly authorized as of the date first above written.

ULTRAGENYX PHARMACEUTICAL INC.

By: /s/ Emil Kakkis
Emil Kakkis, CEO

FORM OF SUBSCRIPTION
(To be signed only upon exercise of Warrant)

To: Ultragenyx Pharmaceutical Inc.

The undersigned, the holder of a right to purchase shares of Series A Preferred Stock of Ultragenyx Pharmaceutical Inc., a California corporation (the "Company"), pursuant to that certain Warrant to Purchase Series A Preferred Stock of Ultragenyx Pharmaceutical Inc. (the "Warrant"), dated as of February __, 2011, hereby irrevocably elects to exercise the purchase right represented by such Warrant for, and to purchase thereunder, _____ (_____) shares of Series A Preferred Stock of the Company and herewith makes payment of _____ Dollars ($_____) therefor by the following method:

Method of Exercise *(Please initial the blank):*

_____ The undersigned elects to exercise the attached Warrant by means of a cash payment, and tenders herewith payment in full for the purchase price of the shares being purchased, together with all applicable transfer taxes, if any.

_____ The undersigned hereby elects to make payment for the aggregate exercise price of this exercise using the Net Issuance method pursuant to Section 2 of such Warrant.

3. Please issue a certificate or certificates representing said Warrant Shares in the name of the undersigned or in such other name as is specified below:

_____
(Name)

_____

_____
(Address)

4. The undersigned hereby represents and warrants that the aforesaid Warrant Shares are being acquired for the account of the undersigned for investment and not with a view to, or for resale, in connection with the distribution thereof, and that the undersigned has no present intention of distributing or reselling such shares and makes to the Company, as of the date hereof, the representations and warranties set forth in Section 3 of the Note and Warrant Purchase Agreement, dated as of February __, 2011, by and among the Company and the investors listed on Exhibit A thereto.

_____
(Signature)

_____
(Name)

_____ _____
(Date) (Title)

ACKNOWLEDGMENT

To: William E. Aliski

The undersigned hereby acknowledges that as of the date hereof, _____ (_____) shares of Series A Preferred Stock remain subject to the right of purchase in favor of William E. Aliski pursuant to that certain Warrant to Purchase Series A Preferred Stock of Ultragenyx Pharmaceutical Inc., dated as of February __, 2011.

DATED:

ULTRAGENYX PHARMACEUTICAL INC.

By: _____

Name: _____

Title: _____

Exhibit 4.6

THIS WARRANT AND THE SHARES PURCHASABLE HEREUNDER HAVE BEEN ACQUIRED FOR INVESTMENT AND HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED, OR QUALIFIED UNDER ANY STATE SECURITIES LAWS. SUCH SECURITIES MAY NOT BE SOLD OR TRANSFERRED IN THE ABSENCE OF SUCH REGISTRATION OR QUALIFICATION OR AN EXEMPTION THEREFROM UNDER SAID ACT AND ANY APPLICABLE STATE SECURITIES LAWS.

THIS WARRANT AND THE SHARES PURCHASABLE HEREUNDER ARE SUBJECT TO RESTRICTIONS ON TRANSFER CONTAINED IN THAT CERTAIN NOTE AND WARRANT PURCHASE AGREEMENT, DATED JUNE 14, 2011, WHICH RESTRICTIONS ON TRANSFER ARE INCORPORATED HEREIN BY REFERENCE.

Dated: June 14, 2011

WARRANT TO PURCHASE

SERIES A PREFERRED STOCK OF

ULTRAGENYX PHARMACEUTICAL INC.

This certifies that the Emil D. Kakkis, M.D., Ph.D., or assigns (collectively, the "Holder"), for value received, is entitled to purchase, at the Stock Purchase Price (as defined below), from Ultragenyx Pharmaceutical Inc., a California corporation (the "Company"), up to that number of fully paid and nonassessable shares of the Company's Series A Preferred Stock (the "Series A Preferred Stock"), equal to the quotient obtained in accordance with the following calculation:

Maximum number of shares of Series A Preferred Stock (the "Warrant Shares") issuable upon exercise of this Warrant	=	**(aggregate Principal Amount of the Note issued by the Company to Holder) x (0.25) the Stock Purchase Price**

This Warrant shall be exercisable at any time from time to time from and after the closing of the Series A Preferred Financing (as defined below) (such date being referred to herein as the "Initial Exercise Date") up to and including 5:00 p.m. (Pacific Time) on the first to occur of (i) the closing date of any reorganization, consolidation or merger of the Company, transfer of all or substantially all of the assets of the Company or any simultaneous sale of more than a majority of the then outstanding securities of the Company other than a mere reincorporation transaction, or (ii) the ten (10) year anniversary of the date hereof (such earlier date being referred to herein as the "Expiration Date"), upon surrender to the Company at its principal office (or at such other location as the Company may advise the Holder in writing) of this Warrant properly endorsed with (i) the Form of Subscription attached hereto duly completed and executed, (ii) payment pursuant to Section 2 of the aggregate Stock Purchase Price for the number of shares for which this Warrant is being exercised determined in accordance with the provisions hereof, and (iii) any documents reasonably requested by the Company to be executed by the Holder as if Holder were an investor in the Series A Preferred Stock Financing, including without limitation a stock purchase agreement, an investors' rights agreement, a right of first refusal and co-sale agreement, and a voting agreement, thereby agreeing to be bound by all obligations and receive all rights thereunder. The Stock Purchase Price and the number of shares purchasable hereunder are subject to adjustment as provided in Section 4 of this Warrant.

For purposes of this Warrant, (a) the term "Purchase Agreement" shall mean that certain Note and Warrant Purchase Agreement, dated as of June 14, 2011 by and among the Company and the investors listed therein and (b) the terms "Note," "Principal Amount," "Series A Preferred Stock Financing" and "Stock Purchase Price" shall have the meanings given them in the Purchase Agreement.

1. Exercise, Issuance of Certificates; Acknowledgement. This Warrant is exercisable at the option of the holder of record hereof, at any time or from time to time from or after the Initial Exercise Date up to the Expiration Date for all or any part of the Warrant Shares (but not for a fraction of a share) which may be purchased hereunder. The Company agrees that the shares of Series A Preferred Stock purchased under this Warrant shall be and are deemed to be issued to the Holder hereof as the record owner of such shares as of the close of business on the date on which this Warrant shall have been surrendered, properly endorsed, the completed, executed Form of Subscription delivered and payment made for such shares. Certificates for the shares of the Series A Preferred Stock so purchased, together with any other securities or property to which the Holder hereof is entitled upon such exercise, shall be delivered to the Holder hereof by the Company at the Company's expense within a reasonable time after the rights represented by this Warrant have been so exercised. Each certificate so delivered shall be in such denominations of the Warrant Shares as may be requested by the Holder hereof and shall be registered in the name of such Holder. In case of a purchase of less than all the Warrant Shares, the Company shall execute and deliver to Holder within a reasonable time an Acknowledgement in the form attached hereto indicating the number of Warrant Shares which remain subject to this Warrant, if any.

2. Payment for Shares. The aggregate purchase price for Warrant Shares being purchased hereunder may be paid either (i) by cash or wire transfer of immediately available funds, (ii) if the fair market value of one (1) share of the Warrant Shares on the date of exercise is greater than the Stock Purchase Price, by surrender of a number of Warrant Shares which have a fair market value equal to the aggregate purchase price of the Warrant Shares being purchased ("Net Issuance") as determined herein, or (iii) any combination of the foregoing. If the Holder elects the Net Issuance method of payment, the Company shall issue to Holder upon exercise a number of shares of Warrant Shares determined in accordance with the following formula:

$$X = \frac{Y(A-B)}{A}$$

Where: X = The number of Warrant Shares to be issued to Holder;

Y = The number of Warrant Shares with respect to which the Holder is exercising its purchase rights under this Warrant;

A = The fair market value of one (1) share of the Warrant Shares on the date of exercise, and

B = The Stock Purchase Price.

No fractional shares arising out of the above formula for determining the number of shares to be issued to the Holder shall be issued, and the Company shall in lieu thereof make payment to the Holder of cash in the amount of such fraction multiplied by the fair market value of one (1) share of the Warrant Shares on the date of exercise. For purposes of the above calculation, the fair market value of one (1) share of the Warrant Shares shall mean (a) if the date of exercise is after the commencement of trading of the Common Stock on a securities exchange or over-the-counter but prior to the closing of the IPO, the price per share to the public set forth on the final prospectus relating to the IPO, multiplied by the number of shares of Common Stock into which each share of the Warrant Shares is then convertible, (b) if the Common Stock is then traded on a securities exchange, the average of the closing prices of such Common Stock on such exchange over the thirty (30) calendar day period (or portion thereof) ending three (3) days prior to the date of exercise, multiplied by the number of shares of Common Stock into which each share of the Warrant Shares is then convertible, (c) if the Common Stock is then regularly traded over-the-counter, the average of the closing sale prices or secondarily the closing bid of such Common Stock over the thirty (30) calendar day period (or portion thereof) ending three (3) days prior to the date of exercise, multiplied by the number of shares of Common Stock into which each share of the Warrant Shares is then convertible, or (d) if there is no active public market for the Common Stock, the fair market value of one share of the Warrant Shares as determined in good faith by the Board of Directors of the Company.

3. Shares to be Fully Paid; Reservation of Shares. The Company covenants and agrees that all shares of Series A Preferred Stock which may be issued upon the exercise of the rights represented by this Warrant (together with all shares of Common Stock issuable upon conversion of such Series A Preferred Stock) will, upon issuance, be duly authorized, validly issued, fully paid and nonassessable and free from all preemptive rights of any shareholder and free of all taxes, liens and charges with respect to the issue thereof. The Company further covenants and agrees that during the period within which the rights represented by this Warrant may be exercised, the Company will at all times have authorized and reserved, for the purpose of issue or transfer upon exercise of the subscription rights evidenced by this Warrant, a sufficient number of shares of authorized but unissued shares of Series A Preferred Stock (together with the number of shares of Common Stock issuable upon conversion of such Series A Preferred Stock), or other securities and property, when and as required to provide for the exercise of the rights represented by this Warrant.

4. Adjustment of Stock Purchase Price and Number of Shares. The Stock Purchase Price and the number of shares purchasable upon the exercise of this Warrant shall be subject to adjustment from time to time upon the occurrence of certain events described in this Section 4. Upon each adjustment of the Stock Purchase Price, the Holder of this Warrant shall thereafter be entitled to purchase, at the Stock Purchase Price resulting from such adjustment, the number of shares obtained by multiplying the Stock Purchase Price in effect immediately prior to such adjustment by the number of shares purchasable pursuant hereto immediately prior to such adjustment, and dividing the product thereof by the Stock Purchase Price resulting from such adjustment.

4.1 Conversion of Preferred Stock. If all of the outstanding Preferred Stock of the Company is converted into shares of Common Stock, then this Warrant shall automatically become exercisable for that number of shares of Common Stock equal to the number of shares of Common Stock that would have been received if this Warrant had been exercised in full and the shares of Preferred Stock received thereupon had been simultaneously converted into shares of Common Stock immediately prior to such event, and the Stock Purchase Price shall be automatically adjusted to equal the number obtained by dividing (i) the aggregate Stock Purchase Price of the shares of Preferred Stock for which this Warrant was exercisable immediately prior to such conversion, by (ii) the number of shares of Common Stock for which this Warrant is exercisable immediately after such conversion

4.2 Subdivisions, Combinations and Dividends. In case the Company shall at any time subdivide its outstanding shares of Series A Preferred Stock into a greater number of shares or pay a dividend in Series A Preferred Stock in respect of outstanding shares of Series A Preferred Stock, the Stock Purchase Price in effect immediately prior to such subdivision or at the record date of such dividend shall be proportionately reduced, and conversely, in case the outstanding shares of the Series A Preferred Stock of the Company shall be combined into a smaller number of shares, the Stock Purchase Price in effect immediately prior to such combination shall be proportionately increased.

4.3 Reclassification. If any reclassification of the capital stock of the Company shall be effected in such a way that holders of Series A Preferred Stock shall be entitled to receive stock, securities, or other assets or property, then, as a condition of such reclassification, lawful and adequate provisions shall be made whereby the Holder hereof shall thereafter have the right to purchase and receive (in lieu of the shares of the Series A Preferred Stock immediately theretofore purchasable and receivable upon the exercise of the rights represented hereby) such shares of stock, securities or other assets or property as may be issued or payable with respect to or in exchange for a number of outstanding shares of such Series A Preferred Stock equal to the number of shares of such Series A Preferred Stock immediately theretofore purchasable and receivable upon the exercise of the rights represented hereby. In any reclassification described above, appropriate provision shall be made with respect to the rights and interests of the Holder of this Warrant to the end that the provisions hereof (including, without limitation, provisions for adjustments of the Stock Purchase Price and of the number of shares purchasable and receivable upon the exercise of this Warrant) shall thereafter be applicable, as nearly as may be, in relation to any shares of stock, securities or assets thereafter deliverable upon the exercise hereof.

4.4 Notice of Adjustment. Upon any adjustment of the Stock Purchase Price or any increase or decrease in the number of shares purchasable upon the exercise of this Warrant, the Company shall give written notice thereof, by first class mail postage prepaid, addressed to the registered Holder of this Warrant at the address of such Holder as shown on the books of the Company. The notice shall be signed by the Company's chief financial officer and shall state the Stock Purchase Price resulting from such adjustment and the increase or decrease, if any, in the number of shares purchasable at such price upon the exercise of this Warrant, setting forth in reasonable detail the method of calculation and the facts upon which such calculation is based. For the avoidance of doubt, the Company acknowledges that the Holder of this Warrant shall be entitled to the benefit of all adjustments in the number of shares of

Common Stock of the Company issuable upon conversion of the Series A Preferred Stock of the Company which occur prior to the exercise of this Warrant, including without limitation, any increase in the number of shares of Common Stock issuable upon conversion as a result of a dilutive issuance of capital stock.

4.5 Other Notices. If at any time:

(1) the Company shall declare any cash dividend upon its Series A Preferred Stock (or Common Stock issuable upon conversion thereof);

(2) there shall be any capital reorganization or reclassification of the capital stock of the Company; or consolidation or merger of the Company with, or sale of all or substantially all of its assets to, another Person;

(3) there shall be a voluntary or involuntary dissolution, liquidation or winding-up of the Company; or

(4) there shall be an IPO;

then, in any one or more of said cases, the Company shall give, by first class mail, postage prepaid, addressed to the Holder of this Warrant at the address of such Holder as shown on the books of the Company, (a) at least ten (10) days prior written notice of the date on which the books of the Company shall close or a record shall be taken for such dividend or for determining rights to vote in respect of any such reorganization, reclassification, consolidation, merger, sale, dissolution, liquidation or winding-up, and (b) in the case of any such reorganization, reclassification, consolidation, merger, sale, dissolution, liquidation, winding-up or public offering, at least ten (10) days prior written notice of the date when the same shall take place; provided, however, that the Holder shall make a best efforts attempt to respond to such notice as early as possible after the receipt thereof Any notice given in accordance with the foregoing clause (a) shall also specify, in the case of any such dividend, the date on which the holders of Series A Preferred Stock (or Common Stock issuable upon conversion thereof) shall be entitled thereto. Any notice given in accordance with the foregoing clause (b) shall also specify the date on which the holders of Series A Preferred Stock (or Common Stock issuable upon conversion thereof) shall be entitled to exchange their Series A Preferred Stock (or Common Stock issuable upon conversion thereof) for securities or other property deliverable upon such reorganization, reclassification, consolidation, merger, sale, dissolution, liquidation, winding-up, conversion or public offering, as the case may be.

5. No Voting or Dividend Rights. Nothing contained in this Warrant shall be construed as conferring upon the Holder hereof the right to vote or to consent to receive notice as a shareholder of the Company or any other matters or any rights whatsoever as a shareholder of the Company. No dividends or interest shall be payable or accrued in respect of this Warrant or the interest represented hereby or the shares purchasable hereunder until, and only to the extent that, this Warrant shall have been exercised.

6. Warrants Transferable. Subject to compliance with applicable federal and state securities laws and the transfer restrictions set forth in the Purchase Agreement, under which this Warrant was issued, this Warrant and all rights hereunder may be transferred, in whole or in part,

without charge to the holder hereof (except for transfer taxes), upon the prior written consent of the Company and, thereafter, upon surrender of this Warrant properly endorsed and in compliance with the provisions of the Purchase Agreement. Each taker and holder of this Warrant, by taking or holding the same, consents and agrees that this Warrant, when endorsed in blank, shall be deemed negotiable, and that the holder hereof, when this Warrant shall have been so endorsed, may be treated by the Company, at the Company's option, and all other persons dealing with this Warrant as the absolute owner hereof for any purpose and as the person entitled to exercise the rights represented by this Warrant, or to the transfer hereof on the books of the Company and notice to the contrary notwithstanding; but until such transfer on such books, the Company may treat the registered owner hereof as the owner for all purposes.

7. Lost Warrants. Upon receipt of evidence reasonably satisfactory to the Company of the loss, theft, destruction, or mutilation of this Warrant and, in the case of any such loss, theft or destruction, upon receipt of an indemnity reasonably satisfactory to the Company, or in the case of any such mutilation upon surrender and cancellation of such Warrant, the Company, at its expense, will make and deliver a new Warrant, of like tenor, in lieu of the lost, stolen, destroyed or mutilated Warrant.

8. Modification and Waiver. Any term of this Warrant and all Warrants issued pursuant to the Purchase Agreement may be amended and the observance of any term of this Warrant and all Warrants issued pursuant to the Purchase Agreement may be waived (either generally or in a particular instance and either retroactively or prospectively) by a writing signed by the Company and the holders of Warrants representing at least a majority of the aggregate number of Warrant Shares issuable upon exercise of all outstanding Warrants issued pursuant to the Purchase Agreement. Any amendment or waiver effected in accordance with this Section shall be binding upon the Company, the Holder and the holders of all Warrants issued pursuant to the Purchase Agreement.

9. Notices. Except as may be otherwise provided herein, all notices, requests, waivers and other communications made pursuant to this Warrant shall be made in accordance with Section 5.6 of the Purchase Agreement.

10. Governing Law. This Warrant is to be construed in accordance with and governed by the laws of the State of California.

IN WITNESS WHEREOF, the Company has caused this Warrant to be duly executed by its officers, thereunto duly authorized as of the date first above written.

ULTRAGENYX PHARMACEUTICAL INC.

By: /s/ John Ditton
John Ditton
Secretary

FORM OF SUBSCRIPTION
(To be signed only upon exercise of Warrant)

To: _____

The undersigned, the holder of a right to purchase shares of Series A Preferred Stock of Ultragenyx Pharmaceutical Inc., a California corporation (the "Company"), pursuant to that certain Warrant to Purchase Series A Preferred Stock of Ultragenyx Pharmaceutical Inc. (the "Warrant"), dated as of June 14, 2011, hereby irrevocably elects to exercise the purchase right represented by such Warrant for, and to purchase thereunder, _____ (_____) shares of Series A Preferred Stock of the Company and herewith makes payment of _____ Dollars ($_____) therefor by the following method:

(Check one of the .following):

_____ (check if applicable) The undersigned hereby elects to make payment of _____ Dollars ($_____) therefor in cash.

_____ (check if applicable) The undersigned hereby elects to make payment for the aggregate exercise price of this exercise using the Net Issuance method pursuant to Section 2 of such Warrant.

The undersigned represents that it is acquiring such securities for its own account for investment and not with a view to or for sale in connection with any distribution thereof and in order to induce the issuance of such securities makes to the Company, as of the date hereof, the representations and warranties set forth in Section 3 of the Note and Warrant Purchase Agreement, dated as of June 14, 2011, by and among the Company and the investors listed on Exhibit A thereto.

DATED: _____

EMIL D. KAKKIS, M.D., Ph.D.

By: _____

ACKNOWLEDGMENT

To: Emit D. Kakkis, M.D., Ph.D.

The undersigned hereby acknowledges that as of the date hereof, _____ (_____) shares of Series A Preferred Stock remain subject to the right of purchase in favor of Emil D. Kalkis, M.D., Ph.D. pursuant to that certain Warrant to Purchase Series A Preferred Stock of Ultragenyx Pharmaceutical Inc., dated as of June 14, 2011.

DATED: _____

ULTRAGENYX PHARMACEUTICAL INC.

By: /s/ Emil Kakkis
Name: Emil Kakkis
Title: CEO

Exhibit 4.7

THIS WARRANT AND THE SHARES PURCHASABLE HEREUNDER HAVE BEEN ACQUIRED FOR INVESTMENT AND HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED, OR QUALIFIED UNDER ANY STATE SECURITIES LAWS. SUCH SECURITIES MAY NOT BE SOLD OR TRANSFERRED IN THE ABSENCE OF SUCH REGISTRATION OR QUALIFICATION OR AN EXEMPTION THEREFROM UNDER SAID ACT AND ANY APPLICABLE STATE SECURITIES LAWS.

THIS WARRANT AND THE SHARES PURCHASABLE HEREUNDER ARE SUBJECT TO RESTRICTIONS ON TRANSFER CONTAINED IN THAT CERTAIN NOTE AND WARRANT PURCHASE AGREEMENT, DATED JUNE 14, 2011, WHICH RESTRICTIONS ON TRANSFER ARE INCORPORATED HEREIN BY REFERENCE.

Dated: June 14, 2011

WARRANT TO PURCHASE

SERIES A PREFERRED STOCK OF

ULTRAGENYX PHARMACEUTICAL INC.

This certifies that Emil D. Kakkis, M.D., Ph.D., or assigns (collectively, the "Holder"), for value received, is entitled to purchase, at the Stock Purchase Price (as defined below), from Ultragenyx Pharmaceutical Inc., a California corporation (the "Company"), up to that number of fully paid and nonassessable shares of the Company's Series A Preferred Stock (the "Series A Preferred Stock"), equal to the quotient obtained in accordance with the following calculation:

Maximum number of shares of Series A Preferred Stock (the "Warrant Shares") issuable upon exercise of this Warrant	=	**(aggregate Principal Amount of the Note issued by the Company to Holder) x (0.25) the Stock Purchase Price**

This Warrant shall be exercisable at any time from time to time from and after the closing of the Series A Preferred Financing (as defined below) (such date being referred to herein as the "Initial Exercise Date") up to and including 5:00 p.m. (Pacific Time) on the first to occur of (i) the closing date of any reorganization, consolidation or merger of the Company, transfer of all or substantially all of the assets of the Company or any simultaneous sale of more than a majority of the then outstanding securities of the Company other than a mere reincorporation transaction, or (ii) the ten (10) year anniversary of the date hereof (such earlier date being referred to herein as the "Expiration Date"), upon surrender to the Company at its principal office (or at such other location as the Company may advise the Holder in writing) of this Warrant properly endorsed with (i) the Form of Subscription attached hereto duly completed and executed, (ii) payment pursuant to Section 2 of the aggregate Stock Purchase Price for the number of shares for which this Warrant is being exercised determined in accordance with the provisions hereof, and (iii) any documents reasonably requested by the Company to be executed by the Holder as if Holder were an investor in the Series A Preferred Stock Financing, including without limitation a stock purchase agreement, an investors' rights agreement, a right of first refusal and co-sale agreement, and a voting agreement, thereby agreeing to be bound by all obligations and receive all rights thereunder. The Stock Purchase Price and the number of shares purchasable hereunder are subject to adjustment as provided in Section 4 of this Warrant.

For purposes of this Warrant, (a) the term "Purchase Agreement" shall mean that certain Note and Warrant Purchase Agreement, dated as of June 14, 2011 by and among the Company and the investors listed therein and (b) the terms "Note," "Principal Amount," "Series A Preferred Stock Financing" and "Stock Purchase Price" shall have the meanings given them in the Purchase Agreement.

1. Exercise; Issuance of Certificates; Acknowledgement. This Warrant is exercisable at the option of the holder of record hereof, at any time or from time to time from or after the Initial Exercise Date up to the Expiration Date for all or any part of the Warrant Shares (but not for a fraction of a share) which may be purchased hereunder. The Company agrees that the shares of Series A Preferred Stock purchased under this Warrant shall be and are deemed to be issued to the Holder hereof as the record owner of such shares as of the close of business on the date on which this Warrant shall have been surrendered, properly endorsed, the completed, executed Form of Subscription delivered and payment made for such shares. Certificates for the shares of the Series A Preferred Stock so purchased, together with any other securities or property to which the Holder hereof is entitled upon such exercise, shall be delivered to the Holder hereof by the Company at the Company's expense within a reasonable time after the rights represented by this Warrant have been so exercised. Each certificate so delivered shall be in such denominations of the Warrant Shares as may be requested by the Holder hereof and shall be registered in the name of such Holder. In case of a purchase of less than all the Warrant Shares, the Company shall execute and deliver to Holder within a reasonable time an Acknowledgement in the form attached hereto indicating the number of Warrant Shares which remain subject to this Warrant, if any.

2. Payment for Shares. The aggregate purchase price for Warrant Shares being purchased hereunder may be paid either (i) by cash or wire transfer of immediately available funds, (ii) if the fair market value of one (1) share of the Warrant Shares on the date of exercise is greater than the Stock Purchase Price, by surrender of a number of Warrant Shares which have a fair market value equal to the aggregate purchase price of the Warrant Shares being purchased ("Net Issuance") as determined herein, or (iii) any combination of the foregoing. If the Holder elects the Net Issuance method of payment, the Company shall issue to Holder upon exercise a number of shares of Warrant Shares determined in accordance with the following formula:

$$X = \frac{Y(A-B)}{A}$$

Where: X = The number of Warrant Shares to be issued to Holder;

Y = the number of Warrant Shares with respect to which the Holder is exercising its purchase rights under this Warrant;

A = the fair market value of one (1) share of the Warrant Shares on the date of exercise; and

B = The Stock Purchase Price.

No fractional shares arising out of the above formula for determining the number of shares to be issued to the Holder shall be issued, and the Company shall in lieu thereof make payment to the Holder of cash in the amount of such fraction multiplied by the fair market value of one (1) share of the Warrant Shares on the date of exercise. For purposes of the above calculation, the fair market value of one (1) share of the Warrant Shares shall mean (a) if the date of exercise is after the commencement of trading of the Common Stock on a securities exchange or over-the-counter but prior to the closing of the IPO, the price per share to the public set forth on the final prospectus relating to the IPO, multiplied by the number of shares of Common Stock into which each share of the Warrant Shares is then convertible, (b) if the Common Stock is then traded on a securities exchange, the average of the closing prices of such Common Stock on such exchange over the thirty (30) calendar day period (or portion thereof) ending three (3) days prior to the date of exercise, multiplied by the number of shares of Common Stock into which each share of the Warrant Shares is then convertible, (c) if the Common Stock is then regularly traded over-the-counter, the average of the closing sale prices or secondarily the closing bid of such Common Stock over the thirty (30) calendar day period (or portion thereof) ending three (3) days prior to the date of exercise, multiplied by the number of shares of Common Stock into which each share of the Warrant Shares is then convertible, or (d) if there is no active public market for the Common Stock, the fair market value of one share of the Warrant Shares as determined in good faith by the Board of Directors of the Company.

3. Shares to be Fully Paid; Reservation of Shares. The Company covenants and agrees that all shares of Series A Preferred Stock which may be issued upon the exercise of the rights represented by this Warrant (together with all shares of Common Stock issuable upon conversion of such Series A Preferred Stock) will, upon issuance, be duly authorized, validly issued, fully paid and nonassessable and free from all preemptive rights of any shareholder and free of all taxes, liens and charges with respect to the issue thereof. The Company further covenants and agrees that during the period within which the rights represented by this Warrant may be exercised, the Company will at all times have authorized and reserved, for the purpose of issue or transfer upon exercise of the subscription rights evidenced by this Warrant, a sufficient number of shares of authorized but unissued shares of Series A Preferred Stock (together with the number of shares of Common Stock issuable upon conversion of such Series A Preferred Stock), or other securities and property, when and as required to provide for the exercise of the rights represented by this Warrant.

4. Adjustment of Stock Purchase Price and Number of Shares. The Stock Purchase Price and the number of shares purchasable upon the exercise of this Warrant shall be subject to adjustment from time to time upon the occurrence of certain events described in this Section 4. Upon each adjustment of the Stock Purchase Price, the Holder of this Warrant shall thereafter be entitled to purchase, at the Stock Purchase Price resulting from such adjustment, the number of shares obtained by multiplying the Stock Purchase Price in effect immediately prior to such adjustment by the number of shares purchasable pursuant hereto immediately prior to such adjustment, and dividing the product thereof by the Stock Purchase Price resulting from such adjustment.

4.1. Conversion of Preferred Stock. If all of the outstanding Preferred Stock of the Company is converted into shares of Common Stock, then this Warrant shall automatically become exercisable for that number of shares of Common Stock equal to the number of shares of Common Stock that would have been received if this Warrant had been exercised in full and the shares of Preferred Stock received thereupon had been simultaneously converted into shares of Common Stock immediately prior to such event, and the Stock Purchase Price shall be automatically adjusted to equal the number obtained by dividing (i) the aggregate Stock Purchase Price of the shares of Preferred Stock for which this Warrant was exercisable immediately prior to such conversion, by (ii) the number of shares of Common Stock for which this Warrant is exercisable immediately after such conversion

4.2. Subdivisions, Combinations and Dividends. In case the Company shall at any time subdivide its outstanding shares of Series A Preferred Stock into a greater number of shares or pay a dividend in Series A Preferred Stock in respect of outstanding shares of Series A Preferred Stock, the Stock Purchase Price in effect immediately prior to such subdivision or at the record date of such dividend shall be proportionately reduced, and conversely, in case the outstanding shares of the Series A Preferred Stock of the Company shall be combined into a smaller number of shares, the Stock Purchase Price in effect immediately prior to such combination shall be proportionately increased.

4.3. Reclassification. If any reclassification of the capital stock of the Company shall be effected in such a way that holders of Series A Preferred Stock shall be entitled to receive stock, securities, or other assets or property, then, as a condition of such reclassification, lawful and adequate provisions shall be made whereby the Holder hereof shall thereafter have the right to purchase and receive (in lieu of the shares of the Series A Preferred Stock immediately theretofore purchasable and receivable upon the exercise of the rights represented hereby) such shares of stock, securities or other assets or property as may be issued or payable with respect to or in exchange for a number of outstanding shares of such Series A Preferred Stock equal to the number of shares of such Series A Preferred Stock immediately theretofore purchasable and receivable upon the exercise of the rights represented hereby. In any reclassification described above, appropriate provision shall be made with respect to the rights and interests of the Holder of this Warrant to the end that the provisions hereof (including, without limitation, provisions for adjustments of the Stock Purchase Price and of the number of shares purchasable and receivable upon the exercise of this Warrant) shall thereafter be applicable, as nearly as may be, in relation to any shares of stock, securities or assets thereafter deliverable upon the exercise hereof.

4.4. Notice of Adjustment. Upon any adjustment of the Stock Purchase Price or any increase or decrease in the number of shares purchasable upon the exercise of this Warrant, the Company shall give written notice thereof, by first class mail postage prepaid, addressed to the registered Holder of this Warrant at the address of such Holder as shown on the books of the Company. The notice shall be signed by the Company's chief financial officer and shall state the Stock Purchase Price resulting from such adjustment and the increase or decrease, if any, in the number of shares purchasable at such price upon the exercise of this Warrant setting forth in reasonable detail the method of calculation and the facts upon which such calculation is based. For the avoidance of doubt, the Company acknowledges that the Holder of this Warrant shall be entitled to the benefit of all adjustments in the number of shares of Common Stock of the Company issuable upon conversion of the Series A Preferred Stock of the Company which occur prior to the exercise of this Warrant, including without limitation, any increase in the number of shares of Common Stock issuable upon conversion as a result of a dilutive issuance of capital stock.

4.5. Other Notices. If at any time:

(1) the Company shall declare any cash dividend upon its Series A Preferred Stock (or Common Stock issuable upon conversion thereof);

(2) there shall be any capital reorganization or reclassification of the capital stock of the Company; or consolidation or merger of the Company with, or sale of all or substantially all of its assets to, another Person;

(3) there shall be a voluntary or involuntary dissolution, liquidation or winding-up of the Company; or

(4) there shall be an IPO;

then, in any one or more of said cases, the Company shall give, by first class mail, postage prepaid, addressed to the Holder of this Warrant at the address of such Holder as shown on the books of the Company, (a) at least ten (10) days prior written notice of the date on which the books of the Company shall close or a record shall be taken for such dividend or for determining rights to vote in respect of any such reorganization, reclassification, consolidation, merger, sale, dissolution, liquidation or winding-up, and (b) in the case of any such reorganization, reclassification, consolidation, merger, sale, dissolution, liquidation, winding-up or public offering, at least ten (10) days prior written notice of the date when the same shall take place; provided, however, that the Holder shall make a best efforts attempt to respond to such notice as early as possible after the receipt thereof. Any notice given in accordance with the foregoing clause (a) shall also specify, in the case of any such dividend, the date on which the holders of Series A Preferred Stock (or Common Stock issuable upon conversion thereof) shall be entitled thereto. Any notice given in accordance with the foregoing clause (b) shall also specify the date on which the holders of Series A Preferred Stock (or Common Stock issuable upon conversion thereof) shall be entitled to exchange their Series A Preferred Stock (or Common Stock issuable upon conversion thereof) for securities or other property deliverable upon such reorganization, reclassification, consolidation, merger, sale, dissolution, liquidation, winding-up, conversion or public offering, as the case may be.

5. No Voting or Dividend Rights. Nothing contained in this Warrant shall be construed as conferring upon the Holder hereof the right to vote or to consent to receive notice as a shareholder of the Company or any other matters or any rights whatsoever as a shareholder of the Company. No dividends or interest shall be payable or accrued in respect of this Warrant or the interest represented hereby or the shares purchasable hereunder until, and only to the extent that, this Warrant shall have been exercised.

6. Warrants Transferable. Subject to compliance with applicable federal and state securities laws and the transfer restrictions set forth in the Purchase Agreement, under which this Warrant was issued, this Warrant and all rights hereunder may be transferred, in whole or in part, without charge to the holder hereof (except for transfer taxes), upon the prior written consent of

the Company and, thereafter, upon surrender of this Warrant properly endorsed and in compliance with the provisions of the Purchase Agreement. Each taker and holder of this Warrant, by taking or holding the same, consents and agrees that this Warrant, when endorsed in blank, shall be deemed negotiable, and that the holder hereof, when this Warrant shall have been so endorsed, may be treated by the Company, at the Company's option, and all other persons dealing with this Warrant as the absolute owner hereof for any purpose and as the person entitled to exercise the rights represented by this Warrant, or to the transfer hereof on the books of the Company and notice to the contrary notwithstanding; but until such transfer on such books, the Company may treat the registered owner hereof as the owner for all purposes.

7. Lost Warrants. Upon receipt of evidence reasonably satisfactory to the Company of the loss, theft, destruction, or mutilation of this Warrant and, in the case of any such loss, theft or destruction, upon receipt of an indemnity reasonably satisfactory to the Company, or in the case of any such mutilation upon surrender and cancellation of such Warrant, the Company, at its expense, will make and deliver a new Warrant, of like tenor, in lieu of the lost, stolen, destroyed or mutilated Warrant.

8. Modification and Waiver. Any term of this Warrant and all Warrants issued pursuant to the Purchase Agreement may be amended and the observance of any term of this Warrant and all Warrants issued pursuant to the Purchase Agreement may be waived (either generally or in a particular instance and either retroactively or prospectively) by a writing signed by the Company and the holders of Warrants representing at least a majority of the aggregate number of Warrant Shares issuable upon exercise of all outstanding Warrants issued pursuant to the Purchase Agreement. Any amendment or waiver effected in accordance with this Section shall be binding upon the Company, the Holder and the holders of all Warrants issued pursuant to the Purchase Agreement.

9. Notices. Except as may be otherwise provided herein, all notices, requests, waivers and other communications made pursuant to this Warrant shall be made in accordance with Section 5.6 of the Purchase Agreement.

10. Governing Law. This Warrant is to be construed in accordance with and governed by the laws of the State of California.

IN WITNESS WHEREOF, the Company has caused this Warrant to be duly executed by its officers, thereunto duly authorized as of the date first above written.

ULTRAGENYX PHARMACEUTICAL INC.

By: /s/ John Ditton
John Ditton
Secretary

FORM OF SUBSCRIPTION

(To be signed only upon exercise of Warrant)

To: _____

The undersigned, the holder of a right to purchase shares of Series A Preferred Stock of Ultragenyx Pharmaceutical Inc., a California corporation (the "Company"); pursuant to that certain Warrant to Purchase Series A Preferred Stock of Ultragenyx Pharmaceutical Inc. (the "Warrant"), dated as of June 14, 2011, hereby irrevocably elects to exercise the purchase right represented by such Warrant for, and to purchase thereunder, _____ (_____) shares of Series A Preferred Stock of the Company and herewith makes payment of _____ Dollars ($_____) therefor by the following method:

(Check one of the following):

_____ (check if applicable) The undersigned hereby elects to make payment of _____ Dollars ($_____) therefor in cash.

_____ (check if applicable) The undersigned hereby elects to make payment for the aggregate exercise price of this exercise using the Net Issuance method pursuant to Section 2 of the Warrant.

The undersigned represents that it is acquiring such securities for its own account for investment and not with a view to or for sale in connection with any distribution thereof and in order to induce the issuance of such securities makes to the Company, as of the date hereof, the representations and warranties set forth in Section 3 of the Note and Warrant Purchase Agreement, dated as of June 14, 2011, by and among the Company and the investors listed on Exhibit A thereto.

DATED:

EMIL D. KAKKIS, M.D., Ph.D.

By: _____

ACKNOWLEDGMENT

To: Emil D. Kakkis, M.D., Ph.D.

The undersigned hereby acknowledges that as of the date hereof, _____ (_____) shares of Series A Preferred Stock remain subject to the right of purchase in favor of Emil D. Kakkis, M.D., Ph.D. pursuant to that certain Warrant to Purchase Series A Preferred Stock of Ultragenyx Pharmaceutical Inc., dated as of June 14, 2010.

DATED: _____

ULTRAGENYX PHARMACEUTICAL INC.

By: _____

Name: _____

Title: _____

Exhibit 4.8

Execution Copy

ULTRAGENYX PHARMACEUTICAL INC.

AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT

Dated as of December 18, 2012

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ULTRAGENYX PHARMACEUTICAL INC.

AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT

THIS AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT (this "Agreement") is made as of December 18, 2012 by and among Ultragenyx Pharmaceutical Inc., a Delaware corporation (the "Company") and the investors listed on Schedule A hereto (each an "Investor" and collectively the "Investors").

RECITALS

WHEREAS, certain of the Investors and the Company previously entered into that certain Investors' Rights Agreement dated June 16, 2011 (the "Prior Agreement"),

WHEREAS, certain of the Investors and the Company are parties to that certain Series B Preferred Stock Purchase Agreement dated as of even date herewith (the "Series B Purchase Agreement") relating to the issue and sale of shares of Series B Preferred Stock of the Company (the "Series B Preferred Stock").

WHEREAS, the obligations of the Company and the Investors under the Series B Purchase Agreement are conditioned, among other things, upon amending and restating the Prior Agreement by the execution and delivery of this Agreement by the Investors and the Company.

WHEREAS, the Prior Agreement may be amended by a written instrument executed by the Company and the holders of at least seventy-five percent (75%) of the then outstanding shares of Series A Preferred Stock.

WHEREAS, the Company and the holders of Series A Preferred Stock that are signatories hereto (constituting at least seventy-five percent (75%) of the outstanding shares of Series A Preferred Stock) desire to amend and restate the Prior Agreement in its entirety in order to induce certain of the Investors to purchase shares of Series B Preferred Stock.

NOW, THEREFORE, in consideration of the mutual premises and covenants set forth herein, the parties hereto agree as follows:

1. Registration Rights. The Company covenants and agrees as follows:

1.1 Definitions. For purposes of this Section 1:

(a) The term "1934 Act" means the Securities Exchange Act of 1934, as amended.

(b) The term "Act" means the Securities Act of 1933, as amended.

(c) The term "Affiliate" means, with respect to any specified person, any other person who or which, directly or indirectly, controls, is controlled by, or is under

common control with such specified person, including, without limitation, any partner, officer, director, member or manager of such person and any venture capital fund now or hereafter existing that is controlled by one or more general partners or managing members of, or is under common investment management with or shares a common investment adviser with, such person.

(d) The term "Certificate" shall mean the Company's Certificate of Incorporation, as amended and/or restated from time to time.

(e) The term "Free Writing Prospectus" means a free-writing prospectus, as defined in Rule 405.

(f) The term "Form S-3" means such form under the Act as in effect on the date hereof or any registration form under the Act subsequently adopted by the SEC that permits inclusion or incorporation of substantial information by reference to other documents filed by the Company with the SEC.

(g) The term "Holder" means any person owning or having the right to acquire Registrable Securities or any assignee thereof in accordance with Section 1.11 hereof.

(h) The term "Initial Offering" means the Company's first firm commitment underwritten public offering of its Common Stock under the Act.

(i) The term "Major Holder" shall mean: (i) any holder of at least 3,650,000 shares of Series A Preferred Stock, or (ii) any holder of at least 1,000,000 shares of Series B Preferred Stock (in each case, as adjusted for Recapitalizations as defined in the Company's Certificate of Incorporation). For purposes of this definition and determining the availability of any rights of a holder under this Agreement, all shares of Preferred Stock held by affiliated entities or persons or entities under common investment management or control shall be aggregated together; accordingly, if such threshold is met by such persons and entities in the aggregate, then each such person or entity shall be considered a "Major Holder" for all purposes of this Agreement. Notwithstanding the foregoing provisions of this Section 1.1(i), none of the following Investors shall be considered a "Major Holder" under this Agreement: Emil D. Kakkis, William Aliski, and the John and Cynthia Klock Trust.

(j) The term "person" means any individual, partnership, limited liability company, joint venture, corporation, association, trust or any other entity or organization.

(k) The term "Qualified Public Offering" shall mean the first sale by the Company of its Common Stock in a firm commitment underwritten public offering pursuant to a registration statement under the Act, which raises gross proceeds of at least $30,000,000 in the aggregate (before deduction of underwriting discounts and commissions).

(l) The terms "register," "registered," and "registration" refer to a registration effected by preparing and filing a registration statement or similar document in compliance with the Act, and the declaration or ordering of effectiveness of such registration statement or document.

(m) The term “Registrable Securities” means (i) the Common Stock issuable or issued upon conversion of the Series A Preferred Stock of the Company (the “Series A Preferred Stock”) and the Series B Preferred Stock (together, the “Preferred Stock”) and (ii) any Common Stock of the Company issued as (or issuable upon the conversion or exercise of any warrant, right or other security that is issued as) a dividend or other distribution with respect to, or in exchange for, or in replacement of, the shares referenced in (i) above, excluding in all cases, however, any Registrable Securities sold by a person in a transaction in which his rights under this Section 1 are not assigned.

(n) The number of shares of “Registrable Securities” outstanding shall be determined by the number of shares of Common Stock outstanding that are, and the number of shares of Common Stock issuable pursuant to then exercisable or convertible securities that are, Registrable Securities.

(o) The term “Rule 144” shall mean Rule 144 under the Act.

(p) The term “Rule 145” shall mean Rule 145 under the Act.

(q) The term “SEC” shall mean the Securities and Exchange Commission.

(r) The term “Significant Holders” shall mean the Major Holders and the Emil Kakkis and Jenny Soriano Living Trust, Dated June 18, 2009 and its assigns, collectively.

1.2 Request for Registration.

(a) Subject to the conditions of this Section 1.2, if the Company shall receive at any time after the earlier of: (i) three (3) years after the date of this Agreement or (ii) six months after the effective date of the Initial Offering, a written request from Investors holding at least fifty percent (50%) of the Registrable Securities outstanding or such lesser percentage if the anticipated aggregate offering price is not less than $10,000,000 (for purposes of this Section 1.2, the “Initiating Holders”) that the Company file a registration statement under the Act covering the registration of Registrable Securities, then the Company shall, within twenty (20) days of the receipt thereof, give written notice of such request to all Holders, and subject to the limitations of this Section 1.2, use best efforts to, as soon as practicable, file a registration statement under the Act with respect to all of the Registrable Securities that the Holders request to be registered in a written request received by the Company within twenty (20) days of the mailing of the Company’s notice pursuant to this Section 1.2(a), and use best efforts to cause such registration statement to be declared effective by the SEC as soon as practicable.

(b) If the Initiating Holders intend to distribute the Registrable Securities covered by their request by means of an underwriting, they shall so advise the Company as a part of their request made pursuant to this Section 1.2 and the Company shall include such information in the written notice referred to in Section 1.2(a). In such event the right of any Holder to include its Registrable Securities in such registration shall be conditioned

upon such Holder's participation in such underwriting and the inclusion of such Holder's Registrable Securities in the underwriting (unless otherwise mutually agreed by a majority in interest of the Initiating Holders and such Holder) to the extent provided herein. All Holders proposing to distribute their securities through such underwriting shall enter into an underwriting agreement in customary form with the underwriter or underwriters selected for such underwriting by a majority in interest of the Initiating Holders (which underwriter or underwriters shall be reasonably acceptable to the Company). Notwithstanding any other provision of this Section 1.2, if the underwriter advises the Company that marketing factors require a limitation on the number of securities underwritten (including Registrable Securities), then the Company shall so advise all Holders of Registrable Securities that would otherwise be underwritten pursuant hereto, and the number of shares that may be included in the underwriting shall be allocated to the Holders of such Registrable Securities pro rata based on the number of Registrable Securities held by all such Holders (including the Initiating Holders). In no event shall any Registrable Securities be excluded from such underwriting unless all other securities are first excluded. Any Registrable Securities excluded or withdrawn from such underwriting shall be withdrawn from the registration.

(c) Notwithstanding the foregoing, the Company shall not be required to effect a registration pursuant to this Section 1.2:

(i) in any particular jurisdiction in which the Company would be required to execute a general consent to service of process in effecting such registration, unless the Company is already subject to service in such jurisdiction and except as may be required under the Act; or

(ii) after the Company has undertaken two (2) registrations pursuant to this Section 1.2 at the request of the Initiating Holders, and such registrations have either been: (a) declared or ordered effective with respect to all of the Registrable Securities requested by the Holders to be included in such registration, or (b) withdrawn after filing by the Company at the request of the Initiating Holders (other than as a result of a material adverse change to the Company) (a "Qualifying Registration"); or

(iii) during the period starting with the date of the filing of and ending on the date one hundred eighty (180) days following the effective date of the Qualified Public Offering or during the period starting with the date of the filing of and ending on the date forty-five days (45) following the effective date of any other Company-initiated registration pursuant to Section 1.3 below; or

(iv) if the Initiating Holders propose to dispose of Registrable Securities that may be registered on Form S-3 pursuant to Section 1.4 hereof; or

(v) if the Company shall furnish to Investors requesting a registration statement pursuant to this Section 1.2 within thirty (30) days of such request a certificate signed by the Company's Chief Executive Officer or Chairman of the Board stating (A) that the Company intends to file a registration statement for its Initial Offering within sixty (60) days following the date of the initial request for registration made by the Initiating Holders pursuant to this Section 1.2 or (B) that in the good faith judgment of the Board of Directors of

the Company, it would be seriously detrimental to the Company and its stockholders for such registration statement to be effected at such time, in which event the Company shall have the right to defer such filing for a period of not more than sixty (60) days after receipt of the request of the Initiating Holders, provided that such right shall be exercised by the Company not more than once in any twelve (12)-month period and provided further that the Company shall not register any securities for the account of itself or any other stockholder during such sixty (60) day period (other than a registration relating solely to the sale of securities of participants in a Company stock plan, a registration relating to a corporate reorganization or transaction under Rule 145 of the Act, a registration on any form that does not include substantially the same information as would be required to be included in a registration statement covering the sale of the Registrable Securities or a registration in which the only Common Stock being registered is Common Stock issuable upon conversion of debt securities that are also being registered).

1.3 Company Registration.

(a) If (but without any obligation to do so) the Company proposes to register (including for this purpose a registration effected by the Company for stockholders other than the Holders that has been expressly approved by the Holders pursuant to Section 1.12) any of its stock or other securities under the Act in connection with the public offering of such securities (other than a registration relating solely to the sale of securities of participants in a Company stock plan, a registration relating to a corporate reorganization or transaction under Rule 145 of the Act, a registration on any form that does not include substantially the same information as would be required to be included in a registration statement covering the sale of the Registrable Securities, or a registration in which the only Common Stock being registered is Common Stock issuable upon conversion of debt securities that are also being registered), the Company shall, at such time, promptly give each Significant Holder written notice of such registration. Upon the written request of each Significant Holder given within twenty (20) days after mailing of such notice by the Company in accordance with Section 1.3(c), the Company shall, subject to the provisions of Section 1.3(c), use reasonable efforts to cause to be registered under the Act all of the Registrable Securities that each such Significant Holder requests to be registered.

(b) Right to Terminate Registration. The Company shall have the right to terminate or withdraw any registration initiated by it under this Section 1.3 prior to the effectiveness of such registration whether or not any Significant Holder has elected to include securities in such registration. The expenses of such withdrawn registration shall be borne by the Company in accordance with Section 1.7 hereof.

(c) Underwriting Requirements. In connection with any offering involving an underwriting of shares of the Company's capital stock, the Company shall not be required under this Section 1.3 to include any of the Significant Holders' securities in such underwriting unless they accept the terms of the underwriting as agreed upon between the Company and the underwriters selected by the Company (or by other persons entitled to select the underwriters) and enter into an underwriting agreement in customary form with such underwriters, and then only in such quantity as the underwriters determine in their sole discretion will not jeopardize the success of the offering by the Company. If the total amount of securities,

including Registrable Securities, requested by stockholders to be included in such offering exceeds the amount of securities sold other than by the Company that the underwriters determine in their sole discretion is compatible with the success of the offering, then the Company shall be required to include in the offering only that number of such securities, including Registrable Securities, that the underwriters determine in their sole discretion will not jeopardize the success of the offering. The Company shall not, without the prior written consent of the holders of at least a majority of the Registrable Securities then held by the Significant Holders exclude any Registrable Securities from such offering unless all other stockholders' securities have been first excluded. In the event that the underwriters determine that less than all of the Registrable Securities requested to be registered can be included in such offering, then the Registrable Securities that are included in such offering shall be apportioned first, to the Company; second, to the Significant Holders on a pro rata basis based on the total number of Registrable Securities held by such Investors; and third, to any stockholder of the Company (other than a Significant Holder) on a pro rata basis so long as the number of Registrable Securities held by the Significant Holders is not reduced. Notwithstanding the foregoing, in no event shall the amount of securities of the selling Significant Holders included in the offering be reduced below thirty percent (30%) of the total amount of securities included in such offering, unless such offering is the initial public offering of the Company's securities, in which case the selling Significant Holders may be excluded if the underwriters make the determination described above and no other stockholder's securities are included in such offering. For purposes of the preceding sentence concerning apportionment, for any selling stockholder that is a Significant Holder of Registrable Securities and that is a venture capital fund, partnership or corporation, the Affiliated venture capital funds, partners, retired partners and stockholders of such Significant Holder, or the estates and family members of any such partners and retired partners and any trusts for the benefit of any of the foregoing persons shall be deemed to be a single "selling Investor," and any pro rata reduction with respect to such "selling Significant Holder" shall be based upon the aggregate amount of Registrable Securities owned by all such related entities and individuals.

1.4 Form S-3 Registration. In case the Company shall receive from the Holders of at least ten percent (10%) of the Registrable Securities outstanding (for purposes of this Section 1.4, the "Initiating Holders") a written request or requests that the Company effect a registration on Form S-3 and any related qualification or compliance with respect to all or a part of the Registrable Securities owned by such Holder or Holders, the Company shall:

(a) promptly give written notice of the proposed registration, and any related qualification or compliance, to all other Holders; and

(b) use reasonable efforts to effect, as soon as practicable, such registration and all such qualifications and compliances as may be so requested and as would permit or facilitate the sale and distribution of all or such portion of such Holders' Registrable Securities as are specified in such request, together with all or such portion of the Registrable Securities of any other Holders joining in such request as are specified in a written request given within fifteen (15) days after receipt of such written notice from the Company, provided, however, that the Company shall not be obligated to effect any such registration, qualification or compliance, pursuant to this Section 1.4:

(i) if Form S-3 is not available for such offering by the Holders;

(ii) if the Holders, together with the holders of any other securities of the Company entitled to inclusion in such registration, propose to sell Registrable Securities and such other securities (if any) at an aggregate price to the public (net of any underwriters' discounts or commissions) of less than $1,000,000;

(iii) if the Company shall furnish to Holders requesting a registration statement pursuant to this Section 1.4 a certificate signed by the Company's Chief Executive Officer or Chairman of the Board stating that in the good faith judgment of the Board of Directors of the Company, it would be seriously detrimental to the Company and its stockholders for such registration statement to be effected at such time, in which event the Company shall have the right to defer such filing for a period of not more than sixty (60) days after receipt of the request of the Initiating Holders, provided that such right shall be exercised by the Company not more than once in any twelve (12)-month period and provided further that the Company shall not register any securities for the account of itself or any other stockholder during such sixty (60) day period (other than a registration relating solely to the sale of securities of participants in a Company stock plan, a registration relating to a corporate reorganization or transaction under Rule 145 of the Act, a registration on any form that does not include substantially the same information as would be required to be included in a registration statement covering the sale of the Registrable Securities or a registration in which the only Common Stock being registered is Common Stock issuable upon conversion of debt securities that are also being registered);

(iv) if the Company has, within the twelve (12) month period preceding the date of such request, already effected three (3) registrations on Form S-3 for the Holders pursuant to this Section 1.4; or

(v) in any particular jurisdiction in which the Company would be required to qualify to do business or to execute a general consent to service of process in effecting such registration, qualification or compliance.

(c) If the Initiating Holders intend to distribute the Registrable Securities covered by their request by means of an underwriting, they shall so advise the Company as a part of their request made pursuant to this Section 1.4 and the Company shall include such information in the written notice referred to in Section 1.4(a). The provisions of Section 1.2(b) shall be applicable to such request (with the substitution of Section 1.4 for references to Section 1.2).

(d) Subject to the foregoing, the Company shall use reasonable efforts to file a registration statement covering the Registrable Securities and other securities so requested to be registered as soon as practicable after receipt of the request or requests of the Initiating Holders. Registrations effected pursuant to this Section 1.4 shall not be counted as requests for registration effected pursuant to Sections 1.2.

1.5 Obligations of the Company. Whenever required under this Section 1 to effect the registration of any Registrable Securities, the Company shall, as expeditiously as reasonably possible:

(a) prepare and file with the SEC a registration statement with respect to such Registrable Securities and use reasonable efforts (and best efforts in the case of registrations under Section 1.2) to cause such registration statement to become effective, and, upon the request of the Holders of a majority of the Registrable Securities registered thereunder, keep such registration statement effective for a period of up to one (1) year or, if earlier, until the distribution contemplated in the registration statement has been completed; provided, however, that (i) such one (1) year period shall be extended for a period of time equal to the period the Holder refrains, at the request of the Company or an underwriter of securities of the Company, from selling any securities included in such registration, and (ii) in the case of any registration of Registrable Securities on Form S-3 that are intended to be offered on a continuous or delayed basis, subject to compliance with applicable SEC rules, such one (1) year period shall be extended for up to ninety (90) additional days, if necessary, to keep the registration statement effective until all such Registrable Securities are sold;

(b) prepare and file with the SEC such amendments and supplements to such registration statement and the prospectus used in connection with such registration statement as may be necessary to comply with the provisions of the Act with respect to the disposition of all securities covered by such registration statement;

(c) furnish to the Holders such number of copies of a prospectus, including a preliminary prospectus and any Free Writing Prospectus, in conformity with the requirements of the Act, and such other documents as they may reasonably request in order to facilitate the disposition of Registrable Securities owned by them;

(d) use reasonable efforts to register and qualify the securities covered by such registration statement under such other securities or Blue Sky laws of such jurisdictions as shall be reasonably requested by the Holders, provided that the Company shall not be required in connection therewith or as a condition thereto to qualify to do business or to file a general consent to service of process in any such states or jurisdictions;

(e) in the event of any underwritten public offering, enter into and perform its obligations under an underwriting agreement, in usual and customary form, with the managing underwriter of such offering;

(f) notify each Holder of Registrable Securities covered by such registration statement at any time when a prospectus or Free Writing Prospectus (to the extent prepared by or on behalf of the Company) relating thereto is required to be delivered under the Act of the happening of any event as a result of which the prospectus included in such registration statement, as then in effect, includes an untrue statement of a material fact or omits to state a material fact required to be stated therein or necessary to make the statements therein not misleading in the light of the circumstances then existing, and, at the request of any such Holder, the Company will, as soon as reasonably practicable, file and furnish to all such Holders

a supplement or amendment to such prospectus or Free Writing Prospectus (to the extent prepared by or on behalf of the Company) so that, as thereafter delivered to the purchasers of such Registrable Securities, such prospectus will not contain an untrue statement of a material fact or omit to state any fact necessary to make the statements therein not misleading in light of the circumstances under which they were made;

(g) cause all such Registrable Securities registered pursuant to this Section 1 to be listed on a national exchange or trading system and on each securities exchange and trading system on which similar securities issued by the Company are then listed;

(h) provide a transfer agent and registrar for all Registrable Securities registered pursuant to this Agreement and a CUSIP number for all such Registrable Securities, in each case not later than the effective date of such registration; and

(i) use reasonable efforts to furnish, at the request of any Holder requesting registration of Registrable Securities pursuant to this Section 1, on the date that such Registrable Securities are delivered to the underwriters for sale in connection with a registration pursuant to this Section 1, if such securities are being sold through underwriters, or, if such securities are not being sold through underwriters, on the date that the registration statement with respect to such securities becomes effective, (i) an opinion, dated as of such date, of the counsel representing the Company for the purposes of such registration, and (ii) a "comfort" letter, dated as of such date, from the independent certified public accountants of the Company, in each case in form and substance as is customarily given to underwriters in an underwritten public offering and reasonably satisfactory to a majority in interest of the Holders requesting registration, addressed to the underwriters and to the Holders requesting registration of Registrable Securities.

Notwithstanding the provisions of this Section 1, the Company shall be entitled to postpone or suspend, for a reasonable period of time but in no event more than 60 days in any twelve (12)-month period, the effectiveness or use of, or trading under, any registration statement if the Company shall determine that the sale of any securities pursuant to such registration statement would in the good faith unanimous judgment of the Board of Directors of the Company require disclosure of material nonpublic information that, if disclosed at such time, would cause a Violation (as defined below); provided, however, that during any such period all executive officers and directors of the Company are also prohibited from selling securities of the Company (or any security of any of the Company's subsidiaries or Affiliates).

In the event of the suspension of effectiveness of any registration statement pursuant to this Section 1.5, the applicable time period during which such registration statement is to remain effective shall be extended by that number of days equal to the number of days the effectiveness of such registration statement was suspended.

1.6 Information from Holder. It shall be a condition precedent to the obligations of the Company to take any action pursuant to this Section 1 with respect to the Registrable Securities of any selling Holder that such Holder shall furnish to the Company such information regarding itself, the Registrable Securities held by it, and the intended method of disposition of such securities as shall be reasonably required to effect the registration of such Holder's Registrable Securities.

1.7 Expenses of Registration. All expenses other than underwriting discounts and commissions incurred in connection with registrations, filings or qualifications pursuant to Sections 1.2, 1.3 and 1.4, including (without limitation) all registration, filing and qualification fees, printers' and accounting fees, fees and disbursements of counsel for the Company and the reasonable fees and disbursements not to exceed $20,000 of one counsel for the selling Holders shall be borne by the Company. Notwithstanding the foregoing, the Company shall not be required to pay for any expenses of any registration proceeding begun pursuant to Section 1.2 or Section 1.4 if the registration request is subsequently withdrawn at the request of the Holders of a majority of the Registrable Securities to be registered (in which case all participating Holders shall bear such expenses pro rata based upon the number of Registrable Securities that were to be included in the withdrawn registration), unless, in the case of a registration requested under Section 1.2 or Section 1.4, the Holders of at least a majority of the Registrable Securities then held by the Investors agree to forfeit their right to one demand registration pursuant to Section 1.2 or Section 1.4 and provided, however, that if at the time of such withdrawal, the Holders have learned of a material adverse change in the condition, business or prospects of the Company from that known to the Holders at the time of their request and have withdrawn the request with reasonable promptness following disclosure by the Company of such material adverse change, then the Holders shall not be required to pay any of such expenses and shall retain their rights pursuant to Section 1.2 and 1.4.

1.8 Delay of Registration. No Holder shall have any right to obtain or seek an injunction restraining or otherwise delaying any such registration as the result of any controversy that might arise with respect to the interpretation or implementation of this Section 1.

1.9 Indemnification. In the event any Registrable Securities are included in a registration statement under this Section 1:

(a) To the extent permitted by law, the Company will indemnify and hold harmless each Holder, the partners, officers, directors and stockholders of each Holder, legal counsel, accountants and investment advisors for each Holder, any underwriter (as defined in the Act) for such Holder and each person, if any, who controls such Holder or underwriter within the meaning of the Act or the 1934 Act, against any losses, claims, damages or liabilities (joint or several) to which they may become subject under the Act, the 1934 Act, any state securities laws or any rule or regulation promulgated under the Act, insofar as such losses, claims, damages, or liabilities (or actions in respect thereof) arise out of or are based upon any of the following statements, omissions or violations (collectively a "Violation"): (i) any untrue statement or alleged untrue statement of a material fact contained in such registration statement, including any preliminary prospectus, final prospectus or Free Writing Prospectus contained therein or any amendments or supplements thereto, any issuer information (as defined in Rule 433 of the Act) filed or required to be filed pursuant to Rule 433(d) under the Act or any other document incident to such registration prepared by or on behalf of the Company or used or

referred to by the Company, (ii) the omission or alleged omission to state in such registration statement, including any preliminary prospectus, final prospectus or Free Writing Prospectus contained therein or any amendments or supplements thereto, any issuer information (as defined in Rule 433 of the Act) filed or required to be filed pursuant to Rule 433(d) under the Act or any other document incident to such registration prepared by or on behalf of the Company or used or referred to by the Company, a material fact required to be stated therein, or necessary to make the statements therein not misleading or (iii) any violation or alleged violation by the Company of the Act, the 1934 Act, any state securities laws or any rule or regulation promulgated under the Act, the 1934 Act or any state securities laws, and the Company will reimburse each such Holder, underwriter, controlling person or other aforementioned person for any legal or other expenses reasonably incurred by them in connection with investigating or defending any such loss, claim, damage, liability or action as such expenses are incurred; provided, however, that the indemnity agreement contained in this subsection 1.9(a) shall not apply to amounts paid in settlement of any such loss, claim, damage, liability or action if such settlement is effected without the consent of the Company (which consent shall not be unreasonably withheld), nor shall the Company be liable in any such case with respect to a specific Holder for any such loss, claim, damage, liability or action to the extent that it arises out of or is based upon a Violation that occurs in reliance upon and in conformity with written information regarding such Holder that is furnished expressly for use in connection with such registration by any such Holder, underwriter, controlling person or other aforementioned person.

(b) To the extent permitted by law, each selling Holder, severally and not jointly, will indemnify and hold harmless the Company, each of its directors, each of its officers who has signed the registration statement, each person, if any, who controls the Company within the meaning of the Act, legal counsel and accountants for the Company, any underwriter, any other Holder selling securities in such registration statement and any controlling person of any such underwriter or other Holder, against any losses, claims, damages or liabilities (joint or several) to which any of the foregoing persons may become subject, under the Act, the 1934 Act, any state securities laws or any rule or regulation promulgated under the Act, the 1934 Act or any state securities laws, insofar as such losses, claims, damages or liabilities (or actions in respect thereto) arise out of or are based upon any Violation, in each case to the extent (and only to the extent) that such Violation occurs in reliance upon and in conformity with written information regarding such Holder that is furnished by such Holder expressly for use in connection with such registration; and each such Holder will reimburse any person intended to be indemnified pursuant to this subsection 1.9(b) for any legal or other expenses reasonably incurred by such person in connection with investigating or defending any such loss, claim, damage, liability or action as such expenses are incurred; provided, however, that the indemnity agreement contained in this subsection 1.9(b) shall not apply to amounts paid in settlement of any such loss, claim, damage, liability or action if such settlement is effected without the consent of the Holder (which consent shall not be unreasonably withheld), and provided that in no event shall any indemnity under this subsection 1.9(b), when aggregated with any contribution obligation under Section 1.9(d), exceed the net proceeds from the offering received by such Holder.

(c) Promptly after receipt by an indemnified party under this Section 1.9 of notice of the commencement of any action (including any governmental action) for which a party may be entitled to indemnification, such indemnified party will, if a claim in respect thereof is to be made against any indemnifying party under this Section 1.9, deliver to the indemnifying party a written notice of the commencement thereof and the indemnifying party shall have the right to participate in and, to the extent the indemnifying party so desires, jointly with any other indemnifying party similarly noticed, to assume the defense thereof with counsel mutually satisfactory to the parties; provided, however, that an indemnified party (together with all other indemnified parties that may be represented without conflict by one counsel) shall have the right to retain one separate counsel, with the fees and expenses to be paid by the indemnifying party, if representation of such indemnified party by the counsel retained by the indemnifying party would be inappropriate due to actual or potential differing interests between such indemnified party and any other party represented by such counsel in such proceeding. The failure to deliver written notice to the indemnifying party within a reasonable time of the commencement of any such action, if prejudicial to its ability to defend such action, shall relieve such indemnifying party of liability to the indemnified party under this Section 1.9 to the extent of such prejudice, but the omission to so deliver written notice to the indemnifying party will not relieve it of any liability that it may have to any indemnified party otherwise than under this Section 1.9.

(d) If the indemnification provided for in this Section 1.9 is held by a court of competent jurisdiction to be unavailable to an indemnified party with respect to any loss, liability, claim, damage or expense referred to herein, then the indemnifying party, in lieu of indemnifying such indemnified party hereunder, shall contribute to the amount paid or payable by such indemnified party as a result of such loss, liability, claim, damage or expense in such proportion as is appropriate to reflect the relative fault of the indemnifying party on the one hand and the indemnified party on the other hand in connection with the statements or omissions that resulted in such loss, liability, claim, damage or expense, as well as any other relevant equitable considerations; provided, however, that no contribution by any Holder, when combined with any amounts paid by such Holder pursuant to Section 1.9(b), shall exceed the net proceeds from the offering received by such Holder. The relative fault of the indemnifying party and the indemnified party shall be determined by reference to, among other things, whether the untrue or alleged untrue statement of a material fact or the omission or alleged omission to state a material fact relates to information supplied by the indemnifying party or by the indemnified party and the parties' relative intent, knowledge, access to information and opportunity to correct or prevent such statement or omission.

(e) Notwithstanding the foregoing, to the extent that the provisions on indemnification and contribution contained in the underwriting agreement entered into in connection with the underwritten public offering are in conflict with the foregoing provisions, the provisions in the underwriting agreement shall control.

(f) The obligations of the Company and Holders under this Section 1.9 shall survive the completion of any offering of Registrable Securities in a registration statement under this Section 1 and otherwise.

1.10 Reports Under the 1934 Act. With a view to making available to the Holders the benefits of Rule 144 and any other rule or regulation of the SEC that may at any time permit a Holder to sell securities of the Company to the public without registration or pursuant to a registration on Form S-3, the Company agrees to:

(a) make and keep public information available, as those terms are understood and defined in Rule 144, at all times after the effective date of the Initial Offering;

(b) file with the SEC in a timely manner all reports and other documents required of the Company under the Act and the 1934 Act; and

(c) furnish to any Holder, so long as the Holder owns any Registrable Securities, forthwith upon request (i) a written statement by the Company that it has complied with the reporting requirements of Rule 144 (at any time after ninety (90) days after the effective date of the first registration statement filed by the Company), the Act and the 1934 Act (at any time after it has become subject to such reporting requirements), or that it qualifies as a registrant whose securities may be resold pursuant to Form S-3 (at any time after it so qualifies), (ii) a copy of the most recent annual or quarterly report of the Company and such other reports and documents so filed by the Company, and (iii) such other information as may be reasonably requested to avail any Holder of any rule or regulation of the SEC that permits the selling of any such securities without registration or pursuant to such form.

1.11 Assignment of Registration Rights. The rights to cause the Company to register Registrable Securities pursuant to this Section 1 may be assigned (but only with all related obligations) by a Holder to a transferee or assignee of such securities that (i) is an Affiliate, subsidiary, parent, partner, limited partner, retired partner, retired stockholder, retired member, member or stockholder of a Holder, (ii) is a member or former member of any Holder that is a limited liability company, (iii) is a Holder's family member or trust for the benefit of an individual Holder, or (iv) after such assignment or transfer, holds at least 425,000 shares of Registrable Securities (subject to appropriate adjustment for Recapitalizations), provided: (a) the Company is, within a reasonable time after such transfer, furnished with written notice of the name and address of such transferee or assignee and the securities with respect to which such registration rights are being assigned; and (b) such transferee or assignee agrees in writing to be bound by and subject to the terms and conditions of this Agreement, including, without limitation, the provisions of Section 2 below.

1.12 Limitations on Subsequent Registration Rights. From and after the date of this Agreement, the Company shall not, without the prior written consent of the Investors holding at least seventy-five percent (75%) of the then outstanding Preferred Stock (determined on an as-converted basis) or, following conversion of all Preferred Stock, seventy-five percent (75%) of Registrable Securities, enter into any agreement with any holder or prospective holder of any securities of the Company that would allow such holder or prospective holder (a) to include any of such securities in any registration filed under Section 1.2, 1.3 or 1.4 hereof, unless under the terms of such agreement, such holder or prospective holder may include such securities in any such registration only to the extent that the inclusion of such securities will not reduce the amount of the Registrable Securities of the Holders that are included, (b) to demand registration of their securities or (c) to exercise other registration rights that are pari passu or senior to those granted to the Holders hereunder.

1.13 Termination of Registration Rights. No Holder shall be entitled to exercise any right provided for in this Section 1, other than a Holder's right to seek indemnification from the Company pursuant to Section 1.9, which shall continue until the expiration of the statute of limitations applicable thereto, (a) after five (5) years following the consummation of the Qualified Public Offering, (b) as to any Holder, such earlier time after the Qualified Public Offering at which such Holder holds one percent (1%) or less of the Company's outstanding Common Stock and all Registrable Securities held by such Holder (together with any Affiliate of the Holder with whom such Holder must aggregate its sales under Rule 144) can be sold in any ninety (90) day period without registration in compliance with Rule 144 and without the requirement that the Company be in compliance with the current public information required under Rule 144(c)(1) or (c) after the consummation of a Liquidation Event, as that term is defined in the Certificate.

1.14 "Market Stand-Off" Agreement.

(a) Each Investor hereby agrees that it will not, directly or indirectly, without the prior written consent of the Company and the managing underwriter, during the period commencing on the date of the final prospectus relating to the Initial Offering and ending on the date specified by the Company and the managing underwriter (such period not to exceed one hundred eighty (180) days) (i) lend, offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of Common Stock or any securities convertible into or exercisable or exchangeable for Common Stock (whether such shares or any such securities are then owned by the Investor or are thereafter acquired), or (ii) enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of the Common Stock, whether any such transaction described in clause (i) or (ii) above is to be settled by delivery of Common Stock or such other securities, in cash or otherwise. The foregoing provisions of this Section 1.14 shall not apply to the sale of any shares to an underwriter pursuant to an underwriting agreement, and shall only be applicable to the Investors if all officers and directors and greater than one percent (1%) stockholders of the Company enter into similar agreements. Any discretionary waiver or termination of the restrictions of any or all of such agreements by the Company or the underwriters shall apply pro rata to all Holders subject to such agreements, based on the number of shares subject to such agreements. The underwriters in connection with the Initial Offering are intended third party beneficiaries of this Section 1.14 and shall have the right, power and authority to enforce the provisions hereof as though they were a party hereto; further, each Investor hereby agrees to enter into a written agreement with such underwriters containing terms substantially equivalent to the terms of this Section 1.14, and each Investor hereby agrees that such underwriters shall be entitled to require each such Investor to enter into such a written agreement. Notwithstanding the foregoing, nothing in this Section 1.14 shall prevent an Investor from making a transfer of any Common Stock that was listed on a national stock exchange, actively traded over-the-counter or traded on the NASDAQ Global Market at the time it was acquired by the Investor or was acquired by such Investor pursuant to Rule 144A of the Act, including any shares acquired in the Initial Offering.

In order to enforce the foregoing covenant, the Company may impose stop-transfer instructions with respect to the Preferred Stock held by each Investor (and the shares or securities of every other person subject to the foregoing restriction) until the end of such period.

(b) Each Holder agrees that a legend reading substantially as follows shall be placed on all certificates representing all Registrable Securities of each Holder (and the shares or securities of every other person subject to the restriction contained in this Section 1.14):

THE SECURITIES REPRESENTED BY THIS CERTIFICATE ARE SUBJECT TO A LOCK-UP PERIOD OF UP TO 180 DAYS AFTER THE EFFECTIVE DATE OF THE ISSUER'S REGISTRATION STATEMENT FILED UNDER THE ACT, AS AMENDED, AS SET FORTH IN AN AGREEMENT BETWEEN THE COMPANY AND THE ORIGINAL HOLDER OF THESE SECURITIES, A COPY OF WHICH MAY BE OBTAINED AT THE ISSUER'S PRINCIPAL OFFICE. SUCH LOCK-UP PERIOD IS BINDING ON TRANSFEREES OF THESE SHARES.

2. Covenants of the Company.

2.1 Delivery of Financial Statements. The Company shall deliver to each Investor:

(a) as soon as practicable after the end of each fiscal year of the Company, and in any event within one hundred eighty (180) days thereafter or such longer period as approved by the Company's Board of Directors, (i) an income statement for such fiscal year, a balance sheet of the Company and statement of stockholder's equity as of the end of such year, and a statement of cash flows for such year, such year-end financial reports to be audited and accompanied by a report and opinion thereon by independent public accountants of national standing selected by the Company's Board of Directors, in reasonable detail, prepared in accordance with generally accepted accounting principles consistently applied ("GAAP") and setting forth in each case in comparative form the figures for the previous fiscal year;

(b) as soon as practicable after the end of each fiscal year of the Company, and in any event within one hundred twenty (120) days thereafter, (i) an unaudited income statement for such fiscal year, an unaudited balance sheet of the Company and an unaudited statement of stockholder's equity as of the end of such year, and an unaudited statement of cash flows for such year;

(c) no later than thirty (30) days after the start of each fiscal year an annual budget and operating plans for such fiscal year (and as soon as available, any subsequent written revisions thereto);

(d) as soon as practicable after the end of each of the first three (3) quarters of each fiscal year of the Company, and in any event within forty-five (45) days after the end of each such fiscal quarter, an unaudited income statement, statement of cash flows for such fiscal quarter and an unaudited balance sheet as of the fiscal quarter, and for the current fiscal year to date, prepared in accordance with generally accepted accounting principles consistently applied with the exception that no notes need be attached to such statements and year-end audit adjustments may not have been made; and

(e) concurrently with the delivery of any of the above, a report including an up-to-date capitalization table and a comparison of the Company's results for the applicable period to the Company's then-current annual budget.

2.2 Inspection. The Company shall permit each Investor at such Investor's expense, and upon reasonable notice, during normal business hours, to visit and inspect the Company's properties, to examine its books of account and records and to discuss the Company's affairs, finances and accounts with its officers, all at such reasonable times as may be reasonably requested by the Investor; provided, however, that the Company shall not be obligated pursuant to this Section 2.2 to provide access to any information that it reasonably and in good faith considers to be a trade secret or similar confidential information, and provided further that the Company may require the Investor to execute a confidentiality and nondisclosure agreement with restrictions that are reasonable under the circumstances prior to any such visit and inspection.

2.3 Right of First Offer. Subject to the terms and conditions specified in this Section 2.3, the Company hereby grants to each Significant Holder a right of first offer with respect to future sales by the Company of its Shares (as hereinafter defined). For purposes of this Section 2.3, "Significant Holder" includes any general partners and Affiliates of a Significant Holder. A Significant Holder shall be entitled to apportion the right of first offer hereby granted it among itself and its partners and affiliates in such proportions as it deems appropriate, so long as such apportionment does not cause the loss of the exemption under Section 4(a)(2) of the Act or any similar exemption under applicable state securities laws in connection with such sale of Shares by the Company.

Each time the Company proposes to offer any shares of, or securities convertible into or exchangeable or exercisable for any shares of, any class of its capital stock (the "Shares"), the Company shall first make an offering of such Shares to each Significant Holder in accordance with the following provisions:

(a) The Company shall deliver a notice in accordance with Section 3.7 (the "Notice") to the Significant Holders stating (i) its bona fide intention to offer such Shares, (ii) the number of such Shares to be offered, and (iii) the price, terms and conditions upon which it proposes to offer such Shares.

(b) By written notification received by the Company, within twenty (20) calendar days after receipt of the Notice, the Significant Holder may elect to purchase or obtain, at the price and on the terms specified in the Notice, up to that portion of

such Shares that equals the proportion that the number of shares of Common Stock issued and held, or issuable upon conversion of the Preferred Stock then held, by such Significant Holder bears to the total number of shares of Common Stock of the Company then outstanding (assuming full conversion and exercise of all outstanding convertible and exercisable securities). The Company shall promptly, in writing, inform each Significant Holder which purchases all the shares available to it ("Fully-Exercising Holder") of any other Significant Holder's failure to do likewise. During the ten (10) day period commencing after receipt of such information, each Fully-Exercising Holder shall be entitled to obtain that portion of the Shares for which Significant Holders were entitled to subscribe but which were not subscribed for by the Significant Holders that is equal to the proportion that the number of shares of Common Stock issued and held, or issuable upon conversion of Preferred Stock then held, by such Fully-Exercising Holder bears to the total number of shares of Common Stock issued and held, or issuable upon conversion of Preferred Stock then held, by all Fully-Exercising Holders who wish to purchase some of the unsubscribed shares.

(c) If all Shares that Significant Holders are entitled to obtain pursuant to Section 2.3(b) are not elected to be obtained as provided in Section 2.3(b) hereof, the Company may, during the ninety (90) day period following the expiration of the period provided in Section 2.3(b) hereof, offer the remaining unsubscribed portion of such Shares to any person or persons at a price not less than, and upon terms no more favorable to the offeree than those specified in the Notice. If the Company does not enter into an agreement for the sale of the Shares within such period, or if such agreement is not consummated within thirty (30) days of the execution thereof, the right provided hereunder shall be deemed to be revived and such Shares shall not be offered unless first reoffered to the Significant Holders in accordance herewith.

(d) The right of first offer in this Section 2.3 shall not be applicable to any of the issuances exempted from the definition of "Additional Stock" in Article IV.B.4.(d)(ii) of the Certificate of Incorporation. In addition to the foregoing, the right of first offer in this Section 2.3 shall not be applicable with respect to any Investor and any subsequent securities issuance, if (i) at the time of such subsequent securities issuance, the Investor is not an "accredited investor," as that term is then defined in Rule 501(a) under the Act, and (ii) such subsequent securities issuance is otherwise being offered only to accredited investors.

(e) The right of first offer set forth in this Section 2.3 may not be assigned or transferred, except that (i) such right is assignable by each Investor to any partner or Affiliate or wholly-owned subsidiary or parent of any such Investor and (ii) such right is assignable to a transferee or assignee who holds after such transfer at least 425,000 shares of the Preferred Stock (subject to appropriate adjustment for Recapitalizations as defined in the Certificate).

2.4 Proprietary Information and Inventions Agreements. The Company shall require all of its employees and consultants to enter into the Company's standard form of proprietary information and inventions agreement, in the form provided to the Investors prior to the date of this Agreement, subject to material change by the Company only with the approval of the Company's Board of Directors.

2.5 Key Man and D/O Insurance. The Company shall purchase and at all times maintain a key-man life insurance policy on Emil Kakkis from financially sound and reputable insurers in the amount of $5,000,000 payable to the Company, or such other amount as elected by the Board of Directors. The Company shall also purchase and at all times maintain from financially sound and reputable insurers Directors and Officers liability insurance in an amount and on terms and conditions satisfactory to the Board of Directors and the Investors, with coverage retroactive to the date hereof and with tail coverage for up to at least six years, and will use commercially reasonable efforts to cause such insurance policies to be maintained until such time as the Board of Directors determines that such insurance should be discontinued. Neither policy contemplated by this Section 2.5 shall be cancelable by the Company without prior approval by the Board of Directors.

2.6 Board Observer Rights. So long as TPG Biotechnology Partners III, L.P. and its Affiliates (collectively, "TPG") continue to hold at least one million (1,000,000) shares of Registrable Securities (as adjusted for Recapitalizations), the Company shall allow TPG to designate one (1) observer (the "TPG Observer") to attend all regular meetings of the Company's Board of Directors in a nonvoting capacity. So long as A.M. Pappas Life Science Ventures IV, L.P. and its Affiliates (collectively, "Pappas") continue to hold at least one million (1,000,000) shares of Registrable Securities (as adjusted for Recapitalizations), the Company shall allow Pappas to designate one (1) observer (the "Pappas Observer") to attend all regular meetings of the Company's Board of Directors in a nonvoting capacity. So long as Emil Kakkis continues to hold at least a majority of the outstanding shares of Common Stock of the Company, the Company shall allow Dr. Kakkis to designate one (1) observer (the "Common Observer") to attend all regular meetings of the Company's Board of Directors in a nonvoting capacity. So long as Adage Capital Partners, L.P. and its Affiliates (collectively, "Adage") continue to hold at least one million (1,000,000) shares of Registrable Securities (as adjusted for Recapitalizations), the Company shall allow Adage to designate one (1) observer (the "Adage Observer") to attend all regular meetings of the Company's Board of Directors in a nonvoting capacity. The Adage Observer shall initially be Steven J. Klaus. Any substitute or replacement Adage Observer shall be subject to the prior approval of the Company's Board of Directors, which approval shall not be unreasonably delayed, conditioned or denied; provided that the appointment of Phill Gross, Alan Sebulsky or Rick Solit as a substitute or replacement Adage Observer shall be deemed approved without any further action. In connection with the observer rights set forth in this Section 2.6, the Company shall give the TPG Observer, the Pappas Observer, the Common Observer and the Adage Observer copies of all notices, minutes, consents and other materials, financial or otherwise, which the Company provides to its Board of Directors; provided, however, that the TPG Observer, the Pappas Observer, the Common Observer and the Adage Observer shall agree to hold in confidence and trust and to act in a fiduciary manner with respect to all information so provided; provided, further, that the Company reserves the right to exclude the TPG Observer, the Pappas Observer, the Common Observer and the Adage Observer from access to any material or meeting or portion thereof if the Company reasonably believes based on advice of counsel (i) such attendance would create a conflict of interest; or (ii) such exclusion is reasonably necessary to preserve attorney-client privilege. The Company shall reimburse TPG, Pappas and Adage for all reasonable out-of-pocket expenses incurred by their respective observers for travel to and attendance at board meetings.

2.7 Stock Vesting. Unless otherwise approved by the Company's Board of Directors, all stock and stock equivalents issued to employees and other service providers of the Company during the term of this Agreement shall be subject to vesting as follows: twenty-five percent (25%) of such shares shall vest at the end of the first year following the issuance thereof, and the remaining seventy-five percent (75%) shall vest monthly over the following three years. If employees are permitted to exercise unvested options, the Company's repurchase option shall provide that, upon termination of the optionee's service to the Company, with or without cause, the Company or its assignee (to the extent permissible under an applicable securities law qualification) may repurchase the unvested shares subject to such option at the lesser of cost or fair market value as determined by the Company's Board of Directors.

2.8 Board Meetings, Board Actions and Committee Rights. Unless otherwise determined by the vote of a majority of the directors then in office, the Board of Directors shall meet at least every other month in accordance with an agreed-upon schedule. The Company shall reimburse the nonemployee directors for all reasonable out-of-pocket travel expenses incurred in connection with attending meetings of the Board of Directors. So long as TPG continues to hold one million (1,000,000) shares of Registrable Securities (as adjusted for any Recapitalizations), the Company shall allow TPG to designate one (1) member of each committee of the Company's Board of Directors. The Company hereby covenants and agrees with each of the Investors that, in addition to any other restrictions set forth in the Certificate, it shall not, without approval of the Board of Directors, enter into, or agree to enter into, or perform any of the transactions or take any of the actions contemplated by Article IV.B.6 of the Certificate.

2.9 Successor Indemnification. If the Company or any of its successors or assignees consolidates with or merges into any other entity and is not the continuing or surviving corporation or entity of such consolidation or merger, then to the extent necessary, proper provision shall be made so that the successors and assignees of the Company assume the obligations of the Company with respect to indemnification of members of the Board of Directors as in effect immediately before such transaction, whether such obligations are contained in the Company's Bylaws, its Certificate of Incorporation, or elsewhere, as the case may be.

2.10 Reservation of Stock. The Company will at all times reserve and keep available, solely for issuance and delivery upon the conversion of the Preferred Stock, all Common Stock issuable from time to time upon such conversion.

2.11 Termination of Certain Covenants. The covenants set forth in this Section 2 shall terminate and be of no further force or effect upon the earlier of the consummation of the Initial Offering or at such time as the Company is required to file reports pursuant to Section 13 or 15(d) of the 1934 Act (the "Termination Date"). This Agreement, except Section 2.9, shall terminate and be of no further force or effect upon the consummation of a Liquidation Event (as such term is defined in the Certificate, which Certificate may be amended from time to time).

3. Miscellaneous.

3.1 Limitations on Disposition.

Each Investor agrees not to make any disposition of all or any portion of such Investor's Registrable Securities unless and until the transferee has agreed in writing for the benefit of the Company to be bound by this Agreement, and:

(a) There is then in effect a registration statement under the Act covering such proposed disposition and such disposition is made in accordance with such registration statement; or

(b) (i) The Investor shall have notified the Company of the proposed disposition and shall have furnished the Company with a detailed statement of the circumstances surrounding the proposed disposition, and (ii) if reasonably requested by the Company, the Investor shall have furnished the Company with an opinion of counsel reasonably satisfactory to the Company that such disposition will not require registration of such shares under the Act. It is agreed that the Company will not require opinions of counsel for transactions made pursuant to Rule 144.

(c) Notwithstanding the provisions of paragraphs (a) and (b) above, no such registration statement or opinion of counsel shall be necessary for a transfer (i) by an Investor to an Affiliate of such Investor or (ii) by the Investor that is a partnership, corporation or limited liability company to a partner of such partnership, or a stockholder of such corporation or a member of such limited liability company or a retired partner of such partnership who retires after the date hereof or a retired stockholder of such corporation who retires after the date hereof or a retired member of such limited liability company who retires after the date hereof, or to the estate of any such partner, retired partner, member or retired member or the transfer by gift, will or intestate succession by any partner or member to his or her spouse or to the siblings, lineal descendants or ancestors of such partner or member or his or her spouse, provided that, in each case, the transferee agrees in writing to be subject to the terms hereof to the same extent as if he or she were an original Investor hereunder.

3.2 Successors and Assigns. Except as otherwise provided herein, the terms and conditions of this Agreement shall inure to the benefit of and be binding upon the respective successors and assigns of the parties (including transferees of any shares of Preferred Stock). Nothing in this Agreement, express or implied, is intended to confer upon any party other than the parties hereto or their respective successors and assigns any rights, remedies, obligations, or liabilities under or by reason of this Agreement, except as expressly provided in this Agreement.

3.3 Governing Law; Venue. This Agreement is to be construed in accordance with and governed by the internal laws of the State of Delaware without giving effect to any choice of law rule that would cause the application of the laws of any jurisdiction other than the internal laws of the State of Delaware to the rights and duties of the parties. All disputes and controversies arising out of or in connection with this Agreement shall be resolved exclusively by the state and federal courts located in New York County in the State of New York, and each party hereto agrees to submit to the jurisdiction of said courts and agrees that venue shall lie exclusively with such courts.

3.4 Specific Enforcement. Each party hereto agrees that its obligations hereunder are necessary and reasonable in order to protect the other parties to this Agreement, and each party expressly agrees and understands that monetary damages would inadequately compensate an injured party for the breach of this Agreement by any party, that this Agreement shall be specifically enforceable, and that, in addition to any other remedies that may be available at law, in equity or otherwise, any breach or threatened breach of this Agreement shall be the proper subject of a temporary or permanent injunction or restraining order, without the necessity of proving actual damages. Further, each party hereto waives any claim or defense that there is an adequate remedy at law for such breach or threatened breach.

3.5 Counterparts. This Agreement may be executed in two or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument. Counterparts may be delivered via facsimile, electronic mail (including by pdf attachment) or other transmission method and any counterpart so delivered shall be deemed to have been duly and validly delivered and be valid and effective for all purposes.

3.6 Titles and Subtitles. The titles and subtitles used in this Agreement are used for convenience only and are not to be considered in construing or interpreting this Agreement.

3.7 Notices; Confidentiality.

(a) Except as may be otherwise provided herein, all notices, requests, waivers and other communications made pursuant to this Agreement shall be in writing and shall be conclusively deemed to have been duly given (a) when hand delivered to the other party; (b) when sent by electronic mail or by facsimile to the email address or to the number set forth below if sent between 8:00 a.m. and 5:00 p.m. recipient's local time on a business day, or on the next business day if sent by electronic mail or by facsimile to the email address or to the number set forth below if sent other than between 8:00 a.m. and 5:00 p.m. recipient's local time on a business day; (c) three business days after deposit in the U.S. mail with first class or certified mail receipt requested postage prepaid and addressed to the other party at the address set forth below; or (d) the next business day after deposit with a national overnight delivery service, postage prepaid, addressed to the parties as set forth below with next business day delivery

guaranteed, provided that the sending party receives a confirmation of delivery from the delivery service provider. A party may change or supplement the addresses given above, or designate additional addresses, for purposes of this Section 4.7 by giving the other party written notice of the new address in the manner set forth above.

(b) The Investors acknowledge that the notices provided from time to time by the Company pursuant to Section 1 of this Agreement may contain material non-public information under the 1934 Act. Accordingly, from and after the Termination Date, the Investors agree to hold in confidence, for a period not to exceed sixty (60) days, any information contained in notices delivered to the Investors pursuant to Section 1 and designated by the Company as material non-public information until such information has been publicly disclosed by the Company in a press release or SEC filing; provided, however, that the Investors may disclose such confidential information to (i) their attorneys, accountants, consultants and other professionals to the extent necessary to obtain their services in connection with monitoring the investment in the Company, (ii) their officers, directors, managers, members, limited partners, and investors who are bound by a duty of confidentiality, and (iii) to the extent required by court order or pursuant to applicable law, regulation or a regulatory organization's rules. Not later than the sixtieth (60th) day following any disclosure of material non-public information by the Company in a notice delivered to an Investor pursuant to Section 1, the Company shall publicly disclose such information in a manner compliant with Regulation FD promulgated under the 1934 Act, as amended from time to time.

(c) Notices and communications to an Investor hereunder shall be sent solely to the person or department set forth on such Investor's signature page hereto and the Company shall not send notices or communications to any other person on behalf of such Investor without the prior written consent of such person or member of such department.

3.8 Expenses. If any action at law or in equity is necessary to enforce or interpret the terms of this Agreement, the prevailing party shall be entitled to reasonable attorney's fees, costs and necessary disbursements in addition to any other relief to which such party may be entitled.

3.9 Amendments and Waivers. Any term of this Agreement may be amended and the observance of any term of this Agreement may be waived (either generally or in a particular instance and either retroactively or prospectively), only with the written consent of the Company and the holders of at least seventy-five percent (75%) of the Preferred Stock then outstanding, voting together as a single class on an as-converted basis; *provided*, *however*, that if any amendment or waiver operates in a manner that treats any Investor different from other Investors, the consent of such Investor shall also be required for such amendment or waiver; *provided*, *further* that, Section 2.6 may not be amended with respect to TPG's right to appoint the TPG Observer and to designate one member of each committee of the Company's Board of Directors, respectively, without the consent of TPG, Section 2.6 may not be amended with respect to Pappas' right to appoint the Pappas Observer without the consent of Pappas and Section 2.6 may not be amended with respect to Dr. Kakkis's right to appoint the Common Observer without the consent of Dr. Kakkis; *provided*, *further* that Section 1.1(i) may not be amended to alter the rights of a Major Holder in a manner that is adverse to such Major Holder

and which differs from the treatment accorded the other Major Holders without the consent of such Major Holder; and provided, further that Section 2.8 may not be amended without the consent of TPG. Any amendment or waiver effected in accordance with this paragraph shall be binding upon each holder of any Registrable Securities, each future holder of all such Registrable Securities and the Company.

3.10 Severability. If one or more provisions of this Agreement are held to be unenforceable under applicable law, such provision shall be excluded from this Agreement and the balance of the Agreement shall be interpreted as if such provision were so excluded and shall be enforceable in accordance with its terms.

3.11 Aggregation of Stock. All shares of Registrable Securities held or acquired by entities advised by the same investment adviser and affiliated entities or persons shall be aggregated together for the purpose of determining the availability of any rights under this Agreement.

3.12 Entire Agreement. This Agreement and the documents referred to herein constitute the entire agreement among the parties with respect to the subject matter hereof and no party shall be liable or bound to any other party in any manner by any warranties, representations or covenants except as specifically set forth herein or therein.

3.13 Amendment of Prior Agreement and Waiver of Right of First Offer. Effective and contingent upon execution of this Agreement by (i) the Company and (ii) the Investors (as defined, for purposes of this Section 3.13, in the Prior Agreement) holding shares representing 75% of the Company's Series A Preferred Stock, and upon the closing of the transactions contemplated by the Series B Purchase Agreement, the Prior Agreement is hereby amended and restated in its entirety to read as set forth in this Agreement, and the Company and the Investors hereby agree to be bound by the provisions hereof as the sole agreement of the Company and the Investors with respect to the rights set forth herein. The Significant Holders (as defined in the Prior Agreement) hereby waive any rights of first offer (including any related notice rights) set forth in Section 2.3 of the Prior Agreement with respect to the issuance of the Series B Preferred Stock pursuant to the Series B Purchase Agreement.

* * *

IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the date first above written.

COMPANY:

ULTRAGENYX PHARMACEUTICAL INC.

By: /s/ Shalini Sharp
Name: Shalini Sharp
Title: Chief Financial Officer

SIGNATURE PAGE TO AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT

IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the date first above written.

INVESTOR:

ADAGE CAPITAL PARTNERS, L.P.

BY: /s/ Phillip T. Gross
NAME: Philip T. Gross
TITLE: Managing Member

ADDRESS:
TELEPHONE:
FACSIMILE:
E-MAIL:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR:

SMALLCAP WORLD FUND, INC.

BY: CAPITAL RESEARCH AND MANAGEMENT COMPANY, ITS INVESTMENT ADVISER

BY: /s/ Michael J. Downer
NAME: Michael J. Downer
TITLE: Senior Vice President and Secretary

ADDRESS:
TELEPHONE:
FACSIMILE:
E-MAIL:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR:

AMERICAN FUNDS INSURANCE SERIES – GLOBAL SMALL CAPITALIZATION FUND

BY: CAPITAL RESEARCH AND MANAGEMENT COMPANY, ITS INVESTMENT ADVISER

BY: /s/ Michael J. Downer
NAME: Michael J. Downer
TITLE: Senior Vice President and Secretary

ADDRESS:
TELEPHONE:
FACSIMILE:
E-MAIL:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR:

PRUDENTIAL SECTOR FUNDS, INC., PRUDENTIAL HEALTH SCIENCES FUND D/B/A PRUDENTIAL JENNISON HEALTH SCIENCES FUND

BY: JENNISON ASSOCIATES LLC, AS INVESTMENT SUB-ADVISER TO THE FUND

BY: /s/ David Chan
NAME: DAVID CHAN
TITLE: MANAGING DIRECTOR

ADDRESS:
TELEPHONE:
FACSIMILE:
E-MAIL:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR:

JENNISON GLOBAL HEALTHCARE MASTER FUND, LTD.

BY: JENNISON ASSOCIATES LLC, AS INVESTMENT ADVISER TO THE FUND

BY: /s/ David Chan
NAME: DAVID CHAN
TITLE: MANAGING DIRECTOR

ADDRESS:
TELEPHONE:
FACSIMILE:
E-MAIL:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR:

NORTH RIVER PARTNERS, L.P.

BY: WELLINGTON MANAGEMENT COMPANY, LLP,
AS INVESTMENT ADVISER

BY: /s/ Steven M. Hoffman
NAME: STEVEN M. HOFFMAN
TITLE: VICE PRESIDENT AND COUNSEL

ADDRESS:
TELEPHONE:
FACSIMILE:
E-MAIL:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR:

NORTH RIVER INVESTORS (BERMUDA) L.P.

BY: WELLINGTON MANAGEMENT COMPANY, LLP,
AS INVESTMENT ADVISER

BY: /s/ Steven M. Hoffman
NAME: STEVEN M. HOFFMAN
TITLE: VICE PRESIDENT AND COUNSEL

ADDRESS:
TELEPHONE:
FACSIMILE:
E-MAIL:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR:

SALTHILL PARTNERS, L.P.

BY: WELLINGTON MANAGEMENT COMPANY, LLP,
AS INVESTMENT ADVISER

BY: /s/ Steven M. Hoffman
NAME: STEVEN M. HOFFMAN
TITLE: VICE PRESIDENT AND COUNSEL

ADDRESS:
TELEPHONE:
FACSIMILE:
E-MAIL:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR:

SALTHILL INVESTORS (BERMUDA) L.P.

BY: WELLINGTON MANAGEMENT COMPANY, LLP,
AS INVESTMENT ADVISER

BY: /s/ Steven M. Hoffman
NAME: STEVEN M. HOFFMAN
TITLE: VICE PRESIDENT AND COUNSEL

ADDRESS:
TELEPHONE:
FACSIMILE:
E-MAIL:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR:

HAWKES BAY MASTER INVESTORS (CAYMAN) LP

BY: WELLINGTON MANAGEMENT COMPANY, LLP,
AS INVESTMENT ADVISER

BY: /s/ Steven M. Hoffman
NAME: STEVEN M. HOFFMAN
TITLE: VICE PRESIDENT AND COUNSEL

ADDRESS:
TELEPHONE:
FACSIMILE:
E-MAIL:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR:

SHIRE LLC

BY: /s/ Mike Chapman
NAME: MIKE CHAPMAN
TITLE: PRESIDENT

ADDRESS:
TELEPHONE:
FACSIMILE:
E-MAIL:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR:

GENZYME CORPORATION

BY: /s/ David P. Meeker
NAME: David P. Meeker
TITLE: President & CEO, Genzyme

ADDRESS:
TELEPHONE:
FACSIMILE:
E-MAIL:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR:

HANS UTSCH

/s/ Hans Utsch

ADDRESS:
TELEPHONE:
FACSIMILE:
E-MAIL:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR:

THOMAS KASSBERG

/s/ Thomas Kassberg

ADDRESS:
TELEPHONE:
FACSIMILE:
E-MAIL:

IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the date first above written.

INVESTOR:

TPG BIOTECHNOLOGY PARTNERS III, L.P.

BY: TPG BIOTECHNOLOGY GENPAR III, L.P.,
ITS GENERAL PARTNER

BY: TPG BIOTECHNOLOGY GENPAR III ADVISORS, LLC,
ITS GENERAL PARTNER

BY: /s/ Ronald Cami
NAME: RONALD CAMI
TITLE: VICE PRESIDENT

ADDRESS:
TELEPHONE:
FACSIMILE:
E-MAIL:

IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the date first above written.

INVESTOR:

BEACON BIOVENTURES FUND II LIMITED PARTNERSHIP

By its sole general partner: Beacon Bioventures Advisors Fund II Limited Partnership
By its sole general partner: BBV II AGP LLC

By: /s/ Stephen Knight
Stephen Knight, Vice President

TELEPHONE:
FACSIMILE:
E-MAIL:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR:

HEALTHCAP VI, L.P.

By: Its General Partner
HEALTHCAP VI GP S.A.

/s/ Francois Kaiser
Name: Franscois Kaiser
Title: Director

/s/ D. Richter
Name: D. Richter
Title: Director

Address:

Mailing address:

TELEPHONE:
FACSIMILE:
E-MAIL:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR:

A.M. PAPPAS LIFE SCIENCE VENTURES IV, L.P.
By: AMP&A Management IV, LLC
Its: General Partner

By: /s/ Ford S. Worthy
Name: Ford S. Worthy
Title: Partner & CFO

PV IV CEO FUND, L.P.
By: AMP&A Management IV, LLC
Its: General Partner

By: /s/ Ford S. Worthy
Name: Ford S. Worthy
Title: Partner & CFO

TELEPHONE:
E-MAIL:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR:

By: /s/ Emil D. Kakkis
Emil D. Kakkis

Address:

Telephone:
Facsimile No.:
E-mail Address:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR:

By: /s/ William Aliski
William Aliski

Address:

Telephone:
Facsimile No.:
E-mail Address:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR:

JOHN AND CYNTHIA KLOCK TRUST

By: /s/ John Klock
Name: John Klock
Title: Trustee

By: /s/ Cynthia Klock
Name: Cynthia Klock
Title: Trustee

Address:

Telephone:
Facsimile No.:
E-mail Address:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

NAME OF INVESTOR: RAMIUS ULTRAGENYX HOLDINGS LLC

BY: /s/ Peter A. Cohen
NAME: Peter A. Cohen
TITLE: Authorized Signatory

ADDRESS:

TELEPHONE:
FACSIMILE:
E-MAIL:

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

T. ROWE PRICE ASSOCIATES, INC., as investment adviser on behalf of the funds and accounts set forth in Attachment A hereto, and listed below, severally and not jointly
T. ROWE PRICE HEALTH SCIENCES FUND, INC.
T. ROWE PRICE HEALTH SCIENCES PORTFOLIO
TD MUTUAL FUNDS—TD HEALTH SCIENCES FUND
VALIC COMPANY I—HEALTH SCIENCES FUND
JOHN HANCOCK VARIABLE INSURANCE TRUST—HEALTH SCIENCES TRUST
JOHN HANCOCK FUNDS II—HEALTH SCIENCES FUND

BY: /s/ Kris Jenner
NAME: Kris Jenner
TITLE: VICE PRESIDENT

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

T. ROWE PRICE ASSOCIATES, INC., as investment adviser on behalf of the funds and accounts set forth in Attachment A hereto, and listed below, severally and not jointly
T. ROWE PRICE NEW HORIZONS FUND, INC.
T. ROWE PRICE NEW HORIZONS TRUST
T. ROWE PRICE H.S. EQUITIES TRUST

BY: /s/ Henry Ellenbogen
NAME: Henry Ellenbogen
TITLE: VICE PRESIDENT

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

NAME OF INVESTOR: BlackRock Health Sciences Trust

BY: BLACKROCK ADVISORS, LLC, ITS INVESTMENT ADVISER

BY: /s/ Erin Xie
NAME: Erin Xie
TITLE: MANAGING DIRECTOR

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

NAME OF INVESTOR: BlackRock Funds: Health Sciences Opportunities Portfollio

BY: BLACKROCK ADVISORS, LLC, ITS INVESTMENT ADVISER

BY: /s/ Erin Xie
NAME: Erin Xie
TITLE: MANAGING DIRECTOR

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

NAME OF INVESTOR: ING Health Sciences Opportunities Portfollio

BY: BLACKROCK ADVISORS, LLC, ITS INVESTMENT ADVISER

BY: /s/ Erin Xie
NAME: Erin Xie
TITLE: MANAGING DIRECTOR

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

NAME OF INVESTOR: BGF World Healthscience Fund

BY: BLACKROCK ADVISORS, LLC, ITS INVESTMENT ADVISER

BY: /s/ Erin Xie
NAME: Erin Xie
TITLE: MANAGING DIRECTOR

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

NAME OF INVESTOR: Columbia Acorn Trust

on behalf of its Columbia Acorn Fund
By Columbia Wanger Asset Management, LLC,
Its investment adviser

BY: /s/ Richard Watson
NAME: Richard Watson
TITLE: Analyst

SCHEDULE A

SCHEDULE OF INVESTORS

Investor Name

Adage Capital Partners, L.P.
SMALLCAP World Fund, Inc.
American Funds Insurance Series – Global Small Capitalization Fund
Prudential Sector Funds, Inc., Prudential Health Sciences Fund d/b/a Prudential Jennison Health Sciences Fund
Jennison Global Healthcare Master Fund, Ltd.
North River Partners, L.P.
North River Investors (Bermuda) L.P.
Salthill Partners, L.P.
Salthill Investors (Bermuda) L.P.
Hawkes Bay Master Investors (Cayman) LP
Shire LLC
Genzyme Corporation
Hans Utsch
Ramius Ultragenyx Holdings LLC
T. Rowe Price Health Sciences Fund, Inc.
T. Rowe Price Health Sciences Portfolio
TD Mutual Funds – TD Health Sciences Fund
VALIC Company I – Health Sciences Fund
John Hancock Variable Insurance Trust – Health Sciences Trust
John Hancock Funds II – Health Sciences Fund
T. Rowe Price New Horizons Fund, Inc.
T. Rowe Price New Horizons Trust
T. Rowe Price U.S. Equities Trust
Columbia Acorn Fund
BlackRock Funds: Health Sciences Opportunities Portfolio
BlackRock Health Sciences Trust
ING BlackRock Health Sciences Opportunities Portfolio
BGF World Healthscience Fund
TPG Biotechnology Partners III, L.P.
Beacon Bioventures Fund II Limited Partnership
Healthcap VI, L.P.
A.M. Pappas Life Science Ventures IV, L.P.
PV IV CEO Fund, L.P.
Emil D. Kakkis
William Aliski
John and Cynthia Klock Trust
Thomas Kassberg

Exhibit 10.1

COLLABORATION AND LICENSE AGREEMENT

by and between

KYOWA HAKKO KIRIN CO., LTD.

and

ULTRAGENYX PHARMACEUTICAL INC.

Dated August 29, 2013

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

TABLE OF CONTENTS

COLLABORATION AND LICENSE AGREEMENT

This Collaboration and License Agreement (this "**Agreement**") is made effective as of August 29, 2013 (the "**Effective Date**"), by and between Kyowa Hakim Kirin Co., Ltd., a company organized and existing under the laws of Japan, with an address at 1-6-1 Ohtemachi, Chiyoda-ku, Tokyo, 100-8185, Japan ("**KHK**"), and Ultragenyx Pharmaceutical Inc., a company organized and existing under the laws of California, U.S.A., with an address at 60 Leveroni Ct. Novato, CA 94949, U.S.A. ("**UGNX**"). KHK and UGNX are sometimes hereinafter referred to each as a "**Party**" and collectively as the "**Parties**."

WITNESSETH:

WHEREAS, KHK desires to grant UGNX, and UGNX desires to accept, certain licenses and other rights subject to the terms of this Agreement regarding the Development and Commercialization of the Licensed Products in the Territory and the European Territory (each as defined below); and

WHEREAS, the Parties desire to set forth the terms and the conditions of such licenses and other rights;

NOW, THEREFORE, in consideration of the foregoing premises and the mutual covenants herein contained, the Parties hereby agree as follows:

ARTICLE 1.
DEFINITIONS

1.1 **Definitions**. When used in this Agreement, capitalized terms will have the meanings as defined below and throughout the Agreement.

1.1.1 "**Affiliate**" with respect to a Party means an individual, trust, business trust, joint venture, partnership, corporation, association or other legal entity that, directly or indirectly, through one or more intermediaries, controls, is controlled by, or is under common control with that Party. For purposes of this definition only, "control" and, with correlative meanings, the terms "controlled by" and "under common control with" means (a) the possession, directly or indirectly, of the power to direct the management or policies of a legal entity, whether through the ownership of voting securities or by contract relating to voting rights or corporate governance, or (b) the ownership, directly or indirectly, of fifty percent (50%) or more of the voting securities or other ownership interest of a legal entity.

1.1.2 "**Applicable Laws**" means any federal, state, local, national and supra-national laws, statutes, rules and/or regulations, including any rules, regulations, guidance, guidelines or requirements of Regulatory Authorities, national securities exchanges or securities listing organizations, that may be in effect from time to time during the Term and apply to a particular activity hereunder and including laws, regulations and guidelines governing the import, export, development, manufacture, marketing, distribution and/or sale of Licensed Products.

1.1.3 "**Business Day**" means a day that is not a Saturday, Sunday or a day on which banking institutions in either Tokyo, Japan, or San Francisco, U.S.A., are required by law to remain closed.

1.1.4 "**Calendar Quarter**" means the period beginning on the Effective Date and ending on the last day of the Calendar Quarter in which the Effective Date falls, and thereafter each successive period of three (3) consecutive calendar months ending on March 31, June 30, September 30 or December 31.

1.1.5 "**Calendar Year**" means the period beginning on the Effective Date and ending on the last day of the Calendar Year in which the Effective Date falls, and thereafter each successive period of twelve (12) months commencing on January 1 and ending on December 31.

1.1.6 "**CKD**" means chronic kidney disease.

1.1.7 "**Clinical Trial**" means a Phase 1 Clinical Trial, a Phase 2 Clinical Trial, a Phase 3 Clinical Trial, a Phase 4 Clinical Trial, a Phase 5 Clinical Trial or a combination of any of the foregoing.

1.1.8 "**Commercialization Costs**" means [***], including [***], incurred by or on behalf of a Party in accordance with this Agreement and attributable to, or reasonably allocable to, the [***] of the [***] in the [***], including [***].

1.1.9 "**Commercialization**" means any and all activities relating to marketing, selling, promoting, distributing, importing, detailing, offering to sell, having sold, and/or selling the Licensed Products, whether before or after the Marketing Approvals and Pricing and/or Reimbursement Approvals for such Licensed Product have been obtained. When used as a verb, "**Commercialize**," means to engage in Commercialization.

1.1.10 "**Commercially Reasonable Efforts**" means, with respect to a Party in the performance of its obligations hereunder, the application by or on behalf of such Party of a level of efforts that a similarly-situated pharmaceutical or biotechnology company, as the case may be, would apply to such activities in relation to a similar pharmaceutical product owned by it or to which it has rights, which product is at a similar stage in its development or product life and is of similar market potential, profit potential and strategic value (in each case as compared to the Licensed Products) taking into account efficacy, safety, expected labeling, the competitiveness of alternative products in the marketplace, the patent and other proprietary position of the product, the likelihood of regulatory approval given the regulatory structure involved, the profitability of the product including the royalties payable to licensors, or patent or other intellectual property rights, alternative products and other relevant factors, based on conditions then prevailing.

1.1.11 "**Confidential Information**" means information of a confidential or proprietary nature disclosed by a Party to the other Party hereunder, whether disclosed in oral, written, graphic or electronic form provided that Confidential Information disclosed in written, graphic or electronic form expressly indicates the confidential nature of such information and that Confidential Information disclosed orally is reduced to writing in summary form indicating the confidential nature of such information within [***] Business Days of disclosure, in connection with this Agreement or the performance of its obligations hereunder, including any such information related to any scientific, clinical, engineering, manufacturing, marketing, financial or personnel matters relating to a Party, or related to a Party's present or future products, sales, suppliers, customers, employees, investors, business plans, Know-How, regulatory filings, data, compounds, research projects, work in progress, future developments or business, in all such cases whether disclosed in oral, written, graphic or electronic form; provided, however, that in any event, Confidential Information excludes any information that: (a) is known by recipient at the time of its receipt, and not through a prior disclosure by or on behalf of the disclosing Party, as documented by contemporaneous business records; (b) is or becomes properly in the public domain through no fault

of the recipient; (c) is subsequently disclosed to the recipient by a Third Party who may lawfully do so and is not directly or indirectly under an obligation of confidentiality to the disclosing Party, as documented by written business records in existence prior to the receipt of such information from the disclosing Party; or (d) is developed by the recipient independently of, and without reference to or use of, Confidential Information received from the disclosing Party.

1.1.12 "**Control**" means with respect to any Patent Rights, Know-How or other intellectual property rights, possession of the right, whether directly or indirectly, and whether by ownership, license or otherwise, to grant a license, sublicense or other right to or under, such Patent Rights, Know-How or other intellectual property rights as provided for herein without violating the terms of any agreement or other arrangements with any Third Party at the time when such license, sublicense or other rights are first granted hereunder.

1.1.13 "**Development**" means, with respect to the [***] and the [***], including the [***]. When used as a verb, "**Develop**" means to engage in Development.

1.1.14 "**Development Costs**" means all [***], including [***], incurred by or on behalf of a Party in accordance with this Agreement and attributable to, or reasonably allocable to, the [***] of the [***] in the [***] and that are [***], including [***].

1.1.15 "**Drug Substance**" means the recombinant human IgG1 monoclonal antibody targeting FGF23 identified as KRN23 with the amino acid sequence set forth on Schedule 1.1.15.

1.1.16 "**European Centralized Approval**" means a [***].

1.1.17 "**European Core Territory**" means [***].

1.1.18 "**European Territory**" means (a) the European Core Territory, if any; (b) any other member states of the European Union (if applicable); and (c) Switzerland and Turkey.

1.1.19 "**European Union**" means the European Union consisting of all member states thereof as of the Effective Date.

1.1.20 "**European Transition Date**" means the date on which Marketing Approval for a Licensed Product for the First Indication is obtained in the European Territory on a country-by-country basis (or, in the case of the European Core Territory, the date on which the European Centralized Approval is obtained for a Licensed Product for the First Indication). By way of illustration, if UGNX [***].

1.1.21 "**FDA**" means the United States Food and Drug Administration or any successor agency thereto.

1.1.22 "**Field**" means the treatment and/or prevention of: (a) any Orphan Disease (whether or not listed in the following clauses); (b) XLH; (c) [***]; (d) [***]; (e) [***]; and (f) [***]; provided, however, that the Field will not include either (i) [***] or (ii) [***].

1.1.23 "**Financial Exhibit**" means the exhibit attached hereto as Exhibit A.

1.1.24 "**First Commercial Sale**" means, with respect to a country in the [***] or [***], the first commercial sale of any Licensed Product to a Third Party for use, consumption or resale in that country after obtaining Marketing Approval and Pricing and/or Reimbursement Approval in that country. For the avoidance of doubt, [***].

1.1.25 "**First Indication**" means [***].

1.1.26 "**First Pediatric Study**" means a first Clinical Trial in pediatric patients in the First Indication.

1.1.27 "**First Pediatric Study Deadline**" is defined in Section 15.3.1(a).

1.1.28 "**Force Majeure Event**" is defined in Section 17.2.

1.1.29 "**FTE**" means a full time equivalent person year (consisting of a total of [***] per year) for personnel supporting [***] and/or [***] and/or [***] of [***] in [***] in accordance with this Agreement. For the avoidance of doubt, [***].

1.1.30 "**FTE Costs**" means all costs for FTEs calculated by multiplying (a) [***] by (b) the [***], provided that, to the extent either Party is unable to fully track the number of FTEs utilized, the Parties shall agree on a mechanism for estimating such number. For clarity, FTE Costs shall in no case include [***], provided that costs [***], may be included.

1.1.31 "**FTE Rate**" means, except as specified in the Financial Exhibit with respect to the Sales Force FTE Rate therein defined, [***] for each of KHK and UGNX (such amount premised on the accuracy of information disclosed and used to determine the amount), with respect to the [***]. With respect to [***]. At the time of any [***] proposed by either Party, the other Party ("**Reviewing Party**") shall have the right to review, upon written request, the

information (including actual costs and applicable assumptions) used by the proposing Party to determine its FTE Rate. The proposing Party shall provide any such information (in an accurate manner) reasonably requested by the Reviewing Party, and in the event the Reviewing Party reasonably believes that additional discussions are required to confirm the basis for the determination of such FTE rate, the Parties shall engage in such discussions in good faith. For clarity, the FTE Rate shall in no case include [***], provided that costs [***], may be included.

1.1.32 "**GAAP**" means generally accepted accounting principles applicable to a Party in a particular country (e.g., Japanese Accounting Standards or U.S. Generally Accepted Accounting Principles) as consistently applied throughout the applicable periods indicated herein by or on behalf of the relevant Party.

1.1.33 "**GMP**" means the then-current good manufacturing practices required by the FDA and as set forth in the laws and regulations in the United States with respect thereto, for the manufacture and testing of pharmaceutical materials, and comparable Applicable Laws and requirements of Regulatory Authorities applicable to the manufacture and testing of pharmaceutical materials in jurisdictions within the Territory, as they may be updated from time to time, including applicable rules and guidelines promulgated under the International Conference on Harmonization.

1.1.34 "**IND**" means an Investigational New Drug Application filed with the FDA pursuant to 21 CFR 312.20, or the corresponding filing required for the clinical testing in humans of a pharmaceutical product in any country or regulatory jurisdiction other than the United States.

1.1.35 "**Initiation**" means, with respect to [***], the date on which [***]. "**Initiate**" shall have a correlative meaning.

1.1.36 "**In-Licenses**" means: (a) [***]; (b) [***]; and (c) [***].

1.1.37 "**Joint Invention**" means any invention made by at least [***].

1.1.38 "**Key Latin American Country**" means any of the following countries: [***].

1.1.39 "**KHK Invention**" means any invention or discovery that relates to a [***] (a) a [***] or (b) [***].

1.1.40 "**KHK Non-Core Development Plan**" means KHK's plan for the KHK Non-Core Development Activities.

1.1.41 "**KHK Out-of-Field Products**" means [***] for use outside of the Field.

1.1.42 "**KHK Regulatory Data**" means all regulatory filings made by or on behalf of KHK (including by its Affiliates and licensees other than UGNX) with respect to the Licensed Products and all data generated by or on behalf of KHK (including by its Affiliates and licensees) in the performance of non-clinical and clinical development activities or regulatory activities, including any information contained in any regulatory filings with respect to the Licensed Products and the results of, and all information generated in connection with, any non-clinical or clinical studies or regulatory activities performed by or on behalf of KHK with respect to the Licensed Products.

1.1.43 "**KHK Trademark**" means any trademark or trade name, including registrations and applications therefor, owned or Controlled by KHK covering KHK's corporate name and/or company logo.

1.1.44 "**Know-How**" means any non-public knowledge, experience, know-how, technology, information and data (including pharmacological, toxicological and clinical data and analytical and quality control data), trade secrets, formulas and formulations, processes, techniques, unpatented inventions, methods, discoveries, specifications, formulations, compositions, materials, ideas, developments, test procedures and results, together with all documents and files embodying the foregoing, but excluding, in any event, any patent rights in any of the foregoing.

1.1.45 "**Latin America**" means Central America and South America, where "**Central America**" means Belize, Costa Rica, El Salvador, Guatemala, Honduras, Mexico, Nicaragua and Panama; and "**South America**" means Argentina, Bolivia, Brazil, Chile, Columbia, Ecuador, Guyana, Paraguay, Peru, Surinam, Uruguay and Venezuela.

1.1.46 "**Licensed Know-How**" means Know-How Controlled by KHK or its Affiliates at any time during the Term which (a) relates to the Drug Substance and/or the Licensed Products, and (b) is necessary or useful for the Development, use or Commercialization of the Drug Substance and/or Licensed Products in the Field. Without limiting the foregoing, "Licensed Know-How" shall include the KHK Clinical Data and KHK Regulatory Data.

1.1.47 "**Licensed Patent Rights**" means all patents and patent applications (and patents issued from the foregoing) Controlled by KHK or its Affiliates at any time during the Term which (a) cover the Drug Substance and/or the Licensed Products or their use and (b) are necessary or useful for the Development, use or Commercialization of the Drug Substance and/or the Licensed Products in the Field. A list of Licensed Patent Rights as of the Effective Date is attached as Schedule 1.1.47.

1.1.48 "**Licensed Products**" means any formulation or product that is comprised of, in whole or in part, or containing the Drug Substance as an active ingredient (regardless of dosage, strength or size).

1.1.49 "**Licensed Technology**" means the Licensed Patent Rights and the Licensed Know-How.

1.1.50 "**Marketing Approval**" means, with respect to a Licensed Product in a country or region, all registrations or authorizations (other than Pricing and/or Reimbursement Approvals) required from Regulatory Authority(ies) to market and sell such Licensed Product in such country or region. For the avoidance of doubt, with respect to the European Core Territory, "Marketing Approval" may consist of a European Centralized Approval.

1.1.51 "**Marketing Materials**" is defined in Section 6.4.

1.1.52 "**Medical Geneticist**" means a medical professional who specializes in the diagnosis, treatment, counseling and/or management of patients with hereditary disorders.

1.1.53 "**Named Patient Sale(s)**" means the sale of a Licensed Product in a country on a "named-patient" basis to meet the needs of particular patients under the order of a physician or other health care professional.

1.1.54 "**Net Sales**" means, with respect to any Licensed Product, the gross amounts invoiced by a Party or its Affiliates **(**"**Selling Party**"**)** to any Third Party for sales of such Licensed Product in any indication in the Field in the Territory or in the European Territory, as applicable, including Named Patient Sales, less the following items, provided that they are bona fide:

(a) actual credits, refunds or allowances to Third Party customers for spoiled, damaged, rejected, recalled, outdated and reasonably returned Licensed Products;

(b) discounts, including cash, volume, quantity and other trade discounts, charge-back payments, and rebates and allowances actually granted, incurred or allowed in the ordinary course of business, as well as government-required discounts and allowances (including government rebates and other price reductions), and other reductions, concessions and allowances that effectively reduce the selling price to the Selling Party;

(c) transportation charges, freight, postage and insurance (but only insurance related to protecting the particular shipment against physical loss or damage); and

(d) sales, use or excise Taxes and import/export duties or tariffs and similar governmental charges actually due or incurred in connection with the sales of such Licensed Products.

Components of Net Sales shall be determined in the ordinary course of business in accordance with GAAP (as applicable in the country of sale), consistently applied. Net Sales shall include, for any Licensed Product, [***]. Notwithstanding the above, to the extent KHK and/or its Affiliates enter into an arrangement that calls for or permits the manufacturing and/or sale of Licensed Products by a Third Party (whether or not a Sublicensee) in consideration for a running royalty or other consideration that operates as a substitute thereof (other than milestone payments or upfront payments) then such Third Party shall be the "Selling Party" and its sales or resales shall be included in definition of "Net Sales". Furthermore, the Parties acknowledge and agree that, with respect to the [***], Net Sales shall be determined based on [***], provided that, in the [***]. For purposes of determining when a sale of any Licensed Products occurs for purposes of calculating Net Sales, the sale will be deemed to occur on date the Licensed Products are shipped. No deductions shall be made for commissions paid to individuals or agents, nor shall any deductions be permitted for the cost of collections. For purposes of determining Net Sales, a "sale" shall not include transfers or dispositions, at no cost or below cost, of Licensed Products for charitable, non-clinical, clinical or regulatory purposes or for promotional samples or free goods. Amounts invoiced by the Selling Party for the sale of Licensed Products to another Affiliate for resale to a Third Party shall not be included in the computation of Net Sales hereunder. In the event that the Selling Party sells the Licensed Products: (a) to a Third Party in a bona fide arm's length transaction, for material consideration, in whole or in part, other than cash (but excluding, for the avoidance of doubt, consideration in the form of non-financial legal terms and conditions incident to sale including, for clarity, the supply of Licensed Products for non-commercial purposes substantially at cost); (b) to a Third Party in other than a bona fide arm's length transaction; or (c) with discounts of Licensed Products that are disproportional to the discounts of other products sold by the Selling Party in conjunction with such Licensed Products, the Net Sales price for such Licensed Product shall be deemed to be the standard invoice price then being invoiced by the Selling Party in an arm's length transaction with similar customers in the same country within the Territory and/or the European Territory. In the event that the Selling Party includes one or more Licensed Product as part of a bundle of products, the price for such Licensed Products shall be deemed to be the standard invoice price for such Licensed Products when sold separately and not as part of a bundle of products. In the event that no separate prices are charged in the applicable transaction, then Net Sales for such bundle shall be determined based on the list price for the Licensed Product and the other products or services in the relevant country during the accounting period in which the sale was made. If no list price exists in such country for the Licensed Product or the other products or services that are part of the bundle, then Net Sales for such bundle shall be equitably determined based on the fair market value of the Product relative to that of the other products or services. Any dispute between the Parties with respect to the determination of such market value shall be finally resolved pursuant to Section 16.4.

1.1.55 "**Non-Core Development Plan**" means, as applicable, the UGNX Non-Core Development Plan and/or the KHK Non-Core Development Plan.

1.1.56 "**Orphan Disease**" means a disease or condition for which in any given country a pharmaceutical treatment meets, as of the Effective Date, the definition of an "orphan product" or "orphan drug" for treatment of a "rare disease" under the U.S. Orphan Drug Act or Regulation No. 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products, each as amended, or any successor laws or regulations thereto.

1.1.57 "**Option Negotiation Right Field**" means all [***] and the Licensed Products outside of the Field, [***]. Option Negotiation Right Field shall not include [***].

1.1.58 “**Patent Rights**” means all issued patents and patent applications, certificates of invention, or applications for certificates of invention, together with any extensions, registrations, confirmations, provisionals, divisionals, continuations, continuations in part (to the extent the claims in such continuation-in-part application are directed to subject matter specifically described in such prior patent application), and patents issuing therefrom, reissues, divisions, continuations, or continuations-in-part, reexaminations, substitutions, renewals, restorations, additions, registrations, and foreign counterparts thereof, as well as extensions and supplementary protection certificates based thereon

1.1.59 “**Person**” means any individual, corporation, association, partnership (general or limited), joint venture, trust, estate, limited liability company, limited liability partnership, unincorporated organization, government (or any agency or political subdivision thereof) or other legal entity or organization.

1.1.60 “**Phase 1 Clinical Trial**” means a human clinical trial performed in accordance with Applicable Laws that provides for the first introduction of a Licensed Product into humans for the purpose of determining human toxicity, metabolism, biomarker, absorption, elimination and other pharmacological action.

1.1.61 “**Phase 2 Clinical Trial**” means a human clinical trial performed in accordance with Applicable Laws in patients with a particular disease or condition which is designed to establish the safety, appropriate dosage, efficacy, and tolerability of a Licensed Product given its intended use and to initially explore its efficacy for such disease or condition and will include such a clinical trial intended to be a pivotal trial.

1.1.62 “**Phase 3 Clinical Trial**” means a registration or pivotal clinical trial performed in accordance with Applicable Laws and conducted in subjects with a particular disease or condition which is designed to establish the efficacy and safety of a Licensed Product given its intended use and to define warnings, precautions and adverse events that are associated with such Licensed Product in the dosage range intended to be prescribed.

1.1.63 “**Phase 4 Clinical Trial**” means a post-registration clinical trial or post-marketing surveillance study performed in accordance with Applicable Laws and required as a condition to, or for the maintenance of, any Marketing Approval or Pricing and/or Reimbursement Approval for a Licensed Product.

1.1.64 “**Phase 5 Clinical Trial**” means a post-registration clinical trial that is not required as a condition to, or for the maintenance of, any Marketing Approval or Pricing and/or Reimbursement Approval for a Licensed Product. Phase 5 Clinical Trials are commonly referred to as “post-marketing clinical trials”.

1.1.65 “**Pricing and/or Reimbursement Approvals**” means, with respect to a Licensed Product in any country or region where Regulatory Authorities may approve or determine pricing or pricing reimbursement for such Licensed Product, such approval or determination.

1.1.66 “**Product Trademarks**” means any trademarks or trade name, and registrations and applications therefor, for a Licensed Product selected in accordance with Section 2.4 for use in connection with the Commercialization of such Licensed Product in the Territory or the European Territory in the Field (excluding, in any event, KHK Trademarks and UGNX Trademarks).

1.1.67 “**Profit Share Territory**” means the U.S. and Canada.

1.1.68 “**Profit Share Territory Transition Date**” means the fifth (5th) anniversary of the First Commercial Sale in the U.S. in the First Indication.

1.1.69 “**Regulatory Authority**” means any applicable governmental regulatory authority involved in granting approvals for the manufacture, Commercialization, reimbursement and/or pricing a Licensed Product. Regulatory Authority includes the FDA in the U.S. or the applicable governmental regulatory authority in any country or region, or any successor agency of the foregoing having regulatory jurisdiction over the manufacture, distribution and sale of drugs in any country or region.

1.1.70 “**Regulatory Filings**” means any filings that may be required for any Marketing Approval, Pricing and/or Reimbursement Approval or otherwise filed or submitted to a Regulatory Authority in an effort to comply with Applicable Laws with respect to the Development or Commercialization of a Licensed Product. Regulatory Filings include any IND (or similar filing outside the U.S.), Biologic Licensing Application (or similar filing outside the U.S.), clinical study protocols and Regulatory Authority briefing books.

1.1.71 “**Rest of the World**” means all countries and territories outside the [***] and the [***].

1.1.72 “**Royalty Term**” means: (a) with respect to each country in the [***] and [***], the period of time commencing on the date of the First Commercial Sale in that country and continuing for as long as any Licensed Product is sold by UGNX or its Affiliates, or KHK or its Affiliates or other Selling Party, as applicable, in or to such country, and (b) with respect to each country in the [***], the period of time commencing on the [***] and continuing for as long as any Licensed Product is sold by KHK or its Affiliates or other Selling Party, as applicable, in any country of the [***].

1.1.73 “**Specifications**” means the specifications of the Licensed Products and the active pharmaceutical ingredient form of the Drug Substance (“**API**”), as determined and updated based on KHK’s actual specifications thereof, that include the minimum shelf life therefor and the tests, references to analytical procedures, and appropriate acceptance criteria that (a) are numerical limits, ranges, or other criteria for the tests, analytical procedures and other criteria described, and (b) establish the set of criteria to which the Licensed Product or the API should conform when tested by the tests, analytical procedures and acceptance criteria listed in the Specifications.

1.1.74 “**Sublicensee**” means a Third Party to whom KHK or any of its Affiliates has granted a license or a sublicense under the Licensed Technology to Develop and/or Commercialize Licensed Products in the Field, but excluding, for clarity, any Third Party distributor that has no rights other than to resell the Licensed Products, and for which resale KHK, its Affiliates or Sublicensees, as applicable, receive no further consideration (such as royalties) beyond the price for the initial sale to the distributor.

1.1.75 “**Tax**” or “**Taxes**” means any taxes, assessments or the like on income, profits, gross receipts, net proceeds, sales, value-added, ad valorem, withholding, or other such taxes or governmental charges.

1.1.76 “**Territory**” means the Profit Share Territory and Latin America, except for any country removed from the Territory in accordance with the express terms and conditions of Section 15.3.

1.1.77 "**Third Party**" means a Person other than KIM, UGNX or their respective Affiliates.

1.1.78 "**Type-C Meeting**" means a meeting (other than a Type A or Type B meeting) between the FDA and a sponsor or applicant regarding the development and review of a product in a human drug.

1.1.79 "**UGNX Non-Core Development Plan**" means UGNX's plan for the UGNX Non-Core Development Activities.

1.1.80 "**UGNX Invention**" means any invention or discovery that relates to a Licensed Product and that is conceived, made or generated during the Term in the performance of activities undertaken pursuant to this Agreement solely by employees, agents, or independent contractors of UGNX or its Affiliates (including any enhancement or modification of a Licensed Product's use, dosage form or formulation).

1.1.81 "**UGNX Regulatory Data**" means all Regulatory Filings made by or on behalf of UGNX with respect to the Licensed Products and all data generated by or on behalf of UGNX in the performance of UGNX Core Development Activities or Regulatory Activities, including any information contained in any Regulatory Filings with respect to Licensed Products and the results of, and all information generated in connection with, any UGNX Core Development Activities or Regulatory Activities performed by or on behalf of UGNX pursuant to this Agreement. UGNX Regulatory Data shall be jointly owned by UGNX and KIM and used in accordance with Section 4.8.1.

1.1.82 "**UGNX Trademark**" means any trademark or trade name, and registrations and applications therefor, owned or Controlled by UGNX in the Territory and covering UGNX's (or its Affiliate's) corporate name or company logo.

1.1.83 **"United States**" or "**U.S.**" means the United States of America and its territories and possessions.

1.1.84 "**XLH**" means x-linked hypophosphatemic rickets/osteomalaica.

1.1.85 "**001 and 002 Studies**" means the Phase 1/2 study (KRN23-INT-001) and the Phase 1/2 extension study (KRN23-INT-002) in adult XLH patients undertaken by KHK as of the Effective Date.

1.2 **Additional Definitions**. In addition, each of the following definitions will have the respective meanings set forth in the Section of this Agreement indicated below:

Definitions	Section
Additional Indication	4.5
Agreement	Preamble
API	1.1.67
Audited Party	9.2.2
Chairman	16.3
Commercial Supply Agreement	8.3.1
Commercial Supply Cost	8.3.2(a)
Committee	3.2.1
Competing Product Infringement	10.5.1

Definitions	Section
Core Development Activities	4.2
Core Development Plan	4.2
Day-to-Day Core Development Activities	Schedule 4.3.2
Delivery	8.2.3
Dispute	16.1
Effective Date	Preamble
European Marketing Approval	15.3.2(a)
ICC	16.2
Indemnifying Party	14.3
Indemnitee	14.3
Inventing Party	10.3
JCC	3.2.1
JDC	3.2.1
JSC	3.1.1
KHK	Preamble
KHK Clinical Data	4.8.2
KHK Core Development Activities	4.3.1(b)
KHK Indemnitees	14.1
KHK. Non-Core Development Activities	4.11.1(a)
KHK Sales Royalties	7.2.3
Lead Development Party	4.3.1(a)
Losses	14.1
Manufacturing-Related Development Activities	4.6
Marketing Budget	6.1.7(b)
Marketing Plan	6.1.7(a)
Negotiation Period	13.1.2
Non-Promoting Third Party(ies)	1.1.54
Pivotal Study Initiation	15.4
Profit-Sharing Period	7.1.3
Notice	17.6
On-Going Clinical Trials	4.3.1(a)
Qualifying Pivotal Study	15.5(c)
Party	Preamble
Product Liability Claim	14.4
Product Liability Shared Losses	14.4(b)(i)
Publication	11.3.1
Regulatory Activities	5.1.1
Responsible Party	4.11.2
Rules	16.2
Safety Agreement	5.4
Sales, Promotion and Marketing Activities	6.1.1
Selling Party	1.1.54
Term	15.1
Third Party Manufacturer	8.5.1
Tribunal	16.3
UGNX	Preamble
UGNX Clinical Data	4.8.1
UGNX Core Development Activities	4.3.1(a)
UGNX Forecast	8.2.1
UGNX Indemnitees	14.2
UGNX IPO	15.4
UGNX Non-Core Development Activities	4.11.1(b)
UGNX Sales Royalties in the European Territory	7.2.1
UGNX Revenue Share in the Profit Share Territory	7.2.2
US Extension Period	15.3 .3(b)

Definitions	Section
US Marketing Approval	15.3.3(a)
VAT	7.6.2

1.3 **Financial Terms**. All financial and accounting terms not otherwise defined in this Agreement, whether capitalized or not, will have the meanings assigned to them in accordance with GAAP.

ARTICLE 2.
LICENSES

2.1 **Licenses Regarding Licensed Technology**.

2.1.1 **Licenses for Profit Share Territory**. Subject to the terms and conditions of this Agreement, KHK hereby grants to UGNX, without right to sublicense: (a) an exclusive (except for KHK and its subcontractors, solely to the extent necessary to exercise KHK's rights and to perform KHK's obligations under this Agreement), royalty-free license under the Licensed Technology to Develop the Licensed Products in the Field in the Profit Share Territory until the later of (i) the day immediately preceding the Profit Share Territory Transition Date and (ii) if applicable, the completion of the UGNX Core Development activities in the Profit Share Territory under this Agreement; and (b) subject to the restrictions in Section 6.1.2, a non-exclusive license, royalty-free under the Licensed Technology to promote the Licensed Products in the Field in the Profit Share Territory until the end of the Term.

2.1.2 **Licenses for Latin America**. Subject to the terms and conditions of this Agreement, KHK hereby grants to UGNX and its Affiliates, without right to sublicense, an exclusive, royalty-bearing (as described in Section 7.2.3) license under the Licensed Technology to Develop, Commercialize, import and export the Licensed Products in the Field in Latin America until the end of the Term.

2.1.3 **Licenses for European Territory**. Subject to the terms and conditions of this Agreement, KHK hereby grants to UGNX a non-exclusive, royalty-free license under the Licensed Technology to Develop the Licensed Products in the Field in the European Territory, on a country-by-country basis, until the later of (a) the day immediately preceding the applicable European Transition Date and (b) if applicable, the completion of the UGNX Core Development activities in the European Territory under the Agreement.

2.1.4 **In-Licenses**. KHK shall not terminate or breach the In-Licenses (or otherwise cause them to be terminated), or amend the In-Licenses in any way that could reasonably be expected to materially conflict or adversely affect the performance of the activities contemplated by or rights granted under this Agreement. For clarity, this Section 2.1.4 shall be subject to Section 17.2 (Force Majeure).

2.2 **No Sublicense Rights**. Subject to Section 13.6, it is agreed between the Parties that UGNX shall exercise the licensed rights granted hereunder and that UGNX shall perform the Development and Commercialization authorized under this grant of license. Accordingly, no sublicense rights are granted hereunder to UGNX, provided that for the avoidance of doubt, UGNX may (a) subject to the obligation to inform the JDC about the engagement of CROs (Contract Research Organizations), use subcontractors for specific functions in connection with Development

and/or Commercialization of Licensed Products in accordance with the terms of the Agreement (including Section 17.10) and/or (b) Commercialize Licensed Products in accordance with the terms of the Agreement through commercially reasonable distributors and/or resellers.

2.3 **KHK Trademarks.**

2.3.1 **License**. Subject to the terms and conditions of this Agreement, KHK hereby grants to UGNX a non-exclusive (except in Latin America, where such license shall be exclusive in the Field) license to use the KHK Trademarks solely in connection with UGNX's exercise of the licenses granted to it pursuant to Section 2.1 above. UGNX shall use the KHK Trademarks: (a) solely in the manner specified in this Agreement in connection with Licensed Products and not for any other goods or services; and (b) in each case, with KHK's Trademark above UGNX's Trademark if aligned vertically or to the left of UGNX's Trademark if aligned horizontally and otherwise only in the form and manner as reasonably prescribed in writing to UGNX in advance from time to time by KHK (provided, however, that UGNX shall have a reasonable period of time to modify any of its promotional, marketing, regulatory or other practices, including in light of Applicable Laws, and/or cease use of the KHK Trademarks, as may be reasonably necessary to comply with any such form and manner prescriptions and/or any changes thereto). Without limiting the foregoing, any use by UGNX of a KHK Trademark for Licensed Products should be accompanied by a trademark notice that states that such KHK Trademark is a trademark (or a registered trademark, if applicable) of Kyowa Hakko Kirin Co., Ltd. Any use by UGNX of the KHK Trademarks, and KHK's maintenance of the KHK Trademarks, shall be in compliance with all Applicable Laws, including those relating to the licensing of trademarks, in the Territory and the European Territory. UGNX agrees to promptly correct any failure to comply with this Section 2.3 (subject to the cure period provided for in Section 15.2.1).

2.3.2 **No Ownership of KHK Trademarks**. UGNX acknowledges KHK's ownership of all right, title and interest in and to the KHK Trademarks, and agrees that it will do nothing inconsistent with such ownership, that all use of the KHK Trademarks by UGNX will inure to the benefit of and be on behalf of KHK, and that any goodwill associated with the use of any KHK Trademark by UGNX will inure to the benefit of KHK. UGNX agrees that nothing in this Agreement will give UGNX any right, title or interest in the KHK Trademarks other than the right to use the KHK Trademarks in accordance with this Agreement. Anything in this Agreement to the contrary notwithstanding, if by virtue of UGNX's use of the KHK Trademarks, UGNX acquires any equity, title or other rights in or to the KHK Trademarks, UGNX hereby agrees all such equity, title or other rights in or to the KHK Trademarks belong to KHK upon creation of the value, and UGNX agrees to and hereby does assign and transfer any such KHK Trademark rights to KHK. UGNX agrees not to use or file any application to register any trademark or trade name that is confusingly similar to any KHK Trademark.

2.4 **UGNX Trademarks.**

2.4.1 **License**. Subject to the terms and conditions of this Agreement, UGNX hereby grants to KHK a non-exclusive, royalty-free license to use the UGNX Trademarks solely to promote Licensed Products in the Field in accordance with this Agreement.

2.4.2 **No Ownership of UGNX Trademarks**. KHK acknowledges UGNX's ownership of all right, title and interest in and to the UGNX Trademarks, and agrees that it will do nothing inconsistent with such ownership, that all use of the UGNX Trademarks by KHK will inure to the benefit of and be on behalf of UGNX, and that any goodwill associated with the use of any UGNX Trademark by KHK will inure to the benefit of UGNX. KHK agrees that nothing in this

Agreement will give KHK any right, title or interest in the UGNX Trademarks other than the right to use the UGNX Trademarks in accordance with this Agreement. Anything in this Agreement to the contrary notwithstanding, if by virtue of KHK's use of the UGNX Trademarks, KHK acquires any equity, title or other rights in or to the UGNX Trademarks, KHK hereby agrees all such equity, title or other rights in or to the UGNX Trademarks belong to UGNX upon creation of the value, and KHK agrees to and hereby does assign and transfer any such UGNX Trademark rights to UGNX. KHK agrees not to use or file any application to register any trademark or trade name that is confusingly similar to any UGNX Trademark.

2.5 **Use of KHK Trademarks and UGNX Trademarks.**

2.5.1 **Product Packaging**.

(a) Subject to Section 2.3 and the requirements of Applicable Laws, for so long as UGNX promotes Licensed Products to Medical Geneticists, UGNX may request KHK to include one or more UGNX Trademarks on the product packaging for all Licensed Products in the Field to be Commercialized in the Profit Share Territory, and KHK shall use Commercially Reasonable Efforts to include such UGNX Trademarks in an appropriate manner.

(b) Subject to Section 2.3 and the requirements of Applicable Laws, UGNX may include one or more UGNX Trademarks on the product packaging for all Licensed Products in the Field to be Commercialized in Latin America.

2.5.2 **Marketing Materials**.

(a) Subject to Section 2.3 and the requirements of Applicable Laws, UGNX shall have the right to use one or more UGNX Trademarks on Marketing Materials to be used (i) in the Profit Share Territory through the day immediately preceding the Profit Share Territory Transition Date and (ii) in Latin America, and UGNX shall reasonably consider any request by KHK to include one or more KHK Trademarks on such Marketing Materials.

(b) Subject to Section 2.4 and the requirements of Applicable Laws, KHK shall have the right to use one or more KHK Trademarks on Marketing Materials to be used in the Profit Share Territory beginning on the Profit Share Territory Transition Date and in the European Territory, and shall reasonably consider any request by UGNX to include one or more UGNX Trademarks on such Marketing Materials.

2.6 **Product Trademarks**. The Parties will jointly select the Product Trademarks for use in connection with the Commercialization of the Licensed Products in the Territory and the European Territory in the Field (which Product Trademarks may or may not be the same as the names, marks and logos used for the Licensed Products in the Rest of the World) provided, however, that in the event of a dispute with respect to such selection, KHK will have the authority to make any final decision. Furthermore, KHK will be responsible for (and will control) the filing, prosecution, maintenance and defense of all registrations of the Product Trademarks in the Territory and the European Territory, and will be solely responsible for the payment of any costs incurred by KHK relating to filing, prosecution, maintenance, defense and enforcement of the Product Trademarks in the Territory and the European Territory. KHK hereby grants to UGNX a non-exclusive license to use the Product Trademarks in the Territory solely in connection with UGNX's exercise of the licenses granted to it pursuant to this Article 2 or otherwise in accordance with this Agreement. The use of the Product Trademarks by UGNX in the Profit Share Territory will be

royalty-free and the royalties for use of the Product Trademarks by UGNX in Latin America will be deemed to be included within the royalty amounts set forth in Section 7.2.3. In the event that UGNX wishes to use any trademark(s) owned by UGNX as Product Trademarks for any Licensed Products, following the Parties' mutual agreement to use such trademark(s), UGNX shall transfer the rights to such trademark(s) free-of-charge to KHK and KHK shall license such trademark(s) to UGNX for use in accordance with this Agreement.

2.7 **Reservation of Rights.**

2.7.1 **Development and Commercialization**. Except as expressly set forth in this Agreement, all rights to Develop and Commercialize the Licensed Products will be retained by KHK. For the avoidance of doubt, subject to the terms of this Agreement, KHK retains the exclusive right to Commercialize the Licensed Products in the European Territory (including through commercially reasonable distributors and/or resellers) and the exclusive right to Develop and Commercialize the Licensed Products in Japan. In the event KHK decides to license or sublicense its right to Develop and/or Commercialize the Licensed Products in the European Territory, (a) each license or sublicense granted by KHK shall be subject to the terms of this Agreement, and KHK shall remain responsible for compliance with the relevant terms of this Agreement by each Sublicensee, and (b) KHK shall provide to UGNX a copy of each license and sublicense agreement within thirty (30) days of its execution, which copy may be reasonably redacted to exclude confidential information of the applicable Sublicensee.

2.7.2 **Licensed Technology, Confidential Information and Regulatory Data**. Except for the rights and licenses specifically granted in this Agreement, KHK reserves all rights to the Licensed Technology, Confidential Information of KHK, KHK Regulatory Data and any regulatory filings owned or held by KHK, and, except for such specifically granted rights and licenses, this Agreement does not include the grant of any right or license, express or implied, to any other intellectual property or other rights owned or Controlled by KHK. Notwithstanding any provision to the contrary, KHK retains all rights under the Licensed Technology, Confidential Information of KHK, KHK Regulatory Data and any regulatory filings owned or held by KHK, as may be required for KHK to perform its express obligations under this Agreement.

ARTICLE 3.
JOINT STEERING COMMITTEE

3.1 **Joint Steering Committee.**

3.1.1 **Establishment and Membership**. Within thirty (30) days of the Effective Date, the Parties shall establish a joint steering committee (the "**JSC**") to coordinate and oversee the Development and Commercialization of the Licensed Products as contemplated by this Agreement.

3.1.2 **Purpose of JSC**. The purposes of the JSC will be: (a) to keep each Party informed about the manufacturing, Development and Commercialization of the Licensed Products; (b) to (i) review any updates or amendments to the Core Development Plan and (ii) approve any material updates or amendments to the Core Development Plan, including any updates or amendments to budgets; (c) to review (but not approve) the Marketing Plans (including monthly sales forecasts) in the Profit Share Territory and the European Territory, and any proposed material updates or amendments proposed thereto; (d) to review and approve the Marketing Budgets in the Profit Share Territory in accordance with Section 6.1.7; (e) to review (but not approve) the

Marketing Plans of each of KHK and UGNX outside the Profit Share Territory and the European Territory, and the Non-Core Development Plans, as well as any proposed material updates or amendments thereto, including the overall strategy for development activities (including proposed pricing, and forecasts); (f) to review the overall strategy for clinical and commercial manufacturing of the Licensed Products, including plans for packaging, labeling, supply chain and trade and distribution activities for the Profit Share Territory and the European Territory, and risk mitigation strategies related thereto. UGNX and KHK agree to carry out all Development of Licensed Products in the Field in the Territory and the European Territory in consultation with the JSC and the JDC, and (g) to (i) provide a forum for the Parties to discuss the working relationship between the Parties' respective personnel, and (ii) provide recommendations to address any issues arising therefrom.

3.1.3 **Membership**. Each Party shall designate an equal number of representatives, to be three (3) each unless the Parties agree otherwise, with appropriate expertise to serve as members of the JSC, and each of such representatives will be an employee of such Party and will have sufficient seniority within the applicable Party to make decisions arising within the scope of the JSC's responsibilities. Subject to the foregoing, each Party may replace its representatives on the JSC at any time upon at least ten (10) days advance Notice to the other Party. The JSC may change its size from time to time by mutual consent of its members.

3.1.4 **Administrative Chair**. One member of the JSC will serve as the administrative chair of the JSC. The administrative chair will be responsible for organizing meetings and preparing and circulating an agenda in advance of each meeting of the JSC and preparing minutes of each meeting, to be approved by both Parties. For the first year, the Party at whose offices the meetings are held will appoint the administrative chair of the JSC. Thereafter, the Parties will alternate in appointing the administrative chair for one (1) year terms.

3.2 **Subcommittees.**

3.2.1 **Establishment**. The JSC may establish and disband the JDC, the JCC and such other subcommittees as deemed necessary by the JSC, provided that neither the JDC nor the JCC may be so disbanded before the later of (a) the end of the Profit-Sharing Period and (b) the end of the last to occur European Transition Date. Each such subcommittee will consist of the same number of representatives designated by each Party, which number will initially be three (3) each, and thereafter shall be as is mutually agreed by the Parties. Each Party will be free to change its representatives on Notice to the other or to send a substitute representative to any subcommittee meeting; provided, however, that each Party shall ensure that at all times during the existence of any subcommittee, its representatives on such subcommittee are appropriate in terms of expertise and seniority for the then-current stage of Development and/or Commercialization of the Licensed Products in the Field in the Territory and the European Territory and have the authority to bind such Party with respect to matters within the purview of the relevant subcommittee. Each Party's representatives and any substitute for a representative will be bound by the obligations of confidentiality set forth in Article 11. Except as expressly provided in this Agreement, no subcommittee will have the authority to bind the Parties hereunder, and each subcommittee will

report to, and any decisions will be made by, the JSC. The initial subcommittees of the JSC will be the Joint Development Committee ("**JDC**") and the Joint Commercialization Committee ("**JCC**"). The JSC, JDC and JCC shall also be referred to as, each, a "**Committee**".

3.2.2 **Joint Development Committee**.

(a) The JDC will oversee Development of Licensed Products in the Field in the Territory and the European Territory. As soon as practicable following the Effective Date (but in no event more than thirty (30) days following the Effective Date), each Party shall designate its initial three (3) representatives on the JDC, and each of such representatives will be an employee of such Party and will have sufficient seniority within the applicable Party to make decisions arising within the scope of the JDC's responsibilities.

(b) Each Party shall appoint a person from among its representatives on the JDC to serve as a co-chairperson of the JDC. The co-chairpersons will not have any greater authority than any other representative on the JDC and shall conduct the following activities of the JDC: (i) calling meetings of the JDC; (ii) preparing and issuing minutes of each such meeting within thirty (30) days thereafter; (iii) preparing and circulating an agenda for the upcoming meeting; and (iv) ensuring that any decision-making delegated to the JDC is carried out in accordance with Section 3.5.

(c) The JDC will have responsibility for: (i) overseeing, reviewing and coordinating the Development of the Licensed Products (including all Clinical Trials and reviewing the protocols, statistical analysis plans and clinical study reports for such Clinical Trials developed by the applicable Lead Development Party) in the Field; (ii) developing and submitting the Core Development Plan, including the budget and proposed amendments or updates thereto, to the JSC; and (iii) as applicable, overseeing, reviewing and coordinating the work being done under the Core Development Plan and any Non-Core Development Plan(s).

(d) UGNX shall periodically, and at least semi-annually, submit comprehensive and complete reports (i) to the JDC, regarding activities previously approved by the JSC and undertaken by or on behalf of UGNX with respect to the Development of Licensed Products in the Field, including their progress, status and outcome as well as major findings and major decision points, as applicable, so as to keep the JDC fully advised of UGNX's Development activities with respect to the Licensed Products in the Field and (ii) to the JCC with respect to the Commercialization of Licensed Products in the Field, including Sales, Promotion and Marketing Activities, and the date of the First Commercial Sale in a country (if applicable) in the relevant half year.

(e) KHK shall periodically, and at least semi-annually, submit reasonably detailed reports (i) to the JDC, regarding activities undertaken by KHK with respect to KHK's Development activities and (ii) to the JCC with respect to KHK's Commercialization activities.

3.2.3 **Joint Commercialization Committee**.

(a) The JCC will oversee the Commercialization of the Licensed Products in the Field in the Territory and the European Territory. Prior to the commencement of the Commercialization of the Licensed Products in the Territory or the European Territory, at a time agreed to by the Parties, each Party shall designate its initial three (3) representatives on the JCC, and each of such representatives will be an employee of such Party and will have sufficient seniority within the applicable Party to make decisions arising within the scope of the JCC's responsibilities.

(b) Each Party shall appoint a person from among its representatives on the JCC to serve as a co-chairperson of the JCC. The co-chairpersons will not have any greater authority than any other representative on the JCC and shall conduct the following activities of the JCC: (i) calling meetings of the JCC; (ii) preparing and issuing minutes of each such meeting within thirty (30) days thereafter; (iii) preparing and circulating an agenda for the upcoming meeting; and (iv) ensuring that any decision-making delegated to the JCC is carried out in accordance with Section 3.5.

(c) The JCC will have responsibility for: (i) overseeing the Commercialization of the Licensed Products; (ii) setting overall strategic objectives and plans (including pricing and reimbursements) related to Commercialization of the Licensed Products in the Field in the Territory and the European Territory; (iii) developing and submitting to the JSC for review the annual Marketing Plan in the Territory at least ninety (90) days prior to the start of each Calendar Year; (iv) developing and submitting to the JSC for review and approval the annual Marketing Budget in the Profit Share Territory in accordance with Section 6.1.7; (v) reviewing marketing plans for the European Territory and proposed material amendments or updates thereto submitted to the JCC by KHK in accordance with Section 6.1.8; (vi) reviewing Commercialization issues for the Licensed Products in the Field in the Territory and the European Territory; (vii) providing a forum for the Parties to discuss the Commercialization of the Licensed Products in the Field in the Territory and the European Territory; and (viii) such other responsibilities as may be assigned to the JCC pursuant to this Agreement or as may be mutually agreed upon by the Parties from time to time.

3.3 **Committee Meetings**. Each Committee shall meet at least once each Calendar Quarter, or more or less often as agreed to by the Parties; provided that either Party may also call a special meeting of any Committee on an *ad hoc* basis upon at least seven (7) days' prior Notice in order to address urgent matters which cannot be reasonably postponed until the next meeting of such Committee. Meetings may be held in person or by means of electronic communication (including telephone, video or web conferences). Each Committee may invite non-members (including consultants and advisors of a Party who are under an obligation of confidentiality consistent with this Agreement) to participate in the discussions and meetings of such Committee, as non-voting observers; provided, however, that all such Third Party attendees at such meetings must be subject to obligations of confidentiality and non-use applicable to the Confidential Information of each Party that are at least as stringent as those set forth in Article 11. Following any Committee meeting, the administrative chair, in the case of the JSC, and a co-chairperson, on an alternating basis, in the case of the JDC or JCC, will be responsible for preparing and issuing minutes of such meeting within thirty (30) days thereafter. Such minutes will not be finalized until a representative of the other Party has reviewed and confirmed the accuracy of such minutes in writing. If a disagreement regarding the accuracy of such minutes cannot be resolved, the minutes will reflect such disagreement.

3.4 **Limitations of Powers**. The JSC and the subcommittees will have only such powers as are specifically delegated to them hereunder and will not be a substitute for the rights of the Parties. Without limiting the generality of the foregoing, neither the JSC nor any subcommittee will have any power to amend, modify or waive compliance with this Agreement (without limiting the right of the JSC to approve amendments to the Core Development Plan) and the JSC and subcommittees are otherwise subject to the express terms and conditions of this Agreement. Any amendment to the terms and conditions of this Agreement may only be implemented pursuant to Section 17.7 below.

3.5 **Decision-Making.**

3.5.1 **Actions by Unanimous Vote**. Subject to the terms of this Section 3.5, the JSC and the subcommittees will make decisions by unanimous vote with each Party having a single vote, irrespective of the number of representatives actually in attendance at a meeting, or by a written resolution signed by the designated representatives to the JSC or a subcommittee, as applicable, of each of the Parties. For the avoidance of doubt, the approval of all Development budgets and Marketing Budgets and any updates or amendments thereto by the JSC will require the unanimous vote with each Party having a single vote. The JSC members shall use good faith efforts to reach agreement on any and all matters submitted to the JSC. If the JSC fails to reach unanimous consent on a particular matter within thirty (30) days of a Party having requested a formal vote on such matter (or, if such matter is urgent, within seven (7) days of such request) or within thirty (30) days of referral of a matter from a subcommittee, then such dispute will be subject to the resolution procedures described in Sections 3.5.2 and 3.5.3.

3.5.2 **Referral of Disputes for Resolution**. If any subcommittee fails to reach unanimous agreement on a matter after it is presented for decision for a period in excess of thirty (30) days (or, if such matter is urgent, within seven (7) days of such request), the matter will be referred to the JSC. Unless the JSC decides to continue discussing the issue, the JSC will have meetings within thirty (30) days of receiving the dispute submission to reach unanimous consensus on a resolution. If the JSC is unable to reach a resolution of any matter referred up to the JSC (either after the first meeting or any mutually agreed continued discussions), then the matter shall be promptly discussed by the senior executives of the Parties.

3.5.3 **Final Resolution of Disputes**. In the event that any dispute within the JSC or a subcommittee is not resolved pursuant to the terms of Sections 3.5.1 and 3.5.2 then final decision-making authority will be as follows: (a) if the matter pertains to a dispute with respect to [***], then the dispute shall be resolved by [***]; provided, however, that [***] will have the authority to make the final decision (except that increases to the unanimously approved budget shall not exceed [***] of the [***] budget without KHK's prior written consent) until the earlier of (i) [***] and (ii) [***] (subject to extensions, if any, applicable pursuant to Sections 15.3.1(b) or 15.3.1(c)(i)) and that [***] will have the authority to make the final decision in the [***] on and after the [***] and in the [***] on and after the [***] except with respect to the [***]; (b) if the matter pertains to a dispute with respect to [***] in the [***], then [***] will have the authority to make the final decision in the [***] through the day immediately preceding the [***], and [***] will have the authority to make the final decision in the [***] beginning on the [***]; (c) if the matter pertains to a dispute with respect to [***] in the [***], then the dispute shall be resolved by [***] through the day immediately preceding the [***] (subject to Section 6.1.7(b)), and, beginning on the [***], [***] will have the authority to make the final decision with respect to such dispute in the [***]; (d) if the matter pertains the [***], then [***] will have the authority to make the final decision; provided that in each case such decision will be consistent with Applicable Laws; and (e) if the matter pertains to the [***], then [***] will have the authority to make the final decision, provided that in each case such decision will be consistent with Applicable Laws.

3.5.4 **Disputes regarding this Agreement**. Notwithstanding this Section 3.5, any dispute regarding the interpretation of this Agreement, the performance or alleged nonperformance of a Party's obligations under this Agreement, or any alleged breach of this Agreement will be resolved in accordance with the terms of Article 16 and will not be subject to resolution by the JSC.

3.6 **Expenses**. Each Party will be responsible for all of its own travel and other costs and expenses for its respective Committee members, designees and non-JSC or subcommittee invitees to attend meetings of, and otherwise participate on, the JSC and any subcommittees or working groups.

3.7 **Relationship of Parties**. Nothing contained in this Agreement shall be deemed to make any member of the JSC or any subcommittee (or any other committees or sub-teams of thereof) a partner, agent or legal representative of the other Party, or to create any fiduciary relationship for any purpose whatsoever. Except as may be explicitly provided in this Agreement, no member of the JSC, any subcommittee (or any other committee or sub-team of thereof) will have any authority to act for, or to assume any obligation or responsibility on behalf of, any other member of the JSC, any subcommittee (or any other committee of sub-team of thereof) of the other Party. For the avoidance of doubt, this Agreement is not intended to create and may not be construed to create a partnership, joint venture, or entity of any kind between the Parties for any legal purpose and/or for any federal, state or local income tax purposes in any jurisdiction in which either of the Parties is resident or may be subject to taxation. The Parties agree not to treat the Agreement as giving rise to a partnership for any U.S., Japanese or other tax purposes, unless otherwise required by a determination of a taxing authority.

ARTICLE 4.
DEVELOPMENT OF PRODUCTS

4.1 **General**. Pursuant to and subject to the terms of this Agreement, the Parties agree to collaborate with respect to the Development of Licensed Products for the First Indication as well as for other indications in the Field, in accordance with the terms set forth in this Article 4.

4.2 **Core Development Plan.**

4.2.1 The Parties shall prepare in writing an overall Development plan and budget (as such plan and budget may be amended from time to time in accordance with this Agreement, the "**Core Development Plan**") covering the entire Development period and the Development activities and costs required in order to obtain and maintain the Marketing Approvals and (if applicable) the Pricing and/or Reimbursement Approvals for the Licensed Products (including Phase 4 Clinical Trials, if applicable) for the First Indication in the Profit Share Territory and the European Territory (such activities, collectively, "**Core Development Activities**"). The Parties acknowledge and agree that it is their intent to seek Marketing Approval in the First Indication for a label that is as broad as reasonably possible (including, for clarity, broad use by age), taking into account, among other things, the requirements of Applicable Laws and the interest in making Licensed Products in the Field commercially available in a timely manner. In addition to Clinical Trial(s) designed to obtain Marketing Approval for pediatric patients from the age of five (5) through the age of eighteen (18), unless otherwise agreed upon in writing by the Parties, the Core Development Plan shall include a Clinical Trial for pediatric patients below the age of 5. For clarity, the Core Development Plan and Core Development Activities shall cover the 001 and 002 Studies. The initial Core Development Plan has been mutually agreed in writing by the Parties as of the date of signing this Agreement, and shall be the operative Core Development Plan until amended with the approval of the JSC.

4.2.2 With the exception of the 2013 and 2014 Budgets (each as defined below), each year by [***], the Parties shall prepare and approve a detailed annual plan and budget covering the portion of the Core Development Plan and Core Development Activities that

will be performed during the [***] months of the following Calendar Year. The detailed annual plan and budget covering the portion of the Core Development Plan and Core Development Activities that will be performed for the period from the Effective Date until December 31, 2013 and that has been agreed upon by the Parties in writing as of the Effective Date (the "**2013 Budget**"). The detailed annual plan and budget covering the portion of the Core Development Plan and Core Development Activities that will be performed during Calendar Year 2014 (the "**2014 Budget**") shall be prepared and updated by the Parties by [***], 2013. The annual plan and budget will also include an allocation of Development activities between the Parties including the number of allocated full time equivalent personnel and the applicable FTE Rate and other expenses to be incurred by each Party during such period.

4.3 **Core Development Activities.**

4.3.1 Subject to the terms of this Agreement and the requirements of Applicable Laws, and except as otherwise mutually agreed upon in writing by the Parties, the Parties Development responsibilities shall be allocated as follows:

(a) UGNX shall be the lead Party for the Development of Licensed Products in the Field in the Profit Share Territory and the European Territory ("**Lead Development Party**") for the following activities beginning on the Effective Date (collectively, "**UGNX Core Development Activities**"): (i) [***] conducted in the Profit Share Territory [***]; and (ii) [***] conducted in the European Territory [***]; provided, however, that UGNX shall in each case under (i) and (ii) continue to be the Lead Development Party for any Phase 4 Clinical Trials and/or Clinical Trials for additional indications in the Field (but excluding for clarity Phase 5 Clinical Trials), if any, commenced (Initiation) by [***] in the [***] or the [***] prior to the [***] or the applicable [***], as applicable, until completion of such respective Clinical Trial ("**On-Going Clinical Trials**").

(b) [***] shall be the Lead Development Party for the following activities (collectively, "[*****] Core Development Activities**"): (i) [***]; (ii) [***]; and (iii) [***], all of the Core Development Activities conducted in the [***], until completion of such studies, excluding, for clarity, in the case of (ii) and (iii), any On-Going Clinical Trials.

4.3.2 The Lead Development Party shall use Commercially Reasonable Efforts to conduct (or have conducted) the Core Development Activities allocated to it under this Section 4.3 (i.e., the UGNX Core Development Activities in the case of UGNX and the KHK Core Development Activities in the case of KHK) in accordance with the Core Development Plan and Applicable Laws. The non-Lead Development Party shall provide the Lead Development Party such timely assistance as reasonably requested by the Lead Development Party to enable such Party to perform its obligations and accomplish the activities allocated to such Party under the Core Development Plan. Subject to the terms of this Agreement and the requirements of Applicable Laws, the Lead Development Party shall make all decisions relating to the Day-to-Day Core Development Activities allocated to it under this Section 4.3, including all decisions related to the matters set forth on Schedule 4.3.2, provided such decisions are consistent with the then-current Core Development Plan. In addition, UGNX will update the JDC or KHK as reasonably requested with respect to the UGNX Core Development Activities. The Lead Development Party shall inform the JDC in writing as soon as reasonably practicable about any unforeseen and/or material results, problems, difficulties or issues in connection with its respective Core Development Activities.

4.4 **INTENTIONALLY DELETED.**

4.5 **Additional Indications**. At any time prior to [***] months before the [***], either Party may propose to Develop in the Profit Share Territory Licensed Products for one or more additional indications in the Field (i.e., in addition to the First Indication) by submitting to the JDC a written proposal for the Development thereof, including a proposed work plan, budget and timeline. Upon mutual written agreement by the Parties to Develop such indication(s), the JDC shall update the Core Development Plan to include such indication consistent with Section 4.2. Until the Profit Share Territory Transition Date, UGNX shall use Commercially Reasonable Efforts to Develop at least one additional indication mutually agreed-upon by the Parties pursuant to the foregoing sentence (each such indication, an "**Additional Indication**") and shall complete any On-Going Clinical Trials. For the avoidance of doubt, subject to the terms of this Agreement, on and after the [***].

4.6 **KHK Manufacturing-Related Development Activities**. Subject to the terms of this Agreement and the requirements of Applicable Laws, KHK will retain all rights and responsibility to conduct at its cost (i.e., not subject to profit/loss sharing under Section 7.1.1) all necessary Development activities related to the manufacture and supply of the Licensed Products, including process development, manufacturing scale-up, development-stage and commercial-stage manufacturing, quality assurance/quality control procedure development, and compilation and reporting of CMC information ("**Manufacturing-Related Development Activities**").

4.7 **Initial Information and Regulatory Filings Transfer**. Within a reasonable period of time after the Effective Date and in accordance with the Core Development Plan, KHK shall provide to UGNX (a) the data (tables, listings and figures) and final report on completed Clinical Trial, (b) the interim data (tables, listings and figures) on Phase 1/2 extension study (KRN23-INT-002), and (c) the final reports on completed non-clinical studies related to the Licensed Products. In addition, within a reasonable period of time following the Effective Date, KHK shall transfer to UGNX the registration as IND holder for the Licensed Products in the U.S. and all Regulatory Filings (for the Profit Share Territory, the European Territory and Latin America), including any INDs, CTAs or their equivalents, including all such filings related to the 001 and 002 Studies, in each case solely for the purpose of the Development and Commercialization of the Licensed Products by UGNX as contemplated by this Agreement. For the avoidance of doubt, KHK will have no obligation to translate any such results and other information into English (or any other language).

4.8 **Development Data.**

4.8.1 **Obligations of UGNX**. The results of all Core Development Activities, including all data collected or analyzed with respect thereto, and all study reports analyzing such data (collectively, "**UGNX Clinical Data**") will be jointly owned by UGNX and KHK. Subject to Applicable Laws, UGNX shall provide KHK free-of-charge with copies of UGNX Clinical Data (in electronic form if requested by KHK and in or reasonably convertible to such electronic form). KHK may use UGNX Clinical Data free-of-charge, and KHK and its Affiliates, licensees and commercialization and development partners will have a right of access, a right of reference and a right to use and incorporate all UGNX Clinical Data in any Regulatory Filings for the Licensed Products in accordance with Applicable Laws in the Profit Share Territory, the European Territory and the Rest of the World. For the avoidance of doubt, KHK may provide the foregoing information (and extend the foregoing rights) to its licensees or commercialization and development partners for the purposes specified in this Section 4.8.1 and otherwise in accordance with this Agreement.

4.8.2 **Obligations of KHK**. During the Term, and subject to Applicable Laws, KHK shall provide UGNX free-of-charge with copies of information or data with respect to the Licensed Products (including non-clinical and clinical study data and results and including all data collected or analyzed with respect thereto, and all study reports analyzing such data) that is (or was prior to the Effective Date) generated by KHK or any Affiliates or licensees or commercialization or development partners of KHK, or by any Third Parties acting on their behalf, which is necessary or useful for UGNX to conduct the UGNX Core Development Activities and UGNX Non-Core Development Activities, or to Commercialize Licensed Products in the Field in the Territory (collectively, "**KHK Clinical Data**"). KHK Clinical Data includes the clinical study reports and Regulatory Filings. However, KHK will have no obligation to provide any translation of KHK Clinical Data to UGNX. UGNX and its Affiliates will have a right of access, a right of reference and a right to use and incorporate all KHK Clinical Data in any Regulatory Filings for the Licensed Products in accordance with Applicable Laws solely for the purposes specified in this Section 4.8.2 and otherwise in accordance with the Agreement.

4.9 **Development Costs**. The obligations of the Parties with respect to Development Costs of the Licensed Products are set forth in this Section 4.9.

4.9.1 **Funding of Core Development Activities**. Development Costs for Core Development Activities shall be allocated as follows:

(a) Pre-Transition Funding. UGNX and KHK shall share equally (50/50) the Development Costs for all of the Core Development Activities incurred after the Effective Date through the day immediately preceding the Profit Share Transition Date (in the Profit Share Territory) or the applicable European Transition Date (in the European Territory), including, for clarity, the 001 and 002 Studies and any On-Going Clinical Trials, until completion of such studies.

(b) Post-Transition Funding. Beginning on the Profit Share Transition Date (in the Profit Share Territory) or the applicable European Transition Date (in the European Territory), KHK will be responsible for [***] ([***]%) of the Development Costs for the Core Development Activities conducted in the Profit Share Territory and the European Territory, respectively, excluding, for clarity, any On-Going Clinical Trials.

(c) Manufacturing-Related Development Costs. Beginning on the Effective Date, KHK shall be solely responsible for one hundred percent (100%) of the Development Costs for any and all Manufacturing-Related Development Activities.

4.9.2 **Funding of Development in Latin America**. UGNX shall be solely responsible for one hundred percent (100%) of the Development Costs for Development activities (other than Core Development Activities) with respect to Licensed Products in the Field (including Development Costs for Phase 4 Clinical Trials) conducted in Latin America.

4.9.3 **Funding of Development for Injection Devices**. The Parties shall separately discuss and agree upon development and cost-sharing for the development of injection devices to be used in connection with the Licensed Products.

4.9.4 **Development Cost Reports**. Each Party shall prepare and deliver to the other Party a [***] report detailing its Development Costs incurred during such period with respect to activities covered by the Core Development Plan. Each Party shall submit any supporting information reasonably requested by the other Party related to such Development Costs included in its report within [***] days after its receipt of such request. The Parties shall conduct a reconciliation of such Development Costs aiming toward agreement within [***] days after receipt

of all such supporting information, and an invoice shall be issued for any unpaid share of the Development Costs identified in such reconciliation. Each Party shall pay all amounts due and payable by such Party under any such invoice within [***] days after its receipt of such invoice and neither Party will have a set-off right with respect to amounts payable pursuant hereto. Each Party shall have the right to audit the records of the other Party with respect to any purported Development Costs included in the reports prepared by such Party in accordance with Section 9.2.

4.10 **No Debarred Personnel**. In performing Development activities under this Agreement, neither Party will use the services of any employee or consultant, who has been debarred by the FDA or any Regulatory Authority or is the subject of debarment proceedings by the FDA or any other Regulatory Authority.

4.11 **Non-Core Development Activities.**

4.11.1 As between UGNX and KHK, subject to the terms of this Agreement and the requirements of Applicable Laws:

(a) KHK will be solely responsible and will have sole discretion and control for all non-clinical, clinical and other Development or Commercialization activities (including regulatory activities) with respect to (i) [***] and (ii) [***] (collectively, the "**KHK Non-Core Development Activities**").

(b) UGNX will be solely responsible and will have sole discretion and control for all non-clinical, clinical and other Development or Commercialization activities (including regulatory activities) with respect to Licensed Products in the Field in Latin America (collectively, the "**UGNX Non-Core Development Activities**").

4.11.2 The responsible Party (i.e., KHK for the KHK Non-Core Development Activities, and UGNX for the UGNX Non-Core Development Activities) ("**Responsible Party**"), through the JDC, shall keep the other Party reasonably informed of all material events and developments, occurring in the course of the non-clinical, clinical and other development activities for the Licensed Products. The Responsible Party shall consider in good faith any recommendations that the other Party may make to the Responsible Party regarding such activities; provided, however, that subject to the terms of this Agreement and the requirements of Applicable Laws, the Responsible Party will have the final decision-making authority with respect thereto.

4.11.3 Notwithstanding the foregoing: (a) KHK shall use Commercially Reasonable Efforts not to cause a material adverse effect on the Development and/or Commercialization of Licensed Products in the Field in the Territory or the European Territory; and (b) UGNX shall use Commercially Reasonable Efforts not to cause a material adverse effect on the Development and/or Commercialization of Licensed Products in the Field in the Territory or the European Territory.

4.12 **Regular Development Updates**. The Parties agree to discuss, through appropriate employees in person or by video or teleconference, the current status of the Development of the Licensed Products in the Field in the Territory and the European Territory as reasonably required or requested by either Party. Updates shall include discussions and consultations between the Parties on topics, in each case as applicable, including: (a) protocol drafting and/or finalization; (b) establishment of, or changes to, the Statistical Analysis Plan (SAP); (c) initial investigator selection after the Effective Date; (d) major investigator meetings; (e) Clinical Study Report (CSR) drafting and/or finalization; (f) regulatory strategy with respect to agency interactions; (g) regulatory meeting requests; (h) agency submissions; and (i) agency responses.

ARTICLE 5.
REGULATORY MATTERS

5.1 **Regulatory Activities**. The obligations of the Parties with respect to Regulatory Activities related to the Licensed Products are set forth in this Article 5.

5.1.1 **Regulatory Activities**. Beginning on the Effective Date and to the extent UGNX remains the Lead Development Party with respect to a particular territory, subject to and in accordance with the terms and conditions of this Agreement and the requirements of Applicable Laws, UGNX, shall: (a) use Commercially Reasonable Efforts to file (or have filed) all Regulatory Filings with respect to the Licensed Products in the Field in order to obtain Marketing Approvals in each country in the Territory and the European Territory (or to obtain the European Centralized Approval in the European Core Territory) and in order to obtain Pricing and/or Reimbursement Approvals in the Profit Share Territory; (b) respond in a timely fashion to requests for data and information from Regulatory Authorities with respect to the Licensed Products in the Field in the Territory and the European Territory; and (c) meet with officials of the Regulatory Authorities at such times as may be requested by such Regulatory Authorities with respect to the Core Development Activities ("**Regulatory Activities**"), provided that KHK will have primary responsibility for obtaining, and UGNX shall provide all assistance reasonably requested by KHK, in relation to Pricing and/or Reimbursement Approvals for the Licensed Products in the Field in the European Territory. For the avoidance of doubt, UGNX will be responsible for obtaining, and KHK will provide all assistance reasonably requested by UGNX, in relation to Pricing and/or Reimbursement Approvals, if any, for the Licensed Products in the Field in the Profit Share Territory as part of the UGNX Core Development Activities, it being understood that the costs incurred by UGNX in connection with such activities will be shared equally (50/50). All such Regulatory Activities will be conducted in a manner consistent with the Core Development Plan and coordinated by the JSC in accordance with Article 3. Without limiting the applicability of the foregoing and the remainder of this Article 5, UGNX shall interface with the applicable Regulatory Authority(ies) and, through the JDC, shall keep KHK reasonably informed of all material events and developments occurring in the course of the Regulatory Activities, including scheduled UGNX regulatory strategy discussions and meetings with Regulatory Authorities in the Territory and the European Territory relating to the Licensed Products in the Field.

5.1.2 **Transfer Regulatory Approvals and Data**. Subject to, and in accordance with, the terms and conditions of this Agreement and the requirements of Applicable Laws, UGNX shall take all steps reasonably necessary to transfer and assign the Marketing Approvals for the Licensed Products in the Field in the Profit Share Territory and the European Territory, the Pricing and/or Reimbursement Approvals for the Licensed Products in the Field in the Profit Share Territory and the UGNX Regulatory Data for the Licensed Products in the Field in the Profit Share Territory and the European Territory to KHK within [***] days after UGNX has obtained Pricing and/or Reimbursement Approval (for the Profit Share Territory) and [***] days after UGNX has obtained Marketing Approval (for the European Territory), in each applicable territory or country, or after such other period required by the regulatory process of such territory or country.

(a) UGNX shall transfer to KHK or KHK's designee all Regulatory Filings (including any INDs, CTAs or their equivalents) unless KHK has provided its prior written consent to the retention of any of the foregoing by UGNX until the day immediately preceding the Profit Share Territory Transition Date or for such longer period as necessary for UGNX to perform Development activities under this Agreement in accordance with Applicable Laws and Marketing Approvals and Pricing and/or Reimbursement Approvals owned by UGNX for the Commercialization of Licensed Products in the Field if and to the extent such transfer is possible, or, if such transfer is not possible under Applicable Laws, then UGNX shall, at KHK's discretion: (i) [***]; or (ii) [***].

(b) UGNX shall provide KHK with copies of all material correspondence between UGNX and any Regulatory Authorities with respect to such Regulatory Filings, Marketing Approvals and Pricing and/or Reimbursement Approvals for the Licensed Products in the Field and all other clinical and non-clinical data, records and tabulations, in all such cases with respect to the Licensed Products in the Field.

(c) Promptly after the Profit Share Territory Transition Date in each country in the Profit Share Territory and the applicable European Transition Date in the European Territory, except to the extent UGNX retains responsibility for the conduct of On-Going Clinical Trials for Licensed Products in the Field, UGNX shall assign to KHK all agreements specific to the conduct of such Clinical Trials in the applicable territory or country (to the extent assignable and excluding any such agreements that also involve clinical trials for other UGNX products that are not Licensed Products), including agreements or contracts with contract research organizations, clinical sites and investigators, between UGNX and any Third Party, subject to any consent required by such Third Party, which consent UGNX will use Commercially Reasonable Efforts to obtain on behalf of KHK.

(d) UGNX shall provide KHK with copies of all reports and data obtained by UGNX or its Affiliates pursuant to this Agreement regarding the Development and the Commercialization of the Licensed Products in the Field under this Agreement, including any UGNX Clinical Data.

5.1.3 **KHK Assistance**. Upon request of UGNX, KHK shall use Commercially Reasonable Efforts to assist UGNX in connection with any meetings with, or requests from, Regulatory Authorities in the Territory and the European Territory related to Licensed Products in the Field; provided, however, that the costs in connection with the Core Development Activities shall be shared equally (50/50), the costs in connection with CMC activities shall be [***] and the costs in connection with the UGNX Non-Core Development Activities shall be [***].

5.1.4 **UGNX Assistance**. Upon request of KHK, following the conclusion of the Profit Sharing Period, UGNX shall use Commercially Reasonable Efforts to assist KHK at KHK's cost in connection with any meetings with, or requests from, Regulatory Authorities with respect to Licensed Products in the Field in any country of the world where KHK is the Lead Development Party.

5.2 **Regulatory Data and Approvals.**

5.2.1 **Regulatory Filings**.

(a) Review. The JDC will coordinate communication and the exchange of information between the Parties with respect to Regulatory Filings to be prepared and submitted by or for UGNX for Licensed Products in the Field in the Territory and the European Territory; and without limiting the foregoing, UGNX shall provide KHK with copies of all such Regulatory Filings (including any protocols to be included in such filings) (in English) in the Territory and the European Territory to review prior to filing thereof. It is acknowledged by the Parties that given time constraints, the Regulatory Filing actually submitted by UGNX to a Regulatory Authority may vary from the matters discussed by the Parties in the JDC because of changes resulting from interactions with Regulatory Authorities and from continued work on such filings by UGNX's regulatory personnel.

(b) Copies. Subject to Applicable Laws, UGNX shall provide to KHK: (i) [***]; (ii) [***]; and (iii) [***]. KHK will have a right of access, a right of reference and the right to use and incorporate all UGNX Regulatory Data in connection with the Licensed Products and in a manner consistent with the relevant terms of this Agreement (e.g., outside the Territory except to the extent required to perform any of its obligations hereunder). For the avoidance of doubt, subject to Article 11 and the relevant terms of the Agreement, KHK may provide copies of such Regulatory Filings (and extend its right of access, right of reference and the right to use and incorporate all UGNX Regulatory Data in connection with Licensed Products into regulatory submissions outside the Territory) to its licensees or commercialization or development partners solely for the Commercialization or the Development of Licensed Products. KHK will have no obligation to translate Regulatory Filings outside the Territory into English (or any other language).

5.2.2 **Regulatory Meetings**. UGNX shall provide KHK with advance notice of any formal, scheduled meetings with any Regulatory Authority in the Territory or the European Territory with respect to Licensed Products in the Field (including any meetings related to the final positioning of labeling and safety claims within the original and subsequent regulatory submissions), and UGNX shall provide a brief description of the topics to be presented or discussed at that meeting. Subject to Applicable Laws, UGNX shall allow KHK and/or its Affiliates to attend any such meeting (without any obligation on KHK to do so), and to participate in the meeting preparation process.

5.3 **Provision of Regulatory Information to UGNX**. KHK shall use Commercially Reasonable Efforts to support UGNX in connection with its conduct of all Regulatory Activities by providing relevant documents and information in KHK's possession. Furthermore, KHK shall provide to UGNX manufacturing and CMC information solely for fulfilling regulatory requirements (e.g., for Regulatory Filings) in the Profit Share Territory, the European Territory and Latin America.

5.4 **Safety; Safety Agreement**. Within [***] months of the Effective Date, the JSC will develop a mutually acceptable safety agreement (to be agreed upon and executed by both Parties) setting forth the Parties' respective obligations in detail regarding pharmacovigilance and the exchange of drug safety data for Licensed Products (the "**Safety Agreement**"). The Safety Agreement will include applicable timelines and scope for reporting (including adverse event data collection and analysis) between UGNX and KHK (or their respective Affiliates) that will: (a) enable each Party to comply with its respective reporting requirements to Regulatory Authorities in the Territory and the European Territory and to satisfy its duty of care with respect to the Drug Substance and the Licensed Products as required by Regulatory Authorities in the Territory and the European Territory (it being understood that, following the transfer of the Marketing Approvals and the Pricing and/or Reimbursement Approvals to KHK, KHK shall be responsible for complying with all associated regulatory requirements, including safety reporting requirements); (b) enable KHK to comply with its reporting requirements to Regulatory Authorities outside the Territory, and (c) ensure worldwide safety surveillance.

5.5 **Recalls and Voluntary Withdrawals.**

5.5.1 For each country in the Territory, UGNX and KHK shall, through the JSC, confer and coordinate regarding their respective internal standard operating procedures (and any changes thereto) regarding product recalls and the treatment of and response to product complaints and inquiries as to safety, quality or efficacy that may be relevant to the Licensed Products.

5.5.2 If either Party becomes aware of information about a Licensed Product indicating that it may not conform to the applicable Specifications or that there are potential adulterations, misbranding and/or other material adverse issues regarding safety of a Licensed Product (or otherwise that a recall or withdrawal of a Licensed Product is potentially at issue), such Party shall notify the other Party as soon as practical (but in any event within such period as the Parties may mutually establish in order to ensure their respective compliance with Applicable Laws). With respect to in the Territory, the Parties shall promptly meet to discuss such circumstances and to consider appropriate courses of action, including Licensed Product recalls.

5.6 **Inspection Rights**. Not more than [***] per year, if either Party (the "**Inspecting Party**") has any reasonable concerns regarding the other Party's or its Affiliates' storage, handling or manufacturing of any Licensed Products, the Inspecting Party will have the right, at the Inspecting Party's expense and on not less than [***] days' prior notice, to inspect the facilities where the other Party or its Affiliates store, handle or manufacture, or have stored, handled or manufactured, any Licensed Products and to audit the procedures of such other Party or its Affiliates for the storage, handling and/or manufacturing of Licensed Products for purposes of quality control. The Safety Agreement will contain additional customary mutual provisions addressing inspection rights for cause.

5.7 **Governmental Inspections and Inquiries**. Each Party shall advise the other Party promptly, but in no event later than [***] days after such Party's receipt of notice thereof, of any planned Regulatory Authority visit to the portion of the facilities of the receiving Party or its Affiliates where Licensed Products are Developed, stored or handled or any material written inquiries by a Regulatory Authority concerning such facilities, the procedures of the receiving Party or its Affiliates for the Development, storage or handling of Licensed Products, or the Commercialization of Licensed Products in the Territory or the European Territory, as applicable. If the Regulatory Authority makes an unannounced or unplanned visit, or if the receiving Party does not have at least [***] days notice of the visit, the receiving Party shall inform the other Party of the visit within one (1) Business Day after the receiving Party obtains actual knowledge of the visit. The receiving Party shall inform the other Party as soon as practicable regarding the purpose and result of such visit or inquiry, and shall provide to the other Party copies of any minutes of the inspection generated by the receiving Party promptly following such inspection and any report or correspondence provided by the receiving Party, or any Affiliate, as the case may be, to the Regulatory Authority or issued by or provided by the Regulatory Authority to the receiving Party, or any Affiliate, as the case may be, in connection with such visit or inquiry. The receiving Party shall advise the other Party of the material aspects of such minutes and correspondence at the next JSC meeting.

5.8 **Regulatory Matters in the Rest of the World**. KHK, through the JSC, shall keep UGNX reasonably informed of significant events occurring in the course of the regulatory activities with respect to the Licensed Products in the Rest of the World or any KHK Out-of-Field Products, to the extent relevant to issues pertaining to the Licensed Products in the Territory and the European Territory. UGNX shall provide all reasonable cooperation to KHK with respect to regulatory activities related to the Licensed Products in the Rest of the World including providing access to UGNX's relevant facilities in the event of an inspection by applicable Regulatory Authorities.

ARTICLE 6.
SALES, PROMOTION AND MARKETING; DILIGENCE OBLIGATIONS

6.1 **Sales, Promotion and Marketing Activities**. The obligations of the Parties with respect to Sales, Promotion and Marketing Activities related to the Licensed Products are set forth in this Section 6.1.

6.1.1 **General**. Subject to and in accordance with the terms and conditions of this Agreement and the requirements of Applicable Laws, each Party shall promote, market and sell, as applicable, the Licensed Products in its respective territory (“**Sales, Promotion and Marketing Activities**”) as set forth in this Section 6.1, and shall keep the other informed through the JCC as to plans and activities regarding Commercialization of the Licensed Products. As appropriate, the Parties shall discuss shared global commercialization activities that benefit the Licensed Products both in the Territory and outside the Territory.

6.1.2 **Sales, Promotion and Marketing in the Profit Share Territory**. KHK shall book sales of the Licensed Products in the Field in the Profit Share Territory. During the [***] following the First Commercial Sale in the U.S., UGNX will have the exclusive right and responsibility, and shall use Commercially Reasonable Efforts, to promote the Licensed Products in the Field in the Profit Share Territory in order to maximize sales of the Licensed Products in the Profit Share Territory. During the period of time commencing on [***], KHK will have the right to increasingly participate in the promotion of the Licensed Products in the Profit Share Territory [***], and UGNX shall continue to promote the Licensed Products in the Profit Share Territory and use Commercially Reasonable Efforts in assisting KHK in the transition of the promotion activities for the Licensed Products in the Profit Share Territory. UGNX shall provide all information, documents (including originals or copies, as applicable) and other assistance reasonably requested by KHK in order to allow KHK to market and promote the Licensed Products in the Profit Share Territory as of the Profit Share Territory Transition Date. Beginning on or after the Profit Share Territory Transition Date, KHK will have the exclusive right, and shall use Commercially Reasonable Efforts, to market and promote the Licensed Products in the Field in the Profit Share Territory [***], provided that UGNX will have the right to continue to promote the Licensed Products in the Profit Share Territory [***] using its own sales force with respect to activity targeting Medical Geneticists and UGNX shall use Commercially Reasonable Efforts to promote the Licensed Products to such key subscribers for Medical Geneticists. During the Profit Sharing Period, if KHK fails to use Commercially Reasonable Efforts to sell the Licensed Products in the Field in the Profit Share Territory, KHK will follow reasonable suggestions from UGNX regarding how KIM should satisfy this obligation.

6.1.3 **Sales, Promotion and Marketing in Latin America**. UGNX shall book sales of the Licensed Products in the Field in Latin America. During the Term, UGNX will have the exclusive right and responsibility to sell, promote and market the Licensed Products in the Field in Latin America. Such right shall include the right to sell and otherwise provide Licensed Products as part of Named Patient Sales in each country in Latin America in accordance with Applicable Laws, and shall be responsible for all associated costs.

6.1.4 **Sales, Promotion and Marketing in the European Territory**. KHK shall book sales, sell, market and promote the Licensed Products in the Field in the European Territory.

6.1.5 **Named Patient Sales in the European Territory**. KHK retains the exclusive right and responsibility to sell and otherwise provide the Licensed Products in the European Territory as part of Named Patient Sales, and shall be responsible for all associated costs (i.e., not subject to cost-sharing). For clarity, UGNX shall not have the right to sell or otherwise provide Licensed Products in the European Territory as part of Named Patient Sales.

6.1.6 **Marketing Guidelines**. Without limiting the generality of the other provisions of this Article 6, the Parties shall agree upon guidelines for the marketing and sale of Products in the Territory and the European Territory at least [***] prior to the expected date of the First Commercial Sale of the Licensed Products in the Territory and the European Territory. Such guidelines shall be included in the relevant Marketing Plans and UGNX and KHK shall abide by the guidelines.

6.1.7 **Marketing Plans and Marketing Budgets**.

(a) Marketing Plans. Without limiting the generality of the other provisions of this Article 6, UGNX shall prepare and submit to the JCC for review (but not approval) a plan containing the strategy and proposed activities (described generally) for marketing and selling Licensed Products in each country in the Territory (as updated pursuant to this Section 6.1.7, the "**Marketing Plan**"). UGNX shall submit a proposed draft of the Marketing Plan, including Phase 5 Clinical Trials, samples, supplies provided to patients as part of "compassionate use" programs in the Profit Share Territory to the JCC for review (but not approval) no later than [***] months prior to the anticipated date of the First Commercial Sale of any Licensed Product in the applicable country. The initial Marketing Plan shall cover the period through the day before the Profit Share Territory Transition Date. UGNX shall annually by [***] update the Marketing plan covering the rest of the period through the day before the Profit Share Territory Transition Date and prepare each year a detailed annual Marketing Plan for each country in the Territory covering the following Calendar Year. This will be subject to review (but not approval) by the JSC. All decisions regarding the Sales, Promotion and Marketing Activities within Latin America consistent with the Marketing Plan will be determined solely by UGNX. Sales forecasts with respect to the Profit Share Territory and Latin America shall be made on a [***] updated [***].

(b) Marketing Budgets. Without limiting the generality of the other provisions of this Article 6, UGNX shall prepare and submit to the ICC for review and approval with each Marketing Plan a marketing budget (the "**Marketing Budget**"), which shall set forth the budgeted amounts for Commercialization Costs for Licensed Products in the Profit Share Territory, it being understood that personnel costs will be allocated based on the percentage of time dedicated by such personnel to the Commercialization of such Licensed Products. Such Marketing Budget shall contain an annual breakdown for the period from the date when pre-commercial Commercialization Costs are posted for the first time through the Profit Share Territory Transition Date. At the end of [***] of each year thereafter, UGNX shall update such Marketing Budget and prepare a detailed annual Marketing Budget with a detailed [***] breakdown covering the following Calendar Year in the Profit Share Territory. Notwithstanding foregoing, in the event actual costs and expenses are expected to exceed the annual Marketing Budget, the solution and/or amendment of the Marketing Budget, shall be promptly agreed at the JSC. The initial Marketing Budget and each annual Marketing Budget in the Profit Share Territory will be subject to the unanimous approval of the JSC. In addition, except as otherwise mutually agreed upon by the Parties, headcount for all sales representatives (including medical liaisons) shall not exceed [***]. UGNX shall submit a proposed draft of the Marketing Budget for a country, or countries, in the Profit Share

Territory to the JCC for review and no later than [***] months prior to the anticipated date when pre-commercial Commercialization Costs are posted for the first time for any Licensed Product in such country. If the initial submission timing falls in [***], the proposed draft shall contain [***] breakdown of the following Calendar Year's budget. In the event that the JSC does not unanimously approve the initial Marketing Budget thereafter, pursuant to Section 3.5.1, either Party may initiate procedures to finally resolve the issue pursuant to Section 16.4.

6.1.8 **Marketing Plans for the European Territory**. KHK shall prepare and submit to the JCC for review (but not approval) a plan containing the strategy and proposed activities (described generally) for marketing and selling Licensed Products in each country in the European Territory no later than [***] months prior to the anticipated date of the First Commercial Sale of any Licensed Product in such country. All decisions regarding the Sales, Promotion and Marketing Activities including budget within the European Territory will be determined solely by KHK.

6.1.9 **Product Complaints**. UGNX shall report to KHK in a manner reasonably designated by KHK from time to time all complaints that are received from any direct or indirect purchasers of the Licensed Products from UGNX involving labeling or packaging of the Licensed Products or other problems not related to the medical effectiveness of the Licensed Products and that could reasonably negatively affect the high-quality image of the Licensed Products, KHK or UGNX. Such reports shall include the following information and/or other information as reasonably specified by KHK: (a) the person or entity that initiated the complaint; (b) the nature of the complaint; (c) the geographic area in which the complaint originated; and (d) whether any return samples of the Licensed Product are available.

6.2 **Sales Forecasts**. UGNX shall provide forecasts with respect to its commercial requirements for sale of the Licensed Products in the Territory in accordance with the terms and conditions to be set forth in the Commercial Supply Agreement.

6.3 **Labeling and Patent Rights Marking**. Subject to Applicable Laws, UGNX shall identify KHK as the licensor or manufacturer of the Licensed Products using the KHK Trademarks as designated by KHK for such use in certain mutually agreed promotional materials for Licensed Products in the Territory where such identification is appropriate, in a manner approved in advance in writing by both Parties and in accordance with (and subject to) the trademark license set forth in Section 2.3. To the extent reasonably and customary in the industry for such products, UGNX shall mark all Licensed Product sold by UGNX with appropriate Product Trademarks and patent numbers, to the extent permitted by Applicable Laws in the country within the Territory where such Licensed Products are sold.

6.4 **Marketing Materials.** All marketing and promotional literature related to the Licensed Products for use in the Field in the Territory and the European Territory by the Parties ("**Marketing Materials**") will be prepared in consultation with the JCC in a manner consistent with Applicable Laws, provided that UGNX will have the authority to make any final decision (a) in Latin America and (b) in the Profit Share Territory through the day immediately preceding the Profit Share Territory Transition Date, and KHK shall have the authority to make any final decision (a) in the European Territory and (b) in the Profit Share Territory beginning on the Profit Share Territory Transition Date. In particular, UGNX shall use Commercially Reasonable Efforts in facilitating a smooth transition in preparation for KHK's Sales, Promotion and Marketing Activities in respect of the Profit Share Territory.

ARTICLE 7.
FINANCIAL TERMS

7.1 **Profit-Sharing.**

7.1.1 Profit Sharing Calculation.

(a) UGNX and KHK shall share equally (50/50) in the net income or net loss derived from sales of Licensed Products in the Profit-Sharing Period in the Field in the Profit Share Territory with net income or loss calculated as follows:

(i) From the Effective Date through [***]:

Net Sales – [***]% of [***]

(ii) From [***] through [***]:

Net Sales – [***]% of [***]

7.1.2 **Commercialization Cost Reports**. Within [***] days following the end of each [***], each Party shall submit to the other Party a [***] report detailing its Commercialization Costs incurred during such period with respect to activities covered by the applicable Marketing Plan for the Licensed Products in the Field in the Profit Share Territory on a country-by-country basis. Each Party shall submit any supporting information reasonably requested by the other Party related to such Commercialization Costs included in its report within [***] days after its receipt of such request. The Parties shall conduct a reconciliation of such Commercialization Costs aiming to reach agreement within [***] days after receipt of all such supporting information, and an invoice will be issued for any unpaid share of the Commercialization Costs identified in such reconciliation. Each Party will have the right to audit the records of the other Party with respect to any purported Commercialization Costs included in the reports prepared by the other Party in accordance with Section 9.2.

7.1.3 **Profit Sharing Period**. The profit-sharing period for the Licensed Products in the Field in the Profit Share Territory means the period commencing on the First Commercial Sale in the U.S. and ending on the day immediately preceding the [***] anniversary of the First Commercial Sale in the U.S. **(**"**Profit-Sharing Period**").

7.1.4 **Pre-Commercial Activity Cost Sharing**. Development Costs will be funded and shared as set forth in Section 4.9. The Parties shall share equally (50/50) all reasonable, mutually agreed upon in writing, pre-commercial Commercialization Costs (other than Development Costs) incurred by UGNX or KHK, if any, in the Field in the Profit Share Territory.

7.2 **Sales Royalties and Revenue Share.**

7.2.1 **Royalties Payable to UGNX in the European Territory.** Subject to the terms and conditions of this Agreement, KHK shall pay royalties to UGNX for each Calendar Year during the applicable Royalty Term in an amount equal to [***] percent ([***]%) of Net Sales of any Licensed Products in the Field in a country in the European Territory ("**UGNX Sales**

Royalties in the European Territory"), provided that, if KHK or any of its Affiliates or Sublicensees obtains Marketing Approval for an additional indication in the Field, the payment of the UGNX Sales Royalties in such country for such additional indication shall be conditioned on UGNX having obtained the Marketing Approval in such country in the First Indication.

7.2.2 **Revenue Share Payable to UGNX in the Profit Share Territory.** Subject to the terms and conditions of this Agreement, KHK shall pay a revenue share to UGNX for each Calendar Year on Net Sales of any Licensed Products in the Field at the rates set forth in this Section 7.2.2 during the applicable Royalty Term in the Profit Share Territory ("**UGNX Revenue Share in the Profit Share Territory**"), provided that, if KHK or any of its Affiliates or Sublicensees obtains Marketing Approval for an Additional Indication, the payment of the UGNX Revenue Share in a country in the Profit Share Territory for such Additional Indication shall be conditioned on UGNX having obtained the Marketing Approval in such country in the First Indication. For the avoidance of doubt, Section 7.1 will not apply in the Profit Share Territory during such Royalty Term.

Net Sales (US$)	Revenue Share (% of Net Sales)
Portion of Net Sales less than or equal to [***]	[***]%
Portion of Net Sales more than [***] but less than or equal to [***]	[***]%
Portion of Net Sales in excess of [***]	[***]%

7.2.3 **Royalties Payable to KHK**. Subject to the terms and conditions of this Agreement, UGNX shall pay royalties to KHK for each Calendar Year during the applicable Royalty Term in an amount equal to [***] percent ([***]%) of the Net Sales of the Licensed Products in the Field in Latin America on a country-by-country basis ("**KHK Sales Royalties**").

7.2.4 **Payments and Reports**. Each royalty or revenue share payment will be non-refundable and non-creditable against any other payments due hereunder. Each Party will make royalty or revenue share payments contemplated by this Section 7.2 in arrears, within [***] days from the end of each Calendar Quarter in which the underlying Net Sales occur. Each royalty or revenue share payment will be accompanied by a report for each country in the Territory and the European Territory in which sales of any Licensed Products occurred in the Calendar Quarter, specifying: (a) the gross sales (if available) and the Net Sales (including a statement of the aggregate deductions taken from gross sales in the calculation of Net Sales) on a Licensed Product-by-Licensed Product and a country-by-country basis, in each country's currency; (b) the applicable royalty or revenue share rate under this Agreement; (c) the royalties or revenue share payable in the country's currency where the Net Sales occurred; (d) the applicable exchange rate to convert from each country's currency to U.S. dollars pursuant to Section 7.5; and (e) the royalties or revenue share payable in U.S. Dollars. For the avoidance of doubt, no royalties or revenue share shall be due or payable by either Party to the other Party with respect to Net Sales of Licensed Products in a given country before or after the Royalty Term for such country.

7.3 **Method of Payment**. Unless otherwise expressly provided, each Party shall make payments owed to the other Party under this Article 7 in arrears, within [***] days from the end of each Calendar Quarter in which such payment accrues. All payments due hereunder will be made by wire transfer of immediately available funds in U.S. Dollars to a bank account or bank accounts designated by the Party to whom the payment is owed.

7.4 **Interest on Overdue Payments**. Any undisputed amounts not paid by either Party when due under this Agreement will be subject to interest from and after the date payment is due through and including the date upon which such Party makes such payment in immediately available funds at an annual rate equal to the sum of 300 basis points over the prime rate of interest quoted in the Money Rates section of the *Wall Street Journal* (New York Edition), calculated daily on the basis of a three hundred sixty (360) day year, or a similar reputable data source, or if lower, the maximum rate permitted by Applicable Laws.

7.5 **Foreign Currency Exchange**. For any currency conversion from the currency of one country in which the Licensed Products are sold into U.S. Dollars (or another currency if applicable) required in determining the amount of Net Sales or any royalties or revenue share due hereunder, such conversion shall be calculated at the conversion rate as reported in the *Wall Street Journal* (New York Edition) (or if that is no longer published, at the exchange rate reported by The Bank of Tokyo-Mitsubishi UFJ, Ltd.) on the last Business Day of the applicable quarterly period in which the Net Sales are determined, or such other method agreed to by the Parties in writing. Likewise in any other situation in which a currency conversion calculation is required, the conversion rate published on an appropriate date by the *Wall Street Journal* (New York Edition) (or if that is no longer published, at the exchange rate reported by The Bank of Tokyo-Mitsubishi UFJ, Ltd.) shall be used.

7.6 **Taxes.**

7.6.1 **No Withholdings.** All payments required to be made by one Party to the other Party under this Agreement shall be made free and clear of, and without reduction for, withholding Tax or similar Taxes; provided, however, that if a Party or any of its Affiliates is required by Applicable Laws to deduct or withhold any such Taxes from payments made under this Agreement, then such Party or its Affiliates, as applicable, shall make such deduction or withholding from such payment and pay the full amount deducted or withheld to the relevant governmental authority in accordance with Applicable Laws. If, under Applicable Laws, such Tax is required to be deducted or withheld, the paying Party or its Affiliates shall promptly furnish the other Party with reasonable evidence of such deduction or withholding and payment thereof to the relevant governmental authority, in electronic or written form. The Parties shall reasonably cooperate in completing and filing documents required under the provisions of any Applicable Laws in connection with the making of any required Tax payment or withholding payment, or in connection with any claim to an exemption from, reduction of, or a refund of or credit for any such payment to the extent available under Applicable Laws.

7.6.2 **Other Tax Liability**. Except as provided to the contrary in this Agreement, each Party shall be solely responsible for all federal, state and local Tax liability arising from this Agreement imposed on such Party by the taxing authority of a jurisdiction in which such Party is resident or is otherwise subject to such Tax liability. In the case of value added or similar Taxes incurred by a Party with respect to payments made to a Party hereunder or the activities underlying such payments ("**VAT**"), each Party and their Affiliates shall use Commercially Reasonable Efforts to secure available exemption(s) from VAT and/or to cooperate with the other Party's efforts to obtain maximum recovery of VAT paid or incurred by such Party or any Affiliate, to the extent permitted by Applicable Laws.

7.6.3 **Treatment of Royalties and Revenue Shares for Tax Purposes**. To the extent permitted by Applicable Laws, the Parties intend and agree to treat for income Tax purposes (i) the revenue shares payable pursuant to Sections 7.2.2 and sub-Sections (d) and (e) of Section 15.5 as business profits within the meaning of Article 7 of the Income Tax Convention for

the Avoidance of Double Taxation between Japan and the United States **(**"**U.S.-Japan Treaty**") and the corresponding (Business Profits) article of any equivalent income tax treaties that may apply to such payments and (ii) the royalties payable pursuant to Sections 7.2.1, 7.2.3 and sub-Sections (a), (b), (c), (f) and (g) of Section 15.5 as consideration for the use of, or the right to use, a patent or patents, a secret process, or information concerning industrial, commercial or scientific experience within the meaning of U.S.-Japan Treaty and any equivalent income tax treaties that may apply to such payments.

7.7 **Prohibited Payments**. Notwithstanding anything to the contrary in this Agreement, if either Party is prohibited from making any payments by virtue of the statutes, laws, codes or governmental regulations of the country from which the payment is to be made, then such payment may be paid by depositing funds in the currency in which it accrued to the other Party's account in a bank reasonably acceptable to the other Party in the country whose currency is involved.

ARTICLE 8.
KHK SUPPLY OF LICENSED PRODUCT; SPECIFICATIONS

8.1 **KHK Obligation to Supply Licensed Product**. UGNX shall obtain one hundred percent (100%) of its requirements of the Licensed Products for Development and Commercialization in the Field in the Territory and the European Territory from KHK, and KHK agrees to manufacture or have manufactured and to supply to UGNX all of UGNX's requirements of the Licensed Products for Development and Commercialization in the Field in the Territory and the European Territory, in all such cases except to the extent otherwise provided in, and in any event subject to and in accordance with, the terms of this Article 8.

8.2 **Supply of Licensed Products for Development**. Subject to the terms and conditions of this Agreement, KHK shall use Commercially Reasonable Efforts to supply UGNX with quantities of the Licensed Products (for purposes of this Section 8.2, "Licensed Products" shall be deemed to refer to "unlabeled, GMP vialed Licensed Products." KHK currently has Licensed Product in the following concentrations and vial sizes: [***]. KHK shall use Commercially Reasonable Efforts to provide Licensed Product, Drug Substance and any placebo required for UGNX's Development activities with respect to the Licensed Products in the Territory and the European Territory in accordance with UGNX's forecasts and orders therefor, as provided below. UGNX shall use the Licensed Products supplied by KHK pursuant to this Section 8.2 solely in order to conduct Development activities in accordance with the terms and conditions of this Agreement, and shall not use such Licensed Products for any other purpose.

8.2.1 **Forecasts and Orders.** UGNX shall keep KHK reasonably informed of its anticipated requirements of the Licensed Products for Core Development Activities and Non-Core Development Activities through the JDC and the Core Development Plan and Non-Core Development Plan by providing a good faith estimate of such requirements on a [***] basis, the first [***] months of which shall be binding, to be updated [***], (collectively, the "**UGNX Forecast**"). UGNX shall order Licensed Products for use in Development from KHK by providing KHK with a binding purchase order (consistent with the terms and conditions of this Agreement) indicating the quantities of the Licensed Products ordered for Development purposes, the requested delivery date and the destination delivery location. Upon receipt of any such binding purchase order, KHK shall use Commercially Reasonable Efforts to manufacture and supply the Licensed Products in accordance therewith. Within [***] Business Days of receiving a binding purchase order, KHK shall notify UGNX with confirmation of such purchase order, it being understood that KHK may not reject a purchase order if such order is materially consistent with the UGNX Forecast and the required delivery date is at least [***] after the date of such purchase order.

8.2.2 **Cost of Supply of Products for Development**. The price for the Licensed Products, the Drug Substance and any placebos required in order for UGNX to conduct non-clinical and clinical studies required for Development will be at (a) [***] and (b) [***].

8.2.3 **Delivery**. KHK shall: (a) deliver the Licensed Products FCA KHK's or the Third Party manufacturer's facility selected in accordance with Section 8.5.1 (Incoterms 2000); (b) if requested by UGNX, obtain any necessary export permits for such delivery, at UGNX's sole expense; (c) deliver all Licensed Products by the delivery dates established under Section 8.2.1 and otherwise in conformance with UGNX's order (except that KHK may elect to split the order into multiple shipments so long as the delivery dates for the entire order are not materially delayed thereby). Title to and risk of loss in the Licensed Products will pass to UGNX upon delivery to the common carrier for delivery to UGNX or its designee (each, a "**Delivery**"), and UGNX shall be responsible for paying any freight, delivery and insurance charges incurred in delivering the Licensed Products to UGNX's designated delivery destination(s) (subject to cost-sharing except in Latin America), provided that KHK shall provide reasonable assistance to UGNX in identifying appropriate carriers. KHK shall provide appropriate documentation (including certificates of analysis, GMP declaration statements and other documentation required by Regulatory Authorities or otherwise to comply with Applicable Laws of the Profit Share Territory and European Territory) with all shipments of Licensed Products hereunder. KHK shall not be responsible for complying with any additional or differing legal or regulatory requirements specific to Licensed Products for Latin America.

8.2.4 **Responsibilities of UGNX**. With respect to the importation of the Licensed Products to Latin America, UGNX will be responsible for the following on a case-by-case basis: (a) obtaining all necessary permits for the importation of the Licensed Products; (b) all customs, duties and other governmental charges relating to the importation of the Licensed Products from the manufacturing site to UGNX; (c) storing and clearing the Licensed Products through all customs and importation requirements; (d) having the Licensed Products delivered to UGNX's labeling and packaging facility; and (e) conducting quality control testing, retention of samples and lot release, labeling and packaging of the Licensed Products for distribution, and conducting any and all release testing required in Latin America, all in full compliance with all Applicable Laws. Notwithstanding the foregoing, based on good faith discussions between the Parties, KHK shall, at [***], either (i) transfer to UGNX any testing procedures and assays solely to the extent reasonably necessary for UGNX to Develop and Commercialize Licensed Products in Latin America, or (ii) conduct any release testing required for any country in Latin America on behalf of UGNX, solely to the extent required by relevant Regulatory Authorities. KHK shall not be responsible for complying with any requirements that are specific to Latin America and are in addition to, or differ from, legal or regulatory requirements applicable to the Profit Share Territory and the European Territory.

8.2.5 **Manufacturing Compliance and Quality Assurance by MIK**. KHK shall manufacture or have manufactured Licensed Products in accordance with GYP and Applicable Laws of the Profit Share Territory and the European Territory. For all Licensed Products delivered to UGNX under this Section 8.2, KHK shall conduct quality control, or will cause its Third Party Manufacturing contractor to conduct such testing, for compliance with Specifications and testing required for compliance with GMP and Applicable Laws of the Profit Share Territory and the European Territory. ICHK shall conduct a quality assurance review of all applicable documents and activities for compliance with Applicable Laws of the Profit Share Territory and European Territory, including GMP, with respect to the Licensed Products prior to shipment thereof. KHK shall not be responsible for compliance with Applicable Laws of Latin America but the Licensed Products must comply with Applicable Laws that would have applied had the Licensed Products been made for the Profit Share Territory or European Territory. Further requirements with respect to Licensed Product to be Developed and Commercialized in Latin America shall be set forth in the Commercial Supply Agreement.

8.2.6 **Limited Warranties**. KHK represents and warrants to UGNX that the Licensed Products supplied pursuant to this Section 8.2 will (a) be manufactured and tested in accordance with GMP and Applicable Laws of the Profit Share Territory or European Territory (but not of Latin America), (b) conform to the Specifications therefore in all material respects, and (c) have a shelf life of at least [***] from shipment. Notwithstanding the above, UGNX agrees that initial shipment(s) to UGNX which are tentatively planned to be made prior to the Type C Meeting will only contain vials with shelf life until [***], provided that, for clarity, UGNX may order after the initial shipment(s) additional Licensed Products with a shelf life of at least [***] from shipment as required for Development pursuant to Section 8.2. KHK further agrees to maintain in inventory sufficient bulk Drug Substance and/or vialed, Licensed Product, in KHK's discretion, the aggregate amount of which shall be equivalent to a [***] month supply of vialed Licensed Product, based on UGNX's most recent forecast for the Territory and the European Territory.

8.2.7 **Nonconforming Licensed Product**.

(a) **Acceptance and Rejection by UGNX**. Each shipment of Licensed Product will contain such quality control documentation (e.g., certificates of analysis and compliance, batch release records) as are necessary to show that Licensed Product conforms to the Specifications in all material respects at the time of Delivery and were manufactured in compliance with GMP and Applicable Laws of the Profit Share Territory or European Territory. In the event that UGNX determines within [***] days of UGNX's receipt of each shipment of Licensed Products that any such Licensed Products did not materially conform with the Specifications at the time of Delivery or otherwise comply with the product warranty in Section 8.2.6 or the requirements of the order (such as matching the quantities ordered), UGNX shall provide Notice to KHK thereof, and, if requested by KHK, ship a sample portion of the affected Licensed Products to KHK or its designated Third Party manufacturing site, freight prepaid and properly insured, along with a reasonably detailed statement of the claimed non-conformity and copy of KHK's invoice therefor. UGNX shall retain the balance of the Licensed Products that are subject to review subject to resolution of the rejection and further disposition in accordance with this Section 8.2.7.

(b) **Replacement by KHK**. In the event that KHK agrees that the returned Licensed Products were non-conforming (or such non-conformance is confirmed under Section 8.2.7(c) below), KHK shall replace all of such non-conforming units of Licensed Product, at no cost to UGNX, and KHK shall as soon as practicable deliver to UGNX, freight prepaid, all replacement units of the Licensed Products, along with reimbursement of the shipment and insurance charges for return of the non-conforming Licensed Product. UGNX shall dispose of all non-conforming Licensed Product at KHK's expense. In the event that the quantities of the Licensed Products delivered to UGNX (in one or more shipments) do not match the quantity ordered in any material respect, KHK shall promptly ship (such shipping at KHK's sole expense) the additional Licensed Products required to make up such shortfall; or if the amount shipped exceeds the amount ordered by a material amount, accept only the amount ordered, in which case upon KHK's request and at KHK's sole expense, such additional quantities shall be returned to KHK.

(c) **Disputes Over Non-Conforming Licensed Product**. In the event that KHK disagrees with UGNX's rejection because the Licensed Products are in fact conforming, the Parties shall cooperate to have both UGNX's returned samples and KHK's retained samples from the same production batch of the Licensed Products in dispute analyzed by a mutually acceptable independent testing laboratory of recognized reputation in the pharmaceutical industry,

using the analytical methods, tests and criteria for conformance set forth in the Specifications. The out-of-pocket external costs of such arrangement shall be shared [***] by the Parties, unless and until an alternative determination is made as provided below. The results of such laboratory testing shall be conclusive and binding on the Parties on the issue of compliance of such units of Licensed Product with the Specifications at the time of Delivery. If such independent testing laboratory determines that UGNX's returned samples of such Licensed Product conform to the Specifications, then (i) the applicable Licensed Product shall be deemed to have been improperly rejected by UGNX, and (ii) UGNX shall bear the cost of the independent laboratory testing and all out-of-pocket external costs and expenses of returning the improperly rejected Licensed Product to UGNX. If such independent testing laboratory determines that UGNX's returned samples of such Licensed Product did not conform to the Specifications and that such returned samples conform to the samples for such batch retained by KHK, then KHK shall bear the cost of the laboratory testing, as well as the costs associated with properly-rejected Licensed Product described in Section 8.2.7(b), and KHK shall promptly supply to UGNX conforming Licensed Products in accordance with Section 8.2.7(b).

(d) **Sole Remedy.** Except for Third Party Claims otherwise indemnified under this Agreement, the remedies expressly provided in this Section 8.2.7 will be UGNX's sole and exclusive remedy for the breach of Section 8.2.6 as a result of the delivery by KHK of non-conforming Licensed Products.

(e) **No Liability for Subsequent Events.** In no event will KHK be liable under this Section 8.2.7 for Licensed Products that conformed to the Specifications at the time of Delivery but that ceased to so conform as a result of any event or occurrence, or any action or omission by UGNX, its Affiliate or a Third Party, following Delivery of such License Products.

8.2.8 **Invoice and Payment.** KHK shall invoice UGNX for each shipment of the Licensed Products upon shipment to UGNX at the supply price (if applicable) determined as set forth herein.

8.3 **Commercial Supply of Licensed Product**. Subject to the terms and conditions of this Agreement, UGNX and its Affiliates shall purchase, and KHK shall supply UGNX and its Affiliates, with all quantities of Licensed Products (for purposes of this Section 8.3 "Licensed Products" shall be deemed to refer to "unlabeled, GMP vialed Licensed Products" in the concentrations and vial sizes to be agreed upon by the Parties as required for UGNX's and its Affiliates' Commercialization of Licensed Products in Latin America). UGNX shall use the Licensed Products supplied by KHK under this provision solely to conduct its Commercialization activities, in accordance with the terms and conditions of this Agreement, and shall not use such Licensed Products for any other purpose.

8.3.1 **Commercial Supply Agreement**. The Parties shall start negotiations as soon as practical after the Effective Date and shall execute a definitive commercial supply agreement ("**Commercial Supply Agreement**") for the supply by KHK of the Licensed Products to UGNX and its Affiliates for marketing and sale of such Licensed Products in Latin America. Such Commercial Supply Agreement shall contain the terms and conditions set forth in this Section 8.3, consistent with those set forth in the remainder of this Article 8 (except as provided otherwise in this Section 8.3) and other reasonable and customary terms and conditions. In the event the Parties fail to enter into such a Commercial Supply Agreement for Latin America, and without diminishing the Parties' obligation to enter into such agreement, UGNX will be obligated to purchase from KHK, and KHK will be obligated to sell to UGNX, all of the requirements of UGNX and its Affiliates for the Licensed Products pursuant to the terms of this Article 8, and either Party may refer the matter for resolution as a Dispute under Section 16.2 (Regular Arbitration).

8.3.2 **Cost of Supply of Licensed Products for Commercial Sales in Latin America, and Non Commercial Uses**.

(a) During the Term, ICHK shall supply the Licensed Products to UGNX and its Affiliates for commercial sales (including Named Patient Sales) in Latin America at a cost (the "**Commercial Supply Cost**") based on a percentage of Net Sales where such percentage is [***] percent ([***]%) of Net Sales for Licensed Products sold prior to [***] and [***] percent ([***]%) of Net Sales for Licensed Products sold thereafter.

(b) During the Term, KHK shall supply Licensed Products to UGNX and its Affiliates for non-commercial uses in each country in the Territory following receipt of Marketing Approval (i.e., Phase 5 Clinical Trials, samples, supplies provided to patients as part of "compassionate use" programs in the U.S.) at a cost to be determined by the Parties.

8.3.3 **Term**. The term of Commercial Supply Agreement will be co-terminus with the Term (plus any extensions as described below), unless the Parties mutually agree to extend the term of the Commercial Supply Agreement to cover periods after this Agreement is terminated or expires. The Commercial Supply Agreement will provide that the term of that agreement will be automatically extended on an annual basis after the end of the Term, unless KHK elects to terminate such Commercial Supply Agreement with at least [***] advance Notice (e.g., [***]); provided that KHK's obligation to supply Licensed Product thereunder shall expire upon the earlier of the end of such [***] notice period or the date on which UGNX has transitioned the manufacture of Licensed Products to a new facility or manufacturer.

8.3.4 **Priority Status**. To the extent the available supply of, or capacity to manufacture, the Licensed Products is less than the requirements of UGNX and its Affiliates hereunder together with the requirements of KHK and its Affiliates and their licensees, KHK shall allocate the available Licensed Product in a fair and reasonable manner as between all such interested entities, which shall not be deemed a breach of KHK's supply obligations under the Commercial Supply Agreement. In the event of any shortage in availability of supply of, or capacity to manufacture, Licensed Products as contemplated by this Section 8.3.4, the Parties will discuss appropriate actions in good faith and will cooperate with each other in order to resolve such situation.

8.4 **Handling of Licensed Products**. UGNX shall use Commercially Reasonable Efforts to maintain all registrations necessary in the Territory and the European Territory for the lawful handling of the Licensed Products other than such registrations as have been transferred to KHK pursuant to Section 5.1.2 and shall immediately notify KHK of any denial, revocation or suspension of any such registration. Standard instructions for safe handling of the Licensed Products will be set forth in instructions provided by KHK to UGNX from time to time.

8.5 **General Manufacturing and Supply Provisions.**

8.5.1 Third Party Manufacturer. UGNX hereby acknowledges and agrees that KHK will be entitled, in its sole discretion, to perform any or all of its obligations under this Article 8 by subcontracting any or all of such obligations to Third Party manufacturers (each, a "**Third Party Manufacturer**") in any country. In the event that KHK elects to subcontract any or all of its

obligations under this Agreement to a Third Party Manufacturer, KHK shall use Commercially Reasonable Efforts to: (a) coordinate the transfer of such obligations with UGNX, including provision to UGNX of the right to audit any facility of the Third Party Manufacturer used or to be used for the manufacture of the applicable Licensed Products and (b) involve UGNX in decisions regarding process changes that could affect the comparability of the Licensed Products. KHK will be solely responsible for Development costs resulting from any change to a Third Party Manufacturer.

8.5.2 **Quality Agreement**. The Parties shall enter into a reasonable and customary GMP quality agreement with respect to the Licensed Products to be manufactured by or for KHK and supplied to UGNX hereunder for use in Development of the Licensed Products in the Territory and in the European Territory within [***] months of the Effective Date. A separate GMP quality agreement with respect to Licensed Products supplied to UGNX for Commercial use in Latin America shall be executed between the Parties within [***] months of execution of a Commercial Supply Agreement between the Parties for Latin America.

ARTICLE 9.
RECORDS AND REPORTING

9.1 **Records**. Each Party shall keep and maintain complete and accurate books and records necessary to permit calculation and verification of amounts due under Sections 4.9, 7.1, 7.2 or 15.5. Each Party shall maintain such books and records for five (5) years after the applicable book or record was created, or such longer period as may be required by Applicable Laws.

9.2 **Audits.**

9.2.1 Each Party may, at the electing Party's expense and upon not less than [***] days' prior written Notice to the other Party, elect to have an independent certified public accountant selected by the electing Party and reasonably acceptable to other Party examine, during regular business hours and under a customary non-disclosure agreement, the books and records of the other Party required to be maintained pursuant to Section 9.1 at the electing Party's expense, not more often than [***] each Calendar Year, for the sole purpose of verifying the accuracy of the payments made under Sections 4.9, 7.1, 7.2 or 15.5, and the associated reports furnished by the other Party with respect thereto solely for prior periods covering no more than the last [***] full Calendar Years. Any amounts shown to be owed but unpaid as a result of such audit shall be paid within [***] days from the accountant's report (plus interest on such amounts pursuant to Section 7.4), unless challenged as provided below. Any amounts shown to have been overpaid shall be refunded to the Party that overpaid within [***] days from the accountant's report. The electing Party shall bear the full cost of such audit unless such audit discloses an underpayment of the amount actually owed during the applicable Calendar Year of more than [***] percent [***]%), in which case the other Party shall bear the full out-of-pocket, external cost of such audit.

9.2.2 If the Party that is the object of an audit (the "**Audited Party**") challenges the results of the audit in good faith, such Party shall be entitled at its own cost and expense to obtain a second independent certified public accountant to confirm the accuracy of the first audit. If the results of the confirmatory audit are substantially similar to the results of the first audit, any amounts owed or overpaid by the Audited Party shall be paid or refunded in accordance with the procedures above. If the results of the confirmatory audit are not substantially similar to the results of the first audit, each Party shall

cause its respective auditors to identify the discrepancy and to agree on a final amount owed or overpaid (as the case may be) by the Audited Party that shall be final and binding on the Parties. If the auditors cannot resolve the discrepancy, the Parties shall mutually agree on a third independent certified public accountant to audit the discrepancy and provide a final amount owed or overpaid (as the case may be) by the Audited Party, which shall be binding on the Parties. The costs of such third audit shall be shared [***] by the Parties. Amounts owed or overpaid as determined by such final audit shall be paid or refunded in accordance with the procedures above.

ARTICLE 10.
INTELLECTUAL PROPERTY PROVISIONS

10.1 **Patent Prosecution and Maintenance.**

10.1.1 KHK shall diligently prepare, file, prosecute and maintain the Licensed Patent Rights at KHK's sole cost and expense. Patent counsel selected by KHK will handle all patent filings and prosecutions. KHK shall provide to UGNX a reasonable opportunity to review and comment on any material filings and correspondence with applicable patent offices with respect thereto, and KHK shall use good faith efforts to consider any such comments. KHK shall notify UGNX in the event KHK desires to abandon its efforts to prosecute and maintain Licensed Patent Rights, or decides not to file patent applications for Licensed Patent Rights (other than [***]), which notification will be given within a reasonable period (i.e., with sufficient time for UGNX to take whatever reasonable action may be necessary) prior to the date on which such Licensed Patent Rights will lapse, go abandoned (other than to file a continuation application for the same subject matter) or otherwise diminish UGNX will then have the right, exercisable upon written notification to KHK, to assume full responsibility, at its discretion and its sole cost and expense, to file, prosecute and maintain the Licensed Patent Rights (other than the [***]) in such country or countries. Should UGNX exercise such right, KHK shall execute such documents and perform such acts as may be reasonably necessary for UGNX to so file, prosecute and maintain such Licensed Patent Rights. With respect to the [***], in the event that KHK desires to abandon its efforts to prosecute and maintained such Licensed Patent Rights, KHK shall work with UGNX in good faith and use Commercially Reasonable Efforts to provide UGNX with the opportunity to discuss directly with [***].

10.1.2 KHK will be responsible for any royalty or any other payments required with regard to patents owned by the licensors under the In-Licenses relevant to the Licensed Products in the Field in the Territory and the European Territory.

10.1.3 If it is determined that a license under the [***] patent owned by [***] is required, KHK shall enter into a license for the applicable patent with [***] as necessary to allow the Parties to exercise the rights and perform the obligations set forth in this Agreement, and KHK shall pay the necessary royalties or any other payments under such license. If it is determined that a license under any Third Party patent is required, Section 10.6 shall apply.

10.2 **Ownership of Inventions.**

10.2.1 KHK will retain ownership of all KHK Inventions and UGNX will retain ownership of all UGNX Inventions and the Parties will jointly own Joint Inventions. The Parties shall reasonably cooperate with respect to, and [***], the preparation, filing, prosecution and maintenance of any patents and/or patent applications on any such Joint Inventions. In connection with the foregoing, the Parties shall agree upon a lead Party to administer such filing, prosecution and maintenance of any such patent applications and/or patents on Joint Inventions and the lead Party shall provide the non-lead Party a reasonable opportunity to review, comment on and approve

(such approval not to be unreasonably withheld) in advance any material filings and correspondence with applicable patent offices with respect thereto. Each Party will have the right to abandon any such patents or patent applications on a patent-by-patent (or application-by-application) or country-by country basis, provided that, if the abandoning Party is the lead Party, the lead Party shall provide reasonable advance notification to the non-lead Party (i.e., with sufficient time for the non-lead Party to take whatever reasonable action may be necessary) prior to the date on which such Licensed Patent Rights will lapse, go abandoned (other than to file a continuation application for the same subject matter) or otherwise diminish. Subject to the licenses granted to each Party hereunder in their respective territories, each Party will have full rights to exploit and license such Joint Inventions (and joint any patent rights therein), without any obligation or requirement of an accounting to the other Party, and each Party hereby consents to such exploitation and licensing of the other Party for Joint Inventions. For the avoidance of doubt, all KHK Inventions (including KHK's rights to any Joint Inventions, but on a royalty-free basis) will be included within the Licensed Technology hereunder and licensed to UGNX pursuant to Article 2.

10.2.2 Subject to the terms and conditions of this Agreement, UGNX hereby grants to KHK and its Affiliates a non-exclusive, royalty-free, sublicenseable (except as provided below) license under the UGNX Inventions (including UGNX's rights to any Joint Inventions) to develop, use, sell, offer for sale, make, import and export (and to have such actions taken on its behalf by agents, contractors and other Third Party service providers) the Drug Substance and the Licensed Products for all indications and all fields in the Rest of the World, and in the Profit Share Territory and the European Territory.

10.2.3 Each Party shall cause all Persons who perform development activities or regulatory activities for such Party relating in whole or in part to the Licensed Products or otherwise to under this Agreement to assign to such Party all their rights and title in any inventions conceived, made or generated or to be conceived, made or generated by them and resulting in whole or in part from such activities, except to the extent Applicable Laws prohibit such a requirement, and further except in the case of governmental, not-for-profit and public institutions which have standard policies against such an assignment (in which case a suitable license, or right to obtain such a license, shall be obtained and maintained).

10.3 **Disclosure**. Each Party ("**Inventing Party**") shall promptly disclose to the other Party, in writing, and shall cause its Affiliates, agents, and independent contractors to disclose to the Inventing Party, all inventions related the Licensed Products conceived, made or generated by the Inventing Party which are, in the Inventing Party's reasonable judgment, potentially patentable.

10.4 **Cooperation**. Each Party agrees to reasonably cooperate in the preparation, filing, prosecution and maintenance of the Licensed Patent Rights in the Territory and the European Territory under this Agreement and in the obtaining and maintenance of any patent extensions, supplementary protection certificates and the like with respect to the Licensed Patent Rights in the Territory and the European Territory. Such cooperation includes: (a) executing all papers and instruments, or requiring its employees or contractors to execute such papers and instruments, in order to effectuate the ownership of inventions set forth in Section 10.2 and of Patents claiming or disclosing such inventions, and to enable the other Party to apply for and to prosecute patent applications in any country, to the extent provided for in this Agreement; (b) consistent with this Agreement, assisting in any license registration processes with applicable governmental authorities that may be available in the Territory or the European Territory for the protection of a Party's interests in this Agreement; and (c) promptly informing the other Party of any matters coming to such Party's attention that may materially affect the preparation, filing, prosecution or maintenance of any such Licensed Patent Rights in the Territory or the European Territory.

10.5 **Enforcement of Licensed Patent Rights against Infringement.**

10.5.1 Initiation. UGNX shall promptly notify KHK of any alleged or threatened infringement of the Licensed Patent Rights by a Third Party, or any alleged or threatened assertion of invalidity of any of the Licensed Patent Rights by a Third Party, in all such cases of which UGNX becomes aware in the Field and in the Territory or the European Territory ("**Competing Product Infringement**"). KHK will have the sole discretionary right, but not the obligation, to prosecute any such infringement at its expense. UGNX will have the right to join as a party to any suit initiated by KHK to recover its damages and participate with its own counsel; provided that KHK will retain control of the prosecution of such suit. KHK agrees to keep UGNX informed with respect to such enforcement action.

10.5.2 **Cooperation**. In the event that KHK initiates an infringement action pursuant to this Section 10.5, UGNX shall cooperate fully with respect to such action, including, if required to bring such action, the furnishing of a power of attorney solely for such purpose or to join or be named as a party such action as a necessary party.

10.5.3 **Recoveries.** Any recoveries resulting from such an action relating to a claim of Competing Product Infringement of the Licensed Patent Rights shall be first applied against repayment of each Party's actual out-of-pocket costs and expenses, or proportionate percentages thereof, in connection therewith. Any remainder will be shared as follows: [***].

10.6 **Defense of Infringement Claims.**

10.6.1 **Infringement in Profit Share Territory.** If the manufacture, sale or use of a Licensed Product in the Field in the Profit Share Territory pursuant to this Agreement results in, or may result in, any claim, suit, or proceeding by a Third Party alleging patent infringement by UGNX (or its Affiliates), the Party that learns of this claim, suit or proceeding shall promptly notify the other Party in writing. KHK will be responsible for the defense of such claim, suit or proceeding using counsel reasonably acceptable to both Parties and both Parties shall share [***] the cost and expense (including attorneys' fees) of such defense and damages or royalties (if any) payable to such Third Party in connection with such claim, suit or proceeding, provided that UGNX shall have the opportunity to provide input with respect to the strategy for such defense as well as the material pleadings, and KHK shall use Commercially Reasonable Efforts to take such input into consideration. The Party controlling the defense shall keep the other Party reasonably informed of all material developments in connection with any such claim, suit or proceeding. Each Party agrees to provide the other Party with copies of all pleadings filed in such action and to allow the other Party reasonable opportunity to participate in the defense of the claims. Any recoveries of attorneys' fees or costs in defense of a claim under this Section 10.6.1, and any sanctions awarded to the Party defending the claim and against a party asserting a claim being defended under this Section 10.6.1.

10.6.2 **Infringement in Latin America.** UGNX acknowledges and agrees that KHK has made no representations or warranties to UGNX in this Agreement or otherwise with respect to infringement (or the lack thereof) of Third Party patents in Latin America. If the manufacture, sale or use of a Licensed Product in the Field in Latin America pursuant to this Agreement results in, or may result in, any claim, suit or proceeding by a Third Party alleging patent infringement by UGNX (or its Affiliates), UGNX shall promptly notify KHK in writing. UGNX will be responsible for the defense of any such claim, suit or proceeding at its own cost and expense

(including attorneys' fees and damages or royalties (if any) payable to such Third Party in connection with such claim, suit or proceeding), using counsel of its own choice. KHK may participate in any such claim, suit or proceeding with counsel of its choice at its own expense. UGNX shall keep KHK reasonably informed of all material developments in connection with any such claim, suit, or proceeding. UGNX agrees to provide KHK with copies of all pleadings filed in such action and to allow KHK reasonable opportunity to participate in the defense of the claims. Any recoveries by UGNX of attorneys' fees or costs in defense of a claim under this Section 10.6.2, and any sanctions awarded to UGNX and against a party asserting a claim being defended under this Section 10.6.2, shall be retained by UGNX.

10.6.3 **Infringement in the European Territory**. If the manufacture, sale or use of a Licensed Product in the Field in the European Territory pursuant to this Agreement results in, or may result in, any claim, suit or proceeding by a Third Party alleging patent infringement by KHK (or its Affiliates), KHK shall promptly notify UGNX in writing. KHK will be responsible for the defense of any such claim, suit or proceeding at its own cost and expense (including attorneys' fees and damages or royalties (if any) payable to such Third Party in connection with such claim, suit or proceeding), using counsel of its own choice. UGNX may participate in any such claim, suit or proceeding with counsel of its choice at its own expense. KHK shall keep UGNX reasonably informed of all material developments in connection with any such claim, suit, or proceeding. KHK agrees to provide UGNX with copies of all pleadings filed in such action and to allow UGNX reasonable opportunity to participate in the defense of the claims. Any recoveries by KHK of attorneys' fees or costs in defense of a claim under this Section 10.6.3, and any sanctions awarded to KHK and against a party asserting a claim being defended under this Section 10.6.3, shall be retained by KHK.

ARTICLE 11.
CONFIDENTIALITY, PUBLICATION AND PUBLICITY

11.1 **Confidentiality**. All Confidential Information disclosed by or on behalf of one Party to the other Party hereunder will be maintained in confidence by the receiving Party and will not be disclosed to a Third Party or used for any purpose other than for purposes of exercising a Party's rights or performing a Party's obligations hereunder pursuant to the terms of this Agreement, except as follows:

11.1.1 If the Confidential Information is required to be disclosed by Applicable Laws or court order, including by governmental or other regulatory agencies in order to obtain patents, to obtain approval to conduct Clinical Trials or to market the Licensed Products (or to otherwise perform a Party's obligations hereunder) or to comply with applicable securities exchange or U.S. Securities and Exchange Commission regulations (or the regulations of counterpart agencies within the Territory or the European Territory), then notice of such disclosure shall be promptly delivered to the disclosing Party to the extent reasonable practicable in order to provide such disclosing Party with an opportunity to challenge or limit the disclosure obligations, provided that the receiving Party works in good faith with the disclosing Party to seek confidential treatment of such disclosure and to disclose only to the extent reasonably necessary to comply with the Applicable Law or court order, such Confidential Information may be disclosed to the extent legally required; and

11.1.2 If it is necessary or useful to disclose the Confidential Information to employees, agents, consultants, Affiliates and/or other Third Parties for the purpose of conducting activities permitted or required in accordance with this Agreement, Confidential Information may be

disclosed to such employees, agents, consultants, Affiliates and/or other Third Parties only to the extent necessary, and only if such Persons agree to be bound by confidentiality obligations at least as protective of such Confidential Information as the terms herein provided that the disclosing Party will be responsible for any disclosure of Confidential Information by any such Person inconsistent with the confidentiality obligations owed by the disclosing Party hereunder.

11.2 **Disclosure of Agreement**. Neither Party will release to any Third Party or publish in any way any non-public information regarding the terms and conditions of this Agreement without the prior written consent of the other Party, which consent will not be unreasonably withheld, conditioned or delayed, except: (a) for KHK 's disclosure to its relevant licensors who are subject to a signed confidentiality agreement; (b) pursuant to Sections 11.3 through 11.5; (c) to the extent required to comply with Applicable Laws (including securities laws, regulations and guidances) or by the requirements of any nationally recognized securities exchange, quotation system or over-the-counter market on which such Party has its securities listed or traded, provided that the disclosing Party shall make Commercially Reasonable Efforts to provide the other Party with Notice beforehand in order to permit the other Party to challenge the necessity of such disclosure and/or to coordinate with the other Party with respect to the wording and timing of any such disclosure; or (d) to the disclosing Party's legal and financial advisors, and to any actual or prospective acquirers, investors, collaborators and lenders (as well as and to their respective legal and financial advisors) who are obligated to keep such information confidential provided that the disclosing Party will be responsible for any disclosure of Confidential Information by any such Person inconsistent with the confidentiality obligations owed by the disclosing Party hereunder.

11.3 **Publications.**

11.3.1 **Publication**. Each Party shall submit any proposed publication or presentation concerning the Drug Substance or the Licensed Products (a "**Publication**") to the other Party at least [***] prior to submitting it to any Third Party (including any editing Person) for publication or presentation. In the event that KHK submits a Publication to UGNX in the Japanese language, KHK shall also submit an abstract of such Publication to UGNX in the English language. For the avoidance of doubt, Publications exclude marketing materials.

11.3.1.1 The other Party will have [***] days after receipt of the draft Publication to review and comment on such draft.

11.3.1.2 Upon Notice within such [***] day period by the other Party that the other Party reasonably believes the Publication would amount to the public disclosure of the other Party's Confidential Information and/or negatively impact the other Party's intellectual property position, submission of the concerned Publication to Third Parties will be delayed for a [***] day period from the date of said Notice for appropriately deleting Confidential Information from the proposed Publication or drafting and filing a patent application with respect to any subject matter to be made public in such Publication. Notwithstanding the foregoing, neither Party will be restricted hereunder from making any publication or disclosure to extent required to comply with Applicable Laws.

11.3.2 For all proposed Publications, each Party shall cooperate in good faith to achieve the business objectives of the proposed Publication and the publishing Party will in good faith take into account reasonable comments from the other Party.

11.4 **Press Releases**. UGNX and KHK shall issue a press release within thirty (30) days of the execution of this Agreement in a form reasonably agreeable to both Parties. Thereafter, UGNX and KHK shall consult in advance and reasonably cooperate in the method and manner of any press release regarding this Agreement and any activity related thereto. For clarity, following any press release issued pursuant to this Section 11.4, UGNX and KHK may each disclose to Third Parties the information set forth in such press release without the need for further approval by the other.

11.5 **Employees and Consultants**. Each Party hereby agrees and covenants that all of its employees and consultants and all of the employees and consultants of its Affiliates who participate in any activities under this Agreement or have access to any Confidential Information are or will, prior to their participation or access, be bound by written obligations to maintain such Confidential Information in confidence and not to use or transfer such information or materials except as expressly permitted hereunder. Each Party agrees to enforce, and to cause its Affiliates to enforce, such obligations.

ARTICLE 12.
REPRESENTATIONS AND WARRANTIES

12.1 **Mutual Representations and Warranties**. Each Party hereby represents and warrants to the other Party that as of the Effective Date:

12.1.1 **Corporate Existence and Power**. It is a corporation duly organized, validly existing and in good standing under the laws of the jurisdiction in which it is incorporated, and has full corporate power and authority and the legal right to own and operate its property and assets and to carry on its business as it is now being conducted and as contemplated in this Agreement, including the right to grant the licenses granted hereunder.

12.1.2 **Authority and Binding Agreement**. (a) It has the corporate power and authority and the legal right to enter into this Agreement and to perform its obligations hereunder; (b) It has taken all necessary corporate action on its part required to authorize the execution and delivery of this Agreement and the performance of its obligations hereunder; and (c) This Agreement has been duly executed and delivered on behalf of such Party, and constitutes a legal, valid and binding obligation of such Party that is enforceable against it in accordance with its terms.

12.1.3 **No Conflict**. It has not entered into any agreement with any Third Party that is in conflict with the rights granted to the other Party under this Agreement, and has not taken any action that would in any way prevent it from granting the rights granted to the other Party under this Agreement, or that would otherwise materially conflict with or adversely affect the rights granted to the other Party under this Agreement. Its performance and execution of this Agreement will not result in a breach of any other contract to which it is a Party.

12.1.4 **No Litigation**. It is aware of no action, suit, inquiry or investigation instituted by any Third Party which questions or threatens the validity or enforceability of this Agreement.

12.1.5 **Consents**. All necessary consents, approvals and authorizations of all governmental authorities and other persons or entities required to be obtained by such Party in connection with the execution and delivery of this Agreement have been obtained.

12.2 **UGNX's Representations and Warranties**. UGNX hereby represents and warrants to KHK that as of the Effective Date there is no pending filing, complaint, matter or action against or involving either UGNX or its Affiliates with any Regulatory Authority that could be reasonably anticipated to have a material adverse effect on its ability to obtain Marketing Approvals and Pricing and/or Reimbursement Approvals for the Licensed Products in any country or region of the Territory or the European Territory.

12.3 **KHK's Representations and Warranties**. KHK hereby represents and warrants to UGNX as of the Effective Date:

12.3.1 **Licensed Patent Rights; Licensed Technology**. KHK owns or Controls the Licensed Patent Rights listed on Schedule 1.1.47 and Schedule 1.1.47 is a complete list of all patents and patent applications owned or Controlled by KHK as of the Effective Date which claim or cover the Drug Substance or the manufacture or use thereof or any Licensed Product in the Territory or the European Territory.

12.3.2 **Title; Encumbrances; Third Party Claims**.

12.3.2.1 KHK has sufficient legal and/or beneficial title, ownership or license, free and clear from any encumbrances of the Licensed Technology, including under the In-Licenses, to grant the licenses to UGNX as purported to be granted pursuant to this Agreement.

12.3.2.2 To KHK's knowledge, the Development and Commercialization of Licensed Products do not infringe any intellectual property rights owned or possessed by any Third Party in the Profit Share Territory or the European Territory and do not and will not breach or infringe any obligation of confidentiality or non-use owed by KHK or its Affiliates to a Third Party in the Profit Share Territory or the European Territory, provided, however, that KHK does not make any representation or warranty under this Section 12.3.2.2 with respect to those Third Party patents disclosed by KIM to UGNX in writing prior to the Effective Date;

12.3.2.3 To KHK's knowledge, there are no pending claims, judgments or settlements against or owed by KHK or its Affiliates, or, to KHK's knowledge, threatened claims or litigation; in each case relating to the Licensed Patent Rights or Licensed Know-How; and

12.3.2.4 To KHK's knowledge, none of the issued Licensed Patent Rights are invalid or unenforceable.

12.3.3 **In-Licenses**. Neither KHK nor its Affiliates have in-licensed any Licensed Patent Rights or Licensed Know-How other than under the In-Licenses.

12.4 **Limitation on Warranties; No Implied Warranties**. EXCEPT AS EXPRESSLY PROVIDED IN THIS AGREEMENT, EACH PARTY MAKES NO AND EXPRESSLY DISCLAIMS ALL OTHER REPRESENTATIONS AND WARRANTIES WITH RESPECT TO THE LICENSED PRODUCTS, THE LICENSED TECHNOLOGY, THE LICENSED PATENT RIGHTS OR ANY OTHER SUBJECT MATTER OF THIS AGREEMENT, WHETHER EXPRESS, IMPLIED OR STATUTORY, INCLUDING WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, NON-INFRINGEMENT OR NON-

MISAPPROPRIATION OF THIRD PARTY INTELLECTUAL PROPERTY RIGHTS. EXCEPT TO THE EXTENT EXPRESSLY PROVIDED FOR HEREIN, NOTHING IN THIS AGREEMENT WILL BE CONSTRUED AS A REPRESENTATION OR WARRANTY BY KHK THAT THE LICENSED PATENT RIGHTS OR THE LICENSED TECHNOLOGY IS NOT INFRINGED BY ANY THIRD PARTY OR THAT THE PRACTICE OF SUCH RIGHTS DOES NOT INFRINGE ANY INTELLECTUAL PROPERTY RIGHTS OF ANY THIRD PARTY. UGNX HAS CONDUCTED ITS OWN INDEPENDENT DUE DILIGENCE REGARDING RISKS OF INFRINGEMENT OF INTELLECTUAL PROPERTY RIGHTS OF THIRD PARTIES AND HAS MADE ITS OWN INDEPENDENT DETERMINATION OF POTENTIAL RISKS WITH THE ASSISTANCE OF COUNSEL.

ARTICLE 13.
OTHER COVENANTS AND AGREEMENTS

13.1 **KHK Licenses to Third Parties.**

13.1.1 **Right of First Negotiation**. In the event that KHK or any of its Affiliates decides to grant to a Third Party a license to develop, sell, offer for sale, import or export Licensed Product(s) in (a) any indication in the Option Negotiation Right Field or (b) any indication in the Field in the Rest of the World, before commencing discussions with respect to any such, license, KHK shall provide Notice to UGNX of such decision. For the avoidance of doubt, except as set forth in this Section 13.1, nothing in this Agreement will be deemed to restrict the activities of KHK or its Affiliates in (i) the Option Negotiation Right Field, or (ii) within the Field in the Rest of the World.

13.1.2 **Exercise of Right**. UGNX will have [***] days following receipt of the Notice delivered pursuant to Section 13.1.1 to provide Notice to KHK about whether UGNX is interested in negotiating with KHK for the exclusive development and Commercialization rights in the Option Negotiation Right Field or in the Field in the Rest of the World. If UGNX elects to exercise its right, UGNX and KHK shall negotiate in good faith on the terms for such license for not less than [***] days from the date of UGNX's receipt of the Notice (the "**Negotiation Period**").

13.1.3 **Decision not to Exercise Right**. If UGNX provides Notice to KHK that UGNX is not interested in exercising the right contemplated by this Section 13.1, if the Parties fail to reach a non-binding term sheet at the end of the Negotiation Period, or if no Notice is provided by UGNX to KHK prior to end of the [***] day response period, KHK will be free to negotiate a license with any Third Party; provided, however, that KHK will not enter into a definitive agreement with any Third Party on more favorable terms than those last offered to UGNX for a period of [***] months from the end of the first to occur of [***], the [***], and, [***].

13.2 **Mutual Covenants**. Each Party hereby covenants and agrees during the Term that such Party: (a) shall carry out the Sales, Promotion and Marketing Activities and its other obligations or activities hereunder in accordance with (i) the terms of this Agreement, (ii) accepted pharmaceutical industry practices and (iii) all Applicable Laws; (b) shall use Commercially Reasonable Efforts to undertake its Development and regulatory responsibilities under this Agreement; (c) shall not enter into any agreement with a Third Party which, in any way, will limit such Party's ability to perform all of the obligations undertaken by it hereunder.

13.3 **Exclusivity**. During the period within the Term in which a Licensed Patent Right exists in any country in the Territory and/or the European Territory, on a country-by-country basis, neither Party nor its Affiliates will engage directly or indirectly in, or grant to any Third Party any right with respect to, the [***] (other than Licensed Products Commercialized under this Agreement) [***]. For the avoidance of doubt, the Parties' obligations under this Section 13.3 will expire on the expiration or earlier termination of this Agreement.

13.4 **Mutual Policing Obligations.**

13.4.1 During the Term, UGNX and its Affiliates shall not knowingly sell, transfer or otherwise provide any Licensed Products, directly or indirectly to any Third Party (a) outside of Latin America or, (b) in Latin America for (i) use outside the Field or (ii) for export outside of Latin America.

13.4.2 During the Term, KHK and its Affiliates shall not knowingly sell, transfer or otherwise provide, directly or indirectly (a) to any Third Party in any country of the world, any KHK Out-of-Field Products for use in the Field, or (b) to any Third Party in the Rest of the World or the European Territory, any Licensed Products for export into Latin America for use in the Field.

13.4.3 In the event of any sales or transfers by a Third Party prohibited by Sections 13.4.1 and 13.4.2, the Parties shall discuss in good faith ways to prevent such sales or transfers in the future and shall use Commercially Reasonable Efforts to prevent such future sales or transfers, in each case subject to and in a manner consistent Applicable Laws.

13.5 **Annual Meetings**. To the extent consistent with Applicable Laws, (a) the Parties, including UGNX's CEO and KHK's senior management shall meet upon KHK's request (but no more often than [***] per Calendar Year) at a mutually agreeable time at UGNX's offices (unless otherwise agreed upon by the Parties), at which time and location UGNX will, on a confidential basis, update KHK on the strategy, plans and other aspects of UGNX's business as reasonably requested by KHK, including UGNX's financing plans and budgets, and an update on UGNX's clinical and R&D programs (provided, however, that UGNX may elect not to disclose any information that it reasonably considers to be competitive or privileged) and (b) UGNX shall provide KHK with copies of financial statements on a quarterly basis. Notwithstanding the above, following the closing of a UGNX IPO, UGNX shall not be required to share with KHK under this Section 13.5 any non-publicly available information.

13.6 **Performance Through Affiliates**. Each Party may discharge any obligation and exercise any right hereunder through any of its Affiliates (without an assignment of this Agreement), provided that such Party shall be responsible for its Affiliates' compliance with the terms of this Agreement.

ARTICLE 14.
INDEMNIFICATION AND INSURANCE

14.1 **Indemnity by UGNX**. UGNX hereby agrees to defend, hold harmless and indemnify KHK and its Affiliates, agents, directors, officers and employees (the "**KHK Indemnitees**") from and against any and all Third Party suits, claims, actions and proceedings and associated expenses (including court costs, legal expenses and attorneys' fees) and damages and

recoveries awarded with respect thereto (collectively "**Losses**") incurred by a KHK Indemnitee in connection with any and all Third Party claims arising or resulting from: (a) any misrepresentation (or alleged misrepresentation) or breach (or alleged breach) of any of the representations, warranties, covenants or agreements made by UGNX under this Agreement; (b) any injury, damage or health complication suffered (or alleged to be suffered) as a result of the Development or Commercialization of the Licensed Products by or for UGNX in the Territory or the Development of the Licensed Products by or for UGNX in the European Territory, as applicable, or (c) any violation by UGNX or its Affiliates of laws and regulations applicable to Named Patient Sales in connection with any such Named Patient Sales made by UGNX or its Affiliates under this Agreement in Latin America, except in the case of either (a) or (b), to the extent such Losses arise from KHK's breach of its representations, warranties or covenants under this Agreement or from KHK's failure to supply Licensed Products hereunder in conformance with the Specifications therefor and in compliance with Applicable Laws (including GMP) in the Profit Share Territory or the European Territory (provided that KHK shall not be responsible for complying with any additional requirements specifically applicable to Licensed Products for Development or Commercialization purposes in Latin America) where such failure could not have been identified or detected by UGNX through its application of reasonable and customary quality assurance and quality control practices, or other inspections or activities required under such Applicable Laws with respect to such Licensed Products, prior to distribution of the Licensed Products in Latin America, and (only with respect to clinical supplies) in the Profit Share Territory or the European Territory.

14.2 **Indemnity by KHK**. KHK hereby agrees to defend, hold harmless and indemnify UGNX and its Affiliates agents, directors, officers and employees (the "**UGNX Indemnitees**") from and against any and all Losses incurred by a UGNX Indemnitee in connection with any and all Third Party claims arising or resulting from (a) any misrepresentation (or alleged misrepresentation) or breach (or alleged breach) of any of the representations, warranties, covenants or agreements made by KHK under this Agreement other than such Losses arising from KHK's failure to supply Licensed Products hereunder in conformance with the Specifications therefor and in compliance with Applicable Laws (including GMP) in the Profit Share Territory or the European Territory (provided that KHK shall not be responsible for complying with any additional requirements specifically applicable to Licensed Products for Development or Commercialization purposes in Latin America) where such failure could have been identified or detected by UGNX through its application of reasonable and customary quality assurance and quality control practices, or other inspections or activities required under such Applicable Laws with respect to such Licensed Products, prior to distribution of the Licensed Products in Latin America, and (only with respect to clinical supplies) in the Profit Share Territory or in the European Territory, as applicable; (b) any injury, damage or health complication suffered (or alleged to be suffered) as a result of the development, commercialization, use, promotion, marketing, distribution or sale of Licensed Product by or for KHK or its Affiliates or licensees in the Rest of the World or in the European Territory; (c) any violation by KHK or its Affiliates of laws and regulations applicable to Named Patient Sales in connection with any such Named Patient Sales made by KHK or its Affiliates under this Agreement in the European Territory, or (d) or UGNX's reasonable and appropriate use of any Product Trademarks or KHK Trademarks in accordance with KHK's instructions in connection with any Development or Commercialization of the Licensed Products in the Territory or in the European Territory, as applicable, except in the case of either (a) or (b), to the extent such Losses arise from UGNX's breach of its representations, warranties or covenants.

14.3 **Procedure for Indemnification.** If a KFIK Indemnitee or UGNX Indemnitee (as the case may be, an "**Indemnitee**") wishes to seek indemnification hereunder, such Indemnitee shall inform the Party obligated to indemnify the Indemnitee hereunder (the "**Indemnifying Party**") of the Third Party claim giving rise to the obligation to indemnify as soon as reasonably practicable

after receiving Notice of such Third Party claim. The Indemnifying Party will have the right to assume and control the defense of any such Third Party claim for which it is obligated to indemnify the Indemnitee under this Agreement. The Indemnitee will cooperate with the Indemnifying Party (and its insurer) as the Indemnifying Party may reasonably request, and at the sole cost and expense of the Indemnifying Party. The Indemnitee will have the right to retain its own counsel, at the expense of the Indemnifying Party, if representation of such Indemnitee by the counsel retained by the Indemnifying Party would be inappropriate because of actual or potential differences in the interests of such Indemnitee and any other Party represented by such counsel. In all other cases, the Indemnitee will have the right to participate in such defense, subject to the Indemnifying Party's control, using its own counsel at its own expense. The Indemnifying Party will have no obligation to indemnify any Indemnitee in connection with any settlement made without the Indemnifying Party's prior written consent; provided that the Indemnifying Party does not unreasonably withhold or delay any such written consent. The Indemnifying Party shall seek the prior written consent of the Indemnitee for any settlement of a Third Party claim subject to indemnification hereunder (such consent to not be unreasonably withheld, delayed or conditioned) if such settlement would materially diminish or materially adversely affect the scope, exclusivity or duration of any intellectual property licensed under this Agreement, would require any payment by such Indemnitee, would require an admission of legal wrongdoing in any way on the part of an Indemnitee, or would affect an amendment of this Agreement (otherwise, no such consent shall be required). If the Indemnifying Party does not assume and conduct the defense of the Third Party claim as provided above, (a) the Indemnitee may defend against, and consent to the entry of any judgment or enter into any settlement with respect to the claim in any manner the Indemnitee may deem reasonably appropriate (and the Indemnitee need not consult with, or obtain any consent from, the Indemnifying Party in connection therewith), and (b) the Indemnifying Party will remain responsible to indemnify the Indemnitee as provided in this Article 14.

14.4 **Losses for Product Liability Claims**. Losses from any Third Party claim alleging product liability, product defect, design, packaging or labeling defect, failure to warn, or any similar action relating to the use or safety of a Licensed Product in the Field in any country of the world (each a "**Product Liability Claim**") shall be allocated as follows:

(a) to the extent the Product Liability Claim is covered by a Party's indemnity obligations under Sections 14.1 or 14.2, the Indemnifying Party shall [***];

(b) to the extent a Product Liability Claim is not covered by either Party's indemnity obligations under Sections 14.1 or 14.2, then:

(i) For Product Liability Claims arising or resulting from Licensed Products sold in the Field in the Profit Share Territory, the Parties shall [***].

(ii) For Product Liability Claims arising or resulting from Licensed Product sold in the Field in the European Territory (except as set forth in Section 14.4(b)(i) above) and/or the Rest of the World, KHK shall [***]; and

(iii) For Product Liability Claims arising or resulting from Licensed Products sold in the Field in Latin America, UGNX shall [***].

14.5 **Insurance**. Each Party shall procure and maintain, at its sole cost and expense, comprehensive general liability insurance (which may be self insurance), including product liability insurance, with bodily injury, death and property limits and amounts of coverage consistent with industry standards, including contractual liability and product liability coverage. It is understood that such insurance will not be construed to create a limit of a Party's liability with respect to its indemnification obligations hereunder. Each Party shall provide the other Party with written evidence of such insurance upon request.

ARTICLE 15.
TERM AND TERMINATION

15.1 **Term**. The term of this Agreement ("**Term**") will commence on the Effective Date and, unless earlier terminated as expressly provided below in this Article 15, will continue for so long as either Party is selling Licensed Products in the Field in the Territory or in the European Territory.

15.2 **Termination by Either Party for Breach or Insolvency**. Either Party will have the right to terminate this Agreement prior to the expiration of the Term upon the occurrence of any of the following:

15.2.1 Upon the material, substantial and ongoing breach of any representation, warranty or obligation by the other Party if the breaching Party has not cured such breach within ninety (90) days after written Notice thereof (describing such breach in reasonable detail) by the non-breaching Party; provided, however, that if a breach is not reasonably capable of being cured within the 90-day cure period described above and the breaching Party is making continuing good faith efforts to cure such breach, the cure period shall be extended to one hundred eighty (180) days.

15.2.2 Immediately upon written Notice if the other Party has filed a petition in bankruptcy, or if an involuntary petition in bankruptcy has been filed against the other Party and such petition is not dismissed within sixty (60) days, or if a receiver or guardian has been appointed for the other Party, or upon or after the cessations of operations of the other Party.

15.3 **Special Termination Rights of KHK.**

15.3.1 **Failure to Initiate First Pediatric Study**.

(a) Subject to the terms of this Agreement, in the event UGNX does not Initiate a First Pediatric Study in the Profit Share Territory or the European Territory within eleven (11) months following a Type-C Meeting for such First Pediatric Study (if the meeting is conducted in the Profit Share Territory) or, if the meeting is conducted in the European Territory, by the earlier to occur of (i) the end of the eleventh (11th) months following the foreign equivalent of a Type-C Meeting and (ii) the end of the fifteenth (15th) months after the date of the Type-C Meeting in the Profit Share Territory (collectively, "**First Pediatric Study Deadline**"), KHK may terminate this Agreement by providing Notice to UGNX within thirty (30) days following the expiration of the First Pediatric Study Deadline.

(b) Notwithstanding Section 15.3.1(a) above, UGNX may extend the First Pediatric Study Deadline in order to reflect any of the following excusable delays: safety issues, KHK's failure to comply with this Agreement, including failure to timely provide information such as final 001 data and interim 002 data, and delays resulting from the application of Sections 3.5.1 and/or 3.5.2 despite each Party acting in good faith.

(c) In addition, (i) notwithstanding Section 15.3.1(a) above, UGNX may in its discretion obtain a one-time extension of the First Pediatric Study Deadline for an additional period of six (6) months upon payment by UGNX to KHK of a fee equal to One Million US Dollars ($1,000,000) and (ii) KHK may, in its discretion, grant to UGNX one or more further extensions of the First Pediatric Study Deadline. For clarity, during any extension period available under this subsection (c), Development Costs in the Profit Share Territory and European Territory shall be shared by the Parties as set forth in Section 4.9.1.

15.3.2 **Failure to obtain Marketing Approval in the European Territory**.

(a) Subject to the terms of this Agreement, in the event a first European Centralized Approval is not obtained in the European Core Territory for the First Indication by December 31, 2019 **(**"**European Marketing Approval**"), KHK may terminate this Agreement solely with respect to the European Territory by providing Notice to UGNX within thirty (30) days following the expiration of such deadline.

(b) Notwithstanding Section 15.3.2(a) above, UGNX may extend the deadline set forth in subsection (a) of this Section 15.3.2 in order to reflect the following excusable delays: safety issues, KHK's failure to comply with this Agreement, delays related to manufacturing and CMC, delays caused by KHK's failure to agree on matters subject to joint decision-making and Force Majeure Events.

15.3.3 **Failure to obtain US Marketing Approval in the U.S.**

(a) Subject to the terms of this Agreement, in the event a first Marketing Approval is not obtained in the U.S. for the First Indication by December 31, 2019 ("**US Marketing Approval**"), then, subject to subsection (b) of this Section 15.3.3, KHK may terminate this Agreement solely with respect to the Profit Share Territory by providing Notice to UGNX within thirty (30) days following the expiration of such deadline.

(b) Notwithstanding Section 15.3.3(a) above, upon receipt of KHK's Notice under the preceding sub-clause (a), UGNX may, by providing Notice to KHK within thirty (30) days of receipt of such KHK's Notice, elect to obtain a one-time extension of the deadline set forth in Section 15.3.3(a) for two (2) years in consideration for the payment to KHK of a fee equal to Ten Million US Dollars ($10,000,000) ("**US Extension Period**"). For clarity, during the US Extension Period, Development Costs in the Profit Share Territory shall be shared by the Parties as set forth in Section 4.9.1. If US Marketing Approval is not obtained by the end of the US Extension Period, KHK may terminate the Agreement solely with respect to the Profit Share Territory by providing Notice to UGNX within thirty (30) days following the expiration of the US Extension Period.

(c) In addition, UGNX may extend the deadline set forth in Section 15.3.3(a) in order to recover and reflect the following excusable delays: safety issues, KHK's failure to comply with this Agreement, delays related to manufacturing and CMC, delays caused by KHK's failure to agree on matters subject to joint decision-making and Force Majeure Events.

15.3.4 **Failure to Commercialize in Latin America**.

(a) Subject to the terms of this Agreement, in the event UGNX does not make the First Commercial Sale within two (2) years of US Marketing Approval in any Key Latin American Country, or within five (5) years of US Marketing Approval in any other country in Latin America (if applicable, pursuant to Section 15.3.4(b)), KHK may terminate this Agreement solely in the applicable country in Latin America, on a country-by-country basis, by providing Notice to UGNX within thirty (30) days following the expiration of the applicable deadline.

(b) Notwithstanding Section 15.3.4(a) above, UGNX may extend the deadline in subsection (a) above in order to reflect the following excusable delays: safety issues, regulatory requirements, KHK's failure to comply with this Agreement, delays related to manufacturing and CMC and Force Majeure Events. Notwithstanding the foregoing, as long as UGNX (i) applies for Marketing Approval, or applies for approval for Named Patient Sales within two (2) years of US Marketing Approval in a Key Latin American Country and (ii) uses Commercially Reasonable Efforts to pursue such application in such Key Latin American Country, then KHK may not terminate this Agreement in such country.

15.3.5 Requests for Extensions. In addition to other extensions available under Sections 15.3.1 through 15.3.4, if at any time UGNX believes that, notwithstanding the use of Commercially Reasonable Efforts, it will not be able meet any of the deadlines set forth in such Sections, then UGNX may request an extension of such deadline by providing Notice to KHK, including a reasonably detailed statement of the factors likely to cause such delay. In such a situation, the Parties will discuss in good faith UGNX's request, along with related alterations or supplements to the Core Development Plan where appropriate, provided that KHK may in its discretion decline UGNX's request.

15.3.6 **Excusable Delays.** The Parties shall discuss in good faith the existence or duration of any excusable delay applicable pursuant to Sections 15.3.1(b), 15.3.2(b), 15.3.3(c) and/or 15.3.4(b). Any dispute between the Parties as to such existence or duration shall be finally resolved pursuant to Sections 16.2 and 16.3. For clarity, during any extension period available under the provisions referred to in this Section 15.3.6, Development Costs in the Profit Share Territory and European Territory shall be shared by the Parties as set forth in Section 4.9.1.

15.4 **Key Man Provision.**

(a) It is the intent of the Parties that Dr. Emil Kakkis, Chief Executive Officer and President of UGNX as of the Effective Date, will remain actively involved with the Development of Licensed Products in the First Indication until the first Marketing Approval.

(b) If Dr. Emil Kakkis does not remain actively involved until at least the earlier of (i) Initiation of a pediatric pivotal study in the First Indication in the Profit Share Territory or the European Territory **(**"**Pivotal Study Initiation**") and (ii) the closing of a public offering of UGNX's securities pursuant to a registration statement under the Securities Act of 1933 (or successor thereof or foreign equivalent) ("**UGNX IPO**"), then UGNX may propose a suitable replacement during the three (3) month period following the date on which Dr. Emil Kakkis is no longer actively involved in the Development of Licensed Products in the First Indication, in which case the Parties shall discuss in good faith UGNX's proposed candidate(s). If the Parties fail to agree, then KHK may terminate this Agreement by providing Notice to UGNX within the following thirty (30) days.

15.5 **Post-Termination Commercialization by KHK**. If KHK Commercializes or permits others to Commercialize Licensed Products following the effective date of the termination of this Agreement under Sections 15.2.1, 15.3 or 15.4(b), then MAX shall make the following payments to UGNX:

(a) Upon termination under Section 15.3.1(a) and subject to UGNX's fulfillment of its obligations under Section 15.7.3 on a country-by-country basis, KHK shall (i) pay a royalty equal to [***] percent ([***]%) of Net Sales of Licensed Products by KHK or its Affiliates or other Selling Party, as applicable in the Field in the Territory and the European Territory for a period of [***] following the First Commercial Sale on a country-by-country basis and (ii) reimburse to UGNX [***] Development Costs incurred by UGNX (i.e., [***]) prior to the effective date of such termination.

(b) Upon termination under Section 15.4(b) and subject to UGNX's fulfillment of its obligations under Section 15.7.3 on a country-by-country basis, KHK shall (i) to the extent Dr. Emil Kakkis had remained actively involved with the Development of Licensed Products upon or beyond the Initiation of a First Pediatric Study in the Profit Share Territory or the European Territory, pay a royalty equal to [***] percent ([***]%) of Net Sales of Licensed Products by KHK or its Affiliates or other Selling Party, as applicable, in the Field in each country in the Territory and the European Territory, which royalty shall be payable for [***] following the First Commercial Sale in such country, on a country-by-country basis and (ii) reimburse to UGNX [***] Development Costs incurred by UGNX (i.e., [***]) prior to the effective date of such termination.

(c) Upon termination under Section 15.2.1, or 15.3.2(a), and subject to UGNX's fulfillment of its obligations under Section 15.7.3 on a country-by-country basis, if following such termination KHK or any of its Affiliates or Sublicensees obtains a European Marketing Approval based on data from a pivotal study commenced by UGNX or its Affiliates only if more than [***] percent ([***]%) of the patients to be enrolled in the Phase 3 Clinical Trial (or pivotal study) would have been dosed with study drugs (including KRN23, placebo, or comparators) at the time of the applicable deadline (a "**Qualifying Pivotal Study**"), then KHK shall pay a royalty equal to [***] percent ([***]%) of Net Sales of Licensed Products by KHK and its Affiliates or other Selling Party, as applicable, in the Field in each country of the European Territory for [***] following the First Commercial Sale in such country, on a country-by-country basis. For clarity, if no data from a Qualifying Pivotal Study is used in obtaining a European Marketing Approval for a Licensed Product in the Field, no royalties or any other amounts shall be payable pursuant hereto by KHK or its Affiliates to UGNX for any Licensed Product. For clarity, in the event of the applicability of paragraph "f" below, this paragraph "c" shall no longer apply.

(d) Upon termination under Section 15.2.1, 15.3.3(a) or 15.3.3(b) and subject to UGNX's fulfillment of its obligations under Section 15.7.3 on a country-by-country basis, if following such termination KHK or any of its Affiliates or Sublicensees obtains a US Marketing Approval based on data from a Qualifying Pivotal Study, then KHK shall pay a revenue share equal to [***] percent ([***]%) of Net Sales of Licensed Products by KHK and its Affiliates or other Selling Party, as applicable, in the Field in each country of the Profit Share Territory for [***] following the First Commercial Sale in such country, on a country-by-country basis. For clarity, if no data from a Qualifying Pivotal Study is used in obtaining a US Marketing Approval for a Licensed Product, no revenue share or any other amounts shall be payable pursuant hereto by KHK or its Affiliates to UGNX for such Licensed Product. For clarity, in the event of the applicability of paragraph "e" below, this paragraph "d" shall no longer apply.

(e) Upon termination under Section 15.2.1, 15.3.3(a) or 15.3.3(b) and subject to UGNX's fulfillment of its obligations under Section 15.7.3 on a country-by-country basis, if following such termination KHK or any of its Affiliates or Sublicensees obtains a US Marketing Approval based on a Biologic Licensing Application (or similar filing outside the U.S.) (BLA) filed by UGNX prior to the effective date of such termination, or UGNX obtains US Marketing Approval prior to the effective date of such termination, KHK shall pay a revenue share at the rates set forth in Section 7.2.2 on Net Sales of Licensed Products by KHK and its Affiliates or other Selling Party, as applicable, in the Field in each country of the Profit Share Territory for [***] following the First Commercial Sale in such country, on a country-by-country basis, or, if UGNX obtains US Marketing Approval prior to the effective date of termination, [***] following such termination, on a country by country basis. For clarity, if no data from a Qualifying Pivotal Study is used in obtaining a US Marketing Approval for a Licensed Product in the Field, no revenue share or any other amounts shall be payable pursuant hereto by KHK or its Affiliates to UGNX for any Licensed Product.

(f) Upon termination under Section 15.2.1 or 15.3.2(a), and subject to UGNX's fulfillment of its obligations under Section 15.7.3 on a country-by-country basis, if following such termination KHK or any of its Affiliates or Sublicensees obtains a European Marketing Approval based on a Marketing Authorization Application filed by UGNX prior to the effective date of such termination, or if UGNX obtains European Marketing Approval prior to the effective date of such termination, KHK shall pay a royalty equal to [***] percent ([***]%) of Net Sales of Licensed Products by KHK and its Affiliates or other Selling Party, as applicable, in the Field in each country of the European Territory for [***] following the First Commercial Sale in such country, on a country-by-country basis, or, if UGNX obtains European Marketing Approval prior to the effective date of termination, [***] following such termination, on a country by country basis. For clarity, if no data from a Qualifying Pivotal Study is used in obtaining a European Centralized Approval for a Licensed Product in the Field, no royalties or any other amounts shall be payable pursuant hereto by MHK or its Affiliates to UGNX for any Licensed Product.

(g) Upon termination under Section 15.2.1, and subject to UGNX's fulfillment of its obligations under Section 15.7.3 on a country-by-country basis, in the event UGNX has achieved a First Commercial Sale or a Named Patient Sale in any country in Latin America prior to the effective date of such termination, then KHK shall pay a royalty equal to [***] percent ([***]%) of Net Sales by KHK and its Affiliates or other Selling Party, as applicable, in such country for [***] following the effective date of termination.

(h) Except as set forth in the preceding sub-clauses of this Section 15.5, upon any termination of this Agreement, notwithstanding anything to the contrary, KIIK shall, subject to the provisions of paragraph "i" below, and subject to UGNX's fulfillment of its obligations under Section 15.7.3 on a country-by-country basis, pay a royalty equal to [***] percent ([***]%) of Net Sales of Licensed Products by KHK and its Affiliates or other Selling Party, as applicable, in the Field in each country of the Territory and/or European Territory for [***] following the First Commercial Sale in such country, on a country-by-country basis.

(i) Notwithstanding the foregoing, no payments shall be due under this Section 15.5 upon termination pursuant to Section 15.2.1 to the extent the breach results from UGNX's intentional or willful misconduct, reckless misconduct or a repeated pattern of bad faith behavior.

15.6 **Effect of Expiration or Termination of this Agreement.**

15.6.1 Upon the expiration or termination of this Agreement by either Party for any reason, the following provisions will apply:

(a) Each Party shall return the originals and any copies of the other Party's Confidential Information; provided that each Party may retain copies of any Confidential Information that is subject to a continuing license hereunder and one copy of the other Party's Confidential Information in possession of its legal counsel for the purposes of monitoring its obligations hereunder and exercising any surviving rights and complying with Applicable Laws; and

(b) Neither Party will be relieved of any liability or obligation of such Party that accrued, or which arose during or relates to any period, prior to the effective date of such termination, including any payment obligations.

15.6.2 The provisions of Sections 5.5 through 5.7, Sections 7.2 through 7.7 (to the extent applicable as a result of the application of Section 15.5), Article 9, Section 10.6 (to the extent the relevant Third-Party claim, suit, or proceeding relate to Licensed Products in the Field sold under this Agreement prior to the effective date of expiration or termination of this Agreement), Article 11, Sections 12.4 and 13.6, Sections 14.1 through 14.3, Section 14.4(a), Sections 15.5 through 15.8, Articles 16 and 17 (other than Section 17.1) will survive any expiration or termination of this Agreement and remain in full force and effect in accordance with their terms. Section 10.5 will survive any expiration or termination of this Agreement and remain in full force and effect in accordance with its terms, except that with respect to infringements covered by Section 10.5 that occur after the Term, UGNX's percentage share of any recoveries remaining after reimbursement of costs and expenses shall equal the percentage royalty or revenue share called for under Section 15.5 to be paid by KHK (if any). Section 14.4(b) will survive any expiration or termination of this Agreement and remain in full force and effect in accordance with its terms, except that KHK will bear [***] of all Losses to the extent resulting from a Product Liability Claim that arises from Licensed Product sold after the Term and that is not covered by either Party's indemnity obligations under Section 14.1 or 14.2.

15.7 **Effect of Certain Terminations**. Upon the termination of this Agreement by either Party pursuant to Section 15.2.1 for breach or Section 15.2.2 for insolvency or by KHK under Section 15.3 or 15.4, the following provisions of this Section 15.7 will apply. In the event any such early termination concerns only a specific country or countries pursuant to Section 15.3, the following shall apply solely with respect to such country(ies) and the Agreement shall otherwise remain in effect in accordance with its terms for all non-terminated countries:

15.7.1 The rights and licenses granted by KIM to UGNX hereunder and UGNX's obligations to share Development Costs pursuant to this Agreement will terminate with respect to the terminated country(ies);

15.7.2 UGNX shall immediately (a) cease conducting any Commercialization activities with respect to the Licensed Products and (b) discontinue making any representation regarding its status as a licensee or distributor of KHK in the Territory for the Licensed Products, subject, in either such case, to requirements of Applicable Laws and to a reasonable wind-down and transition period (not to exceed [***] days);

15.7.3 Subject to Applicable Laws, UGNX shall promptly :

(a) transfer to KHK or KHK's designee all Regulatory Filings (for clarity, including registrations as an IND holder or equivalent), Marketing Approvals and Pricing and/or Reimbursement Approvals owned by UGNX for the Commercialization of the Licensed Products, if such transfer is possible, or, if such transfer is not possible, then at KHK's discretion (i) withdraw any such Regulatory Filings, Marketing Approvals and Pricing and/or Reimbursement Approvals for the Commercialization of the Licensed Products in its name and take all actions necessary or useful to support KHK's or KHK's designee's submission of Regulatory Filings and the achievement of Marketing Approvals and Pricing and/or Reimbursement Approvals in the name of KHK or KHK's designee with respect to the Commercialization of the Licensed Products or (ii) provide KHK with access to, and grant KHK the right and license to use and to reference, such Regulatory Filings, Marketing Approvals and Pricing and/or Reimbursement Approvals then in its name applicable to the Commercialization of the Licensed Products;

(b) provide KHK with copies of all material correspondence between UGNX and Regulatory Authorities with respect to such Regulatory Filings, Marketing Approvals and Pricing and/or Reimbursement Approvals for the Licensed Products, and any and all other clinical and non-clinical data, records and tabulations, in all such cases with respect to the Licensed Products, that UGNX holds as of the date of termination with respect to the Licensed Products;

(c) assign to KHK all agreements specific to the conduct of Clinical Trials for the Licensed Products (to the extent assignable and excluding any such agreements that also involve Clinical Trials for other UGNX products that are not Licensed Products), including agreements or contracts with contract research organizations, clinical sites and investigators, between UGNX and any Third Party, subject to any consent required by such Third Party, which consent UGNX shall use Commercially Reasonable Efforts to obtain on behalf of KHK; and

(d) provide KHK with copies of all reports and data obtained by UGNX or its Affiliates pursuant to this Agreement regarding the Development and the Commercialization of Licensed Products, including any UGNX Clinical Data, which KHK may use for any purpose.

As promptly as possible after any such termination, UGNX shall execute any and all documents of any Regulatory Authorities so as to allow KHK to make immediate use of any data, records and Regulatory Filings transferred by UGNX to KHK pursuant to this Section 15.7.3.

15.7.4 KHK shall make the payments due under Section 15.5, if applicable under the provisions thereof, with respect to the applicable country(ies)

15.8 **Remedies Cumulative and Nonexclusive**. All of the non-breaching Party's remedies will be cumulative, and the exercise of one remedy hereunder by the non-breaching Party will not be deemed to be an election of remedies.

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

ARTICLE 16.
DISPUTE RESOLUTION

16.1 **Disputes**. The Parties recognize that disagreements as to certain matters may from time to time arise out of this Agreement. The Parties agree that such disagreements are to be governed in accordance with this Article 16. Disagreements that are claims, counterclaims, demands, causes of action, disputes or controversies both arising out of this Agreement and related to the performance, enforcement, breach or termination of this Agreement are each, a "**Dispute.**" For the avoidance of doubt, Dispute does not include any claims, counterclaims, demands, causes of action, disputes or controversies regarding a Party's use of any intellectual property rights of the other Party, where such use is not expressly granted by the other Party hereunder, including (with respect to uses by UGNX) any such use in the Rest of the World.

16.2 **Agreement to Arbitrate**. If the Parties fail to resolve any Dispute pursuant to Section 16.1, either Party may submit that Dispute for final resolution by binding arbitration administered by the International Chamber of Commerce ("**ICC**") in accordance with its Rules of Arbitration (the "**Rules**"**)** then in force, to the extent such Rules are not inconsistent with the provisions of this Agreement.

16.3 **Number and Appointment of Arbitrators**. Except as provided by this Section 16.3 or in Section 16.4, the appointment and confirmation of the arbitrators shall be made in accordance with the relevant provisions of the Rules. The arbitral tribunal shall be composed of three (3) arbitrators (the "**Tribunal**") to be appointed in accordance with the Rules, except as expressly provided for herein. Each Party shall select one (1) arbitrator from the list of available ICC arbitrators and such arbitrators shall jointly appoint the third arbitrator who shall act as the chairman of the Tribunal (the "**Chairman**"). In the event any arbitrator becomes unable to serve, that arbitrator will be replaced in the same manner in which he or she was appointed. If either Party fails to appoint an arbitrator within [***] days of the initiation of arbitration, the other Party may request the ICC to appoint such co-arbitrator (for the non-responsive Party). Such appointment shall be binding on the Parties. If the arbitrators selected by the Parties cannot agree on a Chairman within [***] days after they have been selected, then the ICC shall appoint the Chairman upon request by either Party.

16.4 **Special Rules For Certain Specific Disputes (Baseball Arbitration)**. This Section 16.4 shall only apply to the matters expressly identified in this Agreement as subject to resolution pursuant to this Section 16.4. The Tribunal for such matters shall be composed of one (1) arbitrator. In such arbitration, the arbitrator shall be an independent expert (including in the area of the dispute) in the pharmaceutical or biotechnology industry mutually acceptable to the Parties. The Parties shall use their best efforts to mutually agree upon one (1) arbitrator; provided, however, that if the Parties have not done so within [***] Business Days after initiation of arbitration hereunder, or such longer period of time as the Parties have agreed to in writing, then the ICC shall appoint such arbitrator (which arbitrator shall meet the requirements set forth in the preceding sentence) upon request by either Party. Arbitration pursuant to this Section 16.4 shall be limited to choosing one or the other from among the two proposed resolutions prepared by the two Parties with respect to all matters in dispute, and in connection therewith, each Party shall submit to the arbitrator and to each other in writing its position on and desired resolution of (including any specifics or breakdown of the amounts included within their respective figures) each such matter. Such submission shall be made within [***] Business Days of the selection or appointment of the arbitrator, and the arbitrator shall issue its resolution of all such matters within [***] days of receipt of the written submissions by both Parties. For the avoidance of doubt, the arbitrator's resolution of all such matters shall be limited to the selection of one or the other of the two competing resolution methods proposed by the Parties and other than minor modification, the arbitrator may not impose a resolution not proposed by either Party or "split the difference" by creating a compromise resolution based on the desired resolutions proposed separately by the Parties. Except as provided in the preceding sentence, such arbitration shall be conducted in accordance with the relevant provisions of the ICC Rules. The arbitrator's vote shall be final and binding upon the Parties.

16.5 **Conduct of Arbitration**. The arbitration shall be conducted and the award rendered in the English language.

16.6 **Place of Arbitration**. The arbitration shall be held in Singapore.

16.7 **Powers of the Arbitrators, Limitations on Remedies**. With respect to any arbitration conducted pursuant to Section 16.2, the Tribunal will have the power to award all remedies available under Applicable Laws, by a vote of at least two (2) of the three (3) arbitrators.

16.8 **Arbitration Award**. Except as set forth in Section 16.4 (Baseball Arbitration), the Tribunal will make all reasonable efforts to render a written, reasoned decision within [***] days following the closing of the hearing. The decision of the Tribunal will be final and binding on the Parties, and judgment upon the decision may be confirmed in, and judgment upon the award entered by, any court having jurisdiction over the Parties.

16.9 **Confidentiality**. Except to the extent necessary for proceedings relating to enforcement of the arbitration agreement, the award, or other related rights of the Parties, the fact of the arbitration, the arbitration proceeding itself, all evidence, written statements or other documents exchanged or used in the arbitration and the Tribunal's award will be maintained in confidence by the Parties to the fullest extent permitted by law. However, a violation of this covenant will not affect the enforceability of this agreement to arbitrate or of the Tribunal's award.

16.10 **Costs**. Each Party shall bear its own costs and expenses in connection with any Dispute resolution under this Article 16 and shall share equally (50/50) the fees, costs and expenses of the Tribunal and related Third Party arbitration expenses. Notwithstanding the foregoing, the prevailing Party may, as determined by the Tribunal under the circumstances, be awarded its attorneys' fees, costs and expenses of the arbitration, including the arbitrators' fees and expenses, in full. The Tribunal may also fix such costs and expenses proportionate to the extent each Party prevails in the arbitration, as the circumstances may warrant. If a Party fails to proceed with arbitration, unsuccessfully challenges the arbitration award or fails to comply with the arbitration award, the other Party will be entitled to costs, including reasonable attorneys' fees and disbursements, for having to compel arbitration or defend or enforce the award.

16.11 **Injunctive or Other Interim Relief**. Notwithstanding the provisions of this Article 16, the Parties agree that irreparable harm might accrue with respect to a Dispute absent a temporary injunctive or other interim relief and the Parties therefore shall have the right to seek such injunctive or interim relief in a court of competent jurisdiction pending the outcome of Dispute resolution hereunder. In the event interim or injunctive relief is sought by a Party as to a Dispute, and a court of competent jurisdiction grants such interim or injunctive relief, the Parties shall continue to be bound under this Article 16 to resolve by arbitration such Dispute that is the subject of interim or injunctive relief.

16.12 **Continued Performance**. Pending resolution of any Dispute covered by this Article 16, both Parties shall continue their performance under this Agreement of any obligations (including payment obligations) that are not the subject of such Dispute.

16.13 **Governing Law**. Resolution of all Disputes arising out of or related to this Agreement or the performance, enforcement, breach or termination of this Agreement and any remedies relating thereto, will be governed by and construed under the substantive laws of the State of New York, U.S.A., without reference to any choice of law principles thereof that would cause the application of the laws of a different jurisdiction.

16.14 **Separability and Survival of the Agreement to Arbitrate**. The provisions of this agreement to arbitrate are independent of the remaining provisions of this Agreement and the Parties intend that the remaining provisions shall continue in effect even though one or more of the provisions of the Agreement will be determined to be null and void. This agreement to arbitrate will also survive the termination or expiration of this Agreement.

ARTICLE 17.
OTHER PROVISIONS

17.1 **Non-Solicitation of Employees**. During the Term, neither Party nor its Affiliates shall, directly or through its representatives or agents, solicit for employment or hire any officer, director, or employee of the other Party or its Affiliates with whom it has had contact in connection with, or who otherwise is known by it to participate in, the subject matter of this Agreement or the development of the Drug Substance or a Licensed Product; provided, however, a Party will not be prohibited from soliciting and hiring through general public advertisement or other solicitation that is not directed toward the employees of the other Party, and a Party may hire any former employee of the other Party as long as the discussions with the former employee are initiated after termination of employment by the other Party.

17.2 **Force Majeure**. Both Parties will be excused from the performance of their obligations under this Agreement, other than the obligation to make monetary payments, and neither Party will be held liable or responsible to the other Party nor be deemed to have defaulted under or breached this Agreement for failure or delay in fulfilling or performing any term of this Agreement, to the extent that such performance is prevented by force majeure and the nonperforming Party promptly provides Notice thereof to the other Party. Such excuse will be continued so long as the condition constituting a force majeure event continues and the nonperforming Party uses Commercially Reasonable Efforts to remove the condition. For purposes of this Agreement, a force majeure event ("**Force Majeure Event**") will include conditions beyond the reasonable control and without the fault of a Party, such as an act of God, voluntary or involuntary compliance with any regulation, law or order of any government, war, an act of terrorism, civil commotion, labor strike or lock-out, epidemic, failure or default of public utilities or common carriers, destruction of production facilities or materials by fire, earthquake, storm or like catastrophe, inability to procure necessary raw materials in a commercially reasonable manner or default of suppliers or sub-contractors; provided, however, the payment of invoices due and owing hereunder may not be delayed by the payer because of a force majeure affecting the payer.

17.3 **Exclusions of Consequential Damages**. EXCEPT IN THE CASE OF A PARTY'S INTENTIONAL OR WILLFUL MISCONDUCT OR EGREGIOUS BAD FAITH CONDUCT OR DAMAGES AWARDED TO THIRD PARTIES COVERED BY THE INDEMNITY OBLIGATIONS SET FORTH IN SECTION 14, IN NO EVENT WILL EITHER PARTY BE LIABLE TO THE OTHER PARTY FOR LOST PROFITS OR FOR ANY INDIRECT, INCIDENTAL, CONSEQUENTIAL, SPECIAL, PUNITIVE OR EXEMPLARY DAMAGES OF THE OTHER PARTY IN CONNECTION WITH THIS AGREEMENT OR THE TRANSACTIONS CONTEMPLATED BY THIS AGREEMENT, HOWEVER CAUSED, UNDER ANY THEORY OF LIABILITY, AND WHETHER OR NOT SUCH PARTY HAD PRIOR NOTICE THEREOF.

17.4 **Assignment**. Neither Party may assign this Agreement or any of its obligations hereunder without the prior written consent of the other Party; provided, however, that either Party may assign this Agreement in its entirety without such consent to any of its Affiliates, to any purchaser of all, or substantially all, of its assets or to any successor corporation resulting from any merger, consolidation, share exchange, or other similar transaction, and provided further that either Party may assign or sell its rights to receive any amounts due hereunder. This Agreement will inure to the benefit of UGNX and IGIK and their respective successors and permitted assigns. Any assignment of this Agreement that is not made in accordance with this Section 17.4 shall be null and void and of no legal force or effect.

17.5 **Severability**. In the event any one or more of the provisions contained in this Agreement should be held invalid, illegal or unenforceable in any respect, the validity, legality and enforceability of the remaining provisions contained herein will not in any way be affected or impaired thereby, unless the absence of the invalidated provision(s) frustrates the purpose of this Agreement (in which case the Parties will attempt to replace such invalidated provision with an enforceable provision that most clearly implements such purpose). The Parties will in such an instance use their Commercially Reasonable Efforts to replace the invalid, illegal or unenforceable provision(s) with valid, legal and enforceable provision(s) that, insofar as practical, implement the purposes of this Agreement.

17.6 **Notices**. All notices, consents, approvals and other legally operative communications that are required or permitted hereunder ("**Notice**") will be in writing in the English language and sufficient if delivered personally, sent by facsimile or e-mail (and promptly confirmed by personal delivery, registered or certified mail or overnight courier), sent by nationally-recognized overnight courier or sent by registered or certified mail, postage prepaid, return receipt requested, addressed as follows:

Kyowa Hakko Kirin Co., Ltd.
1-6-1 Ohtemachi
Chiyoda-ku, Tokyo, 100-8185, Japan
Attention: Director of Business Development Department
Tel: +81-3-3282-0093
Fax: +81-3-3282-0107

Ultragenyx Pharmaceutical Inc.
60 Leveroni Court
Novato, CA 94949

Attention: Business Development Department/Tom Kassberg
Tel: 415.483.8800
Fax: 415.483.8820
E-mail: Tkassberg@ultragenyx.com

or to such other address as the Party to whom Notice is to be given may have furnished to the other Party in writing in accordance herewith. Any such communication will be deemed delivered: (a) if sent by mail, as aforesaid, on the date upon which the return receipt is signed or delivery is refused or the Notice is designated by the postal authorities as not deliverable, as the case may be; (b) if sent

by facsimile or e-mail, as aforesaid, when sent (with confirmation of receipt); or (c) if sent by courier or hand delivered, as aforesaid, when received. The cost of any translation into English of any communication, document or Notice will be borne solely by the Party providing such communication, document or Notice.

17.7 **Entire Agreement; Amendments**. This Agreement contains the entire understanding of the Parties with respect to the subject matter hereof. All express or implied agreements and understandings, either oral or written, heretofore made are expressly superseded by this Agreement. Except as expressly set forth in this Agreement, this Agreement may be amended, or any term hereof modified, only by a written instrument duly executed by both Parties.

17.8 **Headings**. The captions to the several Articles and Sections hereof are not a part of this Agreement, but are merely guides or labels to assist in locating and reading the several Articles and Sections hereof.

17.9 **Independent Contractors**. It is expressly agreed that KHK and UGNX will be independent contractors and that the relationship between the two Parties will not constitute a partnership, joint venture or agency. Neither KHK nor UGNX will have the authority to make any statements, representations or commitments of any kind, or to take any action, which will be binding on the other, without the prior consent of the other Party.

17.10 **Subcontractors**. Except as otherwise set forth in this Agreement, each Party may engage subcontractors to perform, under its direction, specific and discrete functions that are allocated to it hereunder or that it carries out in the exercise of its rights hereunder, in each case in accordance with this Section 17.10. Each Party shall be fully responsible under this Agreement for the performance hereof by its permitted subcontractors as if such Party so performed this Agreement itself.

17.11 **Waiver**. The waiver by either Party hereto of any right hereunder or the failure to perform or of a breach by the other Party will not be deemed a waiver of any other right hereunder or of any other breach or failure by said other Party whether of a similar nature or otherwise.

17.12 **Counterparts**. This Agreement may be executed in identical duplicate copies exchanged by facsimile or e-mail (PDF form) transmission. The Parties agree to execute two identical original copies of this Agreement after exchanging signed facsimile versions. Each identical counterpart will be deemed an original, but all of which together will constitute one and the same instrument.

17.13 **Waiver of Rule of Construction**. Each Party has had the opportunity to consult with counsel in connection with the review, drafting and negotiation of this Agreement. Accordingly, the rule of construction that any ambiguity in this Agreement will be construed against the drafting Party will not apply.

17.14 **Third Party Beneficiaries**. Except as otherwise expressly provided in this Agreement, nothing herein expressed or implied is intended or will be construed to confer upon or to give to any Third Party any rights or remedies by reason of this Agreement. Except as otherwise expressly provided in this Agreement, there are no intended Third Party beneficiaries under or by reason of this Agreement.

17.15 **Further Assurances**. Upon the other Party's request hereunder, each Party agrees to execute, acknowledge and deliver such further instruments, and to do all such other acts, as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

17.16 **Construction of Agreement**. Unless context otherwise clearly requires, whenever used in this Agreement: (a) the words "include" or "including" shall be construed as incorporating, also, "but not limited to" or "without limitation;" (b) the word "day" or "year" means a calendar day or calendar year unless otherwise specified; (c) the words "hereof," "herein," "hereby" and derivative or similar words refer to this Agreement (including all Exhibits); (d) provisions that require that a Party, the Parties or any committee or team hereunder "agree," "consent" or "approve" or the like shall require that such agreement, consent or approval be specific and in writing, whether by written agreement, letter, approved minutes or otherwise; (e) words of any gender include the other gender; (f) references to any specific Applicable Law or article, section or other division thereof shall be deemed to include the then-current amendments thereto or any replacement Applicable Law thereof; and (g) references to either Party include the successors and permitted assigns of that Party. Except as otherwise stated herein, if the terms of this Agreement conflict with the terms of any Schedule or Exhibit, then the terms of this Agreement will govern.

[Remainder of this page is intentionally left blank.]

IN WITNESS WHEREOF, the Parties have executed this Collaboration and License Agreement to be effective as of the Effective Date.

KYOWA HAKKO KIRIN CO., LTD.

By: /s/ Nobuo Hannai
Name: Nobuo Hanai, Ph.D.
Title: President and CEO
Date: August 29, 2013

ULTRAGENYX PHARMACEUTICAL INC.

By: /s/ Emil D. Kakkis
Name: Emil D. Kakkis, MD, PhD
Title: CEO
Date: August 29, 2013

Schedules

Schedule 1.1.15 –Drug Substance Sequence

Schedule 1.1.47 – Licensed Patent Rights

Schedule 4.3.2 – Decision-Making for Day-to-Day Core Development Activities

Exhibits:

Exhibit A – Financial Exhibit

SCHEDULE 1.1.15

Drug Substance Sequence

Amino acid sequence of KRN23

Light chain

[***]

Heavy chain

[***]

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

SCHEDULE 1.1.47

Licensed Patent Rights

"**Licensed Patent Rights**" shall include the following patents and applications:

1. Title: [***] Applicant/Assignee: [***]

Country	Application No.	Filing Date	Patent No.	Issue Date	Status
[***]	[***]	[***]	[***]	[***]	[***]
[***]	[***]	[***]	[***]	[***]	[***]
[***]	[***]	[***]	[***]	[***]	[***]
[***]	[***]	[***]	[***]	[***]	[***]

2. Title: [***] Applicant/Assignee: [***]

Country	Application No.	Filing Date	Patent No.	Issue Date	Status
[***]	[***]	[***]	[***]	[***]	[***]
[***]	[***]	[***]	[***]	[***]	[***]
[***]	[***]	[***]	[***]	[***]	[***]
[***]	[***]	[***]	[***]	[***]	[***]

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

3. Title: [***] Applicant/Assignee: [***]

Country	Application No.	Filing Date	Patent No.	Issue Date	Status
[***]	[***]	[***]	[***]	[***]	[***]
[***]	[***]	[***]	[***]	[***]	[***]
[***]	[***]	[***]	[***]	[***]	[***]

4. Title: [***] Applicant/Assignee: [***]

Country	Application No.	Filing Date	Patent No.	Issue Date	Status
[***]	[***]	[***]	[***]	[***]	[***]
[***]	[***]	[***]	[***]	[***]	[***]
[***]	[***]	[***]	[***]	[***]	[***]
[***]	[***]	[***]	[***]	[***]	[***]
[***]	[***]	[***]	[***]	[***]	[***]
[***]	[***]	[***]	[***]	[***]	[***]

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

SCHEDULE 4.3.2

Decision-Making for Dav-to-Day Core Development Activities

The Lead Development Party shall make all decisions with respect to day-to-day Core Development Activities ("**Day-to-Day Core Development Activities**") including the following types of decisions and activities:

(a) Number of sites, site selection and site management
(b) Vendor management
(c) Site agreements
(d) Vendor agreements
(e) Site documents
(f) Safety reporting
(g) Recruitment materials
(h) Case Report Form (CRF) design
(i) Database design
(j) Tables, Listings and Graphs (TLGs) design
(k) Study unblinding procedures
(l) Data analysis and report generation
(m) Data Monitoring Committee (DMC) membership, charter and conduct
(n) Regulatory meeting preparations and conduct of meetings
(o) Patient identification
(p) Key opinion leader/Principal investigator interactions

EXHIBIT A

Financial Exhibit

Examples of Commercialization Costs

Commercialization Costs shall include, but shall not be limited to, the following, in each case attributable to, or reasonably allocable to, Commercialization of Licensed Products in the Field:

Detailing costs—costs associated with interactive face-to-face visits by a sales representative with a medical professional who has prescribing authority or is able to influence prescribing decisions, within the target audience during which approved uses, safety, effectiveness, contraindications, side effects, warnings or other relevant characteristics of a pharmaceutical product are discussed in an effort to increase prescribing preferences of a pharmaceutical product for its approved uses.

Distribution costs—costs identifiable to the distribution of a Licensed Product to a customer (and not otherwise deducted from Net Sales), including customer and specialty pharmacy services (including costs associated with establishing and running customer call centers), collection of data about sales to hospitals, clinics and other customers, order entry, billing, shipping, logistics, warehousing, product insurance, freight not paid by customers, credit and collection and other like activities the costs of which are includable in "distribution costs" in accordance with GAAP.

Compassionate Use Programs—costs associated with any program to provide patients with products free of charge. These costs include compassionate use programs, provided however that any compassionate use programs prior to Marketing Approval shall be mutually agreed upon by the Parties in advance.

Health Care Reform fees—costs for fees paid to the U.S. government as defined in the Patient Protection and Affordable Care Act and similar taxes and governmental fees.

Marketing Expenses—costs identifiable to the advertising, promotion and marketing of Licensed Products in the Field, and related professional education, including:

a. **Advertising**, which includes costs associated with media costs, direct mails, production expenses, agency fees, and medical congresses and meetings;

b. **Promotion**, which includes costs associated with professional samples, professional literature, promotional material, patient aids, detailing aids, reimbursement of patient assistance programs, public relations and communications expenses, web and social media expenses, patient advocacy support, development of information and data for national accounts, managed care organizations and group purchasing organizations;

c. **Market Research**, which includes costs associated with market information, focus groups, and market research professional staff and related out-of-pocket costs such as travel, business meals, and training;

d. **Marketing Management**, which includes the costs of product management and sales promotion management compensation and departmental expenses as well as costs associated with developing overall sales and marketing strategies and planning;

e. **Reimbursement/Access Services**, which includes costs incurred to manage reimbursement programs, marketing costs (educational material) as well as coupon or co-pay programs; and

f. **Health Policy/Advocacy**, which includes costs associated with advocacy as well as any specific policy lobbying and trade and government relations related expenses.

Medical Affairs Expenses—costs with respect to: medical affairs and other activities associated with clinical studies conducted after Marketing Approval in the Profit Share Territory (to the extent not otherwise included within Development Costs); medical and scientific information and response to external inquiries or complaints; pharmacovigilance, investigator initiated research if not covered in the Core Development Plan, Phase V Clinical Trials, costs of establishing and maintaining patient registries, medical education, Health Economics and Outcomes Research (HBCOR, HEMAR), speaker programs, advisory boards, educational grants and fellowships, drug safety, government affairs (including costs associated with compliance with the Sunshine Act and other similar government regulation); and field-based medical science liaisons, medical affairs clinical trial management, MD's in field (separate from medical science liaisons), publications, medical communications and field medical education.

Recall Expenses—costs, to the extent not otherwise covered by KHK's indemnity obligations, associated with notification, retrieval and return of Licensed Products in the Field in the Profit Share Territory, destruction of such returned Licensed Products in the Field, and distribution of the replacement Licensed Products in the Field.

Sales Force FTE Costs—costs associated with the sales force, calculated by multiplying the number of sales representative FTEs dedicated to Licensed Products in the Field in the Profit Share Territory by the applicable Sales Force FTE Rate.

Sales Force FTE Rate—an agreed upon multiple of the gross salaries (including any cash bonus or other performance-based cash incentive payments, to the extent based directly on sales or promotion of Licensed Products in the Field) under and in accordance with the Marketing Plan. The sales force FTE rate includes any automobile allowance, meal expenses, travel/housing for meetings and other incidental expenses incurred by such personnel in the ordinary course of employment.

Selling Costs—all out-of-pocket costs and internal costs (other than costs for sales representatives) directly attributable to selling Licensed Product in the Field, including first line sales managers, exhibits at shows or conventions including samples, charges for space, sales aids and brochures, sales meetings, consultants, call reporting and other Third Party monitoring/tracking services, sales training, sales administration and the like.

UGNX shall reasonably consider any requests by KHK with respect to the choice of vendors or subcontractors for Commercialization activities resulting in material Distribution Costs (customer and specialty pharmacy services), Marketing Research, Reimbursement/Access Services, Medical Affairs Expenses and/or Selling Costs.

LICENSE AGREEMENT

This License Agreement is entered into as of the 1st day of March, 2011 (the "EFFECTIVE DATE") between AAIPharma Services Corp., having its principal offices at 2320 Scientific Park Drive, Wilmington, North Carolina 28405 (hereinafter "AAI"), and Ultragenyx Pharmaceutical, Inc., having principal offices at 77 Digital Drive, Suite 210, Novato, California 94949 (hereinafter "ULTRAGENYX").

WHEREAS, AAI has developed and is the owner of certain know how and proprietary intellectual property related to pharmaceutical preparations of controlled release matrix tablet drug delivery formulations; and

WHEREAS, ULTRAGENYX desires to license certain rights to use such technologies on an exclusive basis, and AAI desires to grant such license to ULTRAGENYX, subject to the terms and conditions of this AGREEMENT.

NOW, THEREFORE, in consideration of the mutual covenants and promises in this AGREEMENT and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, AAI and ULTRAGENYX agree as follows:

ARTICLE I — RECITALS

1.01 Incorporation of Recitals. The foregoing recitals are hereby incorporated.

ARTICLE II — DEFINITIONS

"AAI CONFIDENTIAL INFORMATION" shall have the meaning set forth in Section 4.03 herein.

"AAI KNOW-HOW" shall mean all discoveries, methods, ideas which are conceived and reduced to practice, information, data, knowledge, processes, specification, designs, trade secrets, show-how and know-how and/or techniques whether or not patentable, which are presently owned, licensed or controlled by AAI (solely or jointly) or during the term of this AGREEMENT are invented, developed, owned, licensed or controlled by AAI (solely or jointly) and which are necessary or useful in the practice of the TECHNOLOGY or AAI PATENTS, or necessary or useful in the development, manufacture, or promotion or sale of any PRODUCT in the LICENSED FIELD, but shall not mean or include AAI PATENT RIGHTS.

"AAI PATENTS" or "AAI PATENT RIGHTS" shall mean any and all rights to all ideas and inventions embodied in the TECHNOLOGY, any and all claims in any and all patents and patent applications owned, controlled or licensed by AAI (solely or jointly) related to the TECHNOLOGY, and further including claims in any reissues, extensions, substitutions, continuations, divisions, continuations-in-part, supplementary protection certificates, registrations, revalidations, additions, renewals, substitutes in the United States and/or any foreign counterparts of any patents involving the TECHNOLOGY or other patents and patent applications encompassed under this definition. AAI PATENTS include, but are not limited to, any of the foregoing related to AAI's proprietary pharmaceutical formulations technology known as [***].

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

"AFFILIATE" shall mean: (i) a corporation or any other entity that directly, or indirectly through one or more intermediaries, owns or controls, is controlled by, or is under common control with, the designated party, but only for so long as the affiliate relationship exists, or (ii) Nobelpharma Co., Ltd. of Japan, but only for so long as the collaboration agreement between Nobelpharma Co., Ltd. and ULTRAGENYX exists. "Control" shall mean (i) the right to vote, or ownership of, shares of stock or other ownership interests or rights having at least fifty percent (50%) of the voting power entitled to vote for the election of directors or their equivalent in the case of a corporation or other entities, (ii) the right to appoint, directly or indirectly, a majority of the board of directors (or other comparable managers) or (iii) the right to control, directly or indirectly, the management or policies of the entity, whether through the ownership of voting securities, by contract or otherwise.

"AGREEMENT" shall mean this License Agreement and the exhibits hereto, as the same shall be amended from time to time.

"COMMERCIALLY REASONABLE EFFORTS" shall mean for efforts by either party hereto, those efforts that a prudent business person or company would expend in the normal course of business to accomplish an important objective (including marketing an important product, as appropriate), but shall not mean efforts that could, if carried out, have a significant negative impact on that party's relevant business unit as a whole.

"DOMINANT IP" shall mean all intellectual property and proprietary rights (whether patentable or not) that now or at any time during the term of this AGREEMENT are owned, controlled or licensed by AAI (solely or jointly) and that dominate or are otherwise required to practice any of the TECHNOLOGY, AAI KNOW-HOW or AAI PATENTS in the LICENSED FIELD.

"GOVERNMENTAL OR REGULATORY AUTHORITY" shall mean any court, tribunal, arbitrator, authority, agency, commission, official or other instrumentality of any country or any state, county, city or other political subdivision.

"LICENSED FIELD" shall mean use of Sialic Acid for treatment of distal myopathy with rimmed vacuoles or for Hereditary Inclusion Body Myopathy (HIBM), in any and all applications, markets and fields of use, whether now known or hereafter existing.

"LICENSED IP" shall mean the TECHNOLOGY, AAI KNOW-HOW, AAI PATENTS and DOMINANT IP.

"LICENSED TERRITORY" shall mean the territory set forth in Exhibit A attached hereto.

"PERSON" shall mean any natural person, corporation, general partnership, limited partnership, proprietorship, other business organization, trust, union, association or Governmental or Regulatory Authority.

"PRODUCT" shall mean any product (or any product made using a process, or any product that when used practices a method) that is covered by any of the AAI PATENT RIGHTS or DOMINANT IP, or that is based on, uses, includes, incorporates or otherwise embodies any of the LICENSED IP.

"SERVICES DATA" means all reports, analyses and data arising out of services performed by AAI for ULTRAGENYX.

"SUBLICENSE REVENUE" means revenue received by ULTRAGENYX (and its AFFILIATES) from NON-AFFILIATE SUBLICENSEES in exchange for a sublicense to practice under the LICENSED IP (including license fees, technology access fees, milestone payments and PRODUCT royalties). SUBLICENSE REVENUE does not include amounts received in exchange for equity, or amounts received for the sale, license, lease or other conveyance of know-how, patent rights or other intellectual property rights that are not LICENSED IP, or amounts received for the provision of scientific, research, development, clinical or other professional services.

"TECHNOLOGY" means AAI's controlled release matrix solid dose oral tablet consisting of certain combinations of generally recognized as safe pharmaceutically acceptable polymers in combinations that result in a controlled release of pharmaceutical active agents, which is sometimes referred to as ProCR™.

"THIRD PARTY" means an entity or person which is not party to this AGREEMENT or an AFFILIATE thereof nor a licensee nor sublicensee of such party or AFFILIATE.

"ULTRAGENYX CONFIDENTIAL INFORMATION" shall have the meaning as set forth in Section 4.02 herein. To avoid uncertainty, ULTRAGENYX CONFIDENTIAL INFORMATION includes the SERVICES DATA.

Unless the context of this AGREEMENT otherwise requires, (i) words of any gender include every other gender; (ii) the terms "hereof", "herein", "hereby" and derivative or similar words refer to this entire AGREEMENT; and (iii) the terms "Article" and "Section" refer to the specified Article and Section of this AGREEMENT. Whenever this AGREEMENT refers to a number of days, such number shall refer to calendar days unless business days are specified. The terms "including" or "includes", when used herein, shall mean "including, without limitation" and "includes, without limitation".

ARTICLE III — GRANT AND CONSIDERATION

3.01 License Grant. AAI hereby grants to ULTRAGENYX and its AFFILIATES a fully paid up, royalty-free, exclusive (even as to AAI), perpetual and irrevocable license (without right to sublicense, except as permitted in Section 3.03) to research, develop, make, have made, offer to sell, sell, have sold, use, have used and import PRODUCTS, and to otherwise practice, commercialize, exploit, use, modify, improve and make derivative works of the LICENSED IP, in each case, solely in the LICENSED FIELD and in the LICENSED TERRITORY.

3.02 Exclusivity. In granting the foregoing exclusive license under Section 3.01, AAI hereby agrees that (i) except at the request of ULTRAGENYX, it shall not research, develop, make, or have made, or (ii) offer to sell, sell, import or use, any PRODUCT (or otherwise practice, commercialize or exploit any of the LICENSED IP) in the LICENSED FIELD anywhere in the LICENSED TERRITORY, and that it shall not transfer or grant to any THIRD PARTY any right, license or option to do so, and that it shall not covenant not to sue any THIRD PARTY in respect of such prohibited activities.

3.03 Sublicense Rights. This AGREEMENT and license granted hereunder are personal to ULTRAGENYX and may not be assigned by ULTRAGENYX, except as permitted under Section 9.01. In addition, ULTRAGENYX shall not sublicense any of its rights under this AGREEMENT without AAI's written consent (such consent not be unreasonably conditioned, delayed or withheld), except that, without consent, ULTRAGENYX may sublicense any or all of its rights hereunder to: (a) any of its AFFILIATES; and/or (b) any other person or entity that is not an AFFILIATE (a "NON-AFFILIATE SUBLICENSEE"), but only pursuant to a written sublicense agreement between ULTRAGENYX and the NON-AFFILIATE SUBLICENSEE that is entered into on an arms-length basis, that includes payment by the NON-AFFILIATE SUBLICENSEE to ULTRAGENYX of a commercially reasonable fee, and that is executed by the parties thereto after the EFFECTIVE DATE. ULTRAGENYX's AFFILIATES may grant further sublicenses in accordance with the provisions of this Section 3.03, but NON-AFFILIATE SUBLICENSEES may not. ULTRAGENYX agrees not to sublicense any use or practice of the LICENSED IP outside the LICENSED FIELD.

3.04 Technology Transfer. Promptly after the EFFECTIVE DATE and on an ongoing basis during the term of this AGREEMENT (as new information becomes available), AAI shall disclose and transfer to ULTRAGENYX all tangible embodiments of the LICENSED IP in the LICENSED FIELD, including without limitation, all patent applications and patents, specifications, designs, documentation and technology implementations and ULTRAGENYX shall pay AAI for time and materials used in such technical transfer at AAI's standard rates.

3.05 Diligence. AAI acknowledges and agrees that nothing in this AGREEMENT shall be construed as ULTRAGENYX's promise or obligation to use best efforts, diligent efforts, COMMERCIALLY REASONABLE EFFORTS or any other level of diligence in commercializing the LICENSED IP or any PRODUCT in the LICENSED FIELD, and AAI hereby waives any such requirement that may be implied at law or otherwise. AAI agrees that any and all business, technical, clinical and regulatory plans, strategies, designs and decisions applicable to commercializing the LICENSED IP in the LICENSED FIELD shall be within ULTRAGENYX's sole discretion. ULTRAGENYX makes no commitment that its efforts will be continuous or successful, and it shall have the unrestricted right to redirect or abandon such activities, at any time, for any reason or no reason.

3.06 Consideration. In consideration of ULTRAGENYX conducting pre-clinical and clinical studies and making all data from such studies available to AAI for use in advancing the LICENSED IP and marketing, and in consideration of the rights and licenses granted to ULTRAGENYX hereunder and the other subject matter of this AGREEMENT, the parties agree as follows:

(a) [***] Share. Within [***] days after the [***], [***] shall [***] of the [***]. At the same time, [***] shall [***].

(b) Non Interference. Prior to the [***] anniversary of the EFFECTIVE DATE, AAI and its AFFILIATES shall not undertake or engage in any activity or service related to the development of modified release Sialic Acid formulations, extended release Sialic Acid formulations or other formulations of Sialic Acid, for its own benefit or the benefit of any person or entity (other than ULTRAGENYX and its AFFILIATES).

3.07 Audit. During the term of this AGREEMENT and for [***] thereafter, ULTRAGENYX agrees to keep and maintain accurate records and accounts of [***] received hereunder. Upon reasonable advance written notice (but not more than [***] in any [***] period), AAI shall have the right to have an independent certified public accountant (reasonably acceptable to ULTRAGENYX, and subject to execution of ULTRAGENYX's nondisclosure agreement) to verify ULTRAGENYX's compliance with its reporting and payment obligations under Section 3.06(a) and to report the results of its audit simultaneously to AAI and ULTRAGENYX. No period of time may be audited more than once. ULTRAGENYX shall make all directly applicable books and records available for such inspection during normal business hours at its principal place of business. Any such audit shall be at the expense of AAI, unless it discloses an error for the audited period in excess of [***] percent ([***]%), in which case ULTRAGENYX shall reimburse AAI for its reasonable out-of-pocket audit expenses.

ARTICLE IV — CONFIDENTIALITY

4.01 Disclosure. Upon execution of this AGREEMENT, and thereafter during the term hereon, at such times as the parties shall mutually agree, each party shall disclose to the other, in confidence subject to Section 4.02 and 4.03 hereof, such relevant AAI CONFIDENTIAL INFORMATION and/or ULTRAGENYX CONFIDENTIAL INFORMATION, as the case may be, as is reasonably necessary or useful to allow the other party to proceed with the activities contemplated or permitted by this AGREEMENT, subject to any existing confidentiality agreements.

4.02 Obligations of AAI. Except as specifically authorized by this AGREEMENT, AAI shall, for the term of this AGREEMENT and for [***] thereafter, keep confidential, exercise reasonable safeguards to prevent unauthorized access, use and disclosure, not disclose to others and use and copy only for the purposes provided for or permitted under this AGREEMENT, (a) any information supplied by or for ULTRAGENYX to AAI relating to ULTRAGENYX's or any AFFILIATE's business, employees, investors, finances, technologies, clinical trials or regulatory affairs, and (b) all information and data not described in clause (a) hereof but supplied by or for ULTRAGENYX to AAI under this AGREEMENT that is marked or otherwise identified as "Confidential" or proprietary at time of disclosure or that by its nature would be understood by a reasonable person to be proprietary or confidential, and (c) including all copies, analyses and derivatives thereof (collectively, "ULTRAGENYX CONFIDENTIAL INFORMATION"). Notwithstanding the above, AAI shall have no liability to ULTRAGENYX with respect to the following use, or disclosure to others not a party to this AGREEMENT or an AFFILIATE thereof, of ULTRAGENYX CONFIDENTIAL INFORMATION, as AAI can establish to,

(i) have been known by AAI on a non-confidential basis prior to communication by ULTRAGENYX to AAI;

(ii) have been a matter of public knowledge at the time of such disclosure by ULTRAGENYX to AAI;

(iii) have become a matter of public knowledge, without fault on the part of AAI or an agent thereof, subsequent to disclosure by ULTRAGENYX to AAI;

(iv) have been disclosed to AAI or an AFFILIATE thereof on a non-confidential basis from a THIRD PARTY lawfully having possession of the ULTRAGENYX CONFIDENTIAL INFORMATION without an obligation of confidentiality to ULTRAGENYX; or

(v) was independently developed by or for AAI by persons not having knowledge of such ULTRAGENYX CONFIDENTIAL INFORMATION.

4.03 Obligations of ULTRAGENYX. Except as specifically authorized by this AGREEMENT, ULTRAGENYX shall, for the term of this AGREEMENT and thereafter for so long as AAI's PATENT RIGHTS continue (but no less than [***]), keep confidential, exercise reasonable safeguards to prevent unauthorized access, use and disclosure, not disclose to others and use and copy only for the purposes provided for or permitted under this AGREEMENT, (a) any information supplied by or for AAI to ULTRAGENYX relating to LICENSED IP, (b) all information and data not described in clause (a) hereof but supplied by or for AAI to ULTRAGENYX under this AGREEMENT that is marked or otherwise identified as "Confidential" or proprietary at the time of disclosure or that by its nature would be understood by a reasonable person to be proprietary or confidential, and (c) all copies, analyses, and derivatives thereof (collectively, "AAI CONFIDENTIAL INFORMATION"). Notwithstanding the above, ULTRAGENYX shall have no liability to AAI with respect to the following use, or disclosure to others not a party to this AGREEMENT or an AFFILIATE thereof, of AAI CONFIDENTIAL INFORMATION, as ULTRAGENYX can establish to,

(i) have been known by ULTRAGENYX or any AFFILIATE on a non-confidential basis prior to communication by AAI to ULTRAGENYX;

(ii) have been a matter of public knowledge at the time of such disclosure by AAI to ULTRAGENYX;

(iii) have become a matter of public knowledge, without fault on the part of ULTRAGENYX, or agent thereof, subsequent to disclosure by AAI to ULTRAGENYX;

(iv) have been disclosed to ULTRAGENYX or any AFFILIATE on a non-confidential basis from a THIRD PARTY lawfully having possession of the AAI CONFIDENTIAL INFORMATION without an obligation of confidentiality to AAI; or

(v) was independently developed by or for ULTRAGENYX or any AFFILIATE by persons not having knowledge of such AAI CONFIDENTIAL INFORMATION.

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

4.04 Other Exceptions. In addition, notwithstanding the foregoing, AAI CONFIDENTIAL INFORMATION and/or ULTRAGENYX CONFIDENTIAL INFORMATION, may be (a) disclosed to governmental agencies and others where such AAI CONFIDENTIAL INFORMATION or ULTRAGENYX CONFIDENTIAL INFORMATION may be required to be included in regulatory filings permitted under the terms of this AGREEMENT or in patent applications filed within the United States Patent and Trademark Office or corresponding international patent offices; (b) provided to third parties under appropriate terms and conditions including confidentiality provisions substantially protective of the other party's CONFIDENTIAL INFORMATION as those in this AGREEMENT, but only if such third parties have a bona fide need to know for any purpose expressly permitted by this AGREEMENT; (c) published, if and to the extent such publication has been approved by both parties; or (d) disclosed to the extent required by applicable laws or regulations (including the regulations of the U.S. Securities and Exchange Commission, as determined by the outside SEC legal counsel for the party disclosing the other party's CONFIDENTIAL INFORMATION), pursuant to applicable legal process (including subpoenas and civil investigative demands), or pursuant to an order by a court, governmental agency or other regulatory body having competent jurisdiction, providing, however, that the party intending to disclose under Section 4.04(a) or 4.04(d) shall notify the other party prior to such disclosure and shall cooperate with such other party in the event such other party elects, by timely notice to the disclosing party prior to the disclosure, to legally contest such disclosure or request confidential treatment for or otherwise lawfully avoid disclosure of such information; and provided further, upon the request of the other party from time to time, each party agrees to confirm whether or not it has disclosed the other party's CONFIDENTIAL INFORMATION pursuant to Section 4.04(b) to any third party identified in the other party's request. In each of the foregoing cases, the recipient will use COMMERCIALLY REASONABLE EFFORTS to limit the disclosure and maintain confidentiality to the extent possible. Neither party shall disclose the existence or terms of this AGREEMENT without the other party's prior written consent, except that either party may disclose this AGREEMENT or its terms in connection with any legal or regulatory requirement, financing transaction or due diligence inquiry. In the case of any other publication pertaining specifically to or referencing any LICENSED IP or PRODUCT not covered by one of the permitted exceptions to non-publication above, each party recognizes the mutual interest in obtaining valid patent protection. Consequently, either party and its employees or consultants or any other THIRD PARTY wishing to make a publication (including any oral disclosure made without obligation of confidentiality) relating to work performed under this AGREEMENT or any DEFINITIVE AGREEMENT shall transmit to the other party (the "REVIEWING PARTY") a copy of the proposed written publication at least [***] days prior to submission for publication, or an abstract of such oral disclosure at least [***] days prior to submission of the abstract or the oral disclosure, whichever is earlier. The REVIEWING PARTY shall have the right upon timely written notice to the other party (w) to identify any of the REVIEWING PARTY's CONFIDENTIAL INFORMATION, which the publishing party shall delete and not disclose, (x) to propose modifications and redactions to the publication for patent reasons, which the publishing party shall reasonably consider, (y) to require delay in publication or presentation (for up to [***] days) in order to protect patentable information, or (z) to request that the information

be maintained as a trade secret, and, in such case, the publishing party shall consult with the REVIEWING PARTY in good faith. In the event that a party is legally required to provide a copy of this AGREEMENT or any related document to any THIRD PARTY (except in confidence as permitted by this AGREEMENT), such party shall redact CONFIDENTIAL INFORMATION from such document, except as otherwise required by law. Each party shall have the right to review and approve each redacted document prior to its submission to a THIRD PARTY, where practicable. A period of [***] days shall be allowed for review of a redacted document, with the exception of the order of a court or regulatory agency or other legal compulsion for disclosure on a shorter time basis, for which the maximum reasonable amount of time shall be afforded.

4.05 Ownership. Subject to the license rights granted hereunder, all rights, title and interests (including without limitation, intellectual property and proprietary rights) in and to the AAI CONFIDENTIAL INFORMATION and LICENSED IP shall remain with AAI. All rights, title and interests (including without limitation, intellectual property and proprietary rights) in and to all SERVICES DATA (whether currently existing or generated pursuant to future services) and other ULTRAGENYX CONFIDENTIAL INFORMATION shall remain with ULTRAGENYX. Nothing herein is intended to transfer the ownership of any party's CONFIDENTIAL INFORMATION, KNOW-HOW, or PATENT RIGHTS to the other party. In addition, each party to this AGREEMENT maintains the right to use its own CONFIDENTIAL INFORMATION, KNOW-HOW, and PATENT RIGHTS, for any purpose, except as specifically restricted herein.

ARTICLE V — WARRANTIES AND INDEMNIFICATIONS

5.01 Representations and Warranties.

(a) Representations and Warranties of AAI. AAI hereby represents and warrants, the following:

(i) AAI has and will own all right, title and interest in and to LICENSED IP and has and will maintain the right to grant to ULTRAGENYX the exclusive license set forth in Section 3.01 hereunder.

(ii) AAI has full corporate power and authority to execute and deliver this AGREEMENT and to perform its obligations hereunder and to consummate the transactions contemplated hereby.

(iii) except for ULTRAGENYX, no other PERSON presently has any license, option or other right with respect to the manufacture, use and sale of the PRODUCT or the practice of the LICENSED IP, in each case in the LICENSED FIELD and LICENSED TERRITORY.

(iv) there are no adverse actions, suits or claims pending against AAI or any of its AFFILIATES in or before any GOVERNMENTAL OR REGULATORY AUTHORITY with respect to the LICENSED IP and no such actions, suits or claims have been threatened against AAI or any of its AFFILIATES.

(v) to the best of AAI's knowledge there is no reason why any of the LICENSED IP could be rendered invalid or unenforceable upon challenge and prosecution post filing.

(b) AAI further represents and warrants that during the term of this AGREEMENT, it will not develop or commercialize another PRODUCT in the LICENSED FIELD for sale within the LICENSED TERRITORY, except on the request of ULTRAGENYX.

AAI'S WARRANTIES SET FORTH IN THIS AGREEMENT ARE ITS EXCLUSIVE WARRANTIES TO ULTRAGENYX, AND ARE GIVEN AND ACCEPTED IN LIEU OF ANY AND ALL OTHER WARRANTIES, GUARANTEES, CONDITIONS AND REPRESENTATIONS, EXPRESS OR IMPLIED, INCLUDING ANY IMPLIED WARRANTIES OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE. AAI specifically disclaims any warranty of performance of the LICENSED IP in the LICENSED FIELD.

5.02 AAI Indemnity Obligations. AAI agrees to defend, indemnify and hold ULTRAGENYX and its AFFILIATES harmless from any and all costs, expenses, damages, judgments, and liabilities (including reasonable attorneys' fees) incurred by or rendered against ULTRAGENYX or AFFILIATE as a result of any THIRD PARTY claim or suit brought to the extent resulting from a breach by AAI of its covenants or warranties as set forth herein or AAI's negligence or willful misconduct, or arising out of any illegal or unlicensed use by AAI or its transferees or sub-licensees of ULTRAGENYX or any AFFILIATE's patent rights or other intellectual property or proprietary rights, except for claims to the extent arising from a breach of this AGREEMENT that is caused or aggravated in substantial part by ULTRAGENYX's negligence or a breach of a material representation, warranty, covenant or agreement provided herein by ULTRAGENYX. ULTRAGENYX shall give prompt written notice of any such claim or suit, and AAI shall undertake the defense thereof, at AAI's expense. ULTRAGENYX shall cooperate in such defense, to the extent reasonably requested by AAI, at AAI's expense. ULTRAGENYX shall have the right to participate in such defense, at its own expense, to the extent that in its judgment ULTRAGENYX may be prejudiced thereby. In any claim made or suit brought for which ULTRAGENYX seeks indemnification under this Section 5.02, ULTRAGENYX shall not settle, offer to settle, or admit liability or damages without the prior written consent of AAI.

5.03 Negation of Implications. Notwithstanding any provisions set forth in this AGREEMENT to the contrary, nothing in this AGREEMENT shall be construed as:

(i) a warranty or representation by AAI as to the validity or scope of any AAI PATENT RIGHTS; or

(ii) granting by implication, estoppel, or otherwise, any licenses or rights under LICENSED IP, other than as set forth in the grant provisions of Section 3.01.

Except as otherwise set forth in this AGREEMENT, AAI makes no representations or warranties, and assumes no responsibilities or liabilities whatsoever, with respect to use (including clinical efficacy), sale, or other disposition by ULTRAGENYX or its licensees, sub-licensees or vendees, or other transferees or consumers of any PRODUCT in the LICENSED FIELD.

5.04 ULTRAGENYX Warranties. ULTRAGENYX represents and warrants that:

(a) ULTRAGENYX has full corporate power and authority to execute and deliver this AGREEMENT and to perform its obligations hereunder and to consummate the transactions contemplated hereby; and

(b) ULTRAGENYX will use LICENSED IP only for PRODUCTS in the LICENSED FIELD.

ULTRAGENYX'S WARRANTIES SET FORTH IN THIS AGREEMENT ARE ITS EXCLUSIVE WARRANTIES TO AAI, AND ARE GIVEN AND ACCEPTED IN LIEU OF ANY AND ALL OTHER WARRANTIES, GUARANTEES, CONDITIONS AND REPRESENTATIONS, EXPRESS OR IMPLIED, INCLUDING ANY IMPLIED WARRANTIES OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE.

5.05 ULTRAGENYX Indemnity Obligations. ULTRAGENYX agrees to defend, indemnify and hold AAI harmless from any and all costs, expenses, damages, judgments, and liabilities (including reasonable attorneys' fees) incurred by or rendered against AAI as a result of any THIRD PARTY claim or suit brought to the extent resulting from a breach by ULTRAGENYX of its covenants or warranties as set forth herein or ULTRAGENYX's negligence or willful misconduct, or arising out of any illegal or unlicensed use by ULTRAGENYX or its transferees or sub-licensees of LICENSED IP furnished under this AGREEMENT, and/or out of any use, sale, or other disposition in the LICENSED FIELD by ULTRAGENYX or its licensees, sub-licensees or transferees of PRODUCT(S) in the LICENSED FIELD or by any distributors and/or consumers thereof, except for claims to the extent arising from a breach of this AGREEMENT that is caused or aggravated in substantial part by AAI's negligence or a breach of a material representation, warranty, covenant or agreement provided herein by AAI. AAI shall give prompt written notice of any such claim or suit, and ULTRAGENYX shall undertake the defense thereof, at ULTRAGENYX's expense. AAI shall cooperate in such defense, to the extent reasonably requested by ULTRAGENYX, at ULTRAGENYX's expense. AAI shall have the right to participate in such defense, at its own expense, to the extent that in its judgment AAI may be prejudiced thereby. In any claim made or suit brought for which AAI seeks indemnification under this Section 5.05, AAI shall not settle, offer to settle, or admit liability or damages without the prior written consent of ULTRAGENYX.

5.06 Mitigation. In the event of any occurrence which may result in either party becoming liable under Section 5.02 or 5.05, each party shall use COMMERCIALLY REASONABLE EFFORTS to take such actions as may be reasonably necessary to mitigate the damages payable by the other party under Section 5.02 or 5.05, as the case may be.

ARTICLE VI — PATENT PROSECUTION AND INFRINGEMENT

6.01 Prosecution. Using COMMERCIALLY REASONABLE EFFORTS, AAI shall be responsible, at its sole cost and expense, except as otherwise set forth herein, for the prosecution and maintenance of all AAI PATENTS (including applying for available patent extensions) in the United States. AAI will provide to ULTRAGENYX copies of relevant patent applications and substantive communications to and from the U.S. patent offices. At least [***], AAI agrees to make its patent counsel available to meet (in person or by telephone) with ULTRAGENYX's patent counsel to share information about prosecution and maintenance of the PATENT RIGHTS (e.g., status, issues, upcoming filings) and related matters. AAI will incorporate ULTRAGENYX's reasonable suggestions in filing, prosecuting and maintaining AAI PATENTS relative to the LICENSED FIELD but AAI shall have the sole right as to the final content of any such applications and/or communications.

6.02 Infringement Actions.

(a) ULTRAGENYX and AAI each shall promptly notify each other of any infringement of the LICENSED IP, and/or unauthorized use of any LICENSED IP by one or more THIRD PARTY that may come to its respective attention. AAI shall promptly undertake COMMERCIALLY REASONABLE EFFORTS to obtain a discontinuance of the aforesaid infringement or unauthorized use and, if not successful, AAI may, but is not required to, bring suit against such infringer or unauthorized user. At ULTRAGENYX's election, ULTRAGENYX may join such suit initiated by AAI and contribute to the cost of such proceedings, and in such case ULTRAGENYX shall be entitled to share in any sums recovered pro rata in relation to the extent ULTRAGENYX has contributed following reimbursement of each party's respective costs associated with such actions under this Section 6.02(a),

(b) If AAI fails to obtain a discontinuance of said infringement or unauthorized use and elects not to bring suit against such THIRD PARTY within [***] days after notice thereof, or immediately if AAI elects not to pursue such THIRD PARTY, then in any such event AAI shall give notice in writing to ULTRAGENYX of its failure or election not to bring suit against such infringement or unauthorized use, including such evidence of infringement as AAI may possess, the numbers of the AAI PATENT(S) so infringed and the unauthorized use of LICENSED IP. ULTRAGENYX may, but is not required to, assume sole control and authority to (i) obtain a discontinuance of the infringing operation or unauthorized use or (ii) bring suit against such third party. Any suit by ULTRAGENYX may be either in the name of ULTRAGENYX, or in the name of AAI, or jointly by AAI and ULTRAGENYX, as may be required by the laws of the forum, and, in furtherance of such rights, AAI hereby agrees that ULTRAGENYX may join AAI as a party plaintiff in any such suit.

(c) It is understood and agreed that the party to this AGREEMENT that solely institutes a suit or an action hereunder, without the other party hereto joining in such action or joinder of such other party by operation of law, shall solely bear all costs and expenses associated therewith, and shall be entitled to retain and keep any and all sums received, obtained, collected or recovered whether by judgment, settlement or otherwise, as a result of such suit or action. In addition, with respect to any suit for infringement of the LICENSED IP or unauthorized use of LICENSED IP, the party that did not institute suit shall render all reasonable assistance, cooperation and information, at the instituting party's expense, including executing all documents as may be reasonably requested by the party that did institute suit and making available any relevant records, documents, information, evidence, samples and the like for the action providing such records, documents, information, evidence, samples and the like are not detrimental or adverse to the party not instituting suit.

6.03 Infringement or Unauthorized Use of THIRD PARTY Patent, or KNOW-HOW. Each party hereto shall notify the other promptly in the event of the receipt of notice of any action, suit or claim alleging infringement by the PRODUCT, or of unauthorized use, of any patent held or know-how owned, by a THIRD PARTY in the LICENSED TERRITORY arising from the manufacture, use distribution, sale, or use of the PRODUCTS. The party's whose PRODUCTS are at issue shall have the sole right and authority to defend any such action, suit or claim.

6.04 Settlements. Neither party shall settle or enter into any voluntarily final judgment regarding any action, claim or suit under Section 6.02 or 6.03 that is inconsistent with any provision of this AGREEMENT.

ARTICLE VII — FORCE MAJEURE

7.01 Event of Force Majeure. Neither party shall be responsible or liable to the other hereunder for the failure or delay in the performance of this AGREEMENT due to any civil unrest, war, governmental action, fire, earthquake, hurricane, accident or other casualty, strike or labor disturbance, act of God or the public enemy, or any other contingency beyond the party's reasonable control. In the event of the applicability of this Section 7.01, the party failing or delaying performance shall use COMMERCIALLY REASONABLE EFFORTS to eliminate, cure and overcome any of such causes and resume the performance of its obligations.

7.02 Notification. Upon the occurrence of an event of force majeure, the party failing or delaying performance shall promptly notify the other party in writing pursuant to the provisions of Section 11.04 herein, setting forth the nature of the occurrence, its expected duration, and how such party's performance is affected. The failing or delaying party shall resume performance of its obligations hereunder as soon as practicable after the force majeure event ceases.

ARTICLE VIII — TERM AND TERMINATION

8.01 Term. The Term of this AGREEMENT shall commence on the EFFECTIVE DATE and remain in effect until terminated as provided herein.

8.02 Convenience. ULTRAGENYX may terminate this AGREEMENT for any reason or no reason at any time, upon at least thirty (30) days prior written notice to AAI.

8.03 Termination for Material Breach. If either party breaches or defaults in the performance or observance of any of its material obligations under this AGREEMENT (other than pursuant to one or more events of force majeure as provided for in Article VII herein) and such material breach or default is not cured within sixty (60) days after receipt by such party of a written notice from the non-breaching party specifying the material breach or default (or such longer period as is reasonably necessary if the breach is of such nature that it cannot be reasonably cured within such sixty (60) day period), the non-breaching party shall have the right to terminate this AGREEMENT upon an additional thirty (30) days' written notice to the breaching or defaulting party.

8.04 Rights on Termination. Termination of this AGREEMENT for any reason shall terminate all rights, licenses and obligations of the parties hereunder, except that termination shall be without prejudice to:

(i) either party's rights under this AGREEMENT with respect to obligations accruing prior to termination or claims arising out of events occurring prior to termination;

(ii) any other remedies which either party may otherwise have; and

(iii) all sublicenses properly granted to NON-AFFILIATE SUBLICENSEES by ULTRAGENYX or any AFFILIATE prior to termination, which shall remain in effect in accordance with their terms, and ULTRAGENYX agrees to assign to AAI all such sublicense agreements, and AAI hereby agrees to accept assignment thereof (but not to assume ULTRAGENYX's obligations thereunder); *provided*, after any such assignment, each sublicensee may elect, at its sole discretion, to terminate its sublicense agreement upon written notice to AAI.

8.05 Return of Confidential Information. Upon termination or expiration of this AGREEMENT, and at any other time upon receipt of written notice by the party which previously, and pursuant to this AGREEMENT, disclosed its CONFIDENTIAL INFORMATION (the "DISCLOSING PARTY") to the other party hereto (the "RECEIVING PARTY"), the RECEIVING PARTY shall, as soon as reasonably practicable following receipt of such notice, return all CONFIDENTIAL INFORMATION of the DISCLOSING PARTY'S and, unless otherwise set forth herein, immediately cease and desist all use of such CONFIDENTIAL INFORMATION. Notwithstanding the foregoing, RECEIVING PARTY may continue to possess and use that portion of the other's CONFIDENTIAL INFORMATION as to which this AGREEMENT expressly provides RECEIVING PARTY a continuing right or license to use such CONFIDENTIAL INFORMATION (such as, for example, for the purposes permitted under Section 4.04(a) in connection with regulatory filings and applying for patent protection), subject always to all other restrictions in this AGREEMENT regarding the use and disclosure of CONFIDENTIAL INFORMATION.

8.06 Limitations on Liability for Proper Termination. Neither party shall incur any liability to the other by reason of the permitted termination of this AGREEMENT as provided herein, whether for loss of goodwill, anticipated profits or otherwise, and the parties shall accept all rights granted and all obligations assumed hereunder, including those in connection with such termination, in full satisfaction of any claims resulting from such permitted termination.

ARTICLE IX — ASSIGNMENT

9.01 Assignment. This AGREEMENT and its rights and obligations shall not be assigned or delegated by either party without the prior written consent of the other party, such consent not to be unreasonably withheld, except that without consent, either party may assign this AGREEMENT and its rights and duties under this AGREEMENT, in whole or in part, to any successor (including the surviving company in any consolidation, reorganization or merger), or to any assignee or transferee of all or substantially all of its assets or business, or to any AFFILIATE. This AGREEMENT will be binding upon and inure to the benefit of the successors, representatives and permitted assigns of the parties.

ARTICLE X — DISPUTE RESOLUTION

10.01 Dispute Resolution.

(a) Except as otherwise provided in Section 10.01(c), all disputes or claims which may arise under, out of or in connection with this AGREEMENT (each, a "DISPUTE") will be referred in writing by the party raising the DISPUTE to the person designated in Section 11.04 herein for attempted resolution by good faith negotiations. If the DISPUTE remains unresolved for more than [***] business days after the notice of such DISPUTE, the parties will submit the DISPUTE to the next step in the dispute resolution process set forth in subsection (b) of this Section 10.01.

(b) If any DISPUTE is not resolved in accordance with subsection (a), the DISPUTE will be referred in writing (by the party that originally raised the DISPUTE) to ULTRAGENYX's Chief Executive Officer and AAI's Chief Executive Officer for attempted resolution by good faith negotiations. If they are unable to resolve any DISPUTE within [***] business days after the referral of such DISPUTE to them, the parties will submit the DISPUTE to the next step in the dispute resolution process set forth in subsection (c) of this Section 10.01.

(c) If either party desires to pursue any DISPUTE which is no longer subject to the dispute resolution process as provided in Section 10.01 (a) or (b), such party may submit the DISPUTE to any United States District Court or state court of competent jurisdiction.

(d) No DISPUTE under this AGREEMENT will be the subject of formal judicial proceedings between ULTRAGENYX and AAI before following the dispute resolution procedures set forth in Sections 10.01(a) and (b), except for an action to seek specific performance or injunctive relief to protect CONFIDENTIAL INFORMATION or intellectual property rights pursuant to this AGREEMENT. Accordingly, each party agrees that, in the event of any breach or threatened breach of Section 3.01 or Article IV or any other infringement, misappropriation or violation of its intellectual property rights, the non-breaching party will suffer

irreparable damage for which it will have no adequate remedy at law. Accordingly, the non-breaching party shall, in addition to any other legal or equitable remedies, be entitled to seek an order for specific performance, or an injunction or similar equitable relief against such breach or threatened breach, without the necessity of posting any bond.

(e) Notwithstanding anything to the contrary contained in this AGREEMENT, in the event of a DISPUTE arising out of, relating to or in connection with termination of this AGREEMENT pursuant to Section 8.03 herein, the dispute resolution process set forth in this Section 10.01 is intended to and will toll any cure periods and any notice periods set forth in such Section 8.03.

ARTICLE XI — MISCELLANEOUS

11.01 Waiver and Amendment. Any waiver by any party hereto of a breach of any provisions of this AGREEMENT shall not be implied and no consent or waiver shall be valid unless it is recited in writing and signed by such party. Failure of any party to require, in one or more instances, performance by the other party in strict accordance with the terms and conditions of this AGREEMENT shall not be deemed a waiver or relinquishment of the future performance of any such terms or conditions or of any other terms and conditions of this AGREEMENT. A waiver by either party of any term or condition of this AGREEMENT shall not be deemed or construed to be a waiver of such term or condition for any other term. All rights, remedies, undertakings, obligations and agreements contained in this AGREEMENT shall be cumulative and no one of them shall be a limitation of any other remedy, right, undertaking, obligation or agreement of either party. This AGREEMENT may not be amended except in a writing signed by both parties.

11.02 Relationship of the Parties. For all purposes of this AGREEMENT, AAI and ULTRAGENYX shall be deemed to be independent entities and anything in this AGREEMENT to the contrary notwithstanding, nothing herein shall be deemed to constitute AAI and ULTRAGENYX as partners, joint ventures, co-owners, or an association, nor shall this AGREEMENT constitute any party hereto an employee or agent, legal or otherwise, of the other party for any purposes whatsoever. Neither party hereto is authorized to make any statements or representations on behalf of the other party or in any way obligate the other party, except as expressly authorized in writing by the other party. Anything in this AGREEMENT to the contrary notwithstanding, no party hereto shall assume nor shall be liable for any liabilities or obligations of the other party, whether past, present or future.

11.03 Headings. The headings set forth at the beginning of the various Articles and Sections of this AGREEMENT are for reference and convenience and shall not affect the meanings of the provisions of this AGREEMENT.

11.04 Notices. Notices required under this AGREEMENT shall be in writing and delivered in person or sent by registered or certified mail, postage prepaid and return receipt requested, or by facsimile and confirmed by registered or certified mail, postage prepaid and return receipt requested, or by Federal Express or UPS or other recognized express courier (prepaid with confirmation of delivery), and addressed as follows:

If to ULTRAGENYX: Ultragenyx Pharmaceutical, Inc.
77 Digital Drive, Suite 210
Novato, CA 94949
Attention: Chief Executive Officer

If to AAI: AAIPharma Services Corp.
2320 Scientific Park Drive
Wilmington, NC 28405
Attention: Legal Department

All notices shall be deemed to be effective upon receipt. Either party may change the address at which written notice is to be received pursuant to this Section 11.04.

11.05 Severability. If any provision of this AGREEMENT is held by a court of competent jurisdiction to be invalid or unenforceable, it shall be modified, if reasonably possible, to the minimum extent necessary to make it valid and enforceable or, if such modification is not reasonably possible, it shall be stricken to the minimum extent necessary such that the remaining provisions shall remain in full force and effect.

11.06 Survival. The provisions of Article II (as such definitions pertain to surviving Sections and Articles), Articles IV, V, VIII, IX, X and XI and Sections 3.06 and 3.07 shall survive any termination of this AGREEMENT.

11.07 No Conflict. Each party represents that neither this AGREEMENT nor any of its obligations hereunder will conflict or result in a breach of any arrangement or agreement between such party and any THIRD PARTY.

11.08 Entire Agreement. This AGREEMENT, including the attachments and appendices hereto, sets forth the entire understanding between the parties hereto as to the subject matter hereof and supersedes all other documents, agreements, verbal consents, arrangements and understandings by or between the parties with respect to the subject matter hereof. Prior to the execution of this AGREEMENT, the parties have had numerous discussions, conversations and negotiations, and have generated correspondence, writings and other memoranda with respect to the subject matter hereof. Notwithstanding all of such activities, this AGREEMENT (including the attachments and appendices hereto) is intended to define the full extent of the parties respective agreements, arrangements and obligations with respect to the subject matter hereof, and each party represents that it is not relying on any such other discussions, conversations, negotiations, correspondence, writings and memoranda in executing and delivering this AGREEMENT or performing its respective obligations hereunder. This AGREEMENT may be executed in one or more counterparts, each of which shall be an original, but taken together constituting one and the same instrument. Execution of a facsimile or email (.pdf) copy shall have the same force and effect as execution of an original, and a facsimile or email (.pdf) signature shall be deemed an original and valid signature.

11.09 Limitation of Grant. Except for the limited rights and licenses expressly granted hereunder, no other license is granted (by implication, estoppel, or otherwise) and no other use is permitted.

11.10 Governing Law; Courts. This AGREEMENT shall be governed by, and construed and enforced in accordance with the substantive laws of the State of Delaware, without giving effect to such state's rules concerning conflicts of law.

11.11 No Publicity. Except as may be required by applicable laws or regulations, neither party may use any name, trade name, trademark, or other designation of the other party (or its affiliates or employees) in any press release, advertising, marketing, publicity or other promotional activity without the other party's prior written consent.

IN WITNESS WHEREOF, the parties hereto have caused this AGREEMENT to be executed as of the date first written above by their duly authorized representatives.

AAIPharma Services Corp.

By: /s/ Philippe Maitre
Name: Philippe Maitre
Title: Chief Financial Officer

Ultragenyx Pharmaceutical, Inc.

By: /s/ Emil D. Kakkis
Name: Emil D. Kakkis, MD, PhD
Title: Chief Executive Officer

Exhibit A

Territory

The following countries abbreviated by their two-letter codes for the representation of states, other entities and intergovernmental organizations used by the World Intellectual Property Organization (WIPO):

[***]

The following countries abbreviated by their two-letter codes for the representation of states, other entities and intergovernmental organizations used by the African Regional Intellectual Property Org. (ARIPO):

[***]

The following countries abbreviated by their two-letter codes for the representation of states, other entities and intergovernmental organizations used by Eurasian Patent Organization (EAPO):

[***]

The following countries abbreviated by their two-letter codes for the representation of states, other entities and intergovernmental organizations used by European Patent Office (EPO):

[***]

The following countries abbreviated by their two-letter codes for the representation of states, other entities and intergovernmental organizations used by African Intellectual Property Organization (OAPI):

[***]

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

License Agreement

This License Agreement (this "**Agreement**") is made as of September 20, 2012 (the "**Effective Date**"), by and between Ultragenyx Pharmaceutical Inc., a corporation organized and existing under the laws of the State of Delaware, having its principal place of business at 60 Leveroni Court, Novato, CA 94949 ("**Ultragenyx**"), and Baylor Research Institute, a non-profit corporation organized and existing under the laws of the State of Texas, having its principal place of business at 3310 Live Oak Street, Suite 501, Dallas, Texas 75204 ("**BRI**"). Ultragenyx and BRI are referred to in this Agreement individually as a "**Party**" and collectively as the "**Parties**."

Recitals

WHEREAS, Ultragenyx is a biotechnology company focused on the discovery and development of innovative therapeutics for patients with rare and ultra-rare genetic diseases;

WHEREAS, BRI is a research center focused on finding prevention therapies and treatments for diseases and illnesses;

WHEREAS, BRI owns or controls certain intellectual property related to the Compound (as defined below); and

WHEREAS, the Parties desire for Ultragenyx to obtain certain rights and licenses to such intellectual property pertaining to the Compound in order to develop, manufacture and commercialize prophylactic, therapeutic and diagnostic products pursuant to the terms and conditions set forth herein.

NOW, THEREFORE, in consideration of the foregoing premises and the mutual covenants herein contained, the receipt and sufficiency of which are hereby acknowledged, Ultragenyx and BRI hereby agree as follows:

Article 1

Definitions

The terms in this Agreement with initial letters capitalized, shall have the meanings set forth below, or the meaning as designated in the indicated places throughout this Agreement.

1.1 "Active Ingredient" means a therapeutically active material that provides pharmacological activity in a nutritional or pharmaceutical product (excluding formulation components such as coatings, stabilizers, excipients or solvents, adjuvants or controlled release technologies).

1.2 "Affiliate" means, with respect to a Party, any Entity that controls, is controlled by, or is under common control with that Party. For the purpose of this definition, "control" means, direct or indirect, ownership of more than fifty percent (50%) of the shares of stock entitled to vote for the election of directors, in the case of a corporation, or more than fifty percent (50%) of the equity interest in the case of any other type of legal entity, status as a

general partner in any partnership, or any other arrangement whereby the Entity controls or has the right to control the board of directors or equivalent governing body of a corporation or other entity, or the ability to cause the direction of the management or policies of a corporation or other entity.

1.3 "[***]" means [***].

1.4 "[***] Option" means that certain option pursuant to the option and license agreement between BRI and [***], dated [***] and as amended by the amendment agreement dated [***], which grants [***] the option to obtain a license to develop, manufacture and commercialize Compound and Product in the [***].

1.5 "[***] Option Termination" is defined in Section 2.2(a).

1.6 "BRI Indemnitee" is defined in Section 9.2.

1.7 "BRI Know-How" means, subject to Section 10.2(b), any and all Know-How Controlled by BRI or any of its Affiliates as of the Effective Date or thereafter during the Term that relates to, or is otherwise reasonably necessary or reasonably useful for, the use, development, manufacture or commercialization of any Compound or Product.

1.8 "BRI Patents" means any and all Patents Controlled by BRI or its Affiliate(s) as of the Effective Date or thereafter during the Term that: (i) claim the composition of matter of, or the method of manufacturing or using, any Compound or Product; or (ii) that otherwise relate to, or are reasonably necessary for, the use, development, manufacture or commercialization of any Compound or Product, including the Patents set forth in Exhibit A.

1.9 "BRI Technology" means BRI Know-How and BRI Patents.

1.10 "Business Day" means a day other than (a) a Saturday or Sunday, or (b) a day on which commercial banks located in San Francisco, California are authorized or required by law to be closed.

1.11 "Calendar Quarter" means the respective periods of three (3) consecutive calendar months ending on March 31, June 30, September 30 and December 31.

1.12 "Calendar Year" means a period of twelve (12) consecutive months ending on December 31.

1.13 "Claims" means all Third Party demands, claims, actions, proceedings, orders, findings and verdicts (in contract, tort or otherwise), as well as losses of any type, damages and legal costs resulting therefrom, including, without limitation, any product liability or substantially equivalent claims.

1.14 "Combination Product" means:

(i) a Product that contains both a Compound and one or more other Active Ingredients that are not Compounds;

(ii) a product consisting of one or more separate products packaged together with a Product in a single package or as a unit; or

(iii) a drug, device, test, kit or biological product packaged separately that is sold as a unit with a Product.

1.15 "Commencement" means, with respect to a clinical trial of any Compound or Product, the [***].

1.16 "Commercially Reasonable Efforts" means: (a) where applied to carrying out specific tasks and obligations of a Party under this Agreement other than development, manufacture or commercialization of a Product, expending reasonable, diligent, good faith efforts and resources to accomplish such task or obligation as a similarly situated pharmaceutical or biotechnology company (on its own or acting through any of its Affiliates, sublicensees or subcontractors) would normally use to accomplish a similar task or obligation under similar circumstances; and (b) where applied to the development, manufacture or commercialization of a Product, those reasonable efforts and resources customarily used by such Party with respect to a similar pharmaceutical product Controlled by such Party, which product is at a similar stage in its development or product life and is of similar market potential in the applicable market taking into account efficacy, safety profile, labeling, the then-current and expected competition in the applicable market, the likely timing of entry into the market, the expected extent and speed of market penetration, the patent and other proprietary position of the Product, the likelihood of regulatory approval given the regulatory structure involved, the profitability of the Product, including the cost of manufacture, royalties payable to licensors of patent or other intellectual property rights, alternative products and other relevant factors and other scientific, clinical or commercial factors. With respect to subpart (b) of this definition, Commercially Reasonable Efforts shall be determined on a market-by-market and indication-by-indication basis for a particular Product, and it is anticipated that the level of effort shall be different for different markets and different indications, and shall change over time, reflecting changes in the status of the Product and the market(s) and indication(s) involved.

1.17 "Compound" means any of the following: (a) Triheptanoin; (b) [***], and in each case of (a) and (b), including any [***] thereof.

1.18 "Confidential Information" means all proprietary Know-How, unpublished patent applications and other information and data of a financial, commercial, business, operational or technical nature which: (a) the disclosing Party or any of its Affiliates has supplied or otherwise made available to the other Party or any of its Affiliates in connection with this Agreement, whether prior to or during the Term and whether made available orally, by observation, in writing or in electronic form; or (b) the receiving Party has learned from the disclosing Party in the course of this Agreement, in each case including information comprising or relating to concepts, discoveries, inventions, data, designs or formulae in relation to this Agreement.

1.19 "Control" or **"Controlled"** means, with respect to any Know-How, molecule, material, Patents, other intellectual property, or any proprietary or trade secret information, the legal authority or right (whether by ownership, license or otherwise), as of the Effective Date or

during the Term, to: (i) grant ownership of or a license or sublicense to make, use, offer to sell, sell or import such molecule or material; (ii) grant ownership of or a license or a sublicense under such Know-How, Patents, or intellectual property; or (iii) otherwise disclose such proprietary or trade secret information, in each case without breaching the terms of any agreement with, obligation to or other arrangement with a Third Party, or misappropriating the proprietary or trade secret information of a Third Party; in each case as provided in this Agreement.

1.20 "Disclosing Party" is defined in Section 6.1(a).

1.21 "Dollar" or **"$"** means the legal tender of the United States.

1.22 "EMA" means the European Medicines Agency or any successor entity thereto performing substantially the same functions.

1.23 "Entity" means a partnership, limited partnership, limited liability partnership, corporation, limited liability company, business trust, joint stock company, trust, incorporated association, joint venture or similar entity or organization.

1.24 "European Union" means the European Union member states as then constituted; provided, as of the Effective Date, the European Union member states are [***].

1.25 "Fatty Acid Oxidation Disorder" or **"FAOD"** means a group of inherited metabolic disorders associated with a specific enzyme defect in the fatty acid metabolic pathway and affecting utilization of dietary and stored fat. Fatty Acid Oxidation Disorders include, without limitation, disorders of the following enzymes: Carnitine-Acylcarnitine Translocase (CATR), Carnitine Palmitoyltransferase I and II (CPT I, CPT II), Very-Long Chain Acyl-CoA dehydrogenase (VLCAD), L-3-Hydroxy-Acyl-CoA Dehydrogenase (LCHAD), and Mitochondrial Trifunctional Protein (TFP).

1.26 "FD&C Act" means the federal Food, Drug and Cosmetic Act, as amended, and the regulations promulgated thereunder from time to time.

1.27 "FDA" means the United States Food and Drug Administration or any successor entity thereto performing substantially the same functions.

1.28 "Field" means [***].

1.29 "First Commercial Sale" means, with respect to any Product in any country or jurisdiction in the Licensed Territory, the first (1st) *bona fide* commercial sale by or on behalf of Ultragenyx, its Affiliates or sublicensees to a Third Party other than sublicensees for distribution, use or consumption of any such Product in such country or jurisdiction after the Regulatory Approvals and any applicable Pricing Approvals have been obtained for such Product in such country or jurisdiction.

1.30 "Generic Product" means, with respect to a particular Product, and on a country-by-country basis, any nutritional or pharmaceutical product, other than a Product, that contains the same Active Ingredient as such Product and that is commercialized by a Third Party, which

Third Party is not a licensee or sublicensee of Ultragenyx or its Affiliates, or any of their licensees or sublicensees, and has not obtained such pharmaceutical product from a chain of distribution including Ultragenyx or any of its Affiliates, licensees or sublicensees or further sublicensees. The term Generic Product does not include any Product licensed or produced by Ultragenyx or any of its Affiliates or sublicensees (i. e. an authorized generic product).

1.31 "Government Authority" means any federal, state, national, regional, provincial or local government, or political subdivision thereof, or any multinational organization or any authority, agency or commission entitled to exercise any administrative, executive, judicial, legislative, police, regulatory or taxing authority or power, any court or tribunal (or any department, bureau or division thereof, or any governmental arbitrator or arbitral body).

1.32 "IND" means an Investigational New Drug Application submitted to the FDA in conformance with applicable laws and regulations, or the foreign equivalent of any such application in any other country (such as a clinical trial application in the European Union).

1.33 "Indemnified Party" is defined in Section 9.4.

1.34 "Indemnifying Party" is defined in Section 9.4.

1.35 "IMD" is defined in Section 2.4.

1.36 "Know-How" means any and all tangible and intangible information and materials, including research and development data, regulatory submissions and correspondence, manufacturing information and processes, formulations, assays, cell lines, sequences, composition of matter, constructs, discoveries, improvements, modifications, processes, methods, protocols, formulas, utility, data (including physical, chemical, biological, toxicological, pharmacological, analytical, quality control, preclinical, clinical, and veterinary data), results, inventions, know-how and trade secrets, patentable or otherwise, and all other scientific, marketing, financial and commercial information or data, but excluding any of the foregoing to the extent described or claimed in any Patents.

1.37 "Law" means any federal, state, local, foreign or multinational law, statute, standard, ordinance, code, rule, regulation, resolution or promulgation, or any order by any Government Authority, or any license, franchise, permit or similar right granted under any of the foregoing, or any similar provision having the force or effect of law.

1.38 "Licensed Territory" means (a) as of the Effective Date, the United States, Canada and Mexico; or (b) if Ultragenyx exercises the Ultragenyx Option pursuant to Section 2.2(a), worldwide after the Licensed Territory is expanded pursuant to Section 2.2(d).

1.39 "Licensed Territory Product Infringement" is defined in Section 5.3(a).

1.40 "MAA" means a marketing approval application for Regulatory Approval of a Product that is filed with the EMA.

1.41 "Major European Market" means any of the following: [***].

1.42 "Major Market" means [***].

1.43 "Marketing Approval Application" means a BLA, NDA, MAA or similar application for Regulatory Approval that is filed with the applicable Regulatory Authority(ies) in any country or jurisdiction.

1.44 "Net Sales" means, with respect to any Product, the aggregate gross amount invoiced by Ultragenyx, any Affiliate, or sublicensee for sales of such Product to independent, unrelated Third Parties in *bona fide* arms' length transactions, less deductions for:

(a) the costs of packing, transportation, importation, postage, shipping and handling charges, and other charges, such as insurance and customs duties, relating thereto;

(b) any sales, excise or value added taxes imposed on or charged to the selling party and any other charges imposed by a Governmental Authority upon the sale of such Product and actually paid;

(c) trade, quantity, prompt settlement or similar discounts (including chargebacks and allowances) actually granted, allowed or incurred in connection with the sale of such Product that are customary in the trade;

(d) amounts repaid or credited on account of price adjustments, rejection, outdating, billing errors, recalls or return of such Product;

(e) bad debts that have been written off within twelve (12) months after the date of invoice; and

(f) rebates, reimbursements, fees or similar payments to (i) wholesalers and other distributors, pharmacies and other retailers, buying groups (including group purchasing organizations), health care insurance carriers, pharmacy benefit management companies, health maintenance organizations, Governmental Authorities, or other institutions or health care organizations; or (ii) to patients and other Third Parties arising in connection with any program applicable to a Product under which Ultragenyx, its Affiliates, or its sublicensees provides to low income, uninsured or other patients the opportunity to obtain Ultragenyx's pharmaceutical products at no cost or reduced cost.

Sales between Ultragenyx and its Affiliates and sublicensees shall be disregarded for purposes of calculating Net Sales, except if such purchaser is an end user.

If a Product is sold as part of a Combination Product, the Net Sales of such Product for the purpose of calculating royalties owed under this Agreement for sales of such Product under Section 4.4 or determining whether a sales-based milestone payment is due under Section 4.3(a), shall be determined as follows: first, Ultragenyx shall determine the actual Net Sales of such Combination Product (using the above provisions) and then such amount shall be multiplied by the fraction A/(A+B), where A is the invoice price of such Product, if sold separately, and B is the aggregate invoice price for an equivalent dose amount or unit of each other Active Ingredient, drug, device, test, kit or biological product in the Combination Product, if sold

separately. If any other Active Ingredient, drug, device, test, kit or biological product in the Combination Product is not sold separately, Net Sales shall be calculated by multiplying actual Net Sales of such Combination Product by a fraction A/C where A is the invoice price of such Product if sold separately, and C is the invoice price of the Combination Product. If neither the Product nor any other Active Ingredient, drug, device, test, kit or biological product in the Combination Product is sold separately, the adjustment to Net Sales shall be determined by the Parties in good faith to reasonably reflect the fair market value of the contribution of such Product in the Combination Product to the total fair market value of such Combination Product.

1.45 "Option Exercise Fee" is defined in Section 2.2(b).

1.46 "Option Exercise Notice" is defined in Section 2.2(b).

1.47 "Option Period" is defined in Section 2.2(a).

1.48 "Option Territory" means worldwide except for the United States, Canada and Mexico.

1.49 "Patent Counsel" is defined in Section 5.1(a).

1.50 "Patents" means all patents and patent applications and any patents issuing therefrom (which for the purpose of this Agreement shall be deemed to include certificates of invention and applications for certificates of invention), including all divisionals, continuations, substitutions, continuations-in-part, converted provisionals, continued prosecution applications, adjustments, re-examinations, reissues, additions, renewals, revalidations, extensions (including patent term extensions, and supplemental certificates and the like), registrations, pediatric exclusivity periods of any such patents and patent applications, and any and all foreign equivalents of the foregoing.

1.51 "Person" means any individual, Entity or Governmental Authority.

1.52 "Phase 3 Clinical Trial" means a human clinical trial, the principal purpose of which is to establish safety and efficacy in patients and which is designed and intended to serve as a pivotal study to support the filing of an NDA for the indication being studied, all in accordance with the trial protocol.

1.53 "Pricing Approvals" means, with respect to a Product in any country or jurisdiction, all pricing and reimbursement approvals for the Product from Government Authorities required by applicable Law or Governmental Authorities.

1.54 "Product" means any nutritional or pharmaceutical product, including all dosage forms and formulations, containing one or more Compound(s) as an Active Ingredient(s) (alone or as part of a Combination Product). Except when referred to in the Net Sales definition in describing how to calculate the Net Sales of Combination Products, all references to Product in this Agreement shall be deemed to include Combination Products. Two Products shall be deemed the same Product if they contain the same Compound as the Active Ingredient.

1.55 "Product Marks" is defined in Section 3.6(b).

1.56 "Proof of Concept Study" or "POC Study" means a human clinical trial of a Compound or Product conducted by Ultragenyx or any Affiliate to demonstrate preliminary clinical safety and efficacy with a small number of strictly selected patients.

1.57 "Receiving Party" is defined in Section 6.1(a).

1.58 "Regulatory Approval" means, with respect to a Product in any country or jurisdiction, the approvals by the applicable Regulatory Authority in such country or jurisdiction (other than Pricing Approvals) necessary for the commercialization of such Product.

1.59 "Regulatory Authority" means any applicable Government Authority responsible for granting Regulatory Approvals for Products, including the FDA, the EMA and any corresponding national or regional regulatory authorities.

1.60 "Regulatory Exclusivity" means, with respect to a Product, any market exclusivity granted by government or Regulatory Authority to exclude Third Parties from the development and/or commercialization of any Products containing as its Active Ingredient the same Compound as the Product.

1.61 "Regulatory Filings" means, with respect to the Compounds or Products, any submission to a Regulatory Authority of any appropriate regulatory application specific to Compounds or Products, and shall include any submission to a regulatory advisory board and any supplement or amendment thereto. **"Regulatory Filings"** includes any IND, NDA, BLA and any Marketing Approval Application.

1.62 "Retained Territory" means all countries of the world other than the Licensed Territory. For clarity, if Ultragenyx exercises the Ultragenyx Option pursuant to Section 2.2(a), there will be no countries, jurisdictions or territories in the Retained Territory.

1.63 "Retained Territory Product Infringement" is defined in Section 5.4(a).

1.64 "Royalty Report" means a written report or reports showing, with respect to a given Calendar Quarter, on a Product-by-Product basis: (a) the calculation of Net Sales for each such Product during such Calendar Quarter; (b) any applicable currency conversions; and (c) the royalties payable with respect to such Net Sales in United States Dollars.

1.65 "Royalty Term" has the meaning set forth in Section 4.4(b).

1.66 "Safety Data Exchange Agreement" has the meaning set forth in Section 3.4.

1.67 "Term" is defined in Section 7.1.

1.68 "Third Party" means any Person other than a Party or an Affiliate of a Party.

1.69 "Third Party License" is defined in Section 4.4(d).

1.70 "Third Party Patent Proceeding" is defined in Section 5.5.

1.71 "Ultra Orphan Indication" means, on a country-by-country basis, an indication other than FAOD for which a Product has been granted orphan drug exclusivity under Section 527 of the FD&C Act, or has been granted a corresponding exclusivity under the applicable Laws of another country or jurisdiction within the Licensed Territory, and which affects fewer than 20,000 people in the US.

1.72 "Ultragenyx Indemnitee" is defined in Section 9.1.

1.73 "Ultragenyx Option" is defined in Section 2.2(a).

1.74 "Ultragenyx Option Notice" is defined in Section 2.2(a).

1.75 "United States" or **"US"** means the United States of America including its territories and possessions.

1.76 "Valid Claim" means, with respect to any country a claim of any issued and unexpired patent included in the BRI Patents (as may be extended through supplementary protection certificate or patent term extension or the like) that has not been revoked, abandoned, held invalid, unpatentable or unenforceable by a patent office, court or other governmental agency of competent jurisdiction in a final and non-appealable judgment (or judgment from which no appeal was taken within the allowable time period) and which claim has not been disclaimed, denied or admitted to be invalid or unenforceable through reissue, re-examination, opposition or disclaimer or otherwise, or lost in an interference proceeding.

1.77 Interpretation. In this Agreement, unless otherwise specified:

(a) "includes" and "including" means respectively includes and including without limitation;

(b) words denoting the singular shall include the plural and vice versa and words denoting any gender shall include all genders;

(c) the word "or" shall not be deemed to be used in the exclusive sense and shall instead be used in the inclusive sense to mean "and/or";

(d) words such as "herein", "hereof', and "hereunder" refer to this Agreement as a whole and not merely to the particular provision in which such words appear; and

(e) the Exhibits and other attachments form part of the operative provision of this Agreement and references to this Agreement shall include references to the Exhibits and attachments.

Article 2

License

2.1 License to Ultragenyx.

(a) BRI hereby grants to Ultragenyx (i) an exclusive license, with the right to grant sublicenses in multiple tiers, under the BRI Technology to research, develop, make, have made, use, offer to sell, sell, have sold, import and export Compounds and Products in the Field in the Licensed Territory; and (ii) a non-exclusive license, with the right to grant sublicenses in multiple tiers, to use BRI Know-How relating to Products for research purposes, and for commercial purposes for exploitation of any BRI Patents not otherwise exclusively committed to a Third Party outside of the Licensed Territory. For clarity, the license granted to Ultragenyx under this Section 2.1(a) does not include the right for Ultragenyx to practice any BRI Patent or use any BRI Know-How to develop, make, use or sell compounds or products that are proprietary to BRI other than Compounds or Products.

(b) Ultragenyx may exercise its rights and perform its obligations under this Agreement by itself or through any of its Affiliates or Third Party sublicensees or contractors without the prior written consent of BRI. Ultragenyx shall remain responsible for all of its obligations under this Agreement that have been delegated, subcontracted or sublicensed to any of its Affiliates, sublicensees or contractors.

(c) After the expiration of the Royalty Term for a particular Product in a country within the Licensed Territory, the licenses granted to Ultragenyx by BRI under this Section 2.1 shall become fully paid-up, irrevocable and perpetual licenses for such Product in the Field in such country.

(d) All licenses granted to Ultragenyx under this Agreement are granted subject to (i) any limitations imposed by the terms of any government grant, government contract, or government cooperative agreement applicable to the BRI Patent Rights and (ii) applicable requirements of 35 U.S.C. Sections 200 et seq., as amended, and implementing regulations and policies.

2.2 Ultragenyx Option to Expand Licensed Territory.

(a) The Parties acknowledge and agree that, as of the Effective Date, the Licensed Territory is limited to the United States, Canada and Mexico as a result of the [***] Option. The [***] Option, if not exercised by [***], will expire on December 31, 2012. BRI shall promptly provide Ultragenyx with written notice if (i) the [***] Option lapses either as a result of (A) [***] not exercising such [***] Option; or (B) any waiver or early termination of the [***] Option or otherwise (the "[***] **Option Termination**"), and/or (ii) [***] exercising the [***] Option and the resulting license agreement between [***] and BRI subsequently terminating or expiring (such notice in each of (i) and (ii), the "**Ultragenyx Option Notice**"). BRI hereby grants to Ultragenyx an exclusive option, to expand the Licensed Territory to be worldwide (the "**Ultragenyx Option**"), exercisable in the case of (i) above at any time after Ultragenyx receives the Ultragenyx Option Notice and before June 30, 2013 and in the case of (ii) above within three (3) months after Ultragenyx receives the Ultragenyx Option Notice (the "**Option Period**"). If [***] exercises the [***] Option and subsequently obtains a license to develop, manufacture and commercialize Compound and Product in the Option Territory, BRI and Ultragenyx shall use reasonable efforts to approach [***] to discuss in good faith the coordination of such development, manufacture and commercialization of Compound and Product by [***] and Ultragenyx in separate territories, provided, that there is no obligation for any party to agree to any such coordination, except that neither [***] nor Ultragenyx can commercialize Compound or Product in the other party's territory.

(b) Ultragenyx may exercise the Ultragenyx Option by providing written notice to BRI (an "**Option Exercise Notice**") at any during the Option Period and by paying BRI the one-time option exercise fee of Seven Hundred Fifty Thousand Dollars ($750,000) (the "**Option Exercise Fee**").

(c) Within ten (10) days of the date of the Ultragenyx Option Notice, BRI shall provide Ultragenyx with a good faith estimate of the BRI Patent related costs and expenses that BRI anticipates incurring for the BRI Patents for the Option Territory from the date of the Ultragenyx Option Notice through the Option Expiration Date. Upon its receipt of Ultragenyx's Option Exercise Notice, BRI shall provide Ultragenyx with a final reasonably detailed accounting of all BRI Patent related costs and expenses incurred after the date of the Ultragenyx Option Notice until the date of receipt of such Option Exercise Notice. Within thirty (30) days of receipt of such final accounting, Ultragenyx shall reimburse BRI for any such reasonable BRI Patent related costs and expenses to the extent BRI has not been otherwise reimbursed for such costs and expenses.

(d) Upon BRI's receipt of the Option Exercise Notice and the Option Exercise Fee, the definition of Licensed Territory shall automatically be expanded to be worldwide.

2.3 BRI's Retained Rights. BRI retains the right to practice the BRI Technology outside the scope of the license granted to Ultragenyx in Section 2.1 (as may be expanded by Section 2.2), and the right to use the BRI Know-How for internal, not-for-profit research purposes. In addition, BRI shall have the right to continue to hold the IND for and conduct and complete the clinical trial that is being sponsored by BRI as of the Effective Date titled: "[***]" (the "**BRI Ongoing Study**") in accordance with the protocol existing as of the Effective Date; provided, that BRI may only (i) modify the protocol for the Ongoing Study, (ii) increase enrollment for the Ongoing Study or (iii) change the enrollment criteria for the Ongoing Study as follows: BRI shall discuss any such matters with Ultragenyx in good faith and BRI shall use best efforts to incorporate any of Ultragenyx's comments on such matters. During the Term, BRI shall have the right to conduct additional clinical trials using the Compound under the direct supervision of [***]; provided, that BRI shall first notify Ultragenyx of any such proposed clinical trial and discuss any opportunity for collaboration on such clinical trial between BRI and Ultragenyx and, in the event the Parties agree for BRI to conduct such clinical trial independently, BRI shall conduct such clinical trial pursuant to a protocol to be agreed upon by Ultragenyx and BRI.

2.4 Collaborative Research Agreement. The Parties shall negotiate in good faith the terms and conditions for a collaborative research agreement between Ultragenyx and BRI's Institute of Metabolic Disease ("**IMD**") regarding the Compound and such terms and conditions shall include (i) financial and technical support from Ultragenyx for the ongoing Phase 2 Clinical Trial in adult polyglucosan body disease and/or other development work at IMD and (ii) technical and scientific support from IMD to Ultragenyx in support of its development of the Compound and the Products. Neither Party shall be obligated to enter into such collaborative research agreement if the Ultragenyx and BRI cannot reach agreement on such terms and conditions after such good faith negotiations.

2.5 No Implied Licenses. Except as set forth herein, neither Party shall acquire any license or other intellectual property interest, by implication or otherwise, under any trademarks, Patents, Know-How or other intellectual property Controlled by the other Party.

2.6 Technology Transfer. In addition to BRI's obligation to provide information and documents related to BRI Know-How in accordance with Section 3.5(b), promptly after the Effective Date, BRI shall use best efforts, at no additional cost to Ultragenyx, to disclose and provide to Ultragenyx all BRI Know-How pertaining to the manufacture and development of any Compounds or Products that are Controlled by BRI as of the Effective Date, including all data from any and all clinical trials and preclinical studies, to the extent such BRI Know-How has not previously been provided to Ultragenyx. On a continuing basis during the Term, BRI shall, at no additional cost to Ultragenyx, disclose and provide to Ultragenyx additional BRI Know-How pertaining to the manufacture and development of any Compounds or Products that comes into existence or comes to BRI's attention after the Effective Date.

Article 3

Development, Manufacture and Commercialization

3.1 General. Subject to the terms and conditions of this Agreement, as between the Parties, Ultragenyx (on its own or acting through or together with any of its Affiliates, sublicensees or contractor manufacturers) shall have the sole and exclusive right to develop (including making Regulatory Filings and seeking Regulatory Approvals), manufacture and commercialize Compounds and Products in the Field in the Licensed Territory, at its sole discretion and at its cost and expense. The Parties acknowledge and agree that except as otherwise expressly provided in this Agreement, Ultragenyx shall have no obligation to disclose or share with BRI any preclinical or clinical data and information unless or until Ultragenyx exercises the Ultragenyx Option pursuant to Section 2.2(b).

3.2 Diligence.

(a) Ultragenyx (on its own or acting through any of its Affiliates, sublicensees or subcontractors) shall use Commercially Reasonable Efforts to develop and commercialize at least one (1) Product in FAOD and at least (1) Product in [***]. Specifically, Ultragenyx will use Commercially Reasonable Efforts to (a) [***]; (b) [***]; (c) [***]; and (d) within [***] after the Effective Date, perform at least one of the following: (i) [***]; or (ii) [***]; in each case of (a)-(d) above, provided that each such timeline shall be extended to account for any delay resulting from factors beyond Ultragenyx's reasonable control, including regulatory, medical, safety or efficacy delays.

(b) If Ultragenyx shall fail to achieve such milestones within such applicable time frame(s) (as such time frame(s) may be extended pursuant to 3.2(a) above), BRI may provide written notice to Ultragenyx and upon receipt of any such notice, the Parties shall discuss in good faith Ultragenyx's progress for the development of such Product in the

applicable jurisdiction and the Parties may agree on an amended timeline or a plan for Ultragenyx to continue its development of the Product. Each agreed upon [***] extension of the timeline shall be subject to the payment by Ultragenyx of an extension fee of [***]. For clarity, if a delay in timeline is due to new regulatory, development, or safety requirements or other similar actions by regulatory authorities or other government agencies, such timeline is not subject to the payment by Ultragenyx of an extension fee.

(c) For the purposes of this Section 3.2(c), all FAOD indications shall be deemed one "Indication Cluster" and all [***] shall be deemed another "Indication Cluster." On an Indication Cluster-by-Indication Cluster basis, in the event Ultragenyx fails to satisfy such new timeline or plan that is specific to a particular Indication Cluster, then upon written notice by BRI, the license granted to Ultragenyx pursuant to Section 2.1(a) shall become non-exclusive solely for such Indication Cluster, provided that all obligations for payment of royalties or milestones remain the same for such Indication Cluster and provided, that, if Ultragenyx subsequently obtains Regulatory Approval for the Product for such Indication Cluster, the license under Section 2.1(a) shall thereupon automatically convert back to an exclusive license for such Indication Cluster so long as BRI still maintains the right to grant an exclusive license to Ultragenyx at such time. In the event of the conversion of the license to non-exclusive, any sole and/or exclusive rights that Ultragenyx has under this Agreement with respect to such non-exclusive Indication Cluster, including without limitation such rights under Section 3.1, this Section 3.2, and Section 3.3 shall also automatically become non-exclusive. Further, on an Indication Cluster-by-Indication Cluster basis, Ultragenyx's rights regarding BRI Independent Studies under Sections 3.3 (i)-(iv), and Ultragenyx's rights under Sections 5.1(a), 5.3(b), Section 5.4(b), Section 5.5 (a) and (b), and Section 5.6, as well as BRI's corresponding obligations to Ultragenyx with respect to those Sections, in each case as such rights and obligations apply to any non-exclusive Indication Cluster, shall be suspended for so long as such license remains non-exclusive and shall be automatically reinstated if and when such license becomes exclusive pursuant to the foregoing. Failure to achieve such milestones shall not be a material breach of this Agreement. The remedy provided for pursuant to this Section 3.2 shall be BRI's sole and exclusive remedy for or relating to Ultragenyx's failure to achieve any milestone. If the license granted to Ultragenyx pursuant to Section 2.1(a) is converted to a non-exclusive license for a particular Indication Cluster pursuant to the terms of this Section 3.2, BRI shall promptly provide Ultragenyx with written notice of any Third Party license that BRI grants under the BRI Technology relating to Compounds or Products for such Indication Cluster.

3.3 Regulatory. As between the Parties, Ultragenyx (on its own or acting through or together with any of its Affiliates, sublicensees or contractor manufacturers) has the sole right to: (a) make all Regulatory Filings, submissions, reports, updates and supplements with any Regulatory Authority with respect to any Compound or Product in the Licensed Territory; (b) obtain, hold and maintain all INDs (other than the IND for the BRI Ongoing Study), Regulatory Approvals and Pricing Approvals in the Field in the Licensed Territory in the name of Ultragenyx or any of its Affiliates or sublicensees; and (c) conduct all meeting and discussions and handle all correspondence with any Regulatory Authority related to any Compound or Product in the Licensed Territory. For the BRI Ongoing Study and any additional study that BRI independently conducts pursuant to Section 2.3 ("**BRI Independent Studies**"): (i) BRI shall promptly provide Ultragenyx with copies of all material documents, information and

correspondence that are received from any Regulatory Authority; (ii) BRI shall provide Ultragenyx with copies of all material documents, information and correspondence that are planned for submission to any Regulatory Authority and BRI shall cooperate with and consider in good faith any input from Ultragenyx in preparing such submission; (iii) BRI shall provide Ultragenyx with reasonable advance notice of all meetings, conferences and discussions scheduled with any Regulatory Authority concerning such study(ies) and BRI shall consider in good faith any input from Ultragenyx in preparing for such meetings, conferences or discussions; and (iv) to the extent permitted by applicable Laws and by any obligations to Third Parties that (A) exist as of the Effective Date or (B) come into existence after the Effective Date, provided that BRI shall use Commercially Reasonable Efforts to obtain such rights for Ultragenyx in its negotiations with Third Parties after the Effective Date, Ultragenyx shall have the right to participate in any such meetings, conferences or discussions and BRI shall facilitate such participation. If either (x) [***] exercises the [***] Option or (y) Ultragenyx does not exercise the Ultragenyx Option, then, in each case, BRI and Ultragenyx shall each use reasonable efforts to facilitate global coordination of clinical data and safety data between Ultragenyx and [***] and/or any other applicable Third Party licensee of BRI in the Retained Territory in order for Ultragenyx, [***] and/or any Third Party licensee to comply with regulatory requirements.

3.4 Pharmacovigilance. If either (a) [***] exercises the [***] Option or (b) Ultragenyx does not exercise the Ultragenyx Option, then, in each case, BRI and Ultragenyx shall use reasonable efforts to facilitate BRI, Ultragenyx and [***] and/or any other applicable Third Party licensee of BRI in the Retained Territory entering into a safety data exchange agreement ("***Safety Data Exchange Agreement***") setting forth in detail the pharmacovigilance alert process and data exchange with respect to the Product to comply with all applicable legal obligations of Regulatory Authorities in the Licensed Territory and in the Retained Territory. For the Ongoing Study and any additional study that BRI independently conducts pursuant to Section 2.3, BRI shall cooperate with Ultragenyx and BRI shall transfer all safety data relating to the Product to Ultragenyx pursuant to a procedure to be agreed upon by the Parties; in the event of conversion of the license to non-exclusive, Ultragenyx shall also provide a reciprocal transfer of all its safety data to BRI.

3.5 Manufacture; Provision of Know-How.

(a) Subject to Section 3.5(b), Ultragenyx (on its own or acting through any of its Affiliates, sublicensees or subcontractors) shall be responsible for the manufacture and supply of Compounds and Products in the Licensed Territory for use by Ultragenyx, its Affiliates and sublicensees, at its cost and expense, itself or through one or more contract manufacturers or sublicensees. BRI shall use Commercially Reasonable Efforts to assist Ultragenyx in establishing a direct relationship with BRI's current supplier of Compounds.

(b) Promptly after the Effective Date, BRI shall provide Ultragenyx with all BRI Know-How that is necessary or reasonably useful to manufacture Compound or Product, including any and all reports and documentation from BRI's current or former supplier of Compounds that BRI does not have the ability to disclose to Ultragenyx under express or implied obligations of confidentiality.

3.6 Commercialization.

(a) Ultragenyx (on its own or acting through any of its Affiliates, sublicensees or subcontractors) shall book sales for the Products in the Licensed Territory and shall have sole control over pricing and other commercialization decisions with respect to the Products in the Licensed Territory.

(b) As between the Parties, Ultragenyx (on its own or acting through any of its Affiliates, sublicensees or subcontractors) shall have the right to brand commercialized Products using Ultragenyx trademarks and any other trademarks and trade names it determines appropriate for the Products, which may vary by country or within a country ("**Product Marks**"). Ultragenyx (on its own or acting through any of its Affiliates, sublicensees or subcontractors) shall own all rights in the Product Marks and shall be responsible for registration, maintenance, and defense of the Product Marks at its own costs and expense.

Article 4

Financial Provisions

4.1 Upfront Payment. In consideration for the license granted under this Agreement, Ultragenyx shall pay to BRI a one-time, non-refundable, non-creditable upfront payment of Two Hundred Fifty Thousand Dollars ($250,000) within thirty (30) days after the Effective Date.

4.2 Milestone Payments.

(a) Development and Regulatory Milestones. Ultragenyx shall pay to BRI the following one-time, non-refundable, non-creditable development and regulatory milestone payments upon the achievement of the corresponding milestone by Ultragenyx or any of its Affiliates or sublicensees:

Development and Regulatory Milestones	Payments
(i) [***]	$ [***]
(ii) [***]	$ [***]
(iii) [***]	$ [***]
(iv) [***]	$ [***]
(v) [***]	$ [***]
(vi) [***]	$ [***]
(vii) [***]	$ [***]
(viii) [***]	$ [***]
(ix) [***]	$ [***]
(x) [***]	$ [***]

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

(b) The Parties acknowledge and agree that the milestones set forth in Section 4.2(a)(ii), (vii), (viii), (ix) and (x) above will not be due if such Product triggering the milestone is in an indication that is not covered by a Valid Claim and if Ultragenyx has a financial obligation to pay a milestone pursuant to a Third Party License (as defined in Section 4.4(d) below) on such Product for such indication.

(c) The milestones set forth in Section 4.2(a) shall each be due after the first (1st) achievement of such milestone for the first Product to achieve such milestone by or on behalf of Ultragenyx or any of its Affiliates or sublicensees, and each such milestone payment shall be payable only once, regardless of how many Products or how many indications for which such milestone has been achieved.

(d) For clarity, the milestones set forth in sub-Section 4.2(a)(i), (ii), (v), (vi), (ix) and (x) above may only be due if Ultragenyx exercises the Ultragenyx Option pursuant to Section 2.2(b).

(e) Ultragenyx shall notify BRI in writing promptly upon each milestone event set forth in this Section 4.2 and BRI shall thereafter submit an invoice to Ultragenyx for the milestone payment corresponding to such milestone event as set forth in this Section 4.2. Ultragenyx shall make such applicable milestone payment within [***] days after receipt of such invoice from BRI.

4.3 Sales Milestone Payments.

(a) Ultragenyx shall pay to BRI the non-refundable, non-creditable sales milestone payments set forth below. The sales milestones shall each be due after the first achievement of such milestone for annual aggregate Net Sales of all Products by or on behalf of Ultragenyx or any of its Affiliates or sublicensees, and each such milestone payment shall be payable only once, regardless of how many Calendar Years during which such sales milestone have been reached.

Sales Milestones	Payments
First (1st) Calendar Year in which aggregate total Net Sales by Ultragenyx, its Affiliates and sublicensees for all Products throughout the Licensed Territory exceed $[***]	[***]
First (1st) Calendar Year in which aggregate total Net Sales by Ultragenyx, its Affiliates and sublicensees for all Products throughout the Licensed Territory exceed $[***]	$ [***]

(b) Ultragenyx shall notify BRI in writing within [***] days following the end of the Calendar Year during which any milestone event is achieved and BRI shall thereafter submit an invoice to Ultragenyx for the corresponding milestone payment as set forth in Section 4.4(a). Ultragenyx shall make such applicable milestone payment within [***] days following receipt of such invoice.

4.4 Royalty Payments.

(a) Royalty Rates for Products. Subject to the other terms of this Section 4.4, during the Royalty Term, Ultragenyx shall make quarterly royalty payments to BRI equal to [***] percent ([***]%) of the Net Sales of the Products in the Licensed Territory by Ultragenyx and any of its Affiliates or sublicensees.

(b) Royalty Term. On a Product-by-Product basis and country-by-country basis, Ultragenyx's royalty payment obligations under this Section 4.4 shall commence upon the First Commercial Sale of such Product in such country and expire upon the later of: (i) the expiration of the period of the first Regulatory Exclusivity granted by the applicable Regulatory Authority applicable to such Product in such country in connection with approval in such country for either FAOD or an Ultra Orphan Indication; or (ii) the expiration of the last-to-expire Valid Claim included in BRI Patents claiming the composition of matter of, or the method of making or using, such Product in such country in connection with approval in such country for FAOD or an Ultra Orphan Indication ("**Royalty Term**").

(c) Royalty Reduction for a Product Subject to Generic Competition. For a particular Product in a particular country, during any period during the Royalty Term if one (1) or more Generic Product(s) with respect to such Product is being sold in such country then the applicable royalty rate under this Section 4.4 shall be reduced by [***] percent ([***]%) for such Product in such country.

(d) Royalty Reduction for Third Party Payment Obligations. If Ultragenyx (or any of its Affiliates or sublicensees) enters into any agreement with a Third Party under which Ultragenyx obtains rights under any intellectual property (including any Patent or Know-How) Controlled by a Third Party, which is necessary to the use, development, manufacture, commercialization or import of any Compound or Product (including for the use or commercialization of any Compound or Product in a particular indication) (each, a "**Third Party License**"), Ultragenyx shall have the right to credit up to [***] percent ([***]%) of the amounts owed by Ultragenyx under any such Third Party License against Ultragenyx's royalty payments to BRI under this Section 4.4 for the same Product, on a country-by-country basis, provided that, by operation of this Section 4.4(d), Ultragenyx's royalty payment obligation to BRI shall not be reduced by more than [***] percent ([***]%) for the first such Third Party License or [***] percent ([***]%) for the second and any subsequent such Third Party License(s). For clarity, any applicable royalty reduction pursuant to Section 4.4(c) shall be in addition to any applicable royalty reduction pursuant to this Section 4.4(d) and such royalty reduction is not included within the aforementioned limits.

4.5 Reports; Payment of Royalty; Annual Reconciliation. During the Term, following the First Commercial Sale of a Product and on a Calendar Quarter basis, Ultragenyx shall furnish to BRI a Royalty Report. Reports shall be due within [***] days following the close of each Calendar Quarter. Royalties shown to have accrued by each Royalty Report shall be due and payable on the date such royalty report is due. Ultragenyx shall keep complete and accurate records in sufficient detail to enable the royalties payable hereunder to be determined.

4.6 Currency; Exchange Rate.

(a) All payments to be made by Ultragenyx to BRI under this Agreement shall be made in Dollars by bank wire transfer in immediately available funds to a bank account designated by written notice from BRI to Ultragenyx. In the case of sales outside the United States, the rate of exchange to be used in computing the monthly amount of currency equivalent in Dollars due BRI shall be made at the monthly rate of exchange published in *The Wall Street Journal* (U.S., Eastern Edition), prevailing on the last Business Day of the month preceding the month in which such sales are recorded by Ultragenyx.

(b) If pursuant to applicable Law or fiscal policy of a particular country, a remittance of royalties in the currency stipulated in this Section 4.6 is restricted or forbidden, Ultragenyx shall provide notice thereof to BRI, and payment of such royalty shall be made by the deposit thereof in local currency to the credit of BRI in a recognized banking institution designated by BRI or its Affiliates. When in any such country the applicable Law or fiscal policy would then allow the transmittal of such royalty payments, all royalties or other sums that Ultragenyx would have been under obligation to transmit but for the prohibition, shall promptly be transmitted to BRI to the extent allowable.

4.7 Late Payments. If a Party does not receive payment of any sum due to it on or before the due date therefor, simple interest shall thereafter accrue on the sum due to such Party from the due date until the date of payment at a per-annum rate of prime plus [***] percentage points or the maximum rate allowable by applicable Law, whichever is less.

4.8 Taxes.

(a) Taxes on Income. Each Party shall be solely responsible for the payment of all taxes imposed on its share of income arising directly or indirectly from the activities of the Parties under this Agreement.

(b) Tax Cooperation. The Parties agree to cooperate with one another and use reasonable efforts to avoid or reduce tax withholding or similar obligations in respect of royalties, milestone payments, and other payments made by Ultragenyx to BRI under this Agreement. To the extent Ultragenyx is required to deduct and withhold taxes on any payment to BRI, Ultragenyx shall pay the amounts of such taxes to the proper Governmental Authority in a timely manner, and the sum payable to BRI shall be decreased by the same amount. BRI shall provide Ultragenyx any tax forms that may be reasonably necessary in order for Ultragenyx to not withhold tax or to withhold tax at a reduced rate under an applicable bilateral income tax treaty. BRI shall use reasonable efforts to provide any such tax forms to Ultragenyx in advance of the due date. Each Party shall provide the other with reasonable assistance to enable the recovery, as permitted by Law, of withholding taxes or similar obligations resulting from payments made under this Agreement, such recovery to be for the benefit of BRI as the Party bearing such withholding tax under this Section 4.8(b).

4.9 Records and Audit Rights.

(a) Ultragenyx shall keep complete, true and accurate books and records in relation to this Agreement. Upon the written request of BRI and not more than [***] in each Calendar Year, Ultragenyx shall permit an independent certified public accounting firm selected by BRI, and reasonably acceptable to Ultragenyx, to have access during normal business hours to such of the records of Ultragenyx as may be reasonably necessary to verify the accuracy of the royalty reports hereunder for any Calendar Year ending not more than [***] prior to the date of such request. BRI shall treat all financial information subject to review under this Section 4.9 or under any sublicense agreement in accordance with the confidentiality and non-use provisions of this Agreement.

(b) Ultragenyx may require an accounting firm conducting an audit hereunder to sign a non-disclosure agreement to protect the confidentiality of Ultragenyx's Confidential Information before providing such accounting firm access to Ultragenyx's facilities, books or records. Upon completion of any audit hereunder, the accounting firm shall provide both Ultragenyx and BRI a written report disclosing whether the royalty reports submitted by Ultragenyx are correct or incorrect, whether the amounts paid are correct or incorrect, and in each case, the specific details concerning any discrepancies.

(c) BRI shall bear its internal expenses and the out-of-pocket costs for engaging such accounting firm in connection with performing such audits; provided, however, that if any such audit uncovers an underpayment of milestones payments or royalties by Ultragenyx that exceeds [***] percent ([***]%) of the total owed for such payment or payment period, as applicable, then Ultragenyx shall reimburse BRI for the expenses and costs for such audit.

(d) If such accounting firm identifies an underpayment by Ultragenyx during such period, Ultragenyx shall pay BRI the amount of the discrepancy within [***] days of the date BRI delivers to Ultragenyx such accounting firm's written report with the amount of underpayment accruing interest at the rate such forth in Section 4.7. If such accounting firm identifies an overpayment by Ultragenyx during such period, Ultragenyx shall, at its option, have the right to request a refund of such overpaid amount, or credit such overpaid amount against subsequent payment obligations to BRI, and BRI shall make such refund to Ultragenyx within [***] days of Ultragenyx's request if so requested. If Ultragenyx has no future payment obligations under this Agreement, then Ultragenyx may require BRI to refund such overpayment and BRI shall pay such overpaid amount to Ultragenyx within [***] days of Ultragenyx's request.

Article 5

Intellectual Property Rights

5.1 Patent Prosecution in the Licensed Territory.

(a) As between the Parties, Ultragenyx, acting through outside patent counsel of its choice ("**Patent Counsel**"), shall have the first right, but not the obligation, to take the lead in the preparation, filing, prosecution and maintenance of the BRI Patents in the Licensed Territory, at Ultragenyx's cost and expense. BRI shall cooperate with Ultragenyx in filing and prosecution of such BRI Patents in the Licensed Territory, including by providing Ultragenyx with data and other information as appropriate and executing all necessary paperwork. Within [***] days after the Effective Date, BRI shall provide to Ultragenyx those copies of all patent filings, and the correspondence between BRI and patent authorities, for BRI Patents in the Licensed Territory existing as of the Effective Date. Within [***] days after Ultragenyx exercises the Ultragenyx Option pursuant to Section 2.2(a), BRI shall provide Ultragenyx those copies of all patent filings, and the correspondence between BRI and patent authorities, for BRI Patents in the Licensed Territory existing as of the date of exercise of the Ultragenyx Option that have not already been provided to Ultragenyx pursuant to this Section 5.1(a). Ultragenyx will keep BRI reasonably informed of the status of the prosecution of such BRI Patents in the Licensed Territory. For the purpose of this Article 6, "prosecution" shall include any pre-grant and post-grant proceeding including patent interference proceeding, opposition proceeding and other similar proceedings, appeals or petitions to any Board of Appeals in the patent office, appeals to any court for any patent office decisions, reissues and reexamination proceedings, and applications for patent term extensions and the like.

(b) Ultragenyx will notify BRI of any decision not to file for, prosecute or maintain, or not to continue to pay the expenses of prosecution or maintenance of (collectively, "Patent Support"), any BRI Patents in the Licensed Territory. Ultragenyx will provide such notice at least [***] days prior to any filing or payment due date, or any other due date that requires action, in connection with such BRI Patent. In such event, BRI shall have the right, but not the obligation, to file for, or continue prosecution or maintenance of, such BRI Patent in the Licensed Territory, at its expense. In the event BRI does maintain such BRI Patent in the Licensed Territory and such BRI Patent in the Licensed Territory covers the applicable Product that triggers any royalty or milestone payment, Ultragenyx shall continue payment of the applicable royalties and milestones.

5.2 Patent Prosecution in the Retained Territory.

(a) If the Licensed Territory does not expand pursuant to Section 2.2(a), BRI shall use Commercially Reasonable Efforts to prosecute and maintain all of the patents and applications included within the BRI Patents in the Retained Territory. BRI shall: (i) keep Ultragenyx advised of the status of all communications and actual and prospective filings regarding such BRI Patents in the Retained Territory, (ii) give Ultragenyx a reasonable opportunity (but in no event less than [***] business days) to review and comment on any such communications and filings proposed to be sent to any patent authority, and (iii) incorporate all reasonable comments of Ultragenyx before making any such communication or filing related to such BRI Patents in the Retained Territory.

(b) Should BRI (and [***] if the [***] Option has been exercised and [***] has the right) decide that it is no longer interested in maintaining or prosecuting any BRI Patent in the Retained Territory, BRI shall promptly advise Ultragenyx thereof and, upon written notice by Ultragenyx, BRI agrees that Ultragenyx may prosecute and maintain such BRI Patent in its own name, and BRI shall execute all required documents in order to assign any such BRI Patent to Ultragenyx; provided, however, that any such assignment by BRI shall be subject to the [***] Option.

5.3 Patent Enforcement and Defense in the Licensed Territory.

(a) Each Party shall give the other Party written notice of any infringement by a Third Party of any BRI Patents through the development or commercialization of a Product in the Field in the Licensed Territory (a "**Licensed Territory Product Infringement**"), within [***] Business Days after such Licensed Territory Product Infringement comes to such Party's attention.

(b) Ultragenyx shall have the sole and exclusive right, but not the obligation, to bring and control any legal action in connection with such Licensed Territory Product Infringement in the Licensed Territory at its own expense and discretion as it reasonably determines appropriate. BRI shall have the right to be represented in any such action by counsel of its choice at its own expense. Should Ultragenyx decline to bring a legal action in connection with a Licensed Territory Product Infringement in the Licensed Territory, BRI shall have the right, but not the obligation, to bring such a legal action. In the event that the Licensed Territory Product Infringement involves a Generic Product, and Ultragenyx declines to bring a legal action, Ultragenyx shall not be permitted to reduce royalties due BRI pursuant to Section 4.4(c). For clarity, Ultragenyx's right to bring and control any legal action under this Section 5.3(b)shall not apply to a Licensed Territory Product Infringement in an Indication Cluster during the period of time in which Ultragenyx has anon-exclusive license.

(c) At the request of a party bringing a legal action under Section 5.2(b) (the "**Litigating Party**"), the other Party shall reasonably cooperate and provide any information or assistance in connection with any legal action under this Section 5.3, including executing reasonably appropriate documents, cooperating in discovery and joining as a party to the action if required, all at the Litigating Party's expense.

(d) Any recoveries resulting from such an action relating to a claim of Licensed Territory Product Infringement shall be first applied against payment of costs and expenses in connection with the action of the Party which initiated and prosecuted the action. The other Party shall then, to the extent possible, recover its costs and expenses incurred in connection with the action. Solely in the case in which Ultragenyx is the Litigating Party, any such recoveries in excess of such costs and expenses of the Parties shall be retained by Ultragenyx and shall be deemed Net Sales subject to Ultragenyx's royalty payment obligation to BRI under Section 4.4. In the event BRI is the Litigating Party, all recoveries in excess of such costs and expenses of the Parties shall be retained by BRI.

5.4 Patent Enforcement and Defense in the Retained Territory.

(a) If the Licensed Territory does not expand pursuant to Section 2.2(a), each Party shall give the other Party written notice of any infringement by a Third Party of any BRI Patents through the development or commercialization of a Product in the Field in the Retained Territory (a "**Retained Territory Product Infringement**"), within [***] Business Days after such Retained Territory Product Infringement comes to such Party's attention.

(b) As between the Parties, BRI shall have the right, but not the obligation, to bring and control any legal action in connection with such Retained Territory Product Infringement in the Retained Territory at its own expense. BRI shall, if it has brought or controls such legal action, use Commercially Reasonable Efforts to: (i) keep Ultragenyx advised of the status of all such legal actions, (ii) give Ultragenyx a reasonable opportunity to review and comment on any filings, motions, pleadings or other strategic decisions relating to such legal action, and (iii) incorporate all reasonable comments of Ultragenyx in conducting such legal action, and, in each case, BRI shall use Commercially Reasonable Efforts to require [***] or any other applicable Third Party licensee of BRI in the Retained Territory to do so.

5.5 Third Party Patent Proceedings. Each Party will notify the other Party in writing prior to challenging any Patents controlled by a Third Party that are necessary or reasonably useful to use, develop, manufacture, commercialize or import any Compound or Product. Such challenges include declaratory judgment actions, inter parties re-examinations, interferences, oppositions and other similar proceedings (collectively "**Third Party Patent Proceeding**").

(a) Except as the Parties otherwise agree, Ultragenyx shall have the first right to bring and control any legal action in connection with such Third Party Patent Proceeding in the Field in the Licensed Territory, at its own expense and discretion as it reasonably determines appropriate.

(b) At the request of Ultragenyx, BRI shall reasonably cooperate and provide any information or assistance in connection with any legal action under this Section 5.3, including executing reasonably appropriate documents, cooperating in discovery and joining as a party to the action if required, all at Ultragenyx's expense. Ultragenyx shall keep BRI reasonably informed of the status of such action.

5.6 Patent Extensions. The Parties shall cooperate in obtaining patent term restoration (under but not limited to Drug Price Competition and Patent Term Restoration Act), supplemental protection certificates or their equivalents, and patent term extensions with respect to the BRI Patents in any country or region in the Licensed Territory where applicable, provided, that Ultragenyx shall have the final decision making authority on the foregoing.

Article 6

Confidentiality; Publication

6.1 Duty of Confidence. Subject to the other provisions of this Article 6:

(a) all Confidential Information disclosed by or on behalf of a Party or its Affiliates ("**Disclosing Party**") under this Agreement, or in the course of contemplating a transaction under this Agreement prior to the execution of this Agreement, shall be maintained in confidence and otherwise safeguarded by the recipient Party and its Affiliates ("**Receiving Party**"), in the same manner and with the same protection as such Receiving Party maintains its own confidential information, but at least with reasonable protection;

(b) the Receiving Party may only use any such Confidential Information for the purposes of performing its obligations or exercising its rights under this Agreement; and

(c) the Receiving Party may disclose Confidential Information of the other Party to: (i) its Affiliates and sublicensees; and (ii) employees, directors, agents, contractors, consultants and advisers of the Party and its Affiliates, licensees and sublicensees, in each case to the extent reasonably necessary for the purposes of, and for those matters undertaken pursuant to, this Agreement; provided that such Persons are bound to maintain the confidentiality of the Confidential Information in a manner consistent with the confidentiality provisions of this Agreement. Notwithstanding the foregoing, the Parties acknowledge and agree that BRI may not disclose any Confidential Information of Ultragenyx to [***] or any other licensee or sublicensee of the Compound and/or Product in the Option Territory without Ultragenyx's prior written consent. Ultragenyx acknowledges that, pursuant to the [***] Option as amended on February 22, 2012, BRI is required to disclose preclinical and clinical data and information regarding regulatory submissions in the Licensed Territories to [***], and with respect to BRI Independent Studies, shall require no consent from Ultragenyx to do so.

6.2 Exceptions. The foregoing obligations as to particular Confidential Information of a Disclosing Party shall not apply to the extent that the Receiving Party can demonstrate that such Confidential Information:

(a) was known by the Receiving Party at the time of its receipt, and not through a prior disclosure by the Disclosing Party, as documented by the Receiving Party's written records;

(b) was in the public domain before its receipt from the Disclosing Party, or thereafter enters the public domain through no fault of the Receiving Party;

(c) is subsequently disclosed to the Receiving Party by a Third Party who is not under a direct or indirect obligation of confidentiality to the Disclosing Party; or

(d) is developed by the Receiving Party independently and without use of or reference to any Confidential Information received from the Disclosing Party, as documented by the Receiving Party's written records.

Any combination of features or disclosures shall not be deemed to fall within the foregoing exclusions merely because individual features are published or available to the general public or in the rightful possession of the Receiving Party unless the combination itself and principle of operation are published or available to the general public or in the rightful possession of the Receiving Party.

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

6.3 Authorized Disclosures. Notwithstanding the obligations set forth in Sections 6.2 and 6.5, a Party may disclose the other Party's Confidential Information (including this Agreement and the terms herein) solely to the extent:

(a) such disclosure: (i) is reasonably necessary for the filing or prosecuting Patents as contemplated by this Agreement; (ii) is reasonably necessary in connection with Regulatory Filings for Products; (iii) is reasonably necessary for the prosecuting or defending of legal actions, including litigation, as contemplated by this Agreement; or (iv) is made to any Third Party bound by written obligation of confidentiality and non-use similar to those set forth under this Article 6, to the extent otherwise necessary or appropriate in connection with the exercise of its rights or the performance of its obligations hereunder;

(b) such disclosure is reasonably necessary: (i) to such Party's directors, attorneys, independent accountants or financial advisors for the sole purpose of enabling such directors, attorneys, independent accountants or financial advisors to provide advice to the Receiving Party, provided that in each such case on the condition that such directors, attorneys, independent accountants and financial advisors are bound by confidentiality and non-use obligations substantially consistent with those contained in this Agreement; provided, however, that the term of confidentiality for such directors, attorneys, independent accountants and financial advisors shall be no less than [***]; or (ii) to actual or potential investors, sublicensees or acquirors solely for the purpose of evaluating an actual or potential investment, sublicense or acquisition; provided that in each such case on the condition that such actual or potential investors, sublicensees and acquirers are bound by confidentiality and non-use obligations substantially consistent with those contained in this Agreement; provided, however, that the term of confidentiality for such actual or potential investors and acquirers shall be no less than [***]; or

(c) such disclosure is required by judicial or administrative process, provided that in such event such Party shall promptly inform the other Party of such required disclosure and provide the other Party an opportunity to challenge or limit the disclosure obligations. Confidential Information that is disclosed by judicial or administrative process shall remain otherwise subject to the confidentiality and non-use provisions of this Article 7, and the Party disclosing Confidential Information pursuant to law or court order shall take all steps reasonably necessary, including seeking of confidential treatment or a protective order to ensure the continued confidential treatment of such Confidential Information.

6.4 Scientific Publications. Subject to Section 6.3, Ultragenyx or its sublicensee(s) shall have the sole right to make any public publication or presentation of any data regarding any Compound or Product, provided, however, that to the extent such data arises as a result of work performed by a BRI employee or contractor, or with financial support by BRI, BRI shall be provided with a copy of any proposed publication at least [***] days prior to submission for BRI's review and comment, and as appropriate in accordance with scientific journal standards, shall designate the appropriate BRI personnel as co-authors. However, such publication or presentation shall not include any Confidential Information of BRI without the prior written consent of BRI. Subject to Section 6.3, BRI shall make no public publication or presentation of any data regarding any Compound or Product without the prior written consent of Ultragenyx. Notwithstanding the foregoing, this Section 6.4 shall not apply to any publication or presentation of data relating to BRI Know-How or BRI Patents existing as of the Effective Date, provided that BRI shall provide Ultragenyx with a copy of any proposed publication or presentation at least [***] days prior to submission, for Ultragenyx's review and comment.

6.5 Publicity; Use of Names. Notwithstanding anything to the contrary in this Agreement, until the expiration of the [***] Option and Ultragenyx's receipt of the Ultragenyx Option Notice, the existence and the terms of this Agreement are each Party's Confidential Information and such shall be held in strict confidence and not disclosed by either Party, except with the prior express written permission of the other Party or as may be required by applicable Law. Subject to Sections 6.1, 6.2 and 6.3, no other disclosure of the existence or the terms of this Agreement may be made by either Party or its Affiliates except as provided in this Section 6.5, and no Party shall use the name, trademark, trade name or logo of the other Party, its Affiliates or their respective employees in any publicity, promotion, news release or disclosure relating to this Agreement or its subject matter, except as provided in this Section 6.5 or with the prior express written permission of the other Party, except as may be required by applicable Law.

(a) A Party may disclose this Agreement and its terms, and material developments or material information generated under this Agreement, in securities filings with the US Securities and Exchange Commission (or equivalent foreign agency) to the extent required by applicable Law after complying with the procedure set forth in this Section 6.5(a). In such event, the Party seeking such disclosure will prepare a draft confidential treatment request and proposed redacted version of this Agreement to request confidential treatment for this Agreement, and the other Party agrees to promptly (and in any event, no more than seven (7) days after receipt of such confidential treatment request and proposed redactions) give its input in a reasonable manner in order to allow the Party seeking disclosure to file its request within the time lines proscribed by applicable Law. The Party seeking such disclosure shall exercise Commercially Reasonable Efforts to obtain confidential treatment of this Agreement from the US Securities and Exchange Commission (or equivalent foreign agency) as represented by the redacted version reviewed by the other Party.

(b) The Parties agree that any news release or other public announcement relating to the terms and conditions of this Agreement or the performance hereunder shall not be made until after the earlier of (i) expiration or termination of the [***] Option and (ii) [***] exercise of the [***] Option. Any such news release, any further news release or other public disclosure that would disclose information other than that already in the public domain, shall first be reviewed and approved by both Parties (with such approval not to be unreasonably withheld or delayed).

(c) The Parties agree that after a disclosure pursuant to Section 6.5(b), a press release or other public announcement pursuant to Section 6.5(c) has been reviewed and approved by the other Party, the disclosing Party may make subsequent public disclosures reiterating such information without having to obtain the other Party's prior consent and approval.

Article 7

Term and Termination

7.1 Term. The term of this Agreement will commence upon the Effective Date and continue in full force and effect, on a Product-by-Product and country-country basis, until the expiration of the royalty obligations of Ultragenyx with respect to the applicable Product, unless earlier terminated as set forth in Section 7.2 (the "**Term**"). After the expiration of this Agreement for a particular Product in a particular country, Ultragenyx's license in such country shall become fully paid, royalty-free, perpetual and irrevocable.

7.2 Termination.

(a) Termination by Ultragenyx for Convenience. At any time, Ultragenyx may terminate this Agreement, in its entirety or on a Product-by-Product basis, by providing written notice of termination to BRI, which notice includes an effective date of termination at least ninety (90) days after the date of the notice.

(b) Termination for Material Breach. If either Party believes that the other is in breach of its material obligations hereunder (other than Ultragenyx's nonfulfillment of its obligations under Section 3.2, the remedy of which is set forth in Section 3.2), then the non-breaching Party may deliver notice of such breach to the other Party. The allegedly breaching Party shall have ninety (90) days from such notice to dispute or cure such breach. If the Party receiving notice of breach fails to cure, or fails to dispute, that breach within the period set forth above, then the Party originally delivering the notice of breach may terminate this Agreement effective on written notice of termination to the other Party, provided that such non-breaching Party delivers such notice of termination within sixty (60) days of the end of such one hundred ninety(90)-day cure period. If the allegedly breaching Party in good faith disputes such material breach or disputes the failure to cure or remedy such material breach and provides written notice of that dispute to the other Party within the period set forth above, the notifying Party may not terminate this Agreement until one independent industry expert mutually agreeable to both Parties has determined that the allegedly breaching Party is in material breach of this Agreement, and such breaching Party further fails to cure such breach within thirty (30) days after such determination by the independent industry expert (and such termination shall then be effective upon written notification from the notifying Party to the breaching Party).

(c) Termination for Bankruptcy. Either party may terminate this Agreement in the event the other Party ceases doing business as a going concern, makes an assignment for the benefit of creditors, shall not be paying its debts in the ordinary course, shall have become insolvent or shall, voluntarily or involuntarily, become party to insolvency proceeding, or commits any act of bankruptcy. Notwithstanding any other provision of this Agreement to the contrary, in the event that BRI becomes a debtor under the United States Bankruptcy Code (11 U.S.C. §101 et. seq. or any similar law in any other country (the "**Bankruptcy Code**")) and rejects this Agreement pursuant to Section 365 of the Bankruptcy Code (a "**Bankruptcy Rejection**"), (i) the license to the BRI Technology described under this Agreement shall be deemed fully retained by and vested in Ultragenyx as protected intellectual property rights under Section 365(n)(1)(B) of the Bankruptcy Code and further shall be deemed to exist immediately before the commencement of the bankruptcy case in which BRI is the debtor; and (ii) Ultragenyx shall have all of the rights afforded to non-debtor licensees under Section 365(n) of the Bankruptcy Code, subject to its continued compliance with all its obligations under this Agreement. All rights and licenses now or hereinafter granted by BRI to Ultragenyx under or pursuant to any section of this Agreement, including Section 2.1 are rights to "intellectual property" (as defined in the Bankruptcy Code).

7.3 Effect of Termination.

(a) Termination by Ultragenyx for Convenience; Termination by BRI for Breach or Bankruptcy. Upon termination of this Agreement by Ultragenyx pursuant to Section 7.2(a) or by BRI pursuant to Sections 7.2(b) or 7.2(c), the following consequences shall apply to the termination and shall be effective as of the effective date of such termination:

(i) Each Party shall pay all amounts then due and owing to the other Party as of the termination date;

(ii) All licenses and other rights granted to Ultragenyx under the BRI Technology will terminate, and Ultragenyx shall immediately discontinue sales of Product;

(iii) No later than thirty (30) days after the effective date of such termination, Ultragenyx shall return or cause to be returned to BRI all Confidential Information in tangible form received from BRI and all copies thereof and all materials substances or compositions delivered or provided by BRI; provided, however, that Ultragenyx may keep one copy of Confidential Information received from the other Party in its confidential files for record purposes;

(iv) No later than thirty (30) days after the effective date of termination, at BRI's option, the Parties shall negotiate in good faith the terms and conditions (including financial compensation to Ultragenyx) for a license under patent applications, patents, Know-How, and any other information and documentation Controlled by Ultragenyx as of the effective date of such termination necessary for BRI to continue development and commercialization of Products in the Licensed Territory, but solely for those indications, the use of which are claimed by a Valid Claim in the BRI Technology as of the date of such termination. If the Parties have failed to agree upon such terms of compensation within ninety (90) days of initiation of such negotiation, the matter shall be referred to three independent industry experts having expertise in intellectual property valuation and who are mutually agreeable to both Parties. The industry experts shall take into account all pertinent factors, including but not limited to each party's relative contribution to the Ultragenyx Technology, and within thirty (30) days make a joint recommendation of appropriate compensation for the license ("License Compensation"). BRI shall have the right, but not the obligation, to accept the license at the recommended License Compensation. If BRI does not accept the license at the recommended License Compensation, then Ultragenyx shall have no further obligation to negotiate with BRI regarding the terms and conditions for a license pursuant to this Section 7.3(a)(iv) and Ultragenyx shall have no obligation to grant BRI any such license. If BRI does accept the license at the recommended License Compensation, then Ultragenyx shall have the obligation to grant BRI the license at the recommended License Compensation; and

(v) At the written request of any of Ultragenyx's sublicensees under this Agreement, BRI shall negotiate in good faith with such sublicensee an agreement between BRI and such sublicensee under which such sublicensee will obtain a direct license under the BRI Technology. For clarity, under no circumstances shall BRI be required to grant a direct license to any sublicensee which does not compensate BRI at a level substantially equivalent to the compensation received by BRI under this Agreement with respect to the applicable technology, taking into consideration any costs associated with BRI's license to Ultragenyx Technology. Ultragenyx shall indemnify, defend and hold BRI Indemnitees harmless from and against any Claims brought by any sublicensee in connection with the termination of an Ultragenyx sublicense, or the inability of BRI and any sublicensee to negotiate a mutually

acceptable license to BRI Technology, provided that Ultragenyx shall have no indemnification obligation to BRI under this Section 7.3(a)(v) to the extent such Claim arises as a result of BRI's bad faith in negotiation with an Ultragenyx sublicensee.

(b) Termination by Ultragenyx for Breach or Bankruptcy. Upon termination of this Agreement by Ultragenyx pursuant to Sections 7.2(b) or 7.2(c), the following consequences shall apply to the termination and shall be effective as of the effective date of such termination:

(i) Each Party shall pay all amounts then due and owing to the other Party as of the termination date;

(ii) The licenses and other rights granted by BRI to Ultragenyx under the BRI Technology will remain in full force and effect as set forth in Sections 2.1; provided that Ultragenyx fulfills its payment obligations to BRI under Article 4 pursuant to the terms and conditions set forth in Article 4, further provided that, on a Product-by-Product and country by country basis, for any Product that is the subject of the underlying breach, milestone payments under Sections 4.2 and 4.3 and royalty payments under Section 4.4 with respect to such Product shall be reduced by [***] percent ([***]%);

(iii) No later than thirty (30) days after the effective date of such termination, BRI shall return or cause to be returned to Ultragenyx all Confidential Information in tangible form received from Ultragenyx and all copies thereof and all materials, substances or compositions delivered or provided by Ultragenyx; provided, however, that BRI may keep one copy of Confidential Information received from Ultragenyx in its confidential files for record purposes; and

(iv) In addition to the provisions set forth in Section 7.5, the provisions relating to BRI Patents in Sections 6.1, 6.2, 6.3 and 6.4 shall survive the termination of this Agreement for so long as the license to Ultragenyx survives under this Section 7.3(b).

7.4 Survival. Expiration or termination of this Agreement shall not relieve the Parties of any obligation accruing prior to such expiration or termination. Without limiting the foregoing, the provisions of Articles 1, 6, 9 (to the extent the Claims arise out of actions or omissions during the Term) and 10, and Sections 5.1, 7.3, 7.4 and 7.5, and any other provisions which by their nature are intended to survive, shall survive the expiration or termination of this Agreement.

7.5 Termination Not Sole Remedy. Termination is not the sole remedy under this Agreement and, whether or not termination is effected and notwithstanding anything contained in this Agreement to the contrary, all other remedies will remain available except as agreed to otherwise herein.

Article 8

Representations and Warranties

8.1 Representations and Warranties of Each Party. Each Party represents and warrants to the other Party as of the Effective Date that:

(a) it has the full right, power and authority to enter into this Agreement, to perform its obligations hereunder; and

(b) this Agreement has been duly executed by it and is legally binding upon it, enforceable in accordance with its terms, and does not conflict with any agreement, instrument or understanding, oral or written, to which it is a party or by which it may be bound, nor violate any material law or regulation of any court, governmental body or administrative or other agency having jurisdiction over it.

8.2 Representations and Warranties by BRI. BRI represents and warrants to Ultragenyx as of the Effective Date that:

(a) it has not previously assigned, transferred, conveyed or otherwise encumbered its right, title and interest in BRI Patents or BRI Know-How with respect to any of the Compounds or Products other than pursuant to the [***] Option;

(b) it has the right to grant the license and rights herein to Ultragenyx and it has not granted any license, right or interest in, to or under the BRI Patents or BRI Know-How to any Third Party with respect to any of the Compounds or Products other than pursuant to the [***] Option;

(c) to the best of its knowledge, it has provided to Ultragenyx all of the following information relating to the Compound or Product: all communications to and from Regulatory Authorities, all protocols and amendments for any human clinical studies, all safety reports sent to the FDA or other Regulatory Authorities, all Regulatory Filings filed with Regulatory Authorities, including all INDs filed with the FDA, and all clinical, non-clinical, and research study reports in its possession;

(d) the [***] Option does not grant [***] any license rights to BRI Know-How and BRI Patents with respect to the United States, Canada or Mexico;

(e) to the best of its knowledge, except as otherwise stated in this Section 8.2(e), the development, use, sale and import of Compounds or Products in the Licensed Territory do not infringe any valid intellectual property rights owned or possessed by any Third Party and do not breach any obligation of confidentiality or non-use owed by BRI to a Third Party, provided however, that the Parties acknowledge and agree that the following Third Parties may own or possess intellectual property rights covering the method of use of the Compound and/or Product: [***];

(f) there are no claims, judgments or settlements against or owed by BRI and to the best of BRI's knowledge, there are no pending or threatened claims or litigation, in each case relating to any Compounds or Products, or to the BRI Patents or BRI Know-How in the Licensed Territory;

(g) there is no provision in the [***] Option or the agreement to be entered into between BRI and [***] upon [***] exercise of the [***] Option that expressly addresses the performance of clinical trials by BRI or any other party in the Option Territory and during the Term BRI shall not extend the duration of the [***] Option, or amend the terms of such [***] Option or the agreement to be entered into between BRI and [***] upon [***] exercise of the [***] Option (other than financial), in each case without Ultragenyx's prior written consent; and

(h) the list of Patents contained in Exhibit A is a complete list of all BRI Patents.

8.3 No Other Warranties. EXCEPT AS EXPRESSLY STATED IN THIS ARTICLE 8, (A) NO REPRESENTATION, CONDITION OR WARRANTY WHATSOEVER IS MADE OR GIVEN BY OR ON BEHALF OF ULTRAGENYX OR BRI; AND (B) ALL OTHER CONDITIONS AND WARRANTIES WHETHER ARISING BY OPERATION OF LAW OR OTHERWISE ARE HEREBY EXPRESSLY EXCLUDED, INCLUDING ANY CONDITIONS AND WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE OR NON-INFRINGEMENT.

Article 9

Indemnification; Liability

9.1 Indemnification by BRI. BRI shall indemnify and hold Ultragenyx and its Affiliates, and their respective officers, directors, agents and employees ("**Ultragenyx Indemnitees**") harmless from and against any Claims arising under or related to this Agreement against them to the extent arising or resulting from:

(a) the negligence or willful misconduct of any of the BRI Indemnitees; or

(b) the breach of any of the warranties or representations made by BRI to Ultragenyx under this Agreement; or

(c) any breach by BRI of its material obligations pursuant to this Agreement

except in each case, to the extent such Claims result from the material breach by any Ultragenyx Indemnitee of any covenant, representation, warranty or other agreement made by Ultragenyx in this Agreement or the negligence or willful misconduct of any Ultragenyx Indemnitee.

9.2 Indemnification by Ultragenyx. Ultragenyx shall indemnify and hold BRI, its Affiliates, and their respective officers, directors, agents and employees ("**BRI Indemnitees**") harmless from and against any Claims arising under or related to this Agreement against them to the extent arising or resulting from:

(a) the development, manufacture, packaging, use, sale or commercialization of the Compounds or Products, or use of the BRI Technology, by or on behalf Ultragenyx or any of its Affiliates, sublicensees or contractors in the Field in the Licensed Territory; or

(b) the negligence or willful misconduct of any of the Ultragenyx Indemnitees, sublicensees, or contractors; or

(c) the breach of any of the warranties or representations made by Ultragenyx to BRI under this Agreement;

(d) any representation made or warranty given by Ultragenyx, or any of its Affiliates, sublicensees or contractors with respect to Compounds, Products, BRI Patents or BRI Know-How;

(e) any infringement claims relating to Compounds or Products;

(f) any asserted violation of applicable Laws by any Ultragenyx Indemnitees, sublicensees or contractors; or

(g) any breach by Ultragenyx of its material obligations pursuant to this Agreement;

except in each case, to the extent such Claims result from the material breach by any BRI Indemnitee of any covenant, representation, warranty or other agreement made by BRI in this Agreement or the negligence or willful misconduct of any BRI Indemnitee.

9.3 Insurance. From and after the Effective Date, Ultragenyx shall maintain for a period of [***] after the expiration or termination of this Agreement, commercial product liability insurance (including contractual liability insurance and clinical trial insurance) with insurance carriers with, at least, an AM BEST rating of A- VII to cover the activities of Ultragenyx Indemnitees, for minimum limits of [***] dollars ($[***]) per claim and [***] dollars ($[***]) in the aggregate. Such insurance shall cover BRI Indemnitees as additional insureds. Ultragenyx shall furnish a certificate of insurance evidencing such coverage. Ultragenyx will provide thirty days' written notice to BRI of cancellation or material change in coverage. The minimum amounts of insurance coverage required herein shall not be construed as creating any limitation on the Ultragenyx's indemnity obligation under Section 9.2. of this Agreement. If any coverage is written on a Claims Made policy form, the Retroactive Date is to be the first date that the first Claims Made policy was effective and is not to be advanced during the term of the project. Additionally, if the coverage is written on a Claims Made form, the insurance policies will remain in full force and effect, or if canceled or non renewed Ultragenyx shall purchase an Extended Reporting Period "Tail Coverage", for a minimum period of [***] after the termination of this Agreement.

9.4 Indemnification Procedure. If either Party is seeking indemnification under Sections 9.1 or 9.2 (the "**Indemnified Party**"), it shall inform the other Party (the "**Indemnifying Party**") of the claim giving rise to the obligation to indemnify pursuant to such section as soon as reasonably practicable after receiving notice of the claim. The Indemnifying Party shall have the right to assume the defense of any such claim for which it is obligated to indemnify the Indemnified Party. The Indemnified Party shall cooperate with the Indemnifying Party and the Indemnifying Party's insurer as the Indemnifying Party may reasonably request, and at the Indemnifying Party's cost and expense. The Indemnified Party shall have the right to participate, at its own expense and with counsel of its choice, in the defense of any claim or suit that has been assumed by the Indemnifying Party. Neither Party shall have the obligation to indemnify the other Party in connection with any settlement made without the Indemnifying Party's written consent, which consent shall not be unreasonably withheld, conditioned or delayed. If the Parties cannot agree as to the application of Section 9.1 or 9.2 as to any claim, pending resolution of the dispute pursuant to Section 10.6, the Parties may conduct separate defenses of such claims, with each Party retaining the right to claim indemnification from the other Party in accordance with Section 9.1 or 9.2 upon resolution of the underlying claim.

9.5 Mitigation of Loss. Each Indemnified Party will take and will procure that its Affiliates take all such reasonable steps and action as are reasonably necessary or as the Indemnifying Party may reasonably require in order to mitigate any Claims (or potential losses or damages) under this Article 9. Nothing in this Agreement shall or shall be deemed to relieve any Party of any common law or other duty to mitigate any losses incurred by it.

9.6 Special, Indirect and Other Losses. NEITHER PARTY NOR ANY OF ITS AFFILIATES SHALL BE LIABLE IN CONTRACT, TORT, NEGLIGENCE, BREACH OF STATUTORY DUTY OR OTHERWISE FOR ANY CONSEQUENTIAL, SPECIAL OR PUNITIVE DAMAGES OR FOR LOSS OF PROFITS SUFFERED BY THE OTHER PARTY, EXCEPT TO THE EXTENT ANY SUCH DAMAGES ARE REQUIRED TO BE PAID TO A THIRD PARTY AS PART OF A CLAIM FOR WHICH A PARTY PROVIDES INDEMNIFICATION UNDER THIS ARTICLE 9.

Article 10

General Provisions

10.1 Force Majeure. Neither Party shall be held liable to the other Party nor be deemed to have defaulted under or breached this Agreement for failure or delay in performing any obligation under this Agreement to the extent such failure or delay is caused by or results from causes beyond the reasonable control of the affected Party, potentially including embargoes, war, acts of war (whether war be declared or not), acts of terrorism, insurrections, riots, civil commotions, strikes, lockouts or other labor disturbances, fire, floods, earthquakes or other acts of God, or acts, omissions or delays in acting by any Government Authority or the other Party or unavailability of materials related to the manufacture of Compounds or Products. The affected Party shall notify the other Party in writing of such force majeure circumstances as soon as reasonably practical, and shall promptly undertake and continue diligently all reasonable efforts necessary to cure such force majeure circumstances or to perform its obligations in spite of the ongoing circumstances. Notwithstanding the foregoing, neither Party shall be excused from making payments owed hereunder because of a force majeure affecting such Party unless such force majeure event affects the method of payment.

10.2 Assignment.

(a) This Agreement may not be assigned or otherwise transferred, nor may any right or obligation hereunder be assigned or transferred, by either Party without the prior written consent of the other Party. Notwithstanding the foregoing, either Party may, without consent of the other Party, assign this Agreement and its rights and obligations hereunder in whole or in part to an Affiliate of such Party, or to its successor in interest by way of merger, acquisition, or sale of all or substantially all of its assets to which this Agreement relates. Any attempted assignment not in accordance with this Section 10.2 shall be null and void and of no legal effect. Any permitted assignee shall assume all assigned obligations of its assignor under this Agreement. The terms and conditions of this Agreement shall be binding upon, and shall inure to the benefit of, the Parties and their respected successors and permitted assigns.

(b) Notwithstanding anything to the contrary in this Agreement, in the event that a Party undergoes a merger, acquisition, or sale of all or substantially all of its assets to which this Agreement relates, no intellectual property rights of the Third Party assignee, acquiror or successor of such Party or any Affiliate of such Third Party shall be included in the subject matter licensed hereunder, to the extent that such intellectual property rights were held by such Third Party prior to the merger, acquisition or sale, or are created outside of any activities under this Agreement by personnel who were not employees of the acquired Party at the time of the acquisition.

10.3 Severability. If any one or more of the provisions contained in this Agreement is held invalid, illegal or unenforceable in any respect, the validity, legality and enforceability of the remaining provisions contained herein shall not in any way be affected or impaired thereby, unless the absence of the invalidated provision(s) adversely affects the substantive rights of the Parties. The Parties shall in such an instance use their best efforts to replace the invalid, illegal or unenforceable provision(s) with valid, legal and enforceable provision(s) which, insofar as practical, implement the purposes of this Agreement.

10.4 Notices. All notices which are required or permitted hereunder shall be in writing and sufficient if delivered personally, sent by facsimile (and promptly confirmed by personal delivery, registered or certified mail or courier), sent by internationally recognized courier or sent by registered or certified mail, postage prepaid, return receipt requested, addressed as follows:

If to BRI:

Baylor Research Institute
3310 Live Oak Street, Suite 501
Dallas, TX 75204
Attn: Chief Operating Officer
Fax: (214) 820-4952

with a copy to:

Law Department
Baylor Health Care System
4005 Crutcher Street
Dallas, TX 75246
Attn: BRI Attorney
Fax: (214) 820-1535

If to Ultragenyx:

Ultragenyx Pharmaceutical Inc.
60 Leveroni Court
Novato, CA 94949
Attn: Tom Kassberg, Chief Business Officer
Fax: (415) 483-8820

with a copy to:

Cooley LLP
3175 Hanover Street
Palo Alto, CA 94304
Attn: Lila Hope, Esq.
Fax: (650) 849-7400

or to such other address(es) as the Party to whom notice is to be given may have furnished to the other Party in writing in accordance herewith. Any such notice shall be deemed to have been given: (a) when delivered if personally delivered or sent by facsimile on a Business Day (or if delivered or sent on a non-Business Day, then on the next Business Day); (b) on the Business Day after dispatch if sent by internationally recognized overnight courier; or (c) on the fifth (5th) Business Day following the date of mailing, if sent by mail.

10.5 Applicable Law. This Agreement shall be governed by and construed in accordance with the laws of the State of New York and the patent laws of the United States without reference to any rules of conflict of laws.

10.6 Dispute Resolution. The Parties shall negotiate in good faith and use reasonable efforts to settle any dispute, controversy or claim ("**Dispute**") arising from or related to this Agreement or the breach thereof. If any Dispute has not been resolved within [***] days of written notice detailing the nature of the Dispute by one Party to the other, the Parties shall each immediately refer such dispute to respective senior executives at the level of Senior Vice President or above for consideration and resolution. If the Dispute has not been resolved within [***] days of referral to such senior executives, either party may bring an action in a court of competent jurisdiction.

10.7 Compliance. Each Party agrees that in performing its obligations or exercising its rights under this Agreement: (a) it shall comply in all material respects with all applicable Laws; (b) it will not employ or engage any Person who has been debarred by any Regulatory Authority, or, to such Party's knowledge, is the subject of debarment proceedings by a Regulatory Authority; and (c) it will be primarily responsible for any activities performed on its behalf by an Affiliate, licensee, sublicensee, contractor or subcontractor.

10.8 Entire Agreement.

(a) This Agreement, together with the Exhibits, contains the entire understanding of the Parties with respect to the licenses granted hereunder. Any other express or implied agreements and understandings, negotiations, writings and commitments, either oral or written, in respect to the licenses granted hereunder are superseded by the terms of this Agreement. The Exhibits to this Agreement are incorporated herein by reference and shall be deemed a part of this Agreement.

(b) This Agreement supersedes the Mutual Nondisclosure Agreement, dated February 8th, 2012, between Ultragenyx and BRI (the "**Prior NDA**"). All Confidential Information disclosed by one Party to the other Party under the Prior NDA shall be deemed Confidential Information of such disclosing Party under this Agreement and shall be subject to the terms of this Agreement.

10.9 Amendments. This Agreement may be amended, or any term hereof modified, only by a written instrument duly executed by authorized representative(s) of both Parties.

10.10 Headings. The captions to the several Articles, Sections and subsections hereof are not a part of this Agreement, but are merely for convenience to assist in locating and reading the several Articles and Sections hereof.

10.11 Independent Contractors. It is expressly agreed that BRI and Ultragenyx shall be independent contractors and that the relationship between the two Parties shall not constitute a partnership, joint venture or agency. Neither BRI nor Ultragenyx shall have the authority to make any statements, representations or commitments of any kind, or to take any action, which shall be binding on the other Party, without the prior written consent of the other Party.

10.12 Waiver. The waiver by either Party of any right hereunder, or of any failure of the other Party to perform, or of any breach by the other Party, shall not be deemed a waiver of any other right hereunder or of any other breach by or failure of such other Party whether of a similar nature or otherwise.

10.13 Cumulative Remedies. No remedy referred to in this Agreement is intended to be exclusive, but each shall be cumulative and in addition to any other remedy referred to in this Agreement or otherwise available under law.

10.14 Waiver of Rule of Construction. The rule of construction that any ambiguity in this Agreement shall be construed against the drafting Party shall not apply.

10.15 Business Day Requirements. In the event that any notice or other action or omission is required to be taken by a Party under this Agreement on a day that is not a Business Day then such notice or other action or omission shall be deemed to be required to be taken on the next occurring Business Day.

10.16 Counterparts. This Agreement may be executed in counterparts by original signature, facsimile or PDF files, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

[Remainder of page intentionally left blank; signature page follows.]

IN WITNESS WHEREOF, the Parties intending to be bound have caused this Agreement to be executed by their duly authorized representatives.

Baylor Research Institute

By: /s/ Jaime Walkawiak
Name: Jaime Walkawiak
Title: VP Research Operations

Ultragenyx Pharmaceutical Inc.

By: /s/ Emil Kakkis
Name: Emil Kakkis MD PhD
Title: Chief Executive Officer

Exhibit A

BRI Patents Existing as of the Effective Date

Case Number	Case Type	Country	Priority Case Number	Inventor Name	Status, Filing Date, App. Serial No. Pub No. & Date	Pat/Reg No., Issue/Reg Date	Title
[***]							

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

Exhibit 10.4

AMENDMENT LICENSE AGREEMENT

This Amendment to the License Agreement ("Amendment") is made and entered into by and between Ultragenyx Pharmaceutical Inc., a corporation organized and existing under the laws of the State of Delaware, having its principal place of business at 60 Leveroni Court, Novato, CA 94949 ("**Ultragenyx**"), and Baylor Research Institute, a non-profit corporation organized and existing under the laws of the State of Texas, having its principal place of business at 3310 Live Oak Street, Suite 501, Dallas, Texas 75204 ("**BRI**").

RECITALS

A. WHEREAS, Ultragenyx and BRI entered into a License Agreement ("Agreement") dated September 20, 2012.

B. WHEREAS, Both Parties wish to amend the Agreement as set forth below to further clarify the intent of the Parties when the Agreement was initially entered into.

NOW, THEREFORE, in consideration of the mutual covenants and premises herein contained, the parties agree as follows;

1. This Amendment shall be effective as of March 22, 2013 ("the Effective Date")

2. The following shall be inserted to replace Section 5.1 in the Agreement

(a) As between the Parties, Ultragenyx, acting through outside patent counsel of its choice **("Patent Counsel")**, shall have the first right, but not the obligation, to take the lead in the preparation, filing, prosecution and maintenance of the BRI Patents in the Licensed Territory, at Ultragenyx's cost and expense. BRI shall cooperate with Ultragenyx in filing and prosecution of such BRI Patents in the Licensed Territory, including by providing Ultragenyx with data and other information as appropriate and executing all necessary paperwork to enable Patent Counsel to file, prosecute, and defend BRI Patents directly with patent offices. Within [***] days after the Effective Date, BRI shall provide to Ultragenyx those copies of all patent filings, and the correspondence between BRI and patent authorities, for BRI Patents in the Licensed Territory existing as of the Effective Date. Within [***] days after Ultragenyx exercises the Ultragenyx Option pursuant to Section 2.2(a), BRI shall provide Ultragenyx those copies of all patent filings, and the correspondence between BRI and patent authorities, for BRI Patents in the Licensed Territory existing as of the date of exercise of the Ultragenyx Option that have not already been provided to Ultragenyx pursuant to this Section 5.1(a). Ultragenyx will keep BRI reasonably informed of the status of the prosecution of such BRI Patents in the Licensed Territory by providing a copy of any written response to be filed with the USPTO or a copy of any instruction letter to a foreign associate with respect to preparation of a response to be filed with the pertinent foreign patent office to counsel of BRI's choice prior to such filing. BRI has the right to provide comment to Patent Counsel at BRI's cost and expense. Ultragenyx will consider any comment provided by BRI counsel in good faith, but shall not be required to incorporate any such comment.

For the purpose of this Article 6, "prosecution" shall include any pre-grant and post-grant proceeding including patent interference proceeding, opposition proceeding and other similar proceedings, appeals or petitions to any Board of Appeals in the patent office, appeals to any court for any patent office decisions, reissues and reexamination proceedings, and applications for patent term extensions and the like.

(b) Ultragenyx will notify BRI of any decision not to file for, prosecute or maintain, or not to continue to pay the expenses of prosecution or maintenance of (collectively, "Patent Support"), any BRI Patents in the Licensed Territory. Ultragenyx will provide such notice at least [***] days prior to any filing or payment due date, or any other due date that requires action, in connection with such BRI Patent. In such event, BRI shall have the right, but not the obligation, to file for, or continue prosecution or maintenance of, such BRI Patent in the Licensed Territory, at its expense. In the event BRI does maintain such BRI Patent in the Licensed Territory and such BRI Patent in the Licensed Territory covers the applicable Product that triggers any royalty or milestone payment, Ultragenyx shall continue payment of the applicable royalties and milestones.

3. Except as expressly provided in this Amendment, all other terms, conditions and provisions of the Agreement shall continue in full force and effect as provided therein. Capitalized terms used in this Amendment that are not otherwise defined herein shall have the same meanings as such terms are defined in the Agreement

IN WITNESS WHEREOF, the Parties intending to be bound have caused this Amendment to be executed by their duly authorized representatives.

Baylor Research Institute

By: /s/ Paul Convery
Name: Paul Convery
Title: Interim CO

Ultragenyx Pharmaceutical Inc.

By: /s/ Thomas Kassberg
Name: Thomas Kassberg
Title: CBO

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

EXCLUSIVE LICENSE AGREEMENT

THIS EXCLUSIVE LICENSE AGREEMENT including the exhibits referred to herein and attached hereto which are hereby incorporated by reference (the "Agreement"), effective as of April 23, 2012 (the "Effective Date"), is made and entered into by and between the HIBM Research Group, a non-profit public benefit California corporation, having a principal place of business located at 18341 Sherman Way, #201A, Reseda, CA 91335 ("HRG") and Ultragenyx Pharmaceutical Inc., a Delaware corporation having a principal place of business located at 60 Leveroni Ct., Novato, California ("UPI").

RECITALS

A. WHEREAS, HRG and UPI are interested in developing and improving the access to treatment for human inclusion body myopathies ("HIBM") and are desirous of forming a collaboration to further such purpose;

B. WHEREAS, HRG owns or has the right to grant licenses under certain patents, patent applications, technology, trade secrets, data, know-how and other intellectual property relating to the treatment of human inclusion body myopathies ("HIBM") using substrate replacement therapy; and

C. WHEREAS UPI desires to obtain from HRG, and HRG is willing to grant to UPI, an exclusive license under such technology and intellectual property for the development and commercialization of certain products and services under the terms and conditions herein.

NOW, THEREFORE, in consideration of the mutual covenants and obligations set forth herein, and for other good and valuable consideration, the receipt and sufficiency of which is hereby acknowledged, HRG and UPI hereby agree as follows:

1. DEFINITIONS

As used in this Agreement, the following terms shall have the meanings indicated:

1.1. "Affiliate" shall mean any legal person or entity directly or indirectly controlling, controlled by or under common control with a party to this Agreement. For the purposes of this Section, "control" shall mean the possession of the power to direct or cause the direction of the management and policies of an entity, whether by ownership of voting stock or partnership interest, by contract or otherwise, including direct or indirect ownership of more than fifty percent (50%) of the voting interest in the entity in question.

1.2. "Biomarker(s)" shall mean an analyte contained in a biological sample which can be used to diagnose, assess or manage treatment of patients with HIBM or related conditions.

1.3. "Commercially Reasonable Efforts" shall mean, as applied to a party, the performance of such party's obligations under this agreement to apply efforts that are similar to those applied by a similarly situated biotechnology company in relation to a similar or comparable pharmaceutical product owned by it or to which it has rights. Such party is entitled to exercise prudent and justifiable business judgment in meeting its obligations hereunder.

1.4. "**Control**" means, with respect to any Know-How, Patent Rights, other intellectual property rights, or any proprietary or trade secret information, the legal authority or right (whether by ownership, license or otherwise) of a party to grant a license or a sublicense under such Know-How, Patent Rights, or intellectual property rights to the other party, or to otherwise disclose such proprietary or trade secret information to the other party, in each case without breaching the terms of any agreement with a Third Party, or misappropriating the proprietary or trade secret information of a Third Party.

1.5. "**Confidential Information"** shall have the meaning set forth in Section 10.1 below.

1.6. "**FDA"** shall mean the U.S. Food and Drug Administration, or any successor thereto.

1.7. "Field" shall mean [***] related to the treatment or diagnosis of HIBM and related conditions using substrate replacement therapy.

1.8. "**HRG Mice**" shall mean the [***] mice produced by HRG and described in US Patent Application [***], together with any progenies, cross-breeds, modifications and/or derivatives thereof.

1.9. "**Invention(s)"** shall have the meaning set forth in Section 4.1

1.10. "**Know-How**" shall mean technical information, inventions, know-how, processes, procedures, methods, formulae, protocols, techniques, software, documents, reports, data, works of authorship, and materials, but excluded Patent Rights.

1.11. "**Licensed Intellectual Property**" shall mean Licensed Patents and Licensed Know-How.

1.12. "**Licensed Know-How**" shall mean any and all Know-How Controlled by HRG or its Affiliates as of the Effective Date or during the Term and related to the treatment of HIBM or related conditions. Licensed Know-How includes, without limitation, all such information that relates to: (a) the HIBM Mice, (b) the Biomarkers, and (c) any and all data, reports and materials from preclinical studies, toxicology studies, clinical studies, manufacturing, quality, science, and regulatory filings related to substrate replacement therapy for the treatment of HIBM.

1.13. "**Licensed Patents**" shall mean any and all Patent Rights Controlled by HRG or its Affiliates as of the Effective Date or during the Term that: (a) describe, claim, or cover compositions or methods pertaining to the Field; or (b) are necessary for UPI to practice the Licensed Know-How in the Field. Licensed Patents existing as of the Effective Date are set forth on Exhibit A.

1.14. "**Major Market Country"** shall mean [***].

1.15. "**Net Sales**" shall mean the gross amount invoiced by UPI, its Affiliates or sublicensees to third parties with respect to products, less: (a) sales, returns and allowances actually paid, granted or accrued, including trade, quantity and cash discounts and any other

adjustments granted on account of price adjustments or billing errors; (b) rejected goods, damaged or defective goods, recalls, returns; (c) rebates, chargeback rebates, compulsory rebates, reimbursements or similar payments granted or given to wholesalers or other distributors, buying groups, health care insurance carriers or other institutions; (d) adjustments actually granted Third Parties arising from consumer discount programs or other similar programs; (e) non-collectable receivables related to product (provided that if they are later collected, they shall be included in Net Sales in the calendar quarter of collection), (f) customs or excise duties, sales tax, consumption tax, value added tax, and other taxes (except income taxes) separately stated on the invoice; or (g) charges for packing, freight, shipping and insurance (to the extent separately stated on the invoice). Each of the foregoing deductions shall be determined as incurred in the ordinary course of business in type and amount consistent with good industry practice and in accordance with GAAP on a basis consistent with Licensee's audited consolidated financial statements and consistently applied across all products of UPI and its Affiliates. Even if there is overlap between any of deductions (a) - (g), each individual item shall only be deducted once in the overall Net Sales calculation. Any products used for promotional or advertising purposes, used for clinical trials, preclinical trials or other research purposes, free samples, or distributed at no charge to patients unable to purchase the same shall not be included in Net Sales. Donations for charity reasons (to avoid doubt, for which no monetary consideration is received) shall also not be included in Net Sales.

1.16. "**Patent Rights**" means all patents and patent applications (which for the purpose of this Agreement shall be deemed to include certificates of invention and applications for certificates of invention), including all divisionals, continuations, substitutions, continuations-in-part, re-examinations, reissues, additions, renewals, revalidations, extensions, registrations, pediatric exclusivity periods and supplemental protection certificates and the like of any such patents and patent applications, and any and all foreign equivalents of the foregoing.

1.17. "**Phase III Clinical Study**" shall mean the first pivotal human clinical trial conducted in patients and designed to establish the final product safety and efficacy data required to obtain approval for marketing and sale in a particular country or jurisdiction, in accordance with 21 C.F.R. 312.21 (c), as may be amended from time to time, or any foreign equivalent thereto.

1.18. "**Term"** shall have the meaning set forth in Section 9.1.

1.19. "**Third Party"** shall mean any legal person or entity other than HRG or UPI, or an Affiliate of HRG or UPI.

1.20. "**Valid Claim**" shall mean a claim of: (a) an issued and unexpired patent, which has not been disclaimed, revoked, held unenforceable or invalid by a decision of a court or other governmental agency of competent jurisdiction, unappealable or unappealed within the time allowed for appeal, and which has not been admitted to be invalid or unenforceable through reissue or disclaimer or otherwise; or (b) a pending patent application which has not been pending for more than seven (7) years from the date of the first patent application to which it claims priority, provided that, in the event such pending claim issues after the end of such seven (7) year period, such claim shall again be deemed a Valid Claim commencing on the date of such issuance.

2. LICENSES

2.1. License Grant. Subject to the terms and conditions of this Agreement, HRG hereby grants to UPI a worldwide, exclusive license, including the right to grant sublicenses through multiple tiers, in, to and under the Licensed Intellectual Property to research, have researched, develop, have developed, practice, have practiced, manufacture, have manufactured, use, have used, sell, have sold, offer for sale, have offered for sale, import, have imported, export and have exported products in the Field in the Territory (and to have all such rights exercised on UPI's behalf by Third Parties). The license to Licensed Patents is exclusive even as to HRG. UPI shall notify HRG of the general purpose of any sublicense granted by UPI under the license it obtains under this Section 2.1 and, subject to any confidentiality obligation to any Third Party, a summary of material terms of any sublicense agreement governing such sublicense, within thirty (30) days after entering into agreement. HRG agrees that an informal email or verbal communication shall be deemed to satisfy such notice requirement.

2.2. Retained Rights. UPI acknowledges and agrees that HRG and its licensors shall retain ownership of the Licensed Intellectual Property, subject only to the rights and licenses expressly granted herein and the terms and conditions of this Agreement, including this Section 2.2. Except as expressly provided herein, no right, title, or interest is granted by HRG to UPI, implied or otherwise, in, to or under the Licensed Intellectual Property.

2.2.1. HRG retains the right to practice or grant licenses under the Licensed Intellectual Property outside of the Field. In the event that HRG grants a license pursuant to this Section, it shall notify UPI in writing within [***] days after such license grant and provide UPI with a written description of the general purpose of such license grant and, subject to any confidentiality obligation to any Third Party, summary material terms of the agreement governing such license grant.

2.2.2. Third Party Collaborations. In the event that a for-profit Third Party requests access or licenses to HIBM mouse or other Licensed Intellectual Property for internal research use, HRG may propose to UPI a grant of rights to such Third Party and UPI shall have the right, at its sole discretion, to decide whether to allow the grant of such research-use-only limited license.

2.2.3. Other than as specifically set forth in Section 2.2.1 and Section 2.2.2, HRG shall not have the right to practice or grant to any Third Party a license under any Licensed Intellectual Property in the Field, in the Territory.

2.3. Technology Transfer.

2.3.1. Licensed Know-How. Within [***] days after the Effective Date, HRG shall transfer to UPI Licensed Know-How, including those as set forth on Exhibit B hereto.

2.3.2. Regulatory Filings. Subject to the terms and conditions of this Agreement, HRG hereby grants to UPI full rights of access, use and reference to any and all of HRG's regulatory filings or approvals related to the Field, including, without limitation, the information, results and data therein and all appropriate information, consents and notices to the FDA and other regulatory authorities as are useful or necessary for UPI to effectuate the rights of

reference granted to it in this Section 2.1 (collectively, the "Regulatory Materials"). Within [***] days after the Effective Date, HRG shall provide UPI all Regulatory Materials existing as of the Effective Date. From time to time during the Term, HRG shall promptly provide UPI with any new Regulatory Materials come into existence.

3. PAYMENTS AND RELATED OBLIGATIONS

3.1. Initial Payment. Within [***] calendar days after the Effective Date, UPI shall pay to HRG a non-refundable, non-creditable payment of twenty five thousand dollars (US$25,000.00). This payment is in addition to and separate from any payment terms described in the Research Collaboration Agreement.

3.2. Milestone Payments. UPI shall notify HRG in writing within [***] days after the first achievement of each applicable milestone set forth below by or for UPI subject to the achievement of criteria set forth in Sections 3.2(i) through (iii) below, and UPI shall make the following one-time, non-creditable, non-refundable milestone payments to HRG within [***] days after such notice. For the avoidance of doubt, each of the following payments under this Section 3.2 is to be made only once regardless of the number of times such milestone is achieved and the number of products or indications for which such milestone event have been achieved. If UPI [***], UPI shall pay to HRG the following amounts:

(a) [***] dollars (US$[***]) upon [***], and

(b) [***] dollars (US$[***]) upon [***].

3.3. Royalty Payments. UPI shall pay HRG a royalty on products sold by UPI, its Affiliates or sublicensees, on a product-by-product and country-by-country basis: (a) at the rate of [***] percent ([***]%) of Net Sales for a particular product in a particular country, for the period of time commencing on the first commercial sale of such product in such country and ending on the expiration of the last-to-expire Valid Claim included in Licensed Patents claiming the composition of matter of or the method of using such product in such country; or, as the case may be, (b) at the rate of [***] percent ([***]%) of Net Sales for a particular product in a Major Market Country for the period of time when there is no Valid Claim included in the Licensed Patents claiming the composition of matter of or the method of using such product in such Major Market Country but UPI (or its Affiliates or sublicensees) maintains orphan drug exclusivity in such Major Market Country. For clarity, for a particular product, UPI shall at no time have the obligation to pay royalties under both Section 3.3(a) and Section 3.3(b) above and the maximum royalty rate applicable to such product shall be [***] percent ([***]%).

3.4. Taxes. UPI shall be responsible for all taxes, duties and levies directly imposed by all foreign, federal, state, local or other taxing authorities (including, without limitation, export, sales, use, excise, and value-added taxes) based on the transactions or payments under this Agreement, other than taxes imposed or based on HRG's net income. UPI may withhold from any payment to HRG due under this Agreement any taxes required to be withheld by UPI under the applicable laws of the United States or any other country, state, territory or jurisdiction.

3.5. Method of Payments. All payments made by UPI to HRG under this Agreement shall be made in United States dollars, and such payments shall be made by check or wire transfer to one or more bank accounts to be designated in writing by HRG.

4. INTELLECTUAL PROPERTY

4.1. Sole Inventions. All inventions, improvements and discoveries, together with intellectual property rights therein, that are conceived or reduced to practice during the Term solely by employees, consultants or Third Party contractors of a party, or an Affiliate of a party, pursuant to its activities under this Agreement (not including any activities under the Research Collaboration Agreement, the allocation of inventions made under which are subject to the terms and conditions set forth therein) shall be the property of such party (the "**Sole Inventions**"). For the avoidance of doubt, UPI will own all clinical data, and intellectual property rights in, to, and under such data, relating to treatment of HIBM and related conditions developed or acquired by or on behalf of UPI under this Agreement. HRG's interest in any Sole Inventions are included within the Licensed Intellectual Property and subject to the exclusive license granted to UPI hereunder.

4.2. Joint Inventions. All inventions, improvements and discoveries, together with intellectual property rights therein, that are conceived or reduced to practice during the Term jointly by at least one employee, consultant, or Third Party contractor of each party, or an Affiliate of a party, pursuant to its activities under this Agreement (not including any activities under the Research Collaboration Agreement, the allocation of inventions made under which are subject to the terms and conditions set forth therein) (the "**Joint Inventions**"), shall be jointly owned by both parties, with each party having the undivided half-interest in such Joint Inventions and having the right to exploit such Joint Inventions without the duty of accounting or seeking consent from the other party, except that HRG's interest in any Joint Inventions are included within the Licensed Intellectual Property and subject to the exclusive license granted to UPI hereunder.

5. PATENT MAINTENANCE AND PROSECUTION

5.1. Licensed Patents. UPI shall have the right, but not the obligation, to apply for, prosecute and maintain during the term of this Agreement, the Licensed Patents and any Patent Rights claiming Joint Inventions (the "**Joint Patents**"), at its cost and expense. HRG shall provide UPI with all information necessary or useful for the filing and prosecution of such Licensed Intellectual Property and shall cooperate fully with UPI so that UPI may establish and maintain such rights. Patent attorneys chosen by UPI shall handle all patent filings and prosecutions, on behalf of UPI, provided, however, that UPI shall provide HRG with written updates of such filing, prosecution, and maintenance efforts. In the event UPI declines to apply for, prosecute or maintain any Licensed Patents ("**Abandoned Licensed Patents**"), HRG shall have the right to pursue such Abandoned Licensed Patents at HRG's expense and UPI shall have no further rights under such Abandoned Licensed Patents. If UPI decides not to apply for, prosecute or maintain any Licensed Patents, UPI shall give sufficient and timely notice to HRG so as to permit HRG to apply for, prosecute and maintain such Abandoned Licensed Patents. In such event, UPI shall provide HRG with all information necessary or useful for the filing and prosecution of such Abandoned Licensed Patents and shall cooperate fully with HRG so that HRG may establish and maintain such rights.

5.2. Non-Prosecution by HRG. Except as expressly permitted under Section 5.1, HRG shall not have the right to file, prosecute and/or maintain any Licensed Patents or Joint Patents without the prior written consent of UPI, and HRG's non-conformance to this Section 5.2 shall be deemed a material breach of this Agreement.

5.3. Disclosure. HRG shall notify UPI in writing of any and all Sole Invention and/or Joint Invention promptly after its conception, development or reduction to practice. If UPI is unable, after reasonable inquiry, to obtain HRG's (or its employee's or agent's) signature on a document necessary for UPI to obtain ownership and to apply for, secure, and maintain patent or other proprietary protection of such UPI Sole Inventions and Joint Inventions, then HRG hereby appoints UPI as its attorney-in-fact to sign such necessary documents.

6. PATENT ENFORCEMENT

6.1. Infringement Actions. If either party hereto becomes aware that any Licensed Intellectual Property is being or has been infringed by any Third Party in the Field, such party shall promptly notify the other party hereto in writing describing the facts relating thereto in reasonable detail. UPI shall have the sole and exclusive right, but not the obligation, as to HRG to institute, prosecute and control any action, suit or proceeding (an "Action") with respect to such infringement in the Field, including any declaratory judgment action, at its expense, using counsel of its choice and HRG shall cooperate reasonably with UPI, at UPI's request, in connection with any such Action. Any amounts recovered in such Action shall be used first to reimburse UPI and then HRG, to the extent such costs and expenses have not already been reimbursed, for their costs and expenses reasonably incurred in connection with such Action (including attorneys and expert fees) and any remainder relating to infringement in the Field shall be retained by UPI.

6.2. Cooperation. In any Action, HRG shall provide UPI with reasonable cooperation and assistance, including agreeing to be named as a party to such Action, and, upon the request and at the expense of UPI, HRG shall make available, at reasonable times and under appropriate conditions, all relevant personnel, records, papers, information, samples, specimens, and the like in its possession. Notwithstanding any other provision of this Article 6, neither party shall make any settlements of any suit, proceeding or action relating to an infringement of any Licensed Intellectual Property in the Field that would materially and adversely affect the other party or the rights and licenses granted hereunder without first obtaining such other party's prior written consent, such consent not to be unreasonably withheld, conditioned or delayed.

7. REPRESENTATIONS, WARRANTIES AND COVENANTS

7.1. Representations, Warranties and Covenants of HRG. HRG represents and warrants that, as of the Effective Date:

(a) HRG is a nonprofit laboratory, duly organized, validly existing and in good standing under the laws of its state of incorporation;

(1) HRG has the right and authority to grant the rights and licenses granted to UPI under this Agreement;

(2) HRG is not aware of any facts or circumstances that would cause the Licensed Intellectual Property in the Territory to be invalid or unenforceable, and all necessary fees and other actions required in order to maintain the Licensed Intellectual Property have been paid or performed to date;

(3) HRG has not granted any right, license or interest in, to or under the Licensed Intellectual Property and HRG shall not grant during the term of this Agreement any right, license or interest in, to or under the Licensed Intellectual Property that is inconsistent with the rights, licenses and interests granted to UPI hereunder;

(4) HRG has the right to grant to UPI the license hereunder, including the right to grant to UPI the license under the Licensed Patents set forth on Exhibit A and to transfer to UPI the Licensed Know-How set forth on Exhibit B;

(5) HRG has not received any notification of, and is not aware of any threat of, any alleged infringement of any Patent Rights or misappropriation of Know-How of any Third Party in connection with the practice of the Licensed Intellectual Property; and

(6) The execution, delivery and performance of this Agreement have been duly authorized by all necessary corporate action on the part of HRG.

7.2. Representations, Warranties and Covenants of UPI.

7.2.1. UPI represents, warrants and covenants that, as of the Effective Date:

(a) UPI is a corporation, duly organized validly existing and in good standing under the laws of its state of incorporation; and

(b) The execution, delivery and performance of this Agreement has been duly authorized by all necessary corporate action on the part of UPI.

7.2.2. UPI agrees to use Commercially Reasonable Efforts (either by itself of through its Affiliates or sublicensees) to develop and commercialize at least one product that is directly related to substrate replacement therapy for the treatment of HIBM. UPI may conduct such activities itself or through Third Parties.

7.3. Disclaimer. EXCEPT AS EXPRESSLY PROVIDED FOR IN THIS AGREEMENT, NEITHER PARTY MAKES, AND EACH PARTY HEREBY DISCLAIMS, ANY AND ALL REPRESENTATIONS AND WARRANTIES OF ANY KIND, EXPRESS OR IMPLIED, WITH RESPECT TO THE SUBJECT MATTER OF THIS AGREEMENT, INCLUDING WITHOUT LIMITATION, WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE AND NON-INFRINGEMENT AND ANY WARRANTY ARISING OUT OF PRIOR COURSE OF DEALING AND USAGE OF TRADE.

8. INDEMNIFICATION

8.1. HRG Indemnity. HRG shall defend, at its expense, UPI and its directors, officers, employees, agents and consultants (each a "UPI Indemnitee"), against all third party claims, demands, damages, liabilities, losses, costs and expenses, including without limitation attorney's fees (collectively, "Liabilities"), which has resulted in a judgment by a court of competent jurisdiction against UPI after all time to appeal has expired, resulting from or arising out of any material breach by HRG of any of its representations, warranties or covenants hereunder, except to the extent such claim is caused by the gross negligence or willful misconduct of UPI.

8.2. UPI Indemnity. UPI shall indemnify, defend and hold harmless HRG and its directors, officers, employees, agents and consultants (each an "HRG Indemnitee") from and against any and all Liabilities resulting from or arising out of a claim, suit or proceeding brought by a third party against an Indemnitee for personal injury, death, product liability or property damage arising out of or related to its research, development, manufacture, use, or sale of a product arising under this Agreement, or resulting from or arising out of any material breach by UPI of any of its representations, warranties or covenants hereunder, in each case which has resulted in a judgment by a court of competent jurisdiction against UPI after all time to appeal has expired, except to the extent such claim is caused by the gross negligence or willful misconduct of HRG.

8.3. Procedure. For purposes of this Article 8, the indemnified party shall give prompt written notice to the indemnifying party of any claims, suits or proceedings by third parties which may give rise to any claim for which indemnification may be required under this Article 8; provided, however, that failure to give such notice shall not relieve the indemnifying party of its obligation to provide indemnification hereunder except, if and to the extent that such failure materially and adversely affects the ability of such indemnifying party to defend the applicable claim, suit or proceeding. The indemnifying party shall be entitled to assume the defense and control of any such claim at its own cost and expense; provided, however, that the indemnified party shall have the right to be represented by its own counsel at its own cost in such matters. Neither party shall settle or dispose of any such matter in any manner which would adversely affect the rights or interests of the other party (including the obligation to indemnify hereunder) without the prior written consent of the other party, which shall not be unreasonably withheld or delayed. Each party shall reasonably cooperate with the other party and its counsel in the course of the defense of any such suit, claim or demand, such cooperation to include without limitation using reasonable efforts to provide or make available documents, information and witnesses.

9. TERM AND TERMINATION

9.1. Term. Unless and until terminated at an earlier date in accordance with this Section 9, the term of this Agreement shall commence on the Effective Date and continue in full force and effect, on a product-by-product and country-by-country basis, until the expiration date of the last-to-expire patent within the Licensed Patents in such country that claims the composition of matter of, or the method of making or using such product, provided that, with respect to any country that is a Major Market Country, the term of this Agreement with respect to

such product in such Major Market Country shall be extended until, if later, the expiration date of orphan drug exclusivity in such Major Market Country ("Term"). After the expiration (but not early termination) of this Agreement, the license granted to UPI under Section 2.1 shall become irrevocable, perpetual, fully paid and royalty-free.

9.2. Termination by UPI. UPI shall have the right to terminate this Agreement, with or without cause, upon ninety (90) days prior written notice to HRG. In the event that UPI terminates its HIBM substrate replacement therapy program, and has no intention of transferring or conducting development of the Licensed Intellectual Property in the future, UPI shall give HRG written notice to HRG of such termination and this Agreement shall terminate upon ninety (90) days after the date of such notice. 9

9.3. Termination for Cause. If either party materially breaches any term or condition of this Agreement, the other party may notify the breaching party in writing of such breach, setting forth the nature of the breach in reasonable detail. If the breaching party fails to cure such breach within sixty (60) days after the receipt of the foregoing notice from the non-breaching party, then the non-breaching party may terminate this Agreement effective immediately upon a second written notice to the breaching party. Notwithstanding the foregoing, if the party receiving such notice of alleged breach disputes such allegation, the party providing such notification shall not have the right to terminate this Agreement until such dispute is resolved in such party's favor under Section 11.2 and the other party does not cure such breach within thirty (30) days after such determination.

9.4. Termination for Insolvency. Either party may terminate this Agreement immediately if the other party ceases conducting business in the normal course, becomes insolvent, makes a general assignment for the benefit of creditors, suffers or permits the appointment of a receiver for its business or assets, avails itself of or becomes subject to any petition or proceeding under any statute of any state or country relating to insolvency or the protection of the rights of creditors, or any other insolvency or bankruptcy proceeding or other similar proceeding for the settlement of the other party's debt is instituted.

9.5. Effect of Termination; Survival. Section 9.5 and Articles 1, 4, 10 and 11 shall survive expiration or termination of this Agreement. Notwithstanding the foregoing, expiration or termination of this Agreement shall not release either party from any obligation that has accrued prior to such expiration or termination, including without limitation any obligation to pay any amount which became due and payable under the terms and conditions of this Agreement prior to such expiration or termination.

10. CONFIDENTIAL INFORMATION

10.1. Confidentiality. In connection with this Agreement, the parties will provide to each other Confidential Information, including but not limited to each party's know-how, invention disclosures, proprietary materials and/or technologies, economic information, business or research strategies, trade secrets and material embodiments thereof. As used herein, "Confidential Information" means any information of a confidential or proprietary nature disclosed by a party ("Disclosing Party") to this Agreement to the other party ("Recipient") (a) in written form marked "confidential," or (b) in oral form if summarized in a writing marked "confidential" delivered to the receiving party within [***] days after the oral disclosure.

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

10.2. Confidentiality and Non-Use. The Recipient of the Disclosing Party's Confidential Information shall maintain such Confidential Information in confidence, and shall disclose such Confidential Information only to those of its employees, agents, consultants, sublicensees, attorneys, accountants and advisors who have a reasonable need to know such Confidential Information and who are bound by obligations of confidentiality and non-use no less restrictive then those set forth herein. The Recipient shall use such Confidential Information solely to exercise its rights and perform its obligations under this Agreement (including, without limitation, the right to use and disclose such Confidential Information in regulatory applications and filings), unless otherwise mutually agreed in writing. The Recipient shall take the same degree of care to protect the Disclosing Party's Confidential Information that it uses to protect its own confidential and proprietary information of a similar nature and importance (but in any event no less than reasonable care).

10.3. Exclusions. Confidential Information shall not include information that: (a) is in the Recipient's possession prior to receipt from the Disclosing Party as demonstrated by contemporaneous documentation; (b) is or becomes, through no fault of the Recipient, publicly known; (c) is furnished to the Recipient by a Third Party without breach of a duty to the Disclosing Party; (d) is independently developed by the Recipient without use of, application of or reference to the Disclosing Party's Confidential Information as demonstrated by contemporaneous documentation.

10.4. Legal Disclosures. It shall not be a violation of this Article 10 for the Recipient to disclose the Disclosing Party's Confidential Information as required to be disclosed under applicable law, but such disclosure shall be for the sole purpose of and solely to the extent required by such law, and provided that the Recipient, to the extent possible, shall give the Disclosing Party prior written notice of the proposed disclosure and cooperate fully with the Disclosing Party to minimize the scope of any such required disclosure, to the extent possible and in accordance with applicable law.

10.5. Non-Disclosure Agreement. The parties entered into a confidential disclosure agreement dated as of June 22, 2010 ("Confidential Disclosure Agreement"). Upon execution of this Agreement, the Confidential Disclosure Agreement shall be replaced and superseded by the confidentiality provisions set forth in this Agreement with respect to any information disclosed or used hereunder; provided, however, that such Confidential Disclosure Agreement shall remain in full force and effect, in accordance with its terms, with respect to information disclosed prior to the Effective Date and acts or omissions of the parties with respect thereto. For the avoidance of doubt, the terms and conditions of this Article 10 shall apply to information otherwise qualifying as Confidential Information (as defined in Section 10.1) that was already disclosed under the Confidential Disclosure Agreement prior to the Effective Date.

10.6. Publication. HRG shall not publish, present or disclose any data or results in the Field without the prior written consent of UPI, and any non-compliance by HRG under this Section 10.6 shall be deemed a material breach of this Agreement.

10.7. Termination. All obligations of confidentiality and non-use imposed under this Article 10 shall continue to exist indefinitely or until such time as the obligation to such Confidential Information ceases pursuant to Section 10.3, whichever is earlier.

10.8. Return of Confidential Information. Upon termination or expiration of the Agreement, or upon written request of the Disclosing Party, the Recipient shall promptly return to the Disclosing Party or destroy all documents, notes and other tangible materials representing the Disclosing Party's Confidential Information and all copies thereof, provided, however, that each Party may retain a single archival copy of the other Party's Confidential Information for the sole purpose of facilitating compliance with the surviving provisions of this Agreement.

10.9. Injunctive Relief. The parties expressly acknowledge and agree that any breach or threatened breach of this Section 10 may cause immediate and irreparable harm to the Disclosing Party that may not be adequately compensated by damages. Each Party therefore agrees that in the event of such breach or threatened breach and in addition to any remedies available at law, the Disclosing Party shall have the right to secure equitable and injunctive relief, without bond, in connection with such a breach or threatened breach.

11. MISCELLANEOUS

11.1. Governing Law. This Agreement shall be governed by, and construed and interpreted, in accordance with the internal laws of the State of California (as permitted by Section 1646.5 of the California Civil Code or any similar successor provision) without giving effect to any choice of law rule that would cause the application of the laws of any jurisdiction other than the internal laws of the State of California to the rights and duties of the parties.

11.2. Dispute Resolution. The Parties shall endeavor to resolve any disputes under this Agreement through good faith discussions. Either Party shall have the right to refer any such disputes to UPI's chief executive officer and HRG's Founder (Dr. Daniel Darvish) for resolution. If such executive officers of the Parties cannot resolve such dispute within a [***] day period, then either Party shall have the right to initiate binding arbitration under the rules of JAMS, with such arbitration to be conducted in San Francisco, California under one (1) arbitration chosen by the Parties jointly (or, if the Parties cannot agree on such arbitrator, chosen by JAMS).

11.3. Limitation of Liability. EXCEPT WITH RESPECT TO EACH PARTY'S INDEMNITY OBLIGATIONS UNDER ARTICLE 8 AND CONFIDENTIALITY OBLIGATIONS UNDER ARTICLE 10, IN NO EVENT SHALL EITHER PARTY BE LIABLE FOR INCIDENTAL, CONSEQUENTIAL, INDIRECT, PUNITIVE OR SPECIAL DAMAGES OF THE OTHER PARTY ARISING OUT OF OR RELATED TO THIS AGREEMENT, HOWEVER CAUSED, UNDER ANY THEORY OF LIABILITY EVEN IF ADVISED OF THE POSSIBILITY OF SUCH DAMAGES.

11.4. Force Majeure. Neither party shall be held responsible for any delay or failure in performance (with the exception of the payment of money) hereunder to the extent caused by strikes, embargoes, unexpected government requirements, civil or military authorities, acts of God, earthquake, or by the public enemy or other causes reasonably beyond such party's control and without such party's fault or negligence; provided that the affected party notifies the unaffected party as soon as reasonably possible, and resumes performance hereunder as soon as reasonably possible following cessation of such force majeure event.

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

11.5. Independent Contractors. The relationship of HRG and UPI established by this Agreement is that of independent contractors. Nothing in this Agreement shall be constructed to create any other relationship between HRG and UPI. Neither party shall have any right, power or authority to bind the other or assume, create or incur any expense, liability or obligation, express or implied, on behalf of the other.

11.6. Assignment. Neither Party may assign this Agreement without the prior written consent of the other Party, provided that, either Party may transfer or assign its rights and obligations under this Agreement, without consent, to an Affiliate or to a successor to all or substantially all of its business or assets relating to this Agreement whether by sale, merger, operation of law or otherwise. Any assignment not in conformance with this Section 11.6 shall be null, void and of no legal effect.

11.7. Notices. Unless otherwise stated, any notice, report, communication or consent required or permitted by this Agreement shall be in writing and shall be sent (a) by prepaid registered or certified mail, return receipt requested, (b) by overnight express delivery service by a nationally recognized courier, or (c) via confirmed facsimile or telecopy, followed within five (5) days by a copy mailed in the preceding manner, addressed to the other party at the address shown below or at such other address for which such party gives notice hereunder. Such notice will be deemed to have been given when delivered or, if delivery is not accomplished by some fault of the addressee, when tendered.

If to HRG:
HIBM Research Group
Attn: Dr. Daniel Darvish
18341 Sherman Way, #201A
Reseda, CA 91335

If to UPI:
Ultragenyx Pharmaceuticals, Inc.
Attn: Dr. Emil D. Kakkis
60 Leveroni Ct
Novato, California 94949

11.8. Modification; Waiver. This Agreement may not be altered, amended or modified in any way except by a writing signed by the parties. The failure of a party to enforce any rights or provisions of the Agreement shall not be construed to be a waiver of such rights or provisions, or a waiver by such party to thereafter enforce such rights or provision or any other rights or provisions hereunder. No waiver shall be effective unless made in writing and signed by the waiving party.

11.9. Construction. Section headings are included in this Agreement merely for convenience of reference; they are not to be considered part of this Agreement or used in the interpretation of this Agreement. No rule of strict construction will be applied in the interpretation or construction of this Agreement.

11.10. Severability. If any provision of any provision of this Agreement shall be found by a court to be void, invalid or unenforceable, the same shall be reformed to comply with applicable law or stricken if not so conformable, so as not to affect the validity or enforceability of this Agreement; provided that no such reformation or striking shall be effective if the result materially changes the economic benefit of this Agreement to either HRG or UPI. In the event that any provision of this Agreement becomes or is declared by a court of competent jurisdiction to be void, invalid or unenforceable, and reformation or striking of such provision would materially change the economic benefit of this Agreement to either HRG or UPI, HRG and UPI shall modify such provision to obtain a legal, valid and enforceable provision and provide an economic benefit to HRG and UPI, that most nearly effects HRG's and UPI intent on entering into this Agreement.

11.11. Entire Agreement. The parties hereto acknowledge that this Agreement, together with the exhibits attached hereto, set forth the entire agreement and understanding of the parties as to the subject matter hereto, and supersedes all prior and contemporaneous discussions, agreements and writings in respect hereto.

11.12. Headings. The article, section and paragraph headings contained herein are for the purposes of convenience only and are not intended to define or limit the contents of the articles, sections or paragraphs to which such headings apply.

11.13. Public Announcements. Neither UPI nor HRG shall make any public announcement concerning the existence of or the terms of this Agreement, without the prior written approval of the other party with regard to the form, content and precise timing of such announcement, except such as may be required to be made by either party to comply with applicable law, regulations, court order, or tax or securities filings. Such consent shall not be unreasonably withheld or delayed by such other party. Prior to any such public announcement, the party wishing to make the announcement will submit a draft of the proposed announcement to the other party in sufficient time to enable the other party to consider and comment thereon.

11.14. Counterparts. This Agreement may be executed in two or more counterparts, each of which shall be deemed an original and all of which together shall constitute one instrument.

[Signature page follows]

IN WITNESS WHEREOF, HRG and UPI have executed this Agreement by their respective duly authorized representatives.

HIBM RESEARCH GROUP

By: /s/ Daniel Darvish
Name: Dr. Daniel Darvish
Title: Founder HRG

ULTRAGENYX PHARMACEUTICAL, INC.

By: /s/ Emil Kakkis
Name: Emil Kakkis
Title: CEO

EXHIBIT A

List of Licensed Patents

1. U.S. Patent Application Serial No. [***]
   Title: [***]
   Filing Date: [***]

2. U.S. Provisional Application Serial No. [***]
   Title: [***]
   Filing Date: [***]

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

Exhibit 10.6

COLLABORATION AND LICENSE AGREEMENT

BETWEEN

NOBELPHARMA CO., LTD.

AND

ULTRAGENYX PHARMACEUTICALS, INC

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

COLLABORATION AND LICENSE AGREEMENT

This COLLABORATION AND LICENSE AGREEMENT (the "Agreement"), effective as of September 30, 2010 (the "Effective Date"), is made by and between Nobelpharma Co., Ltd., a Japanese corporation, having a principal place of business at Kyodo Bldg. (Horidome), 12-10 Nihonbashi-kobunacho, Chuo-Ku, Tokyo, Japan ("NPC"), and Ultragenyx Pharmaceuticals, Inc., a California corporation, having offices at 77 Digital Drive, Suite 210, Novato, CA 94949, U.S.A. ("UPI") (each a "Party," together the "Parties").

BACKGROUND

WHEREAS, NPC owns or controls certain patent and know-how rights with respect to the Compound (as defined below);

WHEREAS, NPC desires to collaborate with UPI on the research, development and commercialization of the Compound;

WHEREAS, UPI desires to obtain from NPC the licenses set forth herein, and NPC desires for the reasons described above to grant such licenses to UPI, all on the terms and conditions set forth in this Agreement

NOW, THEREFORE, for and in consideration of the covenants, conditions and undertakings hereinafter set forth, it is agreed by and between the Parties as follows:

ARTICLE 1

DEFINITIONS

As used herein, the following terms will have the meanings set forth below:

1.1 "AAA" means the American Arbitration Association.

1.2 “Affiliate” of a Party means any corporation or other business entity which during the Term of this Agreement controls, is controlled by or is under common control with such Party but only for so long as such entity controls, is controlled by, or is under common control with such Party. For the purposes of this definition, with respect to a particular entity “control” means the ownership directly or indirectly of fifty percent (50%) or more of the stock entitled to vote for the election of directors, and for non-stock organizations, of the equity interests entitled to control the management of such entity.

1.3 “Business Day” means a day other than Saturday, Sunday or any day on which the Bank of Japan or commercial banks located in New York, New York are authorized or obligated by applicable Laws to close.

1.4 “Commercialization” means activities directed to obtaining pricing and reimbursement approvals, marketing, promoting, distributing, importing or selling a Product. “Commercialize” means to engage in Commercialization.

1.5 “Commercially Reasonable Efforts” means efforts and resources normally used by a similarly situated therapeutic pharmaceutical company for a product owned by it or to which it has exclusive rights, which is of similar market potential at a similar stage in its development or product life, taking into account issues of safety and efficacy, product profile, the competitiveness of the marketplace, the proprietary position of the compound or product, the regulatory and reimbursement structure involved, the profitability of all applicable products, and other relevant commercial, scientific and other factors.

1.6 “Common Stock” means the common stock of UPI.

1.7 “Compound” means the compound identified as N-acetylneuraminic acid.

1.8 “Confidential Information” of a Party means any data and information of a confidential and proprietary nature (including, but not limited to, trade secrets, know-how, technical and business information, patent information, structures, models, techniques, formula, processes, compositions, compounds, apparatus, specifications, samples and inventions) of such Party previously received or to be received (regardless of whether orally, in writing, by e-mail or any other means) by the other Party in connection with the negotiation, execution or performance of this Agreement, but excludes any information: (i) that is already publicly known when this Agreement is executed or it is received by the other Party; or (ii) that becomes publicly known after the Effective Date without any fault of the other Party.

1.9 “Controlled” or “Controls” means the legal authority or right of a Party hereto (or any of its Affiliates), when used in reference to intellectual property, to grant a license or sublicense of intellectual property rights to another Party, or otherwise to disclose proprietary or trade secret information to such other Party, without breaching the terms of any agreement with a Third Party, or misappropriating the proprietary or trade secret information of a Third Party.

1.10 “Covered” or “Covering” means, with respect to a particular Valid Claim and a Product, that the manufacture, use, sale, offer for sale or importation of such Product, but for the licenses granted herein, would infringe such Valid Claim.

1.11 “Development” means non-clinical and clinical drug development activities related to the development and submission of information to a Regulatory Authority, including, without limitation, toxicology, pharmacology and other discovery and pre-clinical efforts, test method development and stability testing, formulation development, delivery system development, quality assurance and quality control development, statistical analysis, and clinical studies (including, without limitation, pre- and post-approval studies). Development specifically excludes regulatory activities directed to obtaining pricing and reimbursement approvals and all other Commercialization activities. “Develop” means to engage in Development.

1.12 “Development Plan” means the development plan to be prepared jointly by the Parties as soon as reasonably practicable after the execution of this Agreement and as may be revised from time to time in accordance with this Agreement. The Development Plan will set forth the Development activities, including the anticipated costs, on a summary basis. The Parties agree that there does not have to be absolute consensus between them with respect to the activities contained in the Development Plan. Provided that where there is a disagreement between the Parties or a consensus on a Development activity in the Development Plan cannot be reached, then the Development Plan will note such disagreement.

1.13 “Drug Substance” means a quantity of the Compound in bulk form.

1.14 “EMEA” means the European Medicines Agency, or any successor agency thereto.

1.15 “EU” means the European Union, which as of the Effective Date consists of [***]. In the event that the European Union adds one or more new member nations during the Term of this Agreement, the Parties will discuss in good faith whether to amend the definition of “EU” to include such nation(s).

1.16 “Europe” means the [***].

1.17 “FDA” means the United States Food and Drug Administration and any successor thereto having substantially the same functions.

1.18 “First Commercial Sale” means, with respect to a particular Product and Territory, the first bona fide commercial sale of such Product following Regulatory Approval to market such Product to a Third Party in such Territory by or under authority of a Party, its Affiliates or Sublicensees.

1.19 “First UPI Approval” means the first Regulatory Approval for a Product in the United States or Europe, whichever is the earlier.

1.20 “GAAP” means United States Generally Accepted Accounting Principles (as consistently applied by the applicable Party and its Affiliates).

1.21 “GMP” means the then-current good manufacturing practices required by: (a) the provisions of 21 C.F.R., parts 210 and 211 and all applicable rules, regulations, orders and guidances (as the same may from time to time be amended); (b) ICH, Guidance for Industry Q7a Good Manufacturing Practice Guidance for Active Pharmaceutical Ingredients (as the same may from time to time be amended); (c) the provisions of Chapter II of EC Commission Directive 91\356\EEC together with the Guide to Good Manufacturing Practice published by the EC Commission in 1992 (ISBN 92-826-3180-X) (as the same may from time to time be amended); and (d) any other applicable Laws, guidelines, regulations and industry standards, that apply to any manufacturing or processing activities hereunder, or the facilities in which any such activities are performed.

1.22 “IND” means an Investigational New Drug Application (as defined in the United States Food, Drug and Cosmetic Act, as amended, and the regulations promulgated thereunder), or any corresponding application, registration or certification with a Regulatory Body in any jurisdiction.

1.23 “Invention” shall mean any process, method, composition of matter, article of manufacture, discovery or finding, whether or not patentable.

1.24 “JCAA” means the Japan Commercial Arbitration Association.

1.25 “JHSF” means the Japan Health Sciences Foundation.

1.26 “Joint Know-How” shall mean any Know-How, resulting from the Agreement, obtained, developed or invented jointly by at least one employee of NPC or others acting on behalf of NPC and at least one employee of UPI or others acting on behalf of UPI, during the course of Development activities under the Agreement, where inventorship of an Invention, whether patentable or not, is to be determined in accordance with the patent laws of the United States.

1.27 “Joint Patent Rights” shall mean all Patent Rights that claim Joint Know-How.

1.28 “Know-How” means any protocols, formulas, data, Inventions, methods, proprietary information, processes, techniques, technology, materials (including biological or other materials) and trade secrets, patentable or otherwise, and any intellectual property rights (other than Patent Rights) therein.

1.29 “Korosho” means the Japanese Ministry of Health, Labour and Welfare, or any successor agency thereto.

1.30 “Laws” means all laws, statutes, rules, regulations, ordinances and other pronouncements having the effect of law of any federal, national, multinational, state, provincial, county, city or other political subdivision, agency or other body, domestic or foreign.

1.31 “Manufacture” and “Manufacturing” mean, with respect to a product or compound, the manufacturing, processing, formulating, packaging, labeling, storing and quality control testing of such product or compound.

1.32 “Net Sales” means the actual amounts invoiced for Products sold by a Person and its Affiliates and Sublicensees to a Third Party (excluding any sales among such Person and its Affiliates or Sublicensees where the Affiliate or Sublicensee is not itself the user of the Product) less the following amounts related to the Products: (a) credits, allowances, discounts, rebates, and chargebacks for spoiled, damaged, outdated, rejected, and returned Products, (b) freight and insurance costs incurred with respect to the shipment of the Products to customers, (c) duties, surcharges and other governmental charges, (d) sales, use, value-added, excise and other similar taxes (excluding income taxes), (e) cash, quantity, trade and similar discounts, rebates, allowances and other price reductions actually granted or paid by a Person and its Affiliates to the extent that such reductions relate to sales of the Products, and (f) actual uncollectible amounts. If a sale, transfer or other disposition with respect to a product is made for consideration other than cash or is not at arm’s length, then the Net Sales from such sale, transfer or other disposition shall be the arm’s length fair market value thereof. For purposes of this Agreement, “sale” means any transfer or other distribution or disposition, but shall not include

transfers or other distributions or dispositions of product, at no charge, for pre-clinical, clinical or regulatory purposes or in connection with patient assistance programs or other charitable purposes or to physicians or hospitals for promotional purposes. In the event that a Product is sold that includes more than one active ingredient, Net Sales for purposes of determining payments under this Agreement shall be limited to the portion of the Net Sales (determined in accordance with the preceding paragraph) allocated to the Compound rather than the other active ingredient(s), as determined by good faith negotiations between the Parties.

1.33 "NDA" means a New Drug Application (as defined in the United States Food, Drug and Cosmetic Act, as amended, and the regulations promulgated thereunder), or any corresponding application, registration or certification for Regulatory Approval of a Product with a Regulatory Authority in any jurisdiction.

1.34 "[***] Technology" means the technology developed based on studies conducted by [***] related to the [***].

1.35 "North America" means [***].

1.36 "NPC Know-How" means all Know-How that is (i) necessary or useful for the Development and/or Commercialization of the Compound and/or Products; and (ii) Controlled by NPC or its Affiliates as of the Effective Date or during the Term of this Agreement, excluding Joint Know-How.

1.37 "NPC Patent Rights" means any Patent Rights that are (i) necessary or useful for the Development and/or Commercialization of the Compound and/or Products; and (ii) Controlled by NPC or its Affiliates during the Term of this Agreement, excluding Joint Patent Rights.

1.38 “NPC Territory” means [***].

1.39 “Patent Rights” means any patents, patent applications, certificates of invention, or applications for certificates of invention and any supplemental protection certificates, together with any extensions, registrations, confirmations, reissues, substitutions, divisions, continuations or continuations-in-part, reexaminations or renewals thereof, and any foreign counterparts to any of the foregoing.

1.40 “Person” means any individual, firm, corporation, partnership, limited liability company, trust, business trust, joint venture company, governmental authority, association or other entity.

1.41 “Phase II Trials” means human clinical trials, the principal purpose of which is to evaluate both clinical efficacy and safety of an investigational product, and/or to obtain a preliminary evaluation of the dosage regimen of an investigational product, as more fully defined in 21 C.F.R. §312.21(b) or similar clinical study in a country other than the United States.

1.42 “Phase III Trials” means human clinical trials, the principal purpose of which is to establish substantial evidence of both safety and efficacy in patients with the disease or condition being studied, as more fully defined in 21 C.F.R. §312.21(c) or similar clinical study in a country other than the United States. Phase III Trials shall also include any other human clinical trial intended to serve as a pivotal trial to support the submission of an application for Regulatory Approval.

1.43 “Product” means a pharmaceutical preparation which incorporates the Compound as an active drug substance.

1.44 “Regulatory Approval” means approval of the Regulatory Authority in a country necessary for the marketing and sale of a Product in the applicable country. As used herein, “Regulatory Approval” shall not include pricing or reimbursement approval.

1.45 “Regulatory Authority” means the FDA, Korosho, Pharmaceuticals and Medical Devices Agency of Japan and any or all equivalent governmental or administrative authorities outside the United States or Japan whose approval is required for manufacture, marketing, promotion, sale or distribution of the Products.

1.46 “Sublicensee” means a Third Party expressly licensed by a Party to make, use, import, offer for sale or sell Product. The term “Sublicensee” shall not include distributors (i.e. a Third Party who purchases product from a Party for resale).

1.47 “Territory” means the NPC Territory or the UPI Territory, as the case may be.

1.48 “Third Party” means any Person or entity other than NPC and UPI, and their respective Affiliates.

1.49 “Trademark” means any word, name, symbol, color, designation or device or any combination thereof, including, without limitation, any trademark, trade dress, brand mark, house mark, trade name, brand name, logo, or business symbol (whether or not registered or registerable) used or displayed with respect to any Product.

1.50 "UPI Know-How" means all Know-How that is (i) necessary or useful for the Development and/or Commercialization of the Compound and/or Products; and (ii) Controlled by UPI or its Affiliates as of the Effective Date or during the Term of this Agreement, excluding Joint Know-How.

1.51 "UPI Patent Rights" means any Patent Rights that are (i) necessary or useful for the Development and/or Commercialization of the Compound and/or Products; and (ii) Controlled by UPI or its Affiliates during the Term of this Agreement, excluding Joint Patent Rights.

1.52 "UPI Territory" means [***].

1.53 "Valid Claim" means (i) a claim of an issued and unexpired patent (or the equivalent in a supplementary protection certificate), which has not lapsed or become abandoned or been declared invalid or unenforceable by a court of competent jurisdiction or an administrative agency from which no appeal can be or is taken or (ii) a claim of a pending patent application, filed in good faith, which claim shall not have been canceled, withdrawn, abandoned or rejected by an administrative agency from which no appeal can be taken; *provided* that no more than five (5) years has passed since the filing date for such patent application.

1.54 Additional Terms. In addition to the foregoing, the following terms shall have the meaning defined in the corresponding Section below:

Definition	Section Where Defined
Abandonment	9.3.3
Business Year	8.1.2
Competitive Product	5.4
Indemnification Claim	12.3
Indemnitee	12.3
Indemnitor	12.3
Infringement	9.5
Intellectual Properties	9.3.1
JDCC	2.1
JHSF License Agreement	3.1.1
JHSF Royalty	7.6.2
Losses and Claims	12.1
Supply Agreement	6.1
Term	13.1

ARTICLE 2

JOINT DEVELOPMENT AND COMMERCIALIZATION COMMITTEE

2.1 Joint Development and Commercialization Committee. The Parties agree to establish a joint Development and Commercialization committee ("JDCC"), which shall consist of no fewer than two (2) permanent members, with the JDCC having equal representation by each Party on the JDCC. Each Party may replace any or all of its representatives on the JDCC at any time upon written notice to the other Party. Such representatives shall include individuals within or designated by the senior management of each Party. JDCC representatives of each Party shall, individually or collectively, have expertise and/or responsibility in business to provide oversight and management of Development and Commercialization activities undertaken under this Agreement. Any member of the JDCC may designate a substitute to attend and perform the functions of that member at any meeting of the JDCC, and each member of the JDCC may invite such other non-members (subject to the confidentiality provisions set forth in Article 10) as deemed necessary to any meeting of the JDCC as nonvoting observers to help explore and resolve the issues before the JDCC.

2.1.1 Meetings. The JDCC shall meet [***] annually during the Term or more frequently as the Parties agree may be necessary or appropriate, or at such frequency as agreed by the respective committee members. Meetings of the JDCC shall alternate between the facilities of UPI in the United States (or such other location as specified by UPI), and the facilities of NPC in Japan (or such other location as specified by NPC), with the first such meeting to take place in Tokyo, Japan, provided that such meetings may also be held by video conference upon either Party's reasonable request. The JDCC members will otherwise communicate regularly by telephone, electronic mail, facsimile and/or video conference.

2.1.2 Responsibility; Decision Making. The JDCC shall perform the following functions: (i) exchange information concerning the overall strategy and timelines for the Development Plan; (ii) review and evaluate data and progress of the activities under the Development Plan; (iii) resolve disputes or disagreements between the Parties with respect to the Development Plan; (iv) ensure open communication between the Parties as relates to the Development Plan, including making arrangements for a third party service provider to provide for a secure electronic data room to share data, documentation, information and materials generated for or used in the research, development, production or other exploitation of the Compound and Products; (v) make amendments to the Development Plan then in effect; and (vi) taking such other actions as are specifically allocated to the JDCC under this Agreement. Any approval, determination or other action of the JDCC shall require agreement of the members of the JDCC, with each Party having one (1) vote. Action that may be taken at a meeting of the

JDCC also may be taken without a meeting if a written consent setting forth the action so taken is signed by all members of the JDCC. In the event the JDCC is unable to reach consensus on a particular matter within its jurisdiction, the matter shall be referred to executives of the Parties in accordance with Section 14.1, and if such referral does not resolve such matter, then the dispute shall be resolved via arbitration in accordance with Section 14.2.

2.2 Reports; Records; Transfer of Data and Documentation. Each Party shall cooperate fully with the other Party and shall provide the other Party with all data, documentation, information and materials generated or used by the Party in the research, development, production or other exploitation of the Compound and Products, in accordance with this Section 2.2. Each Party shall, following the end of [***] of each calendar year, report to the other Party in reasonable detail at the first meeting of the JDCC to be held after such [***] its Development and Commercialization activities in such Party's Territory (other than those under the Development Plan) during the [***] period until such [***]. Each Party agrees to provide the other Party with any preclinical data obtained during the course of each study specified in the Development Plan and any clinical data obtained during the course of its Development activities within a reasonable time period after the completion of each such study, to the extent a Party has the right to so provide such data. Any such data so provided shall be deemed the Confidential Information of both Parties, and each Party shall be authorized to use such data solely for the Development and Commercialization of Products in their respective Territory or as otherwise permitted under this Agreement.

ARTICLE 3

DEVELOPMENT AND COMMERCIALIZATION IN THE NPC TERRITORY

3.1 Development and Commercialization of Products. NPC shall have responsibility for Development and Commercialization of Products in the NPC Territory at its sole discretion and expense in accordance with this Article 3.

3.1.1 Commercially Reasonable Efforts. NPC shall use Commercially Reasonable Efforts (by itself or through contract service organizations or other permitted Sublicensees) to Develop and Commercialize the Product in the NPC Territory. Without limiting the foregoing, NPC shall use Commercially Reasonable Efforts to initiate, as defined in this Section 3.1.1, such clinical trials as maybe required by the relevant Regulatory Authority for a Product in Japan within [***] after the Effective Date, subject to no intervening action by such Regulatory Authority, *provided* that Nishino Technology is validly and effectively licensed to NPC under a license agreement with the JHSF (the "JHSF License Agreement") within a reasonable period of time after the Effective Date. In this Section 3.1.1, "initiate" shall mean the filing of an IND by NPC (including, but not limited to, an IND of investigator initiated trial sponsored by NPC) or, if applicable, such filing by referencing any existing IND of UPI.

3.1.2 Responsibilities. NPC shall be responsible for the strategy, plans and budgets for marketing and promotion of Products in the NPC Territory, including without limitation establishing and managing marketing, promotion, and distribution capabilities. NPC shall file and be the owner of all regulatory filings in the NPC Territory for Products, including all NDAs and Regulatory Approvals, unless otherwise agreed by the Parties.

3.2 Initial Document and Data Transfer. During the [***] period following the Effective Date NPC shall provide UPI with one (1) electronic or paper copy in English or Japanese of all documents, data or other information Controlled by NPC as of the Effective Date, to the extent (x) that such documents, data and information are (i) subject to the NPC Know-How license under Section 5.1; and (ii) reasonably necessary for UPI to obtain the Regulatory Approval of Products in the UPI Territory and (y) that NPC has the right to so provide. Such documentation shall not be used by UPI for any purpose other than Development, Manufacture and Commercialization of the Compound and Products in the UPI Territory in accordance with this Agreement. NPC shall be responsible for the cost of providing one (1) set of copies only. Any and all such materials delivered to UPI pursuant to this Section 3.2 are and shall remain the property of NPC, *provided* that all such material shall be treated as being Confidential Information of both Parties. The Parties agree that, consistent with the exclusive license granted under Section 5.1, during the term in which such license is in effect: (i) NPC shall not undertake any further Development activities in the UPI Territory; and (ii) NPC shall not engage or correspond with Regulatory Authorities in the UPI Territory without prior written approval of UPI.

3.3 Technical Assistance. Upon reasonable request by UPI, NPC shall reasonably cooperate with UPI to provide technical assistance with respect to Development of Products in the UPI Territory. Such cooperation shall include providing UPI with reasonable access by teleconference or in person at NPC's corporate and manufacturing facilities (subject to NPC's customary rules and restrictions with respect to site visits by non-NPC personnel) to NPC personnel involved in the research, manufacturing and development of the Compound and Products. Upon UPI's request, NPC may in its discretion dispatch NPC personnel to UPI in order to support Development of Products by UPI in the UPI Territory. All expenses for such dispatch including traveling and lodging expenses shall be equally shared by the Parties unless otherwise agreed between the Parties.

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

3.4 Trademarks. NPC shall have the right to select, own, maintain and enforce Trademarks for Products in the NPC Territory. In the event that UPI desires to use for Products in the UPI Territory one or more Product-specific Trademarks that are used in the NPC Territory and owned or Controlled by NPC, NPC shall, upon UPI's reasonable request, grant to UPI a royalty-free exclusive license, with the right to grant and authorize sublicenses, to use, display and enforce such Trademark(s) in connection with Products in the UPI Territory, *provided* that UPI will pay all registration and maintenance costs for such Trademarks and will agree to comply with the terms of a reasonable trademark policy provided by NPC to UPI in writing.

ARTICLE 4

DEVELOPMENT AND COMMERCIALIZATION IN THE UPI TERRITORY

4.1 Development and Commercialization of Products. UPI shall have responsibility for Development and Commercialization of Products in the UPI Territory at its sole discretion and expense in accordance with this Article 4.

4.1.1 Commercially Reasonable Efforts. UPI shall use Commercially Reasonable Efforts (by itself or through contract service organizations or other permitted Sublicensees) to Develop and Commercialize Products in the UPI Territory. Without limiting the foregoing, UPI shall use Commercially Reasonable Efforts to initiate, as defined in this Section 4.1.1, such clinical trials as maybe required by FDA (or EMEA) for a Product in North

America (or a major country in Europe) within [***] after the Effective Date, subject to no intervening FDA (or EMEA) action; provided that all documents, filings and other information reasonably necessary for UPI to achieve such deadline and Controlled by NPC shall be provided by NPC in accordance with terms of this agreement. In this Section 4.1.1, "initiate" means the filing of a separate IND by UPI or, if applicable, such filing by referencing any existing IND of NPC.

4.1.2 Responsibilities. UPI shall be responsible for the establishment and implementation of the strategy, plans and budgets for marketing and promotion of Products in the UPI Territory, including without limitation establishing and managing marketing, promotion, and distribution capabilities. UPI shall file and be the owner of all regulatory filings in the UPI Territory for Products, including all NDAs and Regulatory Approvals, unless otherwise agreed by the Parties. UPI agree that, consistent with the exclusive license granted under Section 5.2, during the term in which such license is in effect: (i) UPI shall not undertake any further Development activities in the NPC Territory; and (ii) UPI shall not engage or correspond with Regulatory Authorities in the NPC Territory without prior written approval of NPC.

4.2 Technical Assistance. Upon reasonable request by NPC, UPI shall reasonably cooperate with NPC to provide technical assistance with respect to Development of Products in the NPC Territory. Such cooperation shall include providing NPC with reasonable access by teleconference or in person at UPI's corporate and manufacturing facilities (subject to UPI's customary rules and restrictions with respect to site visits by non-UPI personnel) to UPI personnel involved in the research, manufacturing and development of the Compound and Products. Upon NPC's request, UPI may in its discretion dispatch UPI personnel to NPC in order to support Development of Products by NPC in the NPC Territory. All expenses for such dispatch including traveling and lodging expenses shall be equally shared by the Parties unless otherwise agreed between the Parties.

4.3 Trademarks. UPI shall have the right to select, own, maintain and enforce Trademarks for Products in the UPI Territory. In the event that NPC desires to use for Products in the NPC Territory one or more Product-specific Trademarks that are used outside the NPC Territory and owned or Controlled by UPI, UPI shall, upon NPC's reasonable request, grant to NPC a royalty-free exclusive license, with the right to grant and authorize sublicenses, to use, display and enforce such Trademark(s) in connection with Products in the NPC Territory, *provided* that NPC will pay all registration and maintenance costs for such Trademarks and will agree to comply with the terms of a reasonable trademark policy provided by UPI to NPC in writing.

ARTICLE 5

LICENSES AND COVENANTS NOT TO COMPETE

5.1 License to UPI. Subject to the terms and conditions of this Agreement, NPC hereby grants to UPI an exclusive license under the NPC Patent Rights and NPC Know-How, with the limited right to sublicense, solely as set forth in Section 5.3, to research, Develop, make and have made, use, import, offer for sale, sell and otherwise exploit and Commercialize Products in the UPI Territory.

5.2 License to NPC. Subject to the terms and conditions of this Agreement, UPI hereby grants to NPC an exclusive license under the UPI Patent Rights and UPI Know-How, with the limited right to sublicense solely as set forth in Section 5.3, to research, Develop, make and have made, use, import, offer for sale, sell and otherwise exploit and Commercialize Products in the NPC Territory.

5.3 License and Sublicense Grants. (a) Each Party shall have the right to grant sublicenses under the NPC Patent Rights, NPC Know-How, UPI Patent Rights and UPI Know How, as applicable, to contract service organizations and similar Third Parties to the extent reasonably necessary for such Party to exercise its rights or perform its obligations under Section 3.1 and 4.1 and the Supply Agreement. (b) Upon Commercialization, each Party shall have the right to sublicense the respective license granted in Section 5.1 and Section 5.2 to a bona fide collaboration partner; provided that such sublicensee shall not have the right to grant any further sublicenses. (c) Any sublicense granted under this Section 5.3 shall be subject to the following: (i) such sublicense agreement shall refer to this Agreement and shall be subordinated to and consistent with the terms and conditions of this Agreement; and (ii) the granting Party shall remain responsible for the performance of this Agreement (including without limitation obligations to participate in the JDCC and use Commercially Reasonable Efforts with respect to Development and Commercialization of the Product).

5.4 Covenant Not to Compete. Each Party hereby covenants that for a period of [***] after the Effective Date, it will not (by itself or through authorization of, or collaboration with, others) (i) conduct any Phase II Trials or Phase III Trials for purposes of seeking Regulatory Approval of a Competitive Product; or (ii) market, sell or promote any Competitive Product. For the purposes of this Section 5.4, "Competitive Product" means [***].

5.5 License on Completion of Term. On completion of the Term, solely due to the passage of time in accordance with Section 13.1, NPC shall grant to UPI an irrevocable, world-wide, fully-paid and royalty-free license under the NPC Patent Rights and NPC Know-How to research, Develop, Manufacture, use, import, offer for sale, sell and otherwise exploit Products in the UPI Territory, and UPI shall grant to NPC an irrevocable, world-wide, fully-paid and royalty-free license under the UPI Patent Rights and UPI Know-How to research, Develop, Manufacture, use, import, offer for sale, sell and otherwise exploit Products in the NPC Territory.

5.6 No Other Rights; No Implied Licenses. Other than the licenses expressly granted under this Agreement, no right, title or interest in NPC Patent Rights and NPC Know-How shall be granted, by implication, estoppel or otherwise, to UPI and no right, title or interest in UPI Patent Rights and UPI Know-How shall be granted, by implication, estoppel or otherwise, to NPC.

ARTICLE 6

SUPPLY

6.1 Manufacturing and Supply. The Parties shall negotiate in good faith and use Commercially Reasonable Efforts to enter into a definitive supply agreement setting forth the terms and conditions for Manufacture and supply to UPI by NPC of Products and/or Drug Substance (the "Supply Agreement") prior to the initiation of a Phase II Trial by UPI for the Product in the United States. Until and unless such Supply Agreement is entered into between the Parties, NPC shall use Commercially Reasonable Efforts to Manufacture and supply Products (and/or Drug Substance upon UPI's request) to UPI. It is understood and agreed between the Parties that under the Supply Agreement NPC shall not be obliged in any event, but shall use Commercially Reasonable Efforts, to Manufacture and supply the Products (and/or Drug Substance). The Supply Agreements shall at a minimum contain the terms and conditions that

are usual and customary in the pharmaceutical industry for a supply agreement of this nature and size, including without limitation that: (i) the Product or Drug Substance will be manufactured in compliance with GMP, and (ii) there shall be a reasonable quantity of inventory of the Product produced for storage in the UPI Territory. The size of such inventory will be agreed upon between the Parties and included in the Development Plan as well as in any plans for Commercialization.

6.2 Termination of Supply. If NPC desires to terminate the supply of Products and/or Drug Substance to UPI in accordance with this Agreement and the Supply Agreement, then NPC shall give UPI [***] written notice of termination if before the First UPI Approval in any major market country of the UPI Territory and [***] written notice of termination if after the First UPI Approval in any major market country of the UPI Territory. During the notice period, NPC must use Commercially Reasonable Efforts to supply the reasonable orders expected to maintain the successful development and commercialization of the Products. Upon the termination of the supply of Products and/or Drug Substance by NPC to UPI, NPC will then use Commercially Reasonable Efforts to obtain and provide to UPI, in a timely fashion, batch records, assays methods and manufacturing details required and customary for the technical transfer of production activities for Products and/or Drug Substance to a new facility of UPI's choice (with respect to such records, methods and details held or owned by a Third Party only to the extent that NPC is permitted to do so in writing by such Third Party), such that such UPI's new facility may be validated and activated for production of Products and/or Drug Substance.

If UPI desires to terminate the supply from NPC of Products and/or Drug Substance in accordance with this Agreement and the Supply Agreement, UPI must give NPC [***] written notice of termination if before the First UPI Approval in any major market country of the UPI

Territory and [***] written notice of termination if after the First UPI Approval in any major market country of the UPI Territory. NPC will provide sufficient technical information in a timely fashion to allow the institution of production of Products and/or Drug Substance at a new UPI manufacturing location at UPI's expense (with respect to such information held or owned by a Third Party only to the extent that NPC is permitted to do so in writing by such Third Party).

6.3 Notwithstanding the aforementioned, in case that UPI proceeds to Manufacture Products and/or Drug Substance independently from NPC, NPC has a right to purchase the Products and/or Drug Substance from UPI, and Supply Agreement shall define the terms and conditions, which are consistent with Article 6.1 and 6.2, for Manufacture and supply to NPC by UPI of the Products and/or Drug Substance. In the event that NPC exercises its rights under this section, UPI shall use Commercially Reasonable Efforts to Manufacture and supply Products (and/or Drug Substance upon NPC's request) to NPC.

ARTICLE 7

PAYMENTS

7.1 Upfront Payments and Equity Issuance.

7.1.1 Upfront License Fee. UPI has paid ¥10,000,000 to NPC as an upfront license fee per the terms of the Term Sheet for Sialic Acid Treatment dated May 28, 2010 entered into between NPC and UPI. Upon the Effective Date of this Agreement such fee shall become non-refundable.

7.1.2 Equity Issuance. As soon as reasonably practicable after the execution of this Agreement, UPI shall issue to NPC and NPC shall purchase 3,000 shares of common stock of UPI (representing approximately [***] percent ([***]%) of the outstanding capital of UPI) pursuant to stock purchase agreement consistent with UPI founder's stock purchase agreement which shall be disclosed to NPC upon the execution of this Agreement and containing such provisions and terms that are customary and usual for a stock purchase agreement with an investment of this size and nature with such reasonable modifications to be mutually agreed upon in good faith by the Parties. The purchase price of the UPI common stock shall be $0.01 per share. UPI shall ensure that such shares of UPI common stock when issued will be duly authorized, fully paid and non-assessable.

7.2 Development Milestones. UPI shall make non-refundable milestone payments to NPC in the amounts set forth below within [***] Business Days of the achievement of each of the milestones set forth therein:

1. Upon completion by NPC of formulation design of Products to be Commercialized in Japan but in no event before December 31, 2010: ¥20,000,000

2. Completion by NPC of teratogenicity study of Products using rats in Japan but in no event before March 31, 2011: ¥20,000,000

Notwithstanding the foregoing, the milestone payment set forth above shall be non-refundable only if NPC has not breached any material obligations hereunder at the time of the achievement of each milestone set forth above.

7.3 Approval Milestones. UPI shall make a nonrefundable milestone payment of ¥200,000,000 to NPC within [***] Business Days of [***].

7.4 Single Milestones. It is understood and agreed that the payments under these Sections 7.2 and 7.3 shall be due only once, upon the first instance of the milestone events described above. Accordingly, if for example UPI makes one or more of the payments due under Sections 7.2 and 7.3 with respect to achievement of the corresponding milestones, and then subsequently achieves the same development milestone again, UPI shall not be obligated to make any additional milestone payment.

7.5 Royalties.

7.5.1 Net Sales by UPI and its Affiliates. UPI shall pay to NPC a royalty of [***] percent ([***]%) on UPI's Net Sales of Products in the UPI Territory.

7.5.2 Net Sales by NPC and its Affiliates. NPC shall pay to UPI a royalty of [***] percent ([***]%) on NPC's Net Sales of Products in the NPC Territory (excluding Japan).

7.6 Additional Terms.

7.6.1 Royalty Term. The royalties due pursuant to Section 7.5 above shall be payable on a country-by-country and Product-by-Product basis commencing on the First Commercial Sale in the Party's Territory and continuing until the expiration of this Agreement.

7.6.2 Third Party Royalty Payments. If (i) UPI or its Affiliate or Sublicensee, as applicable, is legally required to obtain a license from any Third Party under Patent Rights or Know-How covering a Product in order to import, use or sell such Product; and (ii) UPI or its Affiliate or Sublicensee, as applicable, is legally required under such license to pay to such Third Party license fees, milestone payments, or royalties calculated on Net Sales of such Product, then the amount of UPI's royalty obligations under Section 7.5.1 hereof, shall be reduced by [***]

percent ([***]%) of the amount of such license fees, milestone payments or royalties paid to such Third Party, *provided* however, that the royalties payable in any calendar quarter under Section 7.5.1 hereof shall not be reduced in any event below [***] percent ([***]%) of the amounts set forth under Section 7.5.1. If (i) NPC or its Affiliate or Sublicensee, as applicable, is legally required to obtain a license from any Third Party under Patent Rights or Know-How covering a Product in order to import, use or sell such Product; and (ii) NPC or its Affiliate or Sublicensee, as applicable, is legally required under such license to pay to such Third Party license fees, milestone payments, or royalties calculated on Net Sales of such Product, then the amount of NPC's royalty obligations under Section 7.5.2 hereof shall be reduced by [***] percent ([***]%) of the amount of such license fees, milestone payments or royalties paid to such Third Party, *provided* however, that the royalties payable in any calendar quarter under Section 7.5.2 hereof shall not be reduced in any event below [***] percent ([***]%) of the amounts set forth under Section 7.5.2. UPI and NPC shall use Commercially Reasonable Efforts to minimize the amount of any of the foregoing payments owed to Third Parties. Prior to a Party committing to any payments to any Third Party under this Section 7.6.2, it shall provide the other Party with written notice of a potential need to obtain any Third Party license and the Parties shall discuss such potential need in good faith, *provided* that such discussions shall not unreasonably limit or delay such Party's reasonable judgment with respect thereto. Notwithstanding the foregoing, UPI and NPC shall equally share the royalty under the JHSF License Agreement (the "JHSF Royalty"), if any, for use of the Nishino Technology in the UPI Territory. NPC shall pay the license fee to the JHSF and use Commercially Reasonable Efforts to negotiate and obtain the HSTTC license for the Nishino Technology. Each Party shall bear the cost incurred in their respective Territory in connection with the prosecution and maintenance of the intellectual properties related to the Nishino Technology.

ARTICLE 8

PAYMENTS, BOOKS AND RECORDS

8.1.1 Reports; Payments. After the First Commercial Sale of a Product on which royalties are payable by a Party hereunder, such Party shall make and deliver to the other Party quarterly written reports within [***] days after the end of each calendar quarter during the Term stating the quantity, description, and aggregate Net Sales by country of each such Product sold by that Party and its Affiliates and Sublicensees during the calendar quarter. Within thirty [***] of the date of such report, the relevant Party shall pay to the other Party the royalties described above.

8.1.2 After the First Commercial Sale of a Product on which royalties are payable by UPI hereunder, UPI shall make and deliver to NPC annual written reports within [***] days after the end of each business year during the Term, which commences from April 1 and ends on March 31 of the following calendar year (the "Business Year"), stating the sales volume, gross sales, aggregate Net Sales and other items reasonably requested by NPC, by country of each such Product sold by UPI and its Affiliates and Sublicenses during the relevant Business Year. Notwithstanding the provisions of Article 10, NPC may disclose such annual reports to the JHSF subject to the JHSF's entering into a confidentiality agreement reasonably satisfactory to UPI. Further, UPI shall also promptly provide NPC with any information reasonably requested by NPC, and NPC may, notwithstanding the provisions of Article 10, disclose such information to the JHSF subject to the JHSF's entering into a confidentiality agreement reasonably satisfactory to UPI.

8.2 Payment Method. All payments due under this Agreement shall be made by bank wire transfer in immediately available funds to a bank account designated by the Party entitled to receive such payment. All payments hereunder shall be made in Japanese Yen. Any payments that are not paid on the date such payments are due under this Agreement shall bear interest to the extent permitted by applicable Law at a rate equal to the 3-month LIBOR rate at the close of business on the date such payment is due, plus an additional [***]%, calculated on the number of days such payment is delinquent.

8.3 Place of Royalty Payment; Currency Conversion. With respect to gross sales or Net Sales invoiced in a currency other than U.S. Dollars, such gross sales or Net Sales in the quarterly report or annual report as set forth in Section 8.1 above shall be expressed in the invoiced currency, together with the U.S. Dollar and Japanese Yen equivalent (and the royalty payment hereunder shall be made based on such Japanese Yen equivalent), calculated using the arithmetic average of the spot rates on the close of business on the last Business Day of each month of the relevant calendar quarter or the relevant Business Year, in which such gross sales or Net Sales were made. The "closing mid-point rates" found in the "dollar (or yen) spot forward against the dollar (or yen)" table published by The Financial Times or any other publication as agreed to by the Parties shall be used as the source of spot rates to calculate the average as defined in the preceding sentence.

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

8.4 Records; Inspection. Each Party shall keep, and shall ensure that its Affiliates keep, complete, true and accurate books of account and records for the purpose of determining the amounts payable under this Agreement. Such books and records shall be kept at the principal place of business of such Party, for at least [***] following the end of the calendar quarter during the Term to which they pertain. Such records will be open for inspection (i) by a public accounting firm designated by the auditing Party which is reasonably acceptable to the audited Party and subject to such accounting firm entering into a satisfactory confidentiality agreement, solely for the purpose of determining the payments to the auditing Party hereunder, or (ii) by the JHSF if the audited Party is UPI and upon a reasonable request by NPC, which is the auditing Party. Such inspections may be made no more than once each calendar year, at reasonable times and on reasonable advance notice. Inspections conducted under this Section 8.4 shall be at the expense of the auditing Party, unless an error in books of account and records of the audited Party producing an increase exceeding [***] percent ([***]%) of the amount payable under this Agreement stated for the period covered by the inspection is found in the course of such inspection, whereupon all reasonable costs relating to the inspection for such period shall be borne by the audited Party. Any unpaid or overpaid amounts that are discovered during the course of such inspection will be promptly paid or refunded by the appropriate Party, in each case together with interest noted in Section 8.2 thereon from the date such payments were due (if underpaid) or paid (if overpaid).

8.5 Withholding Taxes. Each Party shall pay any and all taxes levied on account of amounts payable to it under this Agreement. If applicable Laws or regulations require that taxes be withheld, the paying Party will (i) deduct those taxes from the remittable payment, (ii) timely pay the taxes to the proper authority, and (iii) send proof of payment to the other Party within [***] days following that payment.

ARTICLE 9

INTELLECTUAL PROPERTY

9.1 Ownership.

9.1.1 Ownership. All intellectual property right and other right, title, and interest in: (i) UPI Patent Rights and UPI Know-How shall be owned solely by UPI; and (ii) NPC Patent Rights and NPC Know-How shall be owned solely by NPC. All intellectual property right and other right, title and interest in Joint Patent Rights and Joint Know-How shall be owned jointly by UPI and NPC.

9.1.2 Disclosure of Joint Know-How. Each Party shall promptly disclose to the other all Inventions within the Joint Know-How.

9.1.3 Inventorship. In the event of any disagreement between the Parties regarding the inventorship of any Invention, the Parties shall subject the disagreement to the arbitration process contained in Section 14.2.

9.1.4 Execution of Documents. Each Party shall execute such documents and perform such acts as may be reasonably requested by the other Party to enable the other Party to use its interest in the Joint Know-How and Joint Patent Rights or to otherwise give effect to the provisions set forth in this Section 9.1.

9.2 Intellectual Property Right Application. If a Party intends to make application in such Party's Territory for patent(s), utility model(s) and/or other intellectual property right(s) concerning any Know-How obtained, developed or invented by such Party during the course of the Development activities under this Agreement (including, but not limited to, Joint Know-How), then such Party shall disclose the contents of such application to the other Party in advance of the filing for review and comment.

9.3.1 NPC Territory. As used herein, “prosecution” shall include interferences, reexaminations, reissues, oppositions, and the like. In the NPC Territory, NPC shall have the sole right to control the preparation, filing, prosecution and maintenance of NPC Patent Rights, NPC Know-How, UPI Patent Rights, UPI Know-How, Joint Patent Rights and Joint Know-How (collectively, the “Intellectual Properties”) using patent counsel of NPC’s choice. UPI shall have the right to review and comment on prosecution and patent applications prior to their filing.

9.3.2 UPI Territory. In the UPI Territory, UPI shall have the sole right to control the preparation, filing, prosecution and maintenance of the Intellectual Properties using patent counsel of UPI’s choice. NPC shall have the right of review and comment on prosecution and patent applications prior to their filing.

9.3.3 Abandonment. Each Party shall give timely notice to the other Party of any decision not to file applications for, or to cease prosecution and/or maintenance of, or not to continue to pay the expenses of prosecution and/or maintenance of, any Joint Patent Rights in their respective Territory (such decision, an “Abandonment”) and, in such case, shall permit the other Party, at its sole discretion and expense, to file or to continue prosecution or maintenance of such Joint Patent Rights in their own Territory. Any such patent rights or patent applications for which the option granted under this Section is exercised shall not be considered part of the Joint Patent Rights thereafter and the claims of such patent rights or patent applications shall not be subject to the license provisions of Article 5 unless otherwise agreed by the Parties.

9.3.4 Intellectual Property Costs. Unless otherwise provided for in this Agreement:

(a) in the UPI Territory, all costs associated with filing, prosecuting, issuing and maintaining UPI Patent Rights, NPC Patent Rights, UPI Know-How and NPC Know-How including interference, opposition, reexamination and reissue actions, shall be borne by UPI;

(b) in the NPC Territory, all costs associated with filing, prosecuting, issuing and maintaining NPC Patent Rights, UPI Patent Rights, NPC Know-How and UPI Know-How including interference, opposition, reexamination and reissue actions shall be borne by NPC; and

(c) all costs associated with filing, prosecuting, issuing and maintaining Joint Patent Rights and Joint Know-How, including interference, opposition, reexamination and reissue actions, shall be equally shared by the Parties.

9.4 Cooperation. Each Party will promptly provide to the other Party information reasonably requested by other Party that is necessary for the prosecution activities pursuant to Section 9.3 above. The Parties agree to cooperate and to take such actions as the Parties mutually agree are reasonable to maximize the protections available under relevant regulations such as the safe harbor provisions of 35 U.S.C. 103(c) for USA patents/patent applications.

9.5 Infringement. UPI and NPC shall promptly notify the other in writing of any alleged or threatened infringement of any of the Intellectual Properties or infringement of any Third Party's intellectual property by developing, making, having made, using, importing, selling or having sold Products or Drug Substance (collectively, the "Infringement"), of which they become aware.

9.5.1 UPI Territory. In the UPI Territory, UPI shall have the sole right to bring and control any action or proceeding with respect to Infringement by counsel of its own choice.

9.5.2 NPC Territory. In the NPC Territory, NPC shall have the sole right to bring and control any action or proceeding with respect to Infringement by counsel of its own choice.

9.5.3 Unless otherwise provided for in this Agreement:

(a) in the UPI Territory, all costs associated with the enforcement of UPI Patent Rights, NPC Patent Rights, UPI Know-How and NPC Know-How and defense against Infringement shall be borne by UPI;

(b) in the NPC Territory, all costs associated with the enforcement of UPI Patent Rights, NPC Patent Rights, UPI Know-How and NPC Know-How and defense against Infringement shall be borne by NPC; and

(c) all costs associated with the enforcement of Joint Patent Rights and Joint Know-How shall be equally shared by the Parties.

9.6 Patent Term Extension; Data Exclusivity. UPI shall use Commercially Reasonable Efforts to obtain patent term extensions, supplemental protection certificates or data exclusivity periods (such as those periods listed in the FDA's Orange Book or its equivalent with the EMEA), or their equivalents in any jurisdiction in Europe and North America, and NPC shall cooperate with UPI in doing so at UPI's expense, with respect to Products and NPC Patent Rights and UPI Patent Rights covering the Products. If elections with respect to obtaining such patent term extensions or data exclusivity periods are to be made, UPI shall have the right to make the election to seek patent term extension or data exclusivity periods, *provided* that such election will be made so as to maximize the period of marketing exclusivity for the Products.

ARTICLE 10

CONFIDENTIALITY

10.1 Confidentiality. During the Term of this Agreement and for a period of [***] following the expiration or earlier termination hereof, each Party shall maintain in confidence the Confidential Information of the other Party, shall not use or grant the use of the Confidential Information of the other Party except as expressly permitted hereby, and shall not disclose the Confidential Information of the other Party (in each case, irrespective of whether such Confidential Information is also the Confidential Information of such Party), except (i) on a need-to-know basis to such Party's directors, officers and employees, (ii) to such Party's consultants performing work contemplated by the Agreement, and to any subcontractor performing work for such Party hereunder, or (iii) to the extent such disclosure is reasonably necessary in connection with such Party's activities under rights and licenses expressly authorized by this Agreement. To the extent that disclosure to any person is authorized by this Agreement, prior to disclosure, a Party shall obtain written agreement of such person to hold in confidence and not disclose, use or grant the use of the Confidential Information of the other Party except as expressly permitted under this Agreement. Each Party shall notify the other Party promptly upon discovery of any unauthorized use or disclosure of the other Party's Confidential Information.

10.2 Permitted Use and Disclosures. The confidentiality obligations under this Article 10 shall not apply to the extent that a Party is required to disclose information by applicable Law, regulation or order of a governmental agency or a court of competent jurisdiction, including filings required by the Securities and Exchange Commission or any similar body, or any securities exchange; *provided*, however, that such Party shall provide written notice thereof to

the other Party (to the extent not prohibited by Law or court order), and consult with the other Party with respect to such disclosure and provide the other Party reasonable opportunity to object to any such disclosure or to request confidential treatment thereof. Notwithstanding the provisions of this Article 10, the Parties agree that: (i) either Party may, to the extent reasonably necessary, disclose Confidential Information of the other Party to any Regulatory Authority in connection with the Development of a Product which it has the right to Develop under this Agreement; (ii) to the extent they have not done so as of the Effective Date, the Parties shall agree upon a press release related to this Agreement. In addition, each Party will consider in good faith any request by the other Party for a public disclosure not otherwise permitted pursuant to this Article 10, with consent for such disclosure not to be unreasonably withheld, conditioned or delayed. In the event of any termination of this Agreement under Article 13, the Parties shall agree on an announcement of such termination; provided that the Parties shall use reasonable efforts to fashion such announcement so as to minimize any negative impact on either Party as a result of such announcement. Once a particular item of information has been publicly disclosed pursuant to this Article 10, further consent will not be needed for further disclosures thereof.

10.3 Additional Nondisclosure. Each of the Parties hereto agrees not to disclose the terms and conditions of this Agreement to any Third Party without the prior written consent of the other Party hereto, which consent shall not be unreasonably withheld, conditioned or delayed, except to (i) the JHSF subject to the JHSF's entering into a confidentiality agreement reasonably satisfactory to UPI, (ii) such Party's attorneys, advisors and actual collaborators on a need to know basis under circumstances that reasonably protect the confidentiality thereof, (iii) Inabata & Co., Ltd., Hisanaga & Co., Ltd. and Development Bank of Japan Inc. (collectively, the "NPC's Shareholders") as long as the NPC's Shareholders maintain ownership of NPC's stock

and are subject to substantially similar terms and conditions of confidentiality obligations of this Agreement, (iv) banks, potential investors and other financial institutions from which NPC or UPI are receiving, will receive or plan to be receiving financing from provided they are subject to confidentiality obligations with substantially similar terms and conditions to the confidentiality obligations of this Agreement, or (v) others to the extent required by Law (and with appropriate requests made for confidential treatment), including disclosures associated with filings required to be made by Law with the Securities and Exchange Commission, any similar body, or any national securities exchange.

10.4 Publication. Any manuscript prepared by UPI or NPC (the "Disclosing Party" in this Section 10.4; for the purpose of this Section 10.4, any manuscript prepared by UPI's or NPC's Affiliate shall be deemed as manuscript prepared by UPI or NPC, respectively) on subject matter in connection with this Agreement to be published or publicly disclosed pursuant to this Article 10, shall be subject to the prior review of the other Party (the "Receiving Party" in this Section 10.4) at least [***] days prior to such publication or disclosure and may not be published or publicly disclosed without the prior written consent of the Receiving Party, which consent shall not be unreasonably withheld, conditioned or delayed. Further, to avoid loss of patent rights as a result of premature public disclosure of patentable information, the Receiving Party shall notify the Disclosing Party in writing within [***] Business Days after receipt of such manuscript of whether the Receiving Party desires to file a patent application on any invention disclosed in such manuscript. In the event that the Receiving Party desires to file such a patent application, the Disclosing Party shall withhold publication or disclosure of such manuscript until the earlier of (i) a patent application is filed thereon, or (ii) the Parties determine after consultation that no patentable invention exists after receipt by the Disclosing Party of the

Receiving Party's written notice of the Receiving Party's desire to file such patent application. Further, if such manuscript contains the information of the Receiving Party that is subject to use and nondisclosure restrictions under this Article 10, the Disclosing Party agrees to remove, upon request of the Receiving Party, such information from the proposed publication or disclosure. The determination of authorship and final editorial control over any publications related to any clinical study or research will be the responsibility of the Party that funded such clinical study or research.

ARTICLE 11

REPRESENTATIONS AND WARRANTIES

11.1 Mutual Representations and Warranties. Each Party represents and warrants to the other Party as of the Effective Date that: (i) it has all requisite corporate power and authority to enter into this Agreement and to perform its obligations under this Agreement; (ii) execution of this Agreement and the performance by such Party of its obligations hereunder have been duly authorized; (iii) this Agreement is legally binding and enforceable on each Party in accordance with its terms; (iv) execution and delivery of this Agreement and the performance of such Party's obligations hereunder do not conflict with or violate any applicable Laws and do not conflict with, or constitute a breach or default under, any contractual obligation of such Party; and (v) all necessary consents, approvals and authorizations of all governmental authorities and other Persons required to be obtained by such Party in connection with entering into this Agreement have been obtained.

11.2 Representations and Warranties of NPC. NPC represents and warrants to UPI that:

11.2.1 Intellectual Property Rights. As of the Effective Date: (i) NPC has not previously assigned, transferred, conveyed or otherwise encumbered its right, title and interest in the NPC Patent Rights or NPC Know-How in the UPI Territory in a manner inconsistent with the rights, options and licenses granted to UPI herein or NPC's obligations to Manufacture and supply Drug Substance and/or Product pursuant to Article 6, including without limitation by authorizing any Third Party to Manufacture, use or sell the Compound or any Products in any country in the UPI Territory; and (ii) NPC is not aware of any facts or circumstances that would cause the UPI Territory to be invalid or unenforceable, and all necessary fees and other actions required in order to maintain the NPC Patent Rights have been paid or performed to date. In particular, as of the Effective Date NPC owns all right, title and interest in and to the NPC Patent Rights in the UPI Territory, and has not granted any right or license under the NPC Patent Rights authorizing any Third Party to Manufacture, use or sell the Compound in any country the UPI Territory that are inconsistent with the terms of this Agreement.

11.2.2 Disclosure. As of the Effective Date, there is no matter within the knowledge of NPC which NPC has intentionally, knowingly, or negligently failed to disclose, which by itself or in connection with any other matters not disclosed by NPC (i) is material to the evaluation of the Compound and/or Products; and (ii) would materially adversely affect the further Development or Commercialization of any Product.

11.3 Representations and Warranties of UPI. UPI represents and warrants to NPC that:

11.3.1 Intellectual Property Rights. As of the Effective Date: (i) UPI has not previously assigned, transferred, conveyed or otherwise encumbered its right, title and interest in the[UPI Patent Rights or] UPI Know-How in the NPC Territory in a manner inconsistent with

the rights, options and licenses granted to NPC herein, including without limitation by authorizing any Third Party to Manufacture, use or sell the Compound or any Products in any country in the NPC Territory; and (ii) UPI is not aware of any facts or circumstances that would cause the NPC Territory to be invalid or unenforceable, and all necessary fees and other actions required in order to maintain the UPI Patent Rights have been paid or performed to date. In particular, as of the Effective Date UPI owns all right, title and interest in and to the UPI Patent Rights in the NPC Territory, and has not granted any right or license under the UPI Patent Rights authorizing any Third Party to Manufacture, use or sell the Compound in any country the NPC Territory that are inconsistent with the terms of this Agreement.

11.3.2 Disclosure. As of the Effective Date, there is no matter within the knowledge of UPI which UPI has intentionally, knowingly, or negligently failed to disclose, which by itself or in connection with any other matters not disclosed by UPI (i) is material to the evaluation of the Compound and/or Products; and (ii) would materially adversely affect the further Development or Commercialization of any Product.

ARTICLE 12

INDEMNIFICATION; PHARMACOVIGILANCE

12.1 UPI Indemnity. UPI shall indemnify, defend and hold harmless NPC and its Affiliates, and their respective officers, directors, employees, agents, licensors, and their respective successors, heirs and assigns and representatives, from and against any and all claims, threatened claims, damages, losses, suits, proceedings, liabilities, costs (including, without limitation, reasonable legal expenses, costs of litigation and reasonable attorney's fees) or judgments, whether for money or equitable relief, of any kind, arising out of any claim, action,

lawsuit or other proceeding brought by a Third Party ("Losses and Claims") arising out of or relating, directly or indirectly, to: (i) the Development or Commercialization of the Compound or any Product in the UPI Territory by UPI, its Affiliates or Sublicensees; (ii) the Commercialization of the Compound or any Product in Europe by UPI or its Affiliates; (iii) breach by UPI of Article 11 or any other provision of this Agreement; or (iv) the negligence, recklessness or willful misconduct of UPI; *except* where such Losses and Claims are attributable to a failure by NPC to comply with applicable Law or the negligence, recklessness or willful misconduct of NPC.

12.2 NPC Indemnity. NPC shall indemnify, defend and hold harmless UPI and its Affiliates, and their respective officers, directors, employees, agents, licensors, and their respective successors, heirs and assigns and representatives, from and against any and all Losses and Claims arising out of or relating, directly or indirectly, to: (i) the Development or Commercialization of the Compound or any Product in the NPC Territory by NPC or its Affiliates or Sublicensees; (ii) breach by NPC of Article 11 or any other provision of this Agreement; or (iii) the negligence, recklessness or willful misconduct of NPC; *except where* such Losses and Claims are attributable to a failure by UPI to comply with applicable Law or the negligence, recklessness or willful misconduct of UPI.

12.3 Indemnification Procedure. A claim to which indemnification applies under Section 12.1 or Section 12.2 shall be referred to herein as an "Indemnification Claim". If any Person or Persons (collectively, the "Indemnitee") intends to claim indemnification under this Article 12, the Indemnitee shall notify the other Party (the "Indemnitor") in writing promptly upon becoming aware of any claim that may be an Indemnification Claim (it being understood and agreed, however, that the failure by an Indemnitee to give such notice shall not relieve the

Indemnitor of its indemnification obligation under this Agreement except and only to the extent that the Indemnitor is actually prejudiced as a result of such failure to give notice). The Indemnitor shall have the right to assume and control the defense of the Indemnification Claim at its own expense with counsel selected by the Indemnitor and reasonably acceptable to the Indemnitee, provided, however, that an Indemnitee shall have the right to retain its own counsel, with the fees and expenses to be paid by the Indemnitee, if representation of such Indemnitee by the counsel retained by the Indemnitor would be inappropriate due to actual or potential differing interests between such Indemnitee and any other Party represented by such counsel in such proceedings. If the Indemnitor does not assume the defense of the Indemnification Claim as aforesaid, the Indemnitee may defend the Indemnification Claim but shall have no obligation to do so. The Indemnitee shall not settle or compromise the Indemnification Claim without the prior written consent of the Indemnitor, and the Indemnitor shall not settle or compromise the Indemnification Claim in any manner which would have an adverse effect on the Indemnitee's interests (including without limitation any rights under this Agreement or the scope or enforceability of the NPC Patents Rights or NPC Know-How), without the prior written consent of the Indemnitee, which consent, in each case, shall not be unreasonably withheld or delayed. The Indemnitee shall reasonably cooperate with the Indemnitor at the Indemnitor's expense and shall make available to the Indemnitor all pertinent information under the control of the Indemnitee, which information shall be subject to Article 10.

12.4 Insurance. Within [***] months after the Effective Date, each Party shall at its own expense procure and maintain during the Term and for a period of [***] years thereafter an insurance policy/policies, adequate to cover its obligations hereunder and which is/are consistent with normal business practices of prudent companies similarly situated. Each Party's insurance

coverage shall include without limitation [***]. Such insurance shall not be construed to create a limit of the insuring Party's liability with respect to its indemnification obligations under this Article 12. Each Party shall provide the other Party with a certificate of insurance or other evidence thereof upon request. Each Party shall provide the other Party with written notice at least [***] days prior to the cancellation, non-renewal or a material change in such insurance or self-insurance which materially adversely affects the rights of the other Party hereunder.

12.5 Global Pharmacovigilance. NPC and UPI shall negotiate in good faith and use reasonable efforts to enter into an agreement regarding the pharmacovigilance of Products within a reasonable period of time after the Effective Date.

ARTICLE 13

TERM AND TERMINATION

13.1 Term. The Term of this Agreement shall commence on the Effective Date, and shall continue in full force and effect on a country-by-country until the date of the first launch of a generic product of the Product in a country, unless earlier terminated as provided in this Article 13 (the "Term").

13.2 Termination for Breach. Subject to Section 13.5, either Party to this Agreement may terminate this Agreement in the event the other Party hereto shall have materially breached or defaulted in the performance of any of its material obligations hereunder (other than failure to use Commercially Reasonable Efforts (by itself or through contract service organizations or other permitted Third Party licensees or sublicensees) to Develop or Commercialize a Product under Section 3.1.1 or Section 4.1.1), and such breach or default shall have continued for thirty

(30) days after written notice thereof was provided to the breaching Party by the non-breaching Party. Any termination shall become effective at the end of such thirty (30) day period unless the breaching Party has cured any such breach or default prior to the expiration of the thirty (30) day period.

13.3 Failure to Develop or Commercialize Products. Five (5) years after the Effective Date or two (2) years after the first Regulatory Approval in a major market country, whichever is the earlier, the Parties shall discuss in good faith on how to deal with the Development and Commercialization of Products in countries where Products have not been commercially sold regardless of whether the Parties have performed their obligations under Section 3.1.1 or 4.1.1.

13.4 Termination For Bankruptcy. Either Party hereto shall have the right to terminate this Agreement forthwith by written notice to the other Party (i) if the other Party is declared insolvent or bankrupt by a court of competent jurisdiction, (ii) if a voluntary or involuntary petition in bankruptcy is filed in any court of competent jurisdiction against the other Party and such petition is not dismissed within thirty (30) days after filing, (iii) if the other Party shall make or execute an assignment of substantially all of its assets for the benefit of creditors, or (iv) substantially all of the assets of such other Party are seized or attached and not released within thirty (30) days thereafter.

13.5 Disputed Breach. If either Party disputes in good faith the existence or materiality of a breach specified in a notice provided by the other Party pursuant to Section 13.2, and the allegedly breaching Party provides notice to the other Party of such dispute within the applicable thirty (30) day cure period, the other Party shall not have the right to terminate this Agreement unless and until the existence of such material breach or failure has been determined in

accordance with Section 14.2 and the allegedly breaching Party fails to cure such breach within thirty (30) days following such determination (except to the extent such breach involves the failure to make a payment when due, which breach must be cured within ten (10) days following such determination). It is understood and acknowledged that while such a dispute is pending all of the terms and conditions of this Agreement shall remain in effect and the Parties shall continue to perform all of their respective obligations hereunder. The Parties further agree that any payments that are made by one Party to the other Party pursuant to this Agreement pending resolution of the dispute shall be promptly refunded if an arbitrator determines pursuant to Section 14.2 that such payments are to be refunded by one Party to the other Party.

13.7 Effect of Termination. Upon expiration or termination of this Agreement for any reason, the rights and obligations of the Parties shall be as set forth in this Section 13.7.

13.7.1 License on Termination for UPI Breach or Bankruptcy. Upon termination of this Agreement by NPC pursuant to Section 13.2 or 13.4 hereof, UPI shall grant to NPC an irrevocable, world-wide, fully-paid and royalty-free license under the UPI Patent Rights and UPI Know-How to research, Develop, Manufacture, use, import, offer for sale, sell and otherwise exploit Products in the NPC Territory.

13.7.2 License on Termination for NPC Breach or Bankruptcy. Upon termination of this Agreement by UPI pursuant to Section 13.2 or 13.4, NPC shall grant to UPI an irrevocable, world-wide, fully-paid and royalty-free license under the NPC Patent Rights and NPC Know-How to research, Develop, Manufacture, use, import, offer for sale, sell and otherwise exploit Products in the UPI Territory.

13.7.3 Survival. The following provisions shall survive termination or expiration of this Agreement for any reason: Sections 5.5, 9.1, 13.7, 15.1 through 15.4, and 15.7 through 15.12, and Articles 1, 8, 10, 12 and 14. Expiration or termination of this Agreement for any reason shall not relieve the Parties of any liability or obligation accruing on or prior to such expiration or termination, or which is attributable to a period prior to such expiration or termination, nor preclude either Party from pursuing any rights and remedies it may have hereunder or at law or in equity which accrued or are based upon any event occurring prior to such expiration or termination. Unless otherwise set forth in this Agreement, any other rights, obligations and provisions hereunder shall terminate and cease to have effect upon expiration or termination of this Agreement.

ARTICLE 14

DISPUTE RESOLUTION

14.1 Escalation to Senior Executives. Other than pursuit of equitable relief as provided in Section 14.4, in the event of a dispute or matter of significant concern arises between the Parties, then at the request of either Party, the matter shall be escalated to the CEO of UPI and the senior executive at NPC with responsibility for pharmaceutical products and authority to make a decision on behalf of the respective Party with respect to such matter. Upon such request, such senior executives shall make themselves reasonably available to meet, and shall meet either by telephone or if specifically requested, in person, to attempt to resolve such matter, and shall thereafter continue to use good faith efforts to attempt to resolve such matter unless it becomes clear that the matter cannot be resolved by mutual agreement. Thereafter, either Party may initiate arbitration pursuant to Section 14.2 below upon written notice to the other Party.

14.2 Arbitration of Disputes. Other than pursuit of equitable relief as provided in Section 14.4, the Parties shall resolve disputes in accordance with this Section 14.2. Any disputes, controversies or differences that may arise under or in relation to this Agreement and which cannot be settled under Section 14.1 shall be settled by arbitration. The arbitration shall be conducted in San Francisco, California in accordance with the applicable rules of the AAA if UPI is the respondent in such dispute or in Tokyo in accordance with the applicable rules of the JCAA if NPC is the respondent in such dispute. Any arbitration conducted under this Section 14.2 shall be conducted in English. The determination of the arbitrator as to the resolution of any dispute shall be binding and conclusive upon all Parties.

14.4 Injunctive Relief. This Article 14 shall not be construed to prohibit either Party from seeking preliminary or permanent injunctive relief, restraining order or decree of specific performance in any court of competent jurisdiction to the extent not prohibited by this Agreement. For avoidance of doubt, any such equitable remedies provided under this Article 14 shall be cumulative and not exclusive and are in addition to any other remedies, which either Party may have under this Agreement or applicable Law.

ARTICLE 15

MISCELLANEOUS

15.1 Governing Laws. This Agreement and any dispute arising from the construction, performance or breach hereof shall be governed by and construed, and enforced in accordance with, the laws of the state of New York, without reference to conflicts of laws principles.

15.2 Waiver. It is agreed that no waiver by either Party hereto of any breach or default of any of the covenants or agreements herein set forth shall be deemed a waiver as to any subsequent and/or similar breach or default.

15.3 Assignment. This Agreement shall not be assignable by either Party without the written consent of the other Party hereto, except either Party may assign this Agreement without such consent to its Affiliates, or to an entity that acquires all or substantially all of the business or assets of such Party related to this Agreement whether by merger, reorganization, acquisition, sale, or otherwise; *provided*, however, that the assignee shall agree in writing to be bound by the terms and conditions of this Agreement.

15.4 Independent Contractors. Both Parties are independent contractors under this Agreement. Nothing herein contained shall be deemed to create an employment, agency, joint venture or partnership relationship between the Parties or any of their agents or employees, or any other legal arrangement that would impose liability upon one Party for the act or failure to act of the other Party. Neither Party shall have any express or implied power to enter into any contracts or commitments or to incur any liabilities in the name of, or on behalf of, the other Party, or to bind the other Party in any respect whatsoever.

15.5 Compliance with Laws. In exercising their rights under this license, the Parties shall fully comply in all material respects with the requirements of any and all applicable Laws, regulations, rules and orders of any governmental body having jurisdiction over the exercise of rights under this license including, without limitation, those applicable to the discovery, development, manufacture, distribution, import and export and sale of Products pursuant to this Agreement.

15.6 Patent Marking. Each Party agrees to mark, and to make sure that its Sublicensees mark, all Products sold by or under authority of such Party pursuant to this Agreement in accordance with the applicable statute or regulations relating to patent marking in the country or countries of manufacture and sale thereof.

15.7 Notices. All notices, requests and other communications hereunder shall be in writing and shall be personally delivered or by registered or certified mail, return receipt requested, postage prepaid, in each case to the respective address specified below, or such other address as may be specified in writing to the other Parties hereto and shall be deemed to have been given upon receipt:

UPI: Ultragenyx Pharmaceutical Inc.
77 Digital Drive, Suite 210
Novato, CA 94949 USA
Attn: Chief Executive Officer

With Copies to (for information purposes only):

Wright Legal Advisory, LLC,
3213 W, Wheeler St, Suite 307
Seattle, WA 91899
Attn: Managing Member

NPC: Nobelpharma Co., Ltd.
Kyodo Bldg. (Horidome)
12-10 Nihonbashi-kobunacho, Chuo-Ku
Tokyo 103-0024, Japan
Attention: Managing Director and CEO

15.8 Severability. In the event that any provision of this Agreement becomes or is declared by a court of competent jurisdiction to be illegal, unenforceable or void, this Agreement shall continue in full force and effect to the fullest extent permitted by law without said provision, and the Parties shall amend the Agreement to the extent feasible to lawfully include the substance of the excluded term to as fully as possible realize the intent of the Parties and their commercial bargain.

15.9 Advice of Counsel. UPI and NPC have each consulted counsel of their choice regarding this Agreement, and each acknowledges and agrees that this Agreement shall not be deemed to have been drafted by one Party or another and will be construed accordingly.

15.10 Complete Agreement. This Agreement constitutes the entire agreement, both written and oral, between the Parties with respect to the subject matter hereof, and all prior agreements respecting the subject matter hereof, either written or oral, express or implied, shall be abrogated, canceled, and are null and void and of no effect. No amendment or change hereof or addition hereto shall be effective or binding on either of the Parties hereto unless reduced to writing and executed by the respective duly authorized representatives of UPI and NPC.

15.11 Headings. The captions to the several Sections and Articles hereof are not a part of this Agreement, but are included merely for convenience of reference and shall not affect its meaning or interpretation.

15.12 Counterparts. This Agreement may be executed in counterparts, each of which shall be deemed to be an original and all of which together shall be deemed to be one and the same agreement.

[remainder of page intentionally left blank]

IN WITNESS WHEREOF, the Parties hereto have caused this Agreement to be duly executed by their authorized representatives and delivered in duplicate originals as of the Effective Date.

Nobelpharma Co., Ltd.

/s/ Jin Shiomura
Name: Jin Shiomura
Title: Managing Director and CEO

Ultragenyx Pharmaceutical Inc.

/s/ Emil Kakkis
Name: Emil Kakkis
Title: CEO and President

LICENSE AGREEMENT

Effective as of September 1, 2012 (the "Effective Date"), St. Jude Children's Research Hospital, having a principal place of business at ("INSTITUTION"), and Ultragenyx Pharmaceutical, Inc., a Delaware corporation having a principal place of business at 60 Leveroni Court, Novato, CA ("LICENSEE"), agree as follows:

1. BACKGROUND

1.1. INSTITUTION has an assignment of certain Technology, as hereinafter defined, from the laboratory of Dr. Alessandra D'Azzo.

1.2. INSTITUTION desires to have the Technology perfected and marketed at the earliest possible time in order that products resulting therefrom may be available for public use and benefit.

1.3. LICENSEE desires a license under said Technology to develop, manufacture, have made, use, and sell product(s) incorporating the Technology.

2. DEFINITIONS

2.1. "Affiliate" means any corporation or other entity that is directly or indirectly controlling, controlled by or under common control with LICENSEE. For the purpose of this definition, "control" shall mean the direct or indirect beneficial ownership of at least fifty percent (50%) of the outstanding shares or other voting rights of the subject entity to elect directors, or if not meeting the preceding, any entity owned or controlled by or owning or controlling at the maximum control or ownership right permitted in the country where such entity exists.

2.2. "Licensed Field" means [***].

2.3. "Licensed Know-How" means scientific, clinical or other information, knowledge and know-how and materials developed in laboratory of Dr. Alessandra D'Azzo as of the Effective Date and for a period of [***] years thereafter that are directed to the Technology, including but not limited to data, reports, and materials from preclinical studies, cell lines, or other specific assay reagents for assays or enzyme production processes, and access to the mouse models for purposes of development of enzyme replacement therapy for galactosialidosis and other materials related to the development and implementation of related products.

2.4. "Licensed Patent(s)" means (i) U.S. provisional application nos. [***] and any future applications that claim priority to any of the foregoing provisional applications, (ii) all divisions, substitutions and continuations of any of the preceding, (iii) all foreign patent applications corresponding to or claiming, priority from any of the preceding, and (iv) all U.S. and foreign patents issuing on any of the preceding, including patents of addition, reexaminations, reissues and extensions.

2.5. "Monogenetic Disorders" means diseases caused by a mutation in a single gene. For the avoidance of doubt, this term does not include Alzheimer's Disease or any form of cancer.

2.6. "Net Sales" means the gross revenue received by LICENSEE and/or its Affiliates or Sublicensees from sales of Products to third parties, less the following items but only insofar as they are included in such gross revenue: (i) import, export, value added, excise and sales taxes, tariffs, and custom duties; (ii) rebates, refunds, and credits for any rejected or returned Products or because of retroactive price reductions, rebates or chargebacks; (iii) charges for packaging, shipping and insurance; and (iv) trade, cash or quantity discounts or rebates to the extent actually granted (including Medicaid and other government-mandated rebates).

2.7. "Orphan Drug Product" means a Product for a specific indication for the which the FDA has granted "Orphan-drug exclusive approval" as defined in 21 CFR Part 316 or which has been granted Orphan Drug exclusivity under similar regulations in the European Union or Japan.

2.8. "Product" means any PPCA protein product discovered, developed, manufactured and/or commercialized by or on behalf of LICENSEE or its Affiliates for which LICENSEE or its Affiliates incorporates and uses any Licensed Know-How in its regulatory filings to support its regulatory approval.

2.9. "Retained Field" means all uses of PPCA outside the Licensed Field.

2.10. "Technology" means the technology developed in the laboratory of Dr. Alessandra D'Azzo as of the Effective Date relating to the use of Protective Protein/Cathepsin A ("PPCA") protein to treat, prevent and/or diagnose galactosialidosis and other Monogenetic Disorders.

3. GRANT

3.1. INSTITUTION hereby grants and LICENSEE hereby accepts a worldwide, exclusive license, with the right to grant sublicense under multiple tiers, under the Licensed Know-How to make, have made, import, use, sell and offer for sale and otherwise commercialize and exploit Products in the Licensed Field, and practice any method, process or procedure within the Licensed Know-How and/or Licensed Patents in connection therewith.

3.2. Notwithstanding Section 3.1, INSTITUTION shall retain the nontransferable right to practice the Licensed Patents for its internal, academic, non-commercial research.

3.3. INSTITUTION hereby grants LICENSEE a right of first negotiation to obtain an exclusive license under the Licensed Patents, together with an exclusive license under the Licensed Know-How in the Retained Field. INSTITUTION shall promptly notify LICENSEE in writing when it is ready to negotiate such a license and in any case prior to INSTITUTION presenting any third party with the opportunity to obtain such a license under such Licensed Patents or Licensed Know-How. LICENSEE shall notify INSTITUTION within [***] days after receiving such notification as to whether it desires to obtain such right. In the event LICENSEE notifies INSTITUTION in writing of its desire to obtain such right, then INSTITUTION shall negotiate in good faith with LICENSEE on an exclusive basis for a period of [***] months to agree upon the terms and conditions under which LICENSEE shall obtain such right. In the event LICENSEE does not notify INSTITUTION of its desire within such [***] day period, or if LICENSEE so notifies INSTITUTION, but INSTITUTION and LICENSEE do not reach

agreement on such terms and conditions within such [***] months period despite good faith negotiations, then INSTITUTION shall have the right to negotiate with any third party with respect to such grant of right, provided that INSTITUTION shall not grant any third party such right on terms and conditions, taken as a whole, that are less favorable to INSTITUTION than those last offered by LICENSEE.

4. GOVERNMENT RIGHTS

The rights for which a license is granted hereunder shall be subject to all of the terms, conditions and limitations of Title 35 United States Code Sections 200 through 212 (the "Bayh-Dole Act") to the extent that such rights constitute a "subject invention" as that term is defined in the Bayh-Dole Act.

5. DILIGENCE. LICENSEE shall use commercially reasonable efforts to develop and commercialize at least one (1) Product, consistent with sound and reasonable business practices and judgments, provided that LICENSEE's interest in any other products designed to treat or prevent Galactosialidosis shall not be taken into account when considering the commercial reasonableness of LICENSEE's efforts with respect to Products. LICENSEE shall submit to INSTITUTION a written progress report within [***] days of January 1 each year after the Effective Date describing all activities conducted during the previous year to diligently develop and commercialize Products. Any efforts of LICENSEE's Affiliates and sublicensees shall be considered efforts of LICENSEE for the sole purpose of determining LICENSEE's compliance with its obligation under this Section 5.

6. PAYMENTS

6.1. LICENSEE agrees to pay to INSTITUTION a nonrefundable, license issue fee of Ten Thousand Dollars ($10,000.00) within [***] days of the Effective Date.

6.2. Subject to Section 6.4, LICENSEE shall pay to INSTITUTION royalties equal to [***] percent ([***]%) of Net Sales of Products sold by LICENSEE or its Affiliates or Sublicensees which are Orphan Drug Products, but only on a Product-by-Product and country-by-country basis for so long as, and only in the country where, such Product has market exclusivity as an Orphan Drug Product.

6.3. In the event that an Orphan Drug Product under this Agreement is sold in a combination product containing other active components, then Net Sales on the combination product shall be calculated using one of the following methods:

(a) By multiplying the net selling price of the combination product by the fraction A/(A+B) where A is the gross selling price, during the royalty-paying period being considered, of such Product sold separately, and B is the gross selling price, during the royalty period in question, of the other active components sold separately; or

(b) In the event that no such separate sales are made of such Product, Net Sales on the combination product for royalty determination shall be as reasonably allocated between such Product and the other active components, based on their relative importance and proprietary protection, as agreed by the parties. If the parties fail to reach agreement such allocation shall be submitted to binding arbitration.

6.4. No more than one royalty payment shall be due with respect to a sale of a particular Product. No royalty shall be payable under Section 6.2 with respect to sales of Products among LICENSEE and its Affiliates or Sublicensees or with respect to Products distributed without charge for use in research and/or development, in clinical trials or as promotional samples or otherwise distributed without charge to third parties.

6.5. For the purpose of determining amounts payable under this Agreement, any Net Sales denominated in currencies other than U.S. dollars shall be converted into U.S. dollars according to LICENSEE's reasonable standard internal conversion procedures, including LICENSEE's standard internal rates and conversion schedule. All payments to INSTITUTION shall be made in U.S. dollars.

7. REPORTS, PAYMENTS AND ACCOUNTING

7.1. Beginning with the first sale of an Orphan Drug Product, LICENSEE shall make written reports of royalty payments due, if any, to INSTITUTION within [***] days of [***] and [***]. This report shall state the number, description, and aggregate Net Sales of the applicable Product(s) received by LICENSEE during the previous completed calendar half year, and resulting calculations of royalty payments due INSTITUTION pursuant to Sections 6.2 through 6.5 for such completed calendar half year. Concurrent with the submission of each such report, LICENSEE shall pay INSTITUTION any royalties due for the [***] covered by such report.

7.2. LICENSEE agrees to keep and maintain records for a period of [***] years showing the sale, use and other disposition of Orphan Drug Products sold or otherwise disposed of under the license herein granted. Such records will include sufficient detail to enable the royalties payable hereunder by LICENSEE to be determined. LICENSEE further agrees to permit its books and records to be examined by an independent certified public accountant selected by INSTITUTION and acceptable to LICENSEE once per calendar year during the term of this Agreement, for the sole purpose of verifying the reports and payments made by LICENSEE. Such examination shall be made at LICENSEE'S place of business during ordinary business hours with at least [***] days prior written notice. Such examination is to be at the expense of INSTITUTION except in the event that the results of the audit reveal an under reporting of payments due INSTITUTION of [***] percent ([***]%) or more, then the audit costs shall be paid by LICENSEE within [***] days of notice by INSTITUTION to LICENSEE.

8. NEGATION OF WARRANTIES

8.1. Nothing in this Agreement is or shall be construed as:

(a) A warranty or representation by INSTITUTION as to the validity or scope of any Licensed Patent(s);

(b) A warranty or representation that anything made, used, sold, or otherwise disposed of under any license granted in this Agreement is or will be free from infringement of patents, copyrights, and other rights of third parties;

(c) An obligation to bring or prosecute actions or suits against third parties for infringement except to the extent and in the circumstances described in Article 13;

(d) Granting by implication, estoppel, or otherwise any licenses or rights under patents or other rights of INSTITUTION or other persons other than to the Licensed Patent(s), regardless of whether such patents or other rights are dominant or subordinate to any Licensed Patent(s); or

(e) An obligation to furnish any technology or technological information, except as expressly set forth in this Agreement.

8.2. Except as expressly set forth in this Agreement, INSTITUTION MAKES NO REPRESENTATIONS AND EXTENDS NO WARRANTIES OF ANY KIND, EITHER EXPRESS OR IMPLIED. THERE ARE NO EXPRESS OR IMPLIED WARRANTIES OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE.

9. REPRESENTATIONS AND WARRANTIES

9.1. INSTITUTION represents and warrants that it owns all right, title and interest in and to the Technology, Licensed Know-How and Licensed Patents, subject to the license set forth in Section 3.1.

9.2. INSTITUTION represents and warrants that it has not granted any third party right or interest in any of the Technology, Licensed Know-How or Licensed Patents that is inconsistent with the rights granted to LICENSEE herein and will not grant any third party such a right during the term of this Agreement.

9.3. INSTITUTION represents and warrants that it has the power to enter into this Agreement and the right to grant the rights granted herein to LICENSEE.

9.4. LICENSEE represents and warrants that it has the power to enter into this Agreement and meet its obligations under this Agreement.

9.5. INSTITUTION represents and warrants that Licensed Patents do not claim the use of Products in the Licensed Field.

10. INDEMNITY

10.1. LICENSEE agrees to indemnify, hold harmless, and defend INSTITUTION and its respective trustees, officers, employees, students, and agents (the “Indemnitees”) against any and all liability, damage, loss or expense incurred by or imposed on the Indemnitees or any one of them, arising out of third party claims arising out of the manufacture, use, sale, or other disposition of Product(s) by LICENSEE or its Affiliates or Sublicensee(s), or the exercise of the license granted herein, except to the extent arising out of the gross negligence or willful misconduct of any Indemnitee.

10.2. NEITHER PARTY SHALL BE LIABLE TO THE OTHER PARTY FOR ANY INDIRECT, SPECIAL, CONSEQUENTIAL, PUNITIVE OR OTHER DAMAGES WHATSOEVER, WHETHER GROUNDED IN TORT (INCLUDING NEGLIGENCE), STRICT LIABILITY, CONTRACT OR OTHERWISE.

11. INSTITUTION NAMES AND MARKS

11.1. LICENSEE agrees not to identify INSTITUTION or use the name of any INSTITUTION faculty member, employee, or student or any trademark, service mark, trade name, or symbol of INSTITUTION or that is associated with INSTITUTION in any promotional advertising or other promotional materials to be disseminated to the public without INSTITUTION's prior written consent, which consent shall not be unreasonably withheld. INSTITUTION and LICENSEE agree that reports in scientific literature and presentations of research and development work are not considered promotional materials. Promotional materials shall also not include disclosures required under any laws or government regulations or by the rules of any stock exchange of any country.

11.2. Notwithstanding Section 11.1, LICENSEE may publicly disclose, on its website or otherwise, that it has obtained from INSTITUTION an exclusive license under the Licensed Know-How.

12. TERM AND TERMINATION

12.1. This Agreement shall be effective as of the Effective Date and, unless earlier terminated in accordance with Sections 12.2 or 12.3, shall expire upon expiration of the last payment obligation of LICENSEE under Section 6.2. After the expiration of this Agreement, the license granted to LICENSEE hereunder shall become fully-paid, royalty-free, perpetual and irrevocable.

12.2. LICENSEE may terminate this Agreement as a whole or solely with respect to any country by giving INSTITUTION notice in writing at least thirty (30) days in advance of the effective date of termination selected by LICENSEE.

12.3. INSTITUTION may terminate this Agreement if LICENSEE is in material breach of any provision hereof and LICENSEE fails to remedy any such breach within sixty (60) days after receipt of written notice thereof by INSTITUTION. Upon any such termination, (i) LICENSEE and its Affiliates shall have six (6) months to complete the manufacture of any Products that then are work in progress and to sell their inventory of Products, provided LICENSEE pays the applicable royalties in accordance with Section 6.2, and (ii) INSTITUTION shall accept an assignment by LICENSEE of any sublicenses granted by LICENSEE to third parties, and any sublicense so assigned shall remain in full force and effect.

12.4. Surviving any termination are:

(a) LICENSEE's obligation to pay royalties accrued prior to termination;

(b) Any cause of action or claim of LICENSEE or INSTITUTION, accrued, because of any breach or default by the other party; and

(c) The provisions of Articles 7, 8, 10, 14 and 16.

13. ASSIGNMENT

Neither party may assign this Agreement or any part hereof without the express written consent of the other, which consent shall not be unreasonably withheld; provided, however, LICENSEE may assign this Agreement or any portion hereof to an Affiliate or to a successor of all or substantially all its assets, stock or business relating hereto without the written consent of INSTITUTION and shall provide INSTITUTION notice of any such assignment.

14. ARBITRATION

14.1. Any controversy arising under or related to this Agreement, and any disputed claim by either party against the other under this Agreement excluding any dispute relating to patent validity or infringement arising under this Agreement, shall be settled by arbitration in accordance with the JAMS rules.

14.2. Upon request by either party, arbitration will be initiated by a third party arbitrator mutually agreed upon in writing by LICENSEE and INSTITUTION within [***] days of such arbitration request. Judgment upon the award rendered by the arbitrator shall be final and nonappealable and may be entered in a court having jurisdiction thereof. The parties agree that any provision of applicable law notwithstanding, they will not request and the arbitrators shall have no authority to award punitive or exemplary damages against any party. The costs of the arbitration, including administrative fees and fees of the arbitrators shall be shared equally by the parties. Each party shall bear the cost of its own attorneys' fees and expert fees.

14.3. Any arbitration shall be held at a location mutually agreed upon by the parties.

14.4. The parties shall maintain the confidential nature of the arbitration proceeding and the Award, including the Hearing, except as may be necessary to prepare for or conduct the arbitration hearing on the merits, or except as may be necessary in connection with a court application for a preliminary remedy, a judicial challenge to an Award or its enforcement, or unless otherwise required by law or judicial decision.

15. NOTICES

All notices under this Agreement shall be deemed to have been fully given when done in writing and deposited in the United States mail, registered or certified, or overnight deliver service (e.g., DHL, Federal Express) and addressed as follows:

To INSTITUTION:

St. Jude Children's Research Hospital
262 Danny Thomas Place
Memphis, TN 38105
Attention: Technology Licensing Director

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

To LICENSEE:	Ultragenyx Pharmaceutical Inc., 60 Leveroni Court, Novato, CA 94949; Attention: Chief Executive Officer

Either party may change its address upon written notice to the other party.

16. CONFIDENTIALITY

INSTITUTION shall maintain this Agreement and the reports and any information provided by LICENSEE to INSTITUTION pursuant to Sections 5 and 7 in confidence and not disclose such information or reports to any third party, except as required by law and disclosed after notice, to LICENSEE and after requesting confidential treatment and a protective order, if available.

17. WAIVER

None of the terms of this Agreement can be waived except by the written consent of the party waiving compliance.

18. APPLICABLE LAW

This Agreement shall be governed by the laws of the State of New York, without reference to principles of conflicts of laws.

19. ENTIRE AGREEMENT

This Agreement constitutes the entire Agreement between LICENSEE and INSTITUTION and supersedes all prior communications, understandings and agreements with respect to the subject matter of this Agreement. This Agreement may not be amended except with a written agreement signed by LICENSEE and INSTITUTION.

IN WITNESS WHEREOF the parties have executed this Agreement effective as of the Effective Date Set forth above.

Ultragenyz Pharmaceutical, Inc.	St. Jude Children's Research Hospital
By: /s/ Tom Kassberg	By: /s/ J. Scott Elmer
Name: Tom Kassberg	Name: J. Scott Elmer
Title: CBO	Title: Director, Office of Technology Licensing

EXCLUSIVE LICENSE AGREEMENT

This License Agreement (the "**Agreement**") is made and entered into as of November 22, 2010, (the "**Effective Date**") by and between Saint Louis University ("**SLU**"), having an address at 3700 West Pine Mall, Fusz Memorial Hall, Second Floor, Saint Louis, Missouri 63108, and Ultragenyx Pharmaceutical Inc. ("**LICENSEE**"), having an address at 77 Digital Drive, Suite 210, Novato, California 94949.

WHEREAS, SLU is the owner by assignment of certain Patent Rights and Technology, as defined below, which SLU owns or controls, and the SLU has the right to grant licenses under such Patent Rights and Technology;

WHEREAS, SLU desires to have the Technology utilized in the public interest;

WHEREAS, LICENSEE has represented to SLU, to induce SLU to enter into this Agreement, that LICENSEE is engaged in the research and development of Beta-glucuronidase for commercial purposes.

NOW, THEREFORE, in consideration of the above premises and the mutual covenants contained herein, the parties hereby agree as follows:

1. DEFINITIONS

1.1. The term "**Affiliate**" means any person or entity which directly or indirectly owns or controls LICENSEE, or which is controlled by or under common control with LICENSEE. For purposes of this definition, "control" means the ownership by LICENSEE, directly or indirectly, of fifty percent (50%) or more of the outstanding equity securities of a corporation entitled to vote in the election of directors or the direct or indirect ownership by a person or entity of fifty percent (50%) or more of the outstanding equity securities of LICENSEE entitled to vote in the election of LICENSEE'S directors.

1.2. The term "**Confidential Information**" shall mean all ideas and information of any kind that are held in confidence by one Party and transferred, disclosed or made available by such Party to a receiving Party and are identified at the time of disclosure as being proprietary or confidential. The obligations in this Agreement with respect to Confidential Information shall not apply to any portion of the Confidential Information that the receiving Party can demonstrate by legally sufficient evidence (i) now or hereafter, through no act or failure to act on the part of the receiving Party, is or becomes public; (ii) is known to the receiving Party or one of its

Affiliates at the time such Party receives such Confidential Information from the disclosing Party; (iii) is hereafter furnished to the receiving Party by an unrelated Third Party without violating any agreement with the disclosing Party; or (iv) is independently developed by the receiving Party or one of its Affiliates without use of any Confidential Information received from the other Party. LICENSEE, prior to disclosure of information believed by LICENSEE to fall into parts (i)-(iv) of this Section, shall provide written notice to SLU with sufficient time so that SLU can make an independent assessment as to whether the information is or is not Confidential Information.

1.3. The term "**Fair Market Value**" mean the cash consideration which LICENSEE or its Sublicensee would realize from an unaffiliated, unrelated buyer in an arm's length sale of an identical item sold in the same quantity and at the same time and place of the transaction.

1.4. The term "**FDA**" means the United States Food and Drug Administration.

1.5. The term "**Field of Use**" means use of the beta-glucuronidase product candidate as a treatment for human disease.

1.6. The term "**First Commercial Sale**" means the first invoiced sale of a Licensed Product to a Third Party by LICENSEE following the receipt of any Regulatory Approval required for the sale of such Licensed Product.

1.7. The term "**Licensed Product**" means any service, product, process, or part thereof the manufacture, use, importation, sale or offer for sale of which in the absence of the license granted hereunder, would infringe a Valid Claim or during the period where any product has a license to Technology granted hereunder has market exclusivity as an Orphan Drug.

1.8. The term "**Net Sales**" means the gross sales of the Licensed Product invoiced by LICENSEE, its Affiliates or Sublicensees to a Third Party, less the following items:

(a) reasonable freight, packaging, shipping, storage, and insurance costs, if separately stated;

(b) amounts repaid or credited by reason of rejections, defects or returns or because of retroactive price reductions;

(c) rebates (price reductions, rebates to social and welfare systems, charge backs or reserves for chargebacks, cash sales incentives, government mandated rebates and similar types of rebates, e.g., Pharmaceutical Price Regulation Scheme and Medicaid and discounts for quantity purchases, cash payments, and for wholesalers and distributors).

No deductions shall be made for commissions paid to individuals whether they are with independent sales agencies or regularly employed by LICENSEE or Sublicensees and on its payroll, or for cost of collections.

1.9. The term "**Non-Commercial Research Purposes**" shall mean use of Patent Rights and Technology for academic research or other not-for-profit scholarly purposes which are undertaken at a non-profit or governmental institution that does not use the Patent Rights and Technology in the production or manufacture of products for sale or the performance of services for a fee.

1.10. The term "**Orphan Drug**" shall mean a Licensed Product for a specific indication for which the FDA has granted "Orphan-drug exclusive approval" as defined in 21 CFR Paid 316 or which be granted Orphan Drug exclusivity under similar regulations in the European Union or Japan.

1.11. The term "**Patent Costs**" means out-of-pocket expenses incurred by SLU in connection with the preparation, filing, prosecution, maintenance, and interference proceedings of patent applications and patents, including the fees and expenses of attorneys and patent agents, foreign and domestic, filing fees, maintenance fees, and annuity fees, but excluding costs associated with any patent infringement actions.

1.12. The term "**Patent Rights**" means the patent applications and patents listed in Exhibit A, including all related international filings, reissues, reexaminations, divisions, or continuations thereof and patent applications and any inventions, whether patented or not, which SLU owns or controls throughout the world, covering or related to the beta-glucuronidase product candidate**.**

1.13. The term "**Party**" means an individual, partnership, limited partnership, joint venture, trustee, trust, corporation, company, unlimited or limited liability company, unincorporated organization or other entity or a government, state or agency or political subdivision thereof.

1.14. "**Parties**" means SLU and LICENSEE.

1.15. The term "**Regulatory Approval**" means any approvals (including pricing and reimbursement approvals), licenses, registrations or authorizations of any national or international or local regulatory agency, department, bureau or other governmental entity, necessary for the manufacture and/or sale of a Licensed Product in a regulatory jurisdiction within the Territory.

1.16. The term "**Sale Transaction**" means the merger of LICENSEE with and into a Third Party, or the sale to, or other acquisition by, a Third Party, of all or substantially all of the assets of the business of LICENSEE in which the Patent Rights and Technology forms part, or all or substantially all, of the equity interests in LICENSEE, but excluding, for greater certainty, the sublicensing by LICENSEE to a Third Party of any of the rights granted to LICENSEE hereunder.

1.17. The term "**Sublicensee**" means any Party to which LICENSEE has sublicensed the rights granted to it under Section 2 below and in accordance with the terms thereof, but the term "Sublicensee" shall not include: (i) any Affiliate of LICENSEE or (ii) any Party who manufactures a Licensed Product for LICENSEE or an Affiliate of LICENSEE but does not sell such Licensed Products other than to LICENSEE or an Affiliate of LICENSEE.

1.18. The term "**Sublicensing Revenue**" means all upfront, milestone and royalty payments and other consideration received by LICENSEE from a Sublicensee in consideration for the sublicensing by LICENSEE of all or part of the rights granted to it under Section 3 below and in accordance with the terms thereof, except for direct reimbursement of research expenditures actually incurred and excluding any research and development grants. Any non-cash consideration so received by LICENSEE from Sublicensees shall be valued at its Fair Market Value as of the date of receipt.

1.19. The term "**Technology**" means scientific information, knowledge and know-how and materials related to [***].

1.20. The term "**Term**" has the meaning ascribed to it in Section 11.1.

1.21. The term "**Territory**" means World Wide.

1.22. The term "**Third Party**" means any Party other than LICENSEE, its Affiliates, Distributors, Sub-Distributors, or SLU.

1.23. The term "**Valid Claim**" means a claim in any unexpired patent or patent application listed in Exhibit A or contained in the Patent Rights that has not been disclaimed, revoked or held invalid or unenforceable by a final unappealable decision of a court or government agency of competent jurisdiction.

2. GRANT OF RIGHTS

2.1. License to Patent Rights and Technology. Subject to the terms and conditions hereof, SLU hereby grants to LICENSEE, who accepts, an exclusive license under the Patent Rights and the Technology in the Field of Use in the Territory, to develop, have developed, to make, have made, to use, have used, to import, have imported, to offer for sale, sell and have sold any Licensed Product. The license granted under this section shall remain in effect throughout the Term and shall, subject to Section 2, be sublicensable by LICENSEE and/or its Sublicensees through multiple tiers.

2.2. Affiliates and Distributors. LICENSEE may exercise its rights and delegate its obligations under this Agreement through and to its Affiliates, distributors and sub-distributors through multiple tiers. Such exercise shall not constitute a sublicense of the rights granted to it hereunder.

2.3. Retained Rights. The exclusivity of the above license is subject to the retained rights of SLU defined by:

(a) make, use and further develop the Patent Rights and Technology for its own educational, research and patient care purposes;

(b) grant to others non-exclusive licenses to make and use for Non-Commercial Research Purposes the subject matter described and claimed in Patent Rights and Technology;

(c) grant licenses to Third Parties outside the Field of Use expressly granted herein.

2.4. Federal Government Rights Reserved. Notwithstanding the exclusive license granted herein, the Federal Government shall receive all the rights to the Patent Rights and Technology as required by law or regulation to be reserved to the government. The Parties agree that the Federal Government is hereby granted a non-exclusive, non-transferable, irrevocable, royalty free license to practice or have practiced on its behalf throughout the world the Patent Rights and Technology. All rights granted in this Agreement are expressly granted subject to the rights of the Federal Government and such rights are specifically reserved to the Federal Government by this Agreement.

2.5. Consulting Agreements. In the event LICENSEE desires to have a consulting agreement with any SLU faculty member(s), any such consulting agreement will be separate and apart from this Agreement, and in accord with SLU policy and procedures.

2.6. Sponsored Research Agreements. In the event LICENSEE desires to fund research at SLU, such research agreements shall be separate and apart from this Agreement, and in accord with SLU policy and procedures.

3. SUBLICENSING

3.1. The right of the LICENSEE to sublicense, in whole or in part, the rights and obligations contained in this Agreement is prohibited without prior written consent from SLU, Should SLU grant to LICENSEE in writing the right to sublicense, in whole or in part the rights and obligations contained in this Agreement, any sublicense will be subject to the following:

(a) LICENSEE shall execute a written sublicense with each Sublicensee which shall be subject to LICENSEE’s rights and obligations under the terms of this Agreement. LICENSEE shall cause any such sublicense

agreement to contain terms that are at least as protective of the Patent Rights and the Technology and Confidential Information of SLU as the terms set forth in this Agreement, and that also include no provisions that would be in violation of the license grant set forth in this Agreement. Any such sublicense agreement shall further: (i) prohibit Sublicensee's further sublicense of the rights delivered hereunder; (ii) name SLU as an intended third party beneficiary of the obligations of Sublicensee without imposition of obligation or liability on the part of SLU to the Sublicensee; and (iii) bear signature from SLU indicating SLU's review and approval of the sublicense agreement. To the extent that any terms, conditions or limitations of any sublicense agreement are inconsistent with this Agreement, those terms, conditions and limitations are null and void against SLU, even through SLU has approved the sublicense in writing.

(b) LICENSEE shall be solely responsible for the enforcement of the terms of any sublicense and for collection of payment due thereunder.

(c) SLU shall be entitled to request a separate accounting from LICENSEE of any Sublicensing Revenue on a yearly or less frequent basis.

(d) Upon termination of this Agreement as provided hereunder, SLU will stand in the place of LICENSEE with respect to the Sublicensee for a period of [***] days during which time SLU will negotiate with Sublicensee in good faith and under reasonable terms and conditions to execute a new license between Sublicensee and SLU. If no new license is completed within the [***] day period, the sublicense will terminate.

4. PAYMENTS

4.1. License Issue Fee. LICENSEE shall pay to SLU a one-time, sign-on fee in the amount of Ten Thousand United States Dollars (USD $10,000.00) (the "License Issue Fee"), payable within [***] days after the Effective Date.

4.2. Milestone Payments. LICENSEE shall provide SLU with written notice within [***] days of its achievement of each of the milestone events set forth below. Within [***] days after delivering each such notice, LICENSEE shall pay to SLU the amounts set forth below:

Milestone	Amount
Approval of a glucuronidase-based enzyme therapy for treatment for MPS VII	$100,000

4.3. Royalty Payments. Subject to section 9.4, beginning after the first $[***] of cumulative worldwide sales of the product and during the Term. LICENSEE shall pay to SLU a royalty of:

4.3.1 [***] percent ([***]%) on Net Sales in [***], where there is a Valid Claim covering a Licensed Product.

4.3.2 [***] ([***]%) on Net Sales in [***] where there is a Valid Claim covering a Licensed Product or during the period where the Licensed Product has market exclusivity as an Orphan Drug.

Royalty payments shall be made in accordance with Section 4.5.

4.4. Additional expenses. Each party shall bear its own legal and professional expenses in connection with the negotiation and execution of this license agreement.

4.5. Reporting and Payment Terms. Commencing on the First Commercial Sale, LICENSEE agrees to deliver to SLU, within [***] days after each [***]-month period, CFO (or equivalent) signed payment of royalties owed and a report showing the information on which provided payments are calculated, including a breakdown of Net Sales of each Licensed Product on a country-by-country basis and to accompany each such report with the payment shown to be due thereby.

4.6. Currency Conversion. If any currency conversion is required in connection with any payments to SLU hereunder, Net Sales shall first be calculated in the relevant foreign currency and then converted to U.S. Dollars against the currency in question on the rate of exchange applicable using the currency exchange rates quoted by *Bloomberg Professional*, a

service of Bloomberg L.P., during the period of such Net Sales, or in the event *Bloomberg Professional* is not available, then *International Financial Statistics* (publisher, International Monetary Fund) during the royalty period of such Net Sales, for the currency of the country in which the sale is made at the average rate of exchange during the royalty period of such Net Sale

4.7. Royalty Stacking. If LICENSEE pays if obliged to pay royalties to a Third Party for patents necessary to the manufacture, use or sale of a Licensed Product(s), then LICENSEE may credit [***] percent ([***]%) of the Third Party royalties paid against royalties otherwise due to SLU for the Net Sales; provided, however that the royalties paid to SLU for the Net Sales shall not be less than [***] percent ([***]%) of those otherwise due. Such reduction of royalties allowed hereunder shall apply on an annual basis with no carryover of Third Party royalty balance from one calendar year to the following calendar year.

4.8. Development Assistance. SLU shall provide LICENSEE with all information related to the Patent Rights and Technology as may be known or possessed by SLU and may be reasonably necessary for LICENSEE to exploit the license granted in this agreement. SLU shall provide LICENSEE with reasonable technical assistance in connection with such transfer of the information related to the Patent Rights and Technology. Technical assistance after completion of the license is not to exceed [***] business hours of work, and LICENSEE shall pay SLU for any necessary technical assistance at a nominal rate of $[***] per hour, plus approved expenses. These expenses may include a single trip for two people to LICENSEE or an agent of LICENSEE to assist in technology transfer of the production process.

5. BEST EFFORTS

LICENSEE shall use its best efforts to (a) develop a Licensed Product and bring the Licensed Product to Market as soon as practicable, consistent with sound and reasonable business practices and judgment; and (b) LICENSEE shall market, promote, manufacture and sell the Licensed Product, whether it be itself or through Sublicensees, throughout the term of this Agreement. LICENSEE's failure to perform in accordance with this Section shall be grounds for SLU to terminate or render this license non-exclusive. In making this determination, SLU shall take into account the normal course of such programs conducted with sound and reasonable business practices and judgment and shall take into account the reports provided hereunder by LICENSEE.

6. INTELLECTUAL PROPERTY

6.1. Each Party shall own and retain all right, title and interest in all intellectual property that: (a) was created, discovered, developed or conceived and reduced to practice by such Party prior to the Effective Date; or (b) was created, discovered, developed or conceived and reduced to practice by such Party after the Effective Date but is unrelated to the subject matter of this Agreement.

6.2. LICENSEE (for itself and Sublicensees) acknowledges and agrees that SLU is and shall remain (as to LICENSEE) the owner of the Patent Rights and the Technology, and that LICENSEE (including its Affiliates and Sublicensees) has no rights in or to the Patent Rights or the Technology except as provided hereunder.

6.3. As between SLU and LICENSEE, any invention that is solely conceived under this Agreement by SLU, including any improvements and enhancements related to the Patent Rights or the Technology, whether patentable or not, shall be owned by SLU, including all intellectual property rights therein. SLU shall license any such improvements (to the extent it is contractually permitted to do so), to LICENSEE under the terms of this Agreement and free of any additional royalty expense. In the event SLU is not contractually permitted to Licenses any such Improvements to LICENSEE, SLU shall offer LICENSEE an option to obtain exclusive license rights to any improvements developed by SLU individually or jointly with others (to the extent contractually permitted to do so). The parties will negotiate in good faith for a period of time not to exceed [***] months to establish the terms of a separate license agreement related to any improvements and enhancements related to the Patent Rights or the Technology. In addition, any invention that is solely conceived under this Agreement by LICENSEE or its Affiliates Sublicensees, including any improvements and enhancements related to the Patent Rights or the Technology, whether patentable or not, shall be owned by LICENSEE, including all intellectual property rights therein.

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

7. DISCLAIMERS

7.1. SLU Representations. SLU represents and warrants that:

(a) it has full authority to enter into this Agreement;

(b) it is not a Party to or bound by any contract or any other obligation whatsoever that limits or impairs its ability to enter into this Agreement;

(c) it has and shall continue during the Term to have the full right and legal capacity to grant the rights granted to LICENSEE hereunder;

(d) it is not aware of any information or fact that would render any of the Patent Rights invalid or unenforceable and that it has exclusive title to and ownership of all Patent Rights and Technology;

(e) neither SLU nor any of its faculty, professors, students, staff or employees received funding from any Third Party to create, discover, develop or conceive and reduce to practice the Patent Rights and/or the Technology, which funding would entitle Third Party to any rights whatsoever to the results of any such funding; and

(f) the patents and patent applications listed in Exhibit A constitute all of the Patent Rights owned or Controlled by SLU in connection with the Technology (as used in this Section, “Controlled” means the ability of SLU to grant a license or sublicense, other than as a result of this Agreement and without violating the terms of any agreement or other arrangement with any Third Party).

7.2. LICENSEE Representations. LICENSEE hereby represents and warrants that:

(a) it has full authority to enter into this Agreement; and

(b) it is not a Party to or bound by any contract or any other obligation whatsoever that limits or impairs its ability to enter into this Agreement or to perform its obligations under this Agreement.

7.3. Warranty Disclaimer. SLU MAKES NO REPRESENTATIONS AND EXTENDS NO WARRANTIES OF ANY KIND, EITHER EXPRESSES OR IMPLIED. Nothing in this Agreement is or shall be construed as:

(a) a warranty or representation by SLU as to the validity or scope of any Patent Rights, issued or pending;

(b) a warranty or representation that anything made, used, sold or otherwise disposed of under any license granted in this Agreement is or will be free from infringement of patents, copyrights and other rights of Third Parties;

(c) a warranty of merchantability or fitness for a particular purpose;

(d) an obligation to bring or prosecute actions or suits against Third Parties for infringement, except to the extent and in the circumstances described in Section 9.3;

(e) a grant by implication, estoppel, or otherwise of any licenses under patent applications or patents of SLU or Third Parties other than as provided in Section 2 hereof; or

(f) a warranty of the usefulness, accuracy or safety of the Licensed Products.

7.4. Additional Disclaimer of Liability. SLU ADDITIONALLY DISCLAIMS ALL OBLIGATIONS AND LIABILITIES ON THE PART OF SLU FOR INDIRECT, PUNITIVE OR CONSEQUENTIAL DAMAGES, ATTORNEYS' FEES, EXPERTS' FEES AND COURT COSTS (EVEN IF SLU HAS BEEN ADVISED OF THE POSSIBILITY OF SUCH DAMAGES, FEES, OR COSTS) ARISING OUT OF OR IN CONNECTION WITH THE MANUFACTURE, USE, OR SALE OF THE PATENT RIGHTS AND TECHNOLOGIES LICENSED UNDER THIS AGREEMENT. LICENSEE, AFFILIATES AND SUBLICENSEE(S) ASSUME ALL RESONSIBILITY AND LIABILITY FOR LOSS OR DAMAGE CAUSED BY A PRODUCT MANUFACTURED, USED, OR SOLD BY LICENSEE, AFFILIATES AND SUBLICENSEE(S) WHICH IS A LICENSED PRODUCT(S) AS DEFINDED IN THIS AGREEMENT.

8. INDEMNIFICATION AND INSURANCE

8.1. Indemnification by LICENSEE. LICENSEE agrees to indemnify, hold harmless and defend SLU, its trustees, officers, employees and agents, against any and all liability and/or damages with respect to any claims, suits, demands, judgments or causes of action (collectively "SLU Losses") arising out of (a) the development, manufacture, storage, sale or other distribution, or any other use of Licensed Products or Patent Rights, or exercise of rights granted hereunder, by LICENSEE or Sublicensees, distributors, agents or representatives; and/or (b) the use by end-users and other Third Parties of Licensed Products. In the event any such claims, demands or actions are made, LICENSEE shall defend SLU at LICENSEE's sole expense by counsel selected by LICENSEE. No settlement, consent judgment or other voluntary final disposition may be entered into without the prior written consent of SLU, which consent shall not be unreasonably withheld.

8.2. Insurance. During the Term of this Agreement, LICENSEE shall procure and maintain occurrence-based comprehensive general liability insurance in amounts not less than $[***] per claim and $[***] annual aggregate and name SLU, its trustees, officers, employees and agents as an additional insured. Such comprehensive general liability insurance shall provide (i) [***] and (ii) [***]. If LICENSEE elects to self-insure all or part of the limits described above, such self-insurance program must be reasonably acceptable to SLU. LICENSEE agrees that no amount greater than the sum of $[***] shall be deductible under LICENSEE's primary coverage for SLU and LICENSEE against any claims or suits arising from alleged defects in Licensed Products. The minimum amounts of insurance coverage required shall not be construed to create or limit LICENSEE's liability with respect to its indemnification under this Agreement.

At the time as any Licensed Product is first used in commerce or in humans, LICENSEE shall provide SLU with a certificate or certificates of insurance evidencing that SLU has been named as an additional insured Party, along with its trustees, officers, employees and agents, and evidencing that the insurer(s) is/are required to notify SLU in writing at least [***] days in advance of any termination of the policy or certificate, or any modification that would cause LICENSEE no longer to be in compliance with the provisions of this Section, or would cause the representation and warranties set forth above in this Section no longer to be true, such

written notification to specify the reason for such termination, the nature of the proposed modification, as the case may be. It is expressly agreed by the Parties that the provisions of this Section regarding insurance shall in no way limit LICENSEE's indemnity obligation, except to the extent that LICENSEE's insurer(s) actually pays SLU amounts for which SLU is entitled to be indemnified under this Agreement, nor shall SLU have any obligation to pursue any insurer as a precondition to its rights to be indemnified by LICENSEE. If LICENSEE does not obtain replacement insurance within such [***] day period specified above, SLU shall have the right to terminate this Agreement effective at the end of such [***] day period without notice or any additional waiting periods.

9. PROSECUTION, MAINTENANCE AND ENFORCEMENT OF PATENT RIGHTS

9.1. Prosecution and Maintenance.

(a) SLU shall have full control over filing, prosecution and maintenance of the patent applications and patents contained in the Patent Rights. SLU will keep LICENSEE advised of the status of patent prosecution by promptly providing LICENSEE with copies of official communications about the patent applications and patents contained in the Patent Rights in sufficient time to allow for review and comment by LICENSEE. LICENSEE shall have reasonable opportunities to advise and cooperate with SLU in such filing, prosecution and maintenance.

9.2. Patent Costs.

(a) All Patent Costs shall be the responsibility of LICENSEE, whether incurred prior to or after the Effective Date of this Agreement. LICENSEE shall reimburse SLU for such Patent Costs incurred by and billed to the SLU prior to the Effective Date of this Agreement will be payable to SLU within 30 days after the Effective Date of this Agreement. Patent Costs incurred after the Effective Date of this Agreement, or Patent Costs incurred before, but billed to SLU after the Effective Date of this Agreement, shall be paid by LICENSEE within [***] days of the receipt of an invoice from SLU. Payments pursuant to this Section 9.2(a) are not creditable against royalties.

(b) LICENSEE may elect to surrender a portion or all of its license rights under the Patent Rights in any country by providing to SLU written notice of such intent at least [***] days contained in the Patent Rights, as set forth at Section 9.1, upon the expiration of the [***] day notice period (or such longer period specified in LICENSEE's notice). In the event LICENSEE elects to surrender any or all of license rights under the Patent Rights, such applicable patent(s) and/or patent application(s) shall be excluded from the definition of "Patent Rights" and from the scope of the license granted under this Agreement, and all rights relating thereto shall revert to SLU, and may thereafter be freely licensed by SLU to Third Parties.

9.3. Enforcement of Patent Rights.

(a) In the event LICENSEE or SLU becomes aware of any actual or potential infringement of any Patent Rights, that Party shall promptly notify the other and the Parties shall discuss the most appropriate action to take. SLU and LICENSEE will cooperate with each other to attempt to terminate such infringement without litigation.

(b) If attempts to abate such infringement are unsuccessful, LICENSEE shall consult with SLU and shall consider the views of SLU regarding the advisability of the proposed action and its effect on the public interest. LICENSEE may (without being required to) bring an action at its own expense, in which event SLU shall cooperate with LICENSEE as reasonably requested, at LICENSEE's expense. No settlement, consent judgment or other voluntary final disposition of the action may be entered into without the prior written consent of SLU, which consent shall not be unreasonably withheld. To the extent LICENSEE's recoveries from such infringement action exceed LICENSEE's expenses, LICENSEE agrees to pay SLU [***] percent ([***]%) of such excess recoveries; provided that any of LICENSEE's expenses not recovered shall be credited towards any future royalties, annual maintenance fees, milestone payments, the Minimum Annual Revenue or any other payments, as provided in Section 3. For greater certainty, such of LICENSEE's expenses in excess of recoveries shall not be credited towards any future Patent Costs, as provided in Section 9.2.

(c) If required by law, SLU shall permit any action under this Section to be brought in its name, including being joined as a Party-plaintiff, provided that LICENSEE shall hold SLU harmless from, and indemnify SLU against, any costs, expenses, or liability that SLU incurs in connection with such action.

(d) In the event that LICENSEE elects not to institute or prosecute any suit to enjoin or recover damages from any infringer, then SLU alone may, in its sole discretion and at its expense, initiate and conduct an infringement action and any settlement or award which may be obtained shall solely belong to SLU.

9.4. Third Party Infringement Claims.

(a) In the event any Licensed Product becomes the subject of a claim for patent or other proprietary right infringement anywhere in the world by virtue of the incorporation of the Patents Rights therein (a "Third Party Infringement Claim"), the Parties shall promptly give notice to the other and meet to consider the Third Party Infringement Claim and the appropriate course of action and LICENSEE shall remit any payments due to SLU under this Agreement in escrow from the date of such Third Party Infringement Claim until final resolution of such claim (the "Period"). LICENSEE shall have the right, but not the obligation, to conduct the defense at its own expense of any such Third Party Infringement Claim brought against LICENSEE and/or SLU, in which event SLU shall cooperate with LICENSEE as reasonably requested, at LICENSEE's expense. In no event may a settlement, consent judgment or other voluntary final disposition of the Third Party Infringement Claim be entered into without the prior written consent of SLU, which consent shall not be unreasonably withheld.

(b) In the event a Third Party Infringement Claim is dismissed by a final unappealable decision, monies held in escrow pending such final unappealable decision ("the Monies") will be remitted to SLU as entire fulfillment of LICENSEE's financial obligations hereunder during the Period.

(c) In the event a Third Party Infringement Claim is upheld, whether by a Court or a settlement, an amount equal to the Monies minus [***] percent ([***]%) of the consideration actually paid to the Third Party with respect to such claim will be remitted to SLU as entire fulfillment of LICENSEE's obligations hereunder during the Period. The balance of the Monies will be remitted to LICENSEE. Thereafter, LICENSEE shall pay to SLU a royalty of [***] percent ([***]%) on Net Sales in the country where such Third Party Infringement Claim was brought.

10. REPORTING, VERIFICATION AND PAYMENT

10.1. Books and Records. LICENSEE agrees to keep proper records of scientific research and keep records of the latest [***] years of Net Sales of Licensed Products in accordance with generally accepted accounting practices. Such records shall include all information necessary for the accurate determination of royalty payments, Sublicensing Revenue and milestone achievement.

10.2. Audit. SLU, at its own expense, shall have the right, [***] per calendar year, and upon [***] days prior written notice, to have a certified public accountant, selected by SLU and reasonably acceptable to LICENSEE, inspect and audit in the location(s) where such records are maintained, the records of LICENSEE during usual business hours for the sole purpose of, and only to the extent necessary for, determining the correctness of payments due hereunder by LICENSEE, with all information disclosed being deemed Confidential Information of LICENSEE. Such audit shall be completed within [***] business days, subject to extension by the auditor if the auditor reasonably determines in good faith that data or information it requires is not available and identifies the data or information required. Results of such audit shall be made available to the Parties. If the audit reflects an underpayment of amounts due to SLU pursuant to this Agreement, such underpayment shall be promptly remitted to SLU by LICENSEE. If the underpayment is equal to or greater than [***] ([***]%) percent of the amount that was otherwise due, SLU shall be entitled to have LICENSEE pay the reasonable out-of-pocket costs incurred by SLU to retain such independent certified public accountant to conduct such review. Any underpayment is subject to the late payment fee of Section 10.5.

10.3. Foreign Payments. Royalties based on Net Sales in any foreign country shall be payable to SLU in United States Dollars. Dollar amounts shall be calculated using the foreign exchange rate, as published by the Wall Street Journal, in effect for such foreign currency on the last business day of each quarter for which a report is required. Where royalties are due for Net Sales in a country where, for reasons of currency, tax or other regulations, transfer of foreign currency out of such country is prohibited, LICENSEE has the right to place SLU's royalties in a

bank account in such country in the name of and under the sole control of SLU; provided, however, that the bank selected be reasonably acceptable to SLU and that LICENSEE inform SLU of the location, account number, amount and currency of money deposited therein. After SLU has been so notified, those monies shall be considered as royalties duly paid to SLU and will be completely controlled by SLU.

10.4. Taxes. Taxes imposed by any foreign or United States governmental agency on any payments to be made to SLU by LICENSEE hereunder shall be paid by LICENSEE without deduction from any payment due to SLU.

10.5. Late Payments. Late payments shall be subject to a charge of [***] percent ([***]%) per month compounded. LICENSEE shall indemnify SLU for all attorneys' fees and costs incurred by SLU in obtaining a full payment of late payments owed to SLU. The payment of such late charges shall not prevent SLU from exercising any other rights it may have as a consequence of the lateness of any payment.

11. TERM AND TERMINATION

11.1. Term. Unless earlier terminated under this Section 11.2, this Agreement shall become effective as of the date of this Agreement and expire the latest date of when the Orphan Drug exclusively expires in the United States, Japan or the European Union, or until the last patent based on technology licensed hereunder shall terminate, lapse or invalidation of the last remaining Valid Claim in the Territory, at which time the licenses granted to LICENSEE under this Agreement to snake, have made, to use, have used, to import, have imported, to offer for sale, sell and have sold any Licensed Product shall be fully paid-up. For greater certainty, SLU acknowledges and agrees that during the Term, no royalties shall be due by LICENSEE to SLU for Net Sales in countries where there is no Valid Claim Covering a Licensed Product in such countries.

11.2. Termination.

(a) Termination by LICENSEE. LICENSEE may terminate this Agreement by giving a ninety (90) written notice to SLU. Further:

(i) LICENSEE shall pay all amounts due and owing as of the effective date of termination; and

(ii) LICENSEE shall submit a report of the type described in Section 10.1.

(b) Termination by SLU. SLU may terminate this Agreement if LICENSEE:

(i) materially breaches this Agreement in a manner that can be cured (including but not limited to failure to pay annual maintenance fees, milestones, royalties or reports thereof) and such breach remains uncured for ninety (90) days following written notice of breach, such notice stating the nature of the defaults claimed by SLU;

(ii) is subject to a petition for relief under any bankruptcy legislation, or makes an assignment for the benefit of creditors, or is subject to the appointment of a receiver for all or a substantial part of LICENSEE's assets, and such petition, assignment or appointment prevents LICENSEE (as a legal or as a practical matter) from performing its obligations under this Agreement, or such petition, assignment or appointment is not otherwise dismissed or vacated within ninety (90) days; or

(iii) files a claim against SLU related to the Licensed Product, Patent Rights and/or Technology.

11.3. Consequences of Expiration or Termination.

(a) In the event of expiration of this Agreement or termination of the Agreement for any reason whatsoever:

(i) neither Party shall be discharged from any liability or obligation to the other that became due or payable prior to the effective date of such expiration or termination;

(ii) SLU is under no obligation to refund any payments made by LICENSEE to SLU prior to the effective date of such termination or expiration;

(iii) the rights and obligations of the Parties under Sections 4.1, 6, 7, 8, 10.1, 10.2, 11.3, 12 and 13 shall survive any expiration or termination of this Agreement; and

(iv) in any sublicense, SLU will stand in the place of LICENSEE with respect to the Sublicensee for a period of ninety (90) days during which time SLU will negotiate in good faith under reasonable terms and conditions with Sublicensee to execute a new license between Sublicensee and SLU, and if no new license is completed within the ninety (90) day period, the sublicense will terminate.

(b) In the event of termination of the Agreement:

(i) if LICENSEE or its Sublicensees then possess Licensed Product in inventory, have started the manufacture thereof or have accepted orders therefore, LICENSEE or its Sublicensees shall have the right, for up to one hundred twenty (120) days following date of termination, to sell their inventories thereof, complete the manufacture thereof and market such fully manufactured Licensed Product, in order to fulfill such accepted orders, subject to the obligation of LICENSEE to pay SLU the royalty payments therefore as provided in Section 3 of this Agreement;

(ii) subject to Section 11.3(b)(i), LICENSEE shall discontinue the manufacture, use, marketing, offering for sale and sale of Licensed Products; and

(iii) LICENSEE shall provide a final report of the type described in Section 10.1, including any allowable post-termination sales, by no later than thirty (30) days following the expiry of the 120-day period referred to in Section 11.3(b)(i).

12. CONFIDENTIAL INFORMATION

Each of SLU and LICENSEE shall hold in confidence any Confidential Information (including trade secrets) disclosed by the other or otherwise obtained by such Party from the other Party as a result of this Agreement, and each of SLU and LICENSEE shall protect the confidentiality thereof with the same degree of care that it exercises with respect to its own information of a like nature, but in no event less than reasonable care. LICENSEE shall have the right to provide Confidential Information to its Affiliates and Sublicensees, subject to the confidentiality obligations imposed by this Section. Without the prior written consent of the disclosing Party, a receiving Party shall not use, disclose, or distribute any Confidential Information, in whole or in part, except as required to perform such Party's obligations under this Agreement or in exercise or furtherance of its rights under this Agreement. Access to the disclosing Party's Confidential Information shall be restricted to the receiving Party's employees, agents and consultants, who, in each case, need to have access to carry out a permitted use and are bound in writing to maintain the use and confidentiality restrictions of such Confidential Information. The obligations set forth in this Section shall survive any termination or expiration of this Agreement in perpetuity (with respect to trade secrets and confidential financial information) and for the length of the life of the patent (with respect to all other Confidential Information).

All Confidential Information communicated by SLU to LICENSEE, including, without limitation, information contained in patent applications, shall be received in strict confidence by LICENSEE, used only for the purposes of this Agreement and not disclosed by LICENSEE or its agents or employees ("Representatives") without the prior written consent of SLU, unless such information is required to be disclosed by law, provided that LICENSEE shall first give notice to SLU of such disclosure and shall have made a reasonable effort to maintain the confidentiality of such information. Nothing contained herein shall prevent LICENSEE from disclosing Confidential Information to Sublicensees so long as such Sublicensees agree to be bound by these confidentiality provisions.

13. CHOICE OF LAW; DISPUTE RESOLUTION

13.1. Governing Law/Venue. This Agreement is made in accordance with and shall be governed and construed in accordance with the laws of the State of Missouri. The Parties agree that any action to interpret and/or enforce the terms of this Agreement shall be brought in the Circuit Court for St. Louis City or the United States District Court for the Eastern District of Missouri, and the Parties consent to the jurisdiction and venue of those courts.

13.2. Amicable Resolution. The Parties shall attempt to settle any controversy between them amicably. To this end, a senior executive from each Party shall consult and negotiate to reach a solution. The Parties agree that the period of amicable resolution shall toll any otherwise applicable statute of limitations. However, nothing in this clause shall preclude any Party from commencing mediation if said negotiations do not result in a signed written settlement agreement within thirty (30) days after written notice that these amicable resolution negotiations have commenced.

13.3. Mediation. If a controversy arises out of or relates to this Agreement, or the breach thereof, and if the controversy cannot be settled through amicable resolution, the Parties agree to try in good faith to settle the controversy by mediation. The Party seeking mediation shall propose [***] mediators, each of whom shall be a lawyer licensed to practice by the state of Missouri, having practiced actively in the field of commercial law for at least [***] years, to the other Party who shall select the mediator from the list. The Parties shall split the cost of the mediator equally. The Parties agree that the period of mediation shall toll any otherwise applicable statute of limitations. However, nothing in this clause shall preclude any Party from commencing other courses of remedy, including but not limited to if said negotiations do not result in a signed written settlement agreement within [***] days after written notice that amicable resolution negotiations have commenced.

13.4. Costs. The Party prevailing on substantially all of its claims shall be entitled to recover its costs, including attorneys' fees, , as well as for any ancillary proceeding.

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

13.5. Survival. The provisions of this Section shall survive expiration or termination of this Agreement.

14. NOTICES; PAYMENT INFORMATION

Except as otherwise provided, payments to be made hereunder to SLU shall be made by wiring the required amount to SLU's bank in accordance with SLU's instructions or by mailing or sending by commercial courier checks to SLU's address. Except as otherwise provided, notices and reports provided for herein shall effectively be given by mailing the same by certified or registered mail or by delivery by commercial courier, in each case properly addressed with charges prepaid. For the purposes of making payments and giving notices, the addresses of the Parties are as follows:

Saint Louis University
3700 West Pine Mall
Fusz Memorial Hall, Second Floor
St Louis, MO 63108
Attn: Director, Office of Innovation and Intellectual Property

Ultragenyx Pharmaceutical Inc.
77 Digital Drive
Suite 210
Novato, CA 94949
Attn: Chief Executive Officer

or to such subsequent addresses as either Party may furnish the other by giving notice thereof as provided in this Section 14.

15. MISCELLANEOUS

15.1. Assignment. Any attempt to assign or transfer this Agreement or any portion thereof in violation of this Section shall be void. This Agreement and all of the provisions hereof shall be binding upon and inure to the benefit of the Parties hereto and their respective successors and permitted assigns, but neither this Agreement nor any of the rights, interests or obligations hereunder shall be assigned by any Party hereto without the prior written consent of the other

Party, nor is this Agreement intended to confer upon any other Party except the Parties hereto any rights or remedies hereunder; provided, however, that LICENSEE may assign any or all of its rights and interests and delegate its obligations hereunder to any of its Affiliates (whether present or future) (provided that no such assignment to an Affiliate shall relieve assignor of its obligations hereunder); and provided further, that LICENSEE may assign its rights and interests and delegate its obligations hereunder (i) in connection with a Sale Transaction or (ii) as collateral to any of LICENSEE's lenders or any of LICENSEE's (or its Affiliate's) lenders. This Agreement shall inure to the benefit of, and be binding upon, the Parties hereto and their successors and permitted assigns. For greater certainty, LICENSEE shall not be required to make any payment whatsoever to SLU for the cash or non-cash proceeds received by LICENSEE or the holders of LICENSEE's equity (as and when received) with respect to any Sale Transaction.

15.2. Headings. The headings used in this Agreement are for convenience of reference only and are not intended to be a part of or to affect the meaning or interpretation of this Agreement.

15.3. Amendment. No amendment or modification hereof shall be valid or binding upon the Parties unless made in writing and signed by the Parties.

15.4. Force Majeure. Any delays in performance by any Party under this Agreement (other than the payment of monies due) shall not be considered a breach of this Agreement if and to the extent caused by occurrences beyond the reasonable control of the Party affected, including but not limited to, acts of god, embargoes, governmental restrictions, strikes or other concerted acts of workers, fire, flood, explosion, riots, wars, civil disorder, rebellion or sabotage. The Party suffering such occurrence shall immediately notify the other Party and any time for performance hereunder shall be extended by the actual time of delay caused by the occurrence.

15.5. Independent Contractors. In making and performing this Agreement, SLU and LICENSEE act and shall act at all times as independent contractors and nothing contained in this Agreement shall be construed or implied to create an agency, partnership or employer and employee relationship between SLU and LICENSEE. At no time shall one Party make commitments or incur any charges or expenses for or in the name of the other Party except as specifically provided herein.

15.6. Use of SLU's Name. Except as otherwise provided herein or required by law, LICENSEE will not originate any publication, news release or other public announcement, written or oral, whether in the public press or otherwise, relating to this Agreement or to the performance hereunder, without the prior written approval of SLU, which approval will not be unreasonably withheld. Such planned publication, news release or other public announcement shall be provided at least fourteen (14) days in advance for approval by SLU. SLU agrees that LICENSEE may make known in promotional and technical literature that the Patent Rights and Technology were developed at SLU; provided, however, that such use shall not state or imply that SLU has any relationship with LICENSEE other than as licensor.

15.7. Markings. LICENSEE shall comply with all applicable United States and foreign statutes related to the marking of Licensed Product(s) and Licensed Product(s) packaging with patent pending, patent number(s), copyrights or other intellectual property notices and legends required to maintain the intellectual property rights licensed in this Agreement.

15.8. Entity Status. If LICENSEE or a Sublicensee does not qualify, or loses qualification, as a small entity as provided by the United States Patent and Trademark Office, LICENSEE must notify SLU immediately.

15.9. Publication. LICENSEE agrees that SLU (including its employees) shall have a right to publish on the Technology in scholarly journals in accordance with its general policies and academic mission, and that this Agreement shall not restrict, in any fashion, SLU's right to publish on the Technology provided however that SLU shall provide LICENSEE with advance notice of any publication.

15.10. Severability. If any term, condition or provision of this Agreement is held to be unenforceable by a court having proper jurisdiction for any reason, it shall, if possible, be interpreted rather than voided, in order to achieve the intent of the Parties to this Agreement to the extent possible. In any event, all other terms, conditions and provisions of this Agreement shall be deemed valid and enforceable to the full extent of the law.

15.11. Compliance with Law. LICENSEE shall comply with and shall insure that any Affiliate or Sublicensee complies with all government statutes and regulations that relate to Licensed Products, including, but not limited to, FDA statutes and regulations and the Export Administration Act of 1979 (50 App. U.S.C. §2401 *et seq*.), as amended, and the regulations promulgated thereunder, and any applicable similar laws and regulations of any other country. Without limiting the generality of the foregoing, LICENSEE agrees that all Licensed Products used or sold in the United States shall be manufactured substantially in the United States to the extent required by Federal law.

15.12. Export Control Regulations. The Technology is subject to, and LICENSEE agrees to comply in all respects with U.S. export controls under the Export Administration Regulations (15 C.F.R. Part 734 et seq.) and U.S. economic sanctions and embargoes codified in 31 C.F.R. Chapter V. LICENSEE agrees that LICENSEE bears sole responsibility for understanding and complying with current U.S. trade controls laws and regulations as applicable to its activities subject to this Agreement. LICENSEE agrees not to sell any goods, services, or technologies subject to this Agreement, or to release or disclose or re-export the same: (i) to any destination prohibited by U.S. law, including any destination subject to U.S. economic embargo; (ii) to any end-user prohibited by U.S. law, including any person or entity listed on the U.S. government's Specially Designated Nationals list, Denied Parties List, Debarred Persons List, Unverified List, or Entities List; (iii) to any foreign national in the U.S. or abroad without prior license if required; or (iv) to any user, for any use, or to any destination without prior license if required.

15.13. Violation of Laws and Reformation. The Parties hereby agree that neither Party intends to violate any public policy, statutory or common law, rule, regulation, treaty or decision of any government agency or executive body thereof of any country or community or association of countries, and that if any word, sentence, paragraph or clause or combination thereof of this Agreement is found, by a court or executive body with judicial powers having jurisdiction over this Agreement or any of the Parties hereto, in a final, unappealable order to be in violation of any such provision in any country or community or association of countries, such words, sentences, paragraphs or clauses or combination shall be inoperative in such country or community or association of countries, and the remainder of this Agreement shall remain binding

upon the Parties hereto. In lieu of such inoperative words, sentences, paragraphs or clauses, or combination of clauses, there will be added automatically as part of this Agreement, a valid, enforceable and operative provision as close to the original language as may be possible which preserves the economic benefits to the Parties.

15.14. Waiver. None of the terms, covenants, and conditions of this Agreement can be waived except by the written consent of the Party waiving compliance. Waiver of one term, covenant or condition, shall not be construed as waiver of any other term, covenant or condition.

15.15. Entire Agreement. This Agreement and Exhibits attached hereto contain the entire agreement and understanding between the Parties with respect to the subject matter hereof, and merges all prior discussions, representations and negotiations with respect to the subject matter of this Agreement.

15.16. Counterparts. This Agreement may be executed in counterparts, each of which shall be deemed an original and all of which when taken together shall be deemed but one instrument.

IN WITNESS WHEREOF, the parties hereto have executed this Agreement by their duly authorized officers or representatives.

SAINT LOUIS UNIVERSITY

By: /s/ Raymond C. Tait
Title: Vice President, Research

ULTRAGENYX PHARMACEUTICAL, INC.

By: /s/ Emil D. Kakkis
Title: President and Chief Executive Officer

SUPPLY AGREEMENT

between

CREMER OLEO GmbH & **Co KG,** Glockengiesserwall 3, 20095 Hamburg,

Germany

— hereinafter referred to as Cremer —

and

Ultragenyx Pharmaceutical Inc, 60 Leveroni Court, Suite 200, Novato, California 94949, United States of America

— hereinafter referred to as Ultragenyx —

- each party also referred to as a "Party" and jointly as the "Parties" –

Preamble

Whereas, Cremer is a producer of oleo chemical products;

Whereas, Ultragenyx is a biotechnology company committed to bringing life-enhancing therapeutics for patients with rare and ultra-rare genetic diseases, also known as orphan diseases, to market;

Whereas, the Parties desire that Cremer supplies to Ultragenyx the product Triheptanoin (hereinafter also referred to as the "**Product**") in bulk form pursuant to the terms and conditions of this Agreement;

Whereas, Ultragenyx intends to process the Product into a pharmaceutical product in the meaning of Sec. 2 German Pharmaceuticals Act (Arzneimittelgesetz —AMG) and to market the processed Product in the Field (as defined below) (hereinafter referred to as the "Purpose"); and

Whereas, Ultragenyx intends to obtain regulatory approval for the processed Product as a pharmaceutical product in the meaning of Sec. 2 AMG.

Now therefore, the Parties hereto agree as follows:

Article 1 Supply of Product

1) Subject to the terms and conditions set forth in this Agreement Cremer shall supply Ultragenyx with the Product free from defect and meeting the product specification attached to this Agreement as **Annex A** (the "Product Specifications").

2) Cremer shall supply Ultragenyx exclusively with the Product worldwide. The aforesaid exclusivity is limited to [***] (collectively, the "Field"). Cremer may supply the Product to other customers outside of the Field.

3) Ultragenyx shall purchase the Product exclusively from Cremer.

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

Article 2 Orders and Delivery

1) The Product will be ordered by Ultragenyx through purchase orders. Purchase orders shall be submitted in any written or electronic form or by facsimile, setting out the quantity of Product required and the date for delivery. Cremer shall give its order confirmation in writing stating the quantity and Price (as defined below). Cremer shall not be obliged to deliver the Product in the absence of a written order confirmation given to Ultragenyx.

2) Delivery of the Product in bulk form by Cremer shall be EXW (Incoterms 2010), unless otherwise agreed in writing by the Parties.

3) Within [***] days of execution of this Agreement, Cremer shall deliver to Ultragenyx the Master Batch Record for the Product for Ultragenyx to review.

4) All Product shall be delivered with the applicable certificate of analysis and batch records for the Product delivered and an invoice for the quantity of Product delivered.

5) If Ultragenyx obtains regulatory approval for the processed Product, the Parties shall enter into a separate commercial supply agreement for the Product that sets forth the forecasting and ordering mechanism for commercial supply of the Product, enablement of the manufacturing process in the event of a failure to supply, the term of such commercial supply agreement and other customary terms and conditions.

Article 3 Prices and payment

1) The prices payable by Ultragenyx to Cremer for the Product (the “Price”) shall be agreed [***] every contract year; provided, that the Price may not increase more than the [***] for such period or [***]%, whichever is higher. At the date of signing the Parties agree on a Price of €[***] per kilogram for the Product.

2) If the parties cannot agree on a price for the Product by the beginning of a following contract year, Cremer may refuse to deliver the Product to Ultragenyx until the Parties agreed on a respective price.

3) Payments shall be made by Ultragenyx in Euro and within [***] days after receipt of a proper invoice.

4) Transfer of title with respect to any Product shall be subject to full payment and settlement of all claims Cremer may have against Ultragenyx in connection with the execution of this Agreement.

Article 4 Specification; Warranties; Cremer’s Liability; Indemnification

1) The Parties assume that the Product constitutes an active pharmaceutical ingredient in the meaning of Sec. 4 para. 19 AMG. Ultragenyx shall process the Product into a pharmaceutical product in the meaning of Sec. 2 AMG and market the processed Product as a pharmaceutical product in the meaning of Sec 2 AMG and to perform clinical trials. Cremer does not participate in the processing, manufacturing and marketing of the respective pharmaceutical product or in the clinical trials.

2) Upon execution of this Agreement and any purchase order, Cremer shall provide Ultragenyx with following documentation regarding the Product: Certificate of Analysis and the applicants' part of the Drug Master File once compiled.

3) Cremer represents and warrants that all quantities of Product delivered under the Agreement were manufactured in accordance with GMP. The Product shall be free from defects if it is within the specifications according to Annex A.

4) Cremer represents and warrants that it has not received any written notice from a third party alleging that the manufacture, use or sale of the Product infringes intellectual property rights of a third party.

5) Ultragenyx will perform final release of the Product. Ultragenyx may rely on the documentation provided by Cremer and Ultragenyx will not need to independently test the Product unless Ultragenyx determines such independent testing is necessary. In the event that the Product fails to conform to the Product Specifications, and/or GMP, Ultragenyx may reject the Product by giving written notice to Cremer within [***] days after receipt of the Product and all documentation (except such [***] day period will not apply for any latent defect). Within [***] days following receipt of the rejected and returned Product from Ultragenyx, Cremer will, at Ultragenyx's choice, replace such quantity of Product with Product conforming to the Product Specifications, and GMP or refund Ultragenyx the Price paid for such Product.

6) Cremer does not warrant or represent that the Product is effective in a pharmaceutical way within the meaning of Sec. 4 para. 19 AMG. Cremer does not warrant or represent that the Product is safe in a pharmaceutical and pharmacological way. Cremer does not warrant or represent that the Product is suitable for the intended Purpose by Ultragenyx. Cremer is not a pharmaceutical manufacturer within the meaning of Sec. 4 para. 18 AMG. Cremer's liability in connection with the Purpose and the processing and marketing of a pharmaceutical product is excluded. No. 9 below applies.

7) Except for a claim arising out of Cremer's intentional misconduct or gross negligence under this Agreement, in the event of legal proceedings being instituted against Cremer by a third party arising out of Ultragenyx's development, processing and commercialization of the Product, Ultragenyx shall indemnify and keep indemnified Cremer in full against all damages, losses, injuries, costs and expenses in connection with such legal proceedings. Cremer will inform Ultragenyx about any legal proceedings being instituted against Cremer without delay. Ultragenyx shall control the respective legal proceedings but shall not settle any claim that admits fault on behalf of Cremer without Cremer's consent (not be unreasonably withheld).

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

8) In the event of legal proceedings being instituted against Ultragenyx by a third party arising out of Cremer's intentional misconduct or gross negligence under this Agreement, Cremer shall indemnify and keep indemnified Ultragenyx in full against all damages, losses, injuries, costs and expenses in connection with such legal proceedings. Ultragenyx will inform Cremer about any legal proceedings being instituted against Ultragenyx without delay. Cremer shall control the respective legal proceedings but shall not settle any claim without Ultragenyx's consent (not be unreasonably withheld).

9) Cremer's liability arising from this Agreement is limited to intentional misconduct or gross negligence. This limitation of liability does not apply to the injury of the life, body or health of a person, to claims according to the Product Liability Act (Produkthaftungsgesetz) or any other coercive legal liability claims.

10) NEITHER PARTY MAY CLAIM AND NEITHER PARTY IS LIABLE FOR CLAIMS FOR INDIRECT DAMAGES AND LOSSES, SUCH AS SPECIAL OR CONSEQUENTIAL LOSS OR DAMAGE, ANY LOSS OF ACTUAL OR ANTICIPATED PROFIT, OR REVENUE, ANTICIPATED SAVINGS OR BUSINESS OR DAMAGE TO GOODWILL OR BRAND EQUITY, ARE EXCLUDED.

Article 5 Term and Termination

1) This Agreement shall become effective on the date of its execution and shall remain in force for [***] years (the "Initial Term"). Thereafter, the Agreement shall be automatically renewed for additional [**] year periods (each a "Renewal Term", the Initial Term and all Renewal Terms, the "Term") unless either Party notifies the other Party of its intention not to renew in writing at least three calendar months before the expiration of the then current Term.

2) If a Party materially breaches an obligation under this Agreement and does not cure such breach within sixty (60) days of receiving notice of such breach from the non-breaching Party, the non-breaching Party may terminate this Agreement immediately upon written notice to the breaching Party.

3) Every termination has to be in writing.

Article 6 General Terms and Conditions

The application of General Terms and Conditions of any Party is excluded.

Article 7 Product Development

At the request and expense of Ultragenyx, Cremer shall perform development work for Ultragenyx to develop new formulations of the Product. All such work shall be performed pursuant to a statement of work (including a budget) to be agreed upon by the Parties and attached as an annex to this Agreement (each, a "Statement of Work"). In the event that in the course of performing a Statement of Work new Product know-how and intellectual property rights may result, can be created or have been created the Parties will enter into a separate Agreement in order to define the rights and duties regarding the aforesaid know how and intellectual property rights.

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

Article 8 Invalidity

In the event that any individual clauses of these terms and conditions are, or shall become, invalid, this shall not affect the validity of the remaining clauses. An invalid condition shall be deemed to have been replaced by such provision which is legally valid and corresponds nearest to the economic purpose of the clause originally deemed invalid.

Article 9 Applicable Law; Modifications; Annexes; Miscellaneous

1) The laws of the Federal Republic of Germany shall apply to the Agreement and any legal relations thereof, especially any purchase order, between Cremer and Ultragenyx shall be governed by that law. The law of the United Nations Conventions of the formation of Agreements for the international sale of goods (CISG) is excluded. Exclusive place of Jurisdiction is Hamburg, Germany.

2) No addition or modification to this Agreement shall be valid unless made in writing and signed by the Parties.

3) The Annex attached to this Agreement form an integral part of the Agreement.

4) This Agreement, including the Annexes and any Statement of Work, constitutes the entire agreement between the Parties concerning the subject matter hereof and supersedes all written or oral prior agreements or understandings with respect thereto except the Confidentiality Agreement between the parties dated September 26th, 2012. This Agreement shall be binding upon and shall inure to the benefit of the Parties hereto, their successors and assigns.

5) All waivers must be in writing and signed by the Party to be charged. Any waiver or failure to enforce any provision of this Agreement on one occasion will not be deemed a waiver of any other provision or of such provision on any other occasion.

6) Each Party must deliver all notices, consents, and approvals required or permitted under this Agreement in writing to the other Party at the address specified above, by personal delivery, by certified or registered mail (postage prepaid and return receipt requested), by a nationally-recognized overnight carrier, or by facsimile transmission with electronic confirmation of transmission. Notice will be effective upon receipt or refusal of delivery. Each Party may change its address for receipt of notice by giving notice of such change to the other Party.

7) This Agreement may be executed in counterparts by original signature, facsimile or PDF files, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

Hamburg, November 19th, 2012

/s/ Thomas Kassberg
Ultragenyx Pharmaceutical Inc

/s/ Dr. R. Stephan
CREMER OLEO GmbH & Co KG

CREMER OLEO GmbH & Co. KG
Postfach 10 11 20, D-20007 Hamburg
Tel: 040/320 11-0, Telefax 320 11-400

Annex A — Specification of the Product
Trihepatanoin (Heptansäuretriglycerid)

No	Test	EP method	Limits
1	[***]	[***],	[***]
2	[***]	[***]	[***]
3	[***]	[***]	[***]
4	[***]	[***]	[***]
5	[***]	[***]	[***]
6	[***]	[***]	[***]
7	[***]	[***]	[***]
8	[***]	[***]	[***]
9	[***]	[***]	[***]
10	[***]	[***]	[***]
11	[***]	[***]	[***]
12	[***]		[***]
13	[***]	[***]	[***]
18	[***]	[***]	[***]

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

Exhibit 10.10

Development and Clinical Supply Agreement
Contract Number: E86A1D4C-B3F9

Between

Rentschler Biotechnologie GmbH

- and -

Ultragenyx Pharmaceutical Inc.

[***] Certain information in this document has been omitted and filed separately with the Securities and Exchange Commission. Confidential treatment has been requested with respect to the omitted portions.

CONTENTS

This Agreement (this "Agreement") is made on the date of the last signature shown below ("**Effective Date**")

Between:

(1) **Rentschler Biotechnologie GmbH**, Erwin-Rentschler-Straße 21, 88471 Laupheim, Germany (hereafter referred to as **"Rentschler"**); and,

(2) **Ultragenyx Pharmaceutical Inc.**, 60 Leveroni Court, Novato, CA 94949, USA (hereafter referred to as **"Ultragenyx").**

Purpose and Scope:

(A) Rentschler is in the business of providing biotechnology development services including without limitation process development, validation, scale up services, production and product manufacturing services, quality assurance, regulatory support, analytical development, fill and finish services and quality control analysis in respect of intermediate drug products and bulk form active pharmaceutical ingredients; and

(B) Ultragenyx is in the business of drug development and wishes to engage and contract with Rentschler for development services pursuant to which Rentschler is to further develop Ultragenyx's laboratory scale process used in the manufacture of the recombinant enzyme beta-glucuronidase known as UX003 for initial scale up and validation of the process (**"Project"**). The Project is more fully described in the initial Project Plan in **Schedule Two**; **and,**

(C) Rentschler is willing to provide the Services pursuant to the Project to Ultragenyx which Ultragenyx is willing to accept on the terms and conditions set out in this Agreement. This Agreement relates to the Quality Agreement with the contract number: 0467A7F4-15C7-.

Now it is Agreed as follows:

1 Definitions

For purposes of this Agreement, the terms defined in this Section shall have the respective meanings set forth below:

1.1 "Affiliate" means any Company, partnership or other entity which directly or indirectly Controls, is controlled by or is under common control with the relevant Party to this Agreement. "Control" means the ownership of more than fifty percent (50%) of the issued share capital or the legal power to direct or cause the direction of the general management and policies of the Party in question;

1.2 "Agreement" means this Development and Clinical Supply Agreement in its entirety including all Schedules;

1.3 “Applicable Laws” means all applicable laws, rules, regulations and guidelines that apply in Germany to the Services;

1.4 “Binding Reservation” means a reservation made by Ultragenyx to Rentschler to use the Rentschler facility for GMP manufacturing of Drug Substance, which reservation start date shall be agreed and documented in writing by the Parties.

1.5 “Business Day” means any day which is not a Saturday, a Sunday or a German public holiday and references to any time shall be to German time (GMT+one hour);

1.6 “Cell Line” means the cell line as described in **Schedule One** or any other cell line agreed between the Parties in accordance with **Section 3.2** below. Where a strain has been specified in the Specification, references in this Agreement to Cell Line refer to that specified strain;

1.7 “Drug Substance” means the recombinant enzyme beta-glucuronidase;

1.8 “Drug Product” means the finished dosage form of the Drug Substance;

1.9 “Gewaehrleistungen” means certain contractual obligations hereunder, which in case of a breach have the consequences as ascribed to them in this Agreement, provided that in case of damages such damages must be caused by negligence or willful misconduct;

1.10 “GMP” means Good Manufacturing Practices as regulated under the German Pharmaceuticals Act (“Arzneimittelgesetz”), the German Regulation for Manufacturing of Medicinal Products and Active Ingredients (Arzneimittel- und Wirkstoffherstellungsverordnung), and the Guidelines to Good Manufacturing Practice of Medicinal Products for Human and Veterinary Use of the European Union, each of the foregoing as amended from time to time. Additionally, it shall mean the compliance with the Code of Federal Regulations, Title 21, Chapter I, Parts 210 and 211, as amended from time to time;

1.11 “Change Order” has the meaning set forth in **Section 2.7**;

1.12 “Confidential Information” means any and all information of whatever nature relating to either Party and their business affairs and all such other information relating to technology of whatever nature and application including, without limitation, all information relating to the Cell Line, Process, Product and strain, regardless of whether such information is specifically designated as confidential and regardless of whether such information is in written, oral, electronic or other form;

1.13 “Group” means the relevant Party and Affiliates from time to time;

1.14 “Intellectual Property Rights” means all intellectual property rights, including (without limitation) patents, supplementary protection certificates, petty patents, utility models, trade marks, database rights, rights in designs, copyrights and topography rights (whether or not any of these rights are registered, and including applications and the right to apply for registration of any such rights) and all inventions, know-how, trade secrets, techniques and confidential information and other proprietary knowledge and information, and all rights and forms of protection of a similar nature or having equivalent or similar effect to any of these which may subsist anywhere in the world, in each case for their full term, and together with any renewals or extensions;

1.15 “Party” shall mean Rentschler or Ultragenyx individually and “Parties” shall mean Rentschler and Ultragenyx jointly;

1.16 “Permitted Recipients” shall mean the employees of Rentschler, Inc., (the sales and distribution unit of Rentschler located at 400 Oser Ave,, Suite 1650, Hauppauge, NY 11788, USA) and the directors, officers or employees of the respective Party who are required, on a strict need to know basis, in the course of their duties to receive and consider the Confidential Information for the purpose of enabling the relevant Party to perform its obligations under this Agreement provided that such persons are under obligations of confidence no less onerous than those contained herein at **Section 11** which are imposed on the recipient Party;

1.17 “Price” means the price for the Services as defined in **Schedule Two**;

1.18 “Process” means the method of manufacture and processing of the Product from the Cell Line which, pursuant to the Services, is to be further developed by Rentschler under this Agreement;

1.19 “Product” means Drug Substance or Drug Product as required by the context;

1.20 “Project Plan” means the plan describing all activities to be performed under this Agreement, including any update and amendment of the Project Plan, as evidenced by a Change Order. The initial Project Plan is included in **Schedule Two**;

1.21 “Quarantine Production” means any production of Product with materials that were not released or were delivered without a certificate of release;

1.22 “Quarantine Shipment” means any transport of a batch of Product before a certificate of release has been issued;

1.23 “Schedule” means the schedule (or schedules as appropriate) to this Agreement which specify the Services, payment terms and other relevant details referred to under this Agreement;

1.24 “Services” means any or all parts of the services to be performed under this Agreement in pursuance of the Project as more fully described in **Schedule Two**;

1.25 "Specifications" means the specifications of the Product as described in **Schedule Three**, as amended from time to time;

1.26 "Steering Committee" means the body organised in accordance with and pursuant to the rules under **Section 7**;

1.27 "Testing Laboratories" means any third party qualified according to Rentschler's standard operating procedures that is instructed by Rentschler to carry out tests on the Cell Line, the Process, Ultragenyx Materials or the Product pursuant to the performance of the Services under this Agreement;

1.28 "Ultragenyx Materials" means the Cell Line and all other materials provided by Ultragenyx, its Affiliates or agents to Rentschler and as described in **Schedule One**;

1.29 "Working Package" means a particular activity or series of activities that constitute a main step in the performance of the Services and which is more clearly identified in **Schedule Two** by the breakdown of the Services into separate stages;

2 Provision of Services

2.1 Rentschler will perform the Services diligently using its professional skill and care in accordance with the terms of this Agreement and the Project Plan and will use its commercially reasonable endeavours to meet the Specifications. Rentschler shall perform the Services under this Agreement according to the Project Plan, in accordance with GMP (as applicable per **Section 2.2**), and subject to terms and conditions as set out herein. Rentschler will conduct all of its activities under this Agreement in compliance with all Applicable Laws.

2.2 Only those parts of the Services will comply with GMP that are explicitly designated to comply with GMP in **Schedule Two**. Should during the term of this Agreement new interpretations of GMP (the **"Interpretations"**) become available, the Parties will discuss the Interpretations and decide on their application regarding the Services. Should the Parties be unable to reach an agreement on the applicability of Interpretations, the final decision shall be with Ultragenyx. However, Rentschler is in any such case entitled to increase the Price if and to the extent Interpretations require additional Services directed specifically to the Process and/or Product (i.e., not to include services which Rentschler would have to perform to be able to provide GMP conforming services also to other customers).

2.3 As of the Effective Date, Ultragenyx is committed to engaging Rentschler to provide the Services and to paying Rentschler for such Services, in each case, as generally described in **Schedule Two**. However, because of the inherent uncertainty in timelines for Product development, the Parties hereby agree to discuss and finalize, after the Effective Date, a Binding Reservation for Rentschler's GMP manufacturing of Drug Substance. Once a Binding

Reservation has been made, but subject to the last paragraph in this **Section 2.3,** if Ultragenyx cancels the Binding Reservation, the Exit Fee is as follows:

2.3.1 In case of a cancellation during the period from and including the [***] to and including the [***] month before the start date of the Binding Reservation [***]% of the total remuneration for the respective Services;

2.3.2 In case of a cancellation during the period from and including the [***] to and including the [***] month before the start date of the Binding Reservation [***]% of the total remuneration for the respective Services;

2.3.3 In case of a cancellation during the period from the [***] to and including the [***] month before the start date of the Binding Reservation [***]% of the total remuneration for the respective Services;

2.3.4 In case of a cancellation within [***] month before the start date of the Binding Reservation [***]% of the total remuneration for the respective Services.

Rentschler shall use commercially reasonable efforts to mitigate any losses or damages it may incur due to Ultragenyx's cancellation of the Binding Reservation. If Rentschler mitigates such losses or damages so that it incurs no damage or significantly less damage as a result of the cancellation, Ultragenyx must only reimburse Rentschler for the unmitigated costs of the cancellation. This applies in particular if Rentschler can use the production time for another comparable manufacturing.

For clarity, if (1) Ultragenyx cancels the Binding Reservation because [***], then in either case, the foregoing Exit Fee [***].

2.4 If (1) Ultragenyx cancels or postpones the Services concerning Drug Product for a reason within Ultragenyx's control and not the result of Rentschler's policies, or (2) other deviations to the respective timelines stated in **Schedule Two** are caused by reasons within Ultragenyx's control and not the result of Rentschler's policies, then in either case, Rentschler is entitled to require that Ultragenyx pay the following reimbursement for the unused production facilities:

2.4.1 In case of a cancellation, postponement or delay during the period from the [***] to and including the [***] week before the production time: [***]% of the remuneration for the respective Services;

2.4.2 In case of a cancellation, postponement or delay during the period from the [***] week to and including the [***] week before the production time: [***]% of the remuneration for the respective Services;

2.4.3 In case of a cancellation, postponement or delay during the period from the [***] week to and including the [***] week before the production time: [***]% of the remuneration for the respective Services.

Rentschler shall use commercially reasonable efforts to mitigate any losses or damages it may incur due to Ultragenyx's cancellation, postponement or delay regarding the Drug Product. If Rentschler mitigates such losses or damages so that it incurs no damage or significantly less damage as a result of the cancellation, postponement or delay in the provision of materials or information, Ultragenyx must only reimburse Rentschler for the unmitigated costs of the cancellation, postponement or delay. This in particular applies if Rentschler can use the production time for another order.

For clarity, if Ultragenyx notifies Rentschler of a cancellation, postponement or delay prior to the [***] week before the production time, then the foregoing reimbursement shall [***].

2.5 Rentschler shall make available to Ultragenyx within a reasonable period of time upon completion of any Working Package as outlined in the Project Plan and – irrespective of the status of the Services – upon reasonable request of Ultragenyx, copies of all relevant documentation and information resulting from the Services in order to enable Ultragenyx to evaluate the status and to meet any statutory or regulatory requirements.

2.6 In the performance of the Services Rentschler may subcontract certain part(s) of the Services to a Testing Laboratory and is permitted to sublicense to such Testing Laboratory, but only for the purpose of performing those part(s) of the Services, any license from or grant of rights by Ultragenyx to Rentschler in respect of Ultragenyx Intellectual Property Rights and Ultragenyx Materials. Rentschler shall procure that all Testing Laboratories are subject to confidentiality obligations and intellectual property obligations corresponding to the obligations under **Section 11** and **Section 12** below. For clarity, Rentschler shall obtain Ultragenyx's prior written consent before subcontracting Services to any third party that is not a Testing Laboratory.

2.7 The Services (including the Price thereof) as described on the Project Plan may be amended by a written change order that specifies the particular change(s), including any changes to the nature of the Services, the timeline for such Services, and the Price associated with such Services (a **"Change Order"**). A Change Order may be submitted by either Party to the other, provided that such Change Order shall not be effective until signed by Ultragenyx and Rentschler in accordance with the provisions of **Section 18.1**.

2.8 Ultragenyx acknowledges and accepts that the Services are dependent upon living systems and that they are experimental in nature. Accordingly, the Parties agree that because of these factors and the unpredictable nature of the Services, the timelines and anticipated results expected by the Parties that are more fully described in the Schedules are estimates only and that provided Rentschler has complied with **Section 2.1** it shall not be liable for failing to achieve any target results, milestones or timelines or completing the Services.

2.9 As Ultragenyx wishes to have a prompt start with the clinical program, Rentschler will manufacture the first batch of Drug Substance after the consolidation run according to GMP (**"First Batch"**). Ultragenyx is aware that a successful GMP engineering run is required to confirm feasibility of the manufacturing of Drug Substance. Therefore, the Parties agree that Rentschler shall not be obligated to meet any Specifications for the First Batch and Ultragenyx assumes all risks, responsibilities and costs associated with the manufacturing of the First Batch, except in case of damages caused by Rentschler's gross negligence or willful misconduct. For the avoidance of doubt, manufacturing as used in this Section shall also comprise the testing of the Product. The provisions in this section also apply to the first batch of Drug Product manufactured by Rentschler for Ultragenyx as, upon request by Ultragenyx, there will not be a technical fill to confirm feasibility of the GMP-conform manufacturing of Drug Product.

2.10 As of the Effective Date, the Parties intend that Rentschler shall be the First Supplier to Ultragenyx of Product to the extent Ultragenyx demands such Product and Rentschler has available capacity and the manufacturing is reasonably feasible at Rentschler's facility. In such case, the Parties intend that **"First Supplier"** means that Ultragenyx will not source larger quantities of Product from another supplier than from Rentschler per year. The Parties shall endeavor in good faith to negotiate a definitive agreement for commercial supply of Product in a timely manner. To this end, the Parties shall initiate negotiations, if possible before the planned production start for the commercial supply, however, not later than the first submission of a BLA (US) or MAA (EU) for the Drug Product. For clarity, this **Section 2.10** describes the Parties' good faith intentions as of the Effective Date but shall not be interpreted as a binding commitment on either Party with respect to Rentschler being a First Supplier to Ultragenyx nor the Parties concluding a definitive commercial supply agreement.

3 Material and Technology Transfer

3.1 Ultragenyx shall reasonably and timely support Rentschler as required to perform and to facilitate the proper provision of the Services and Ultragenyx shall deliver to Rentschler, DDP Rentschler's facility at Laupheim (Incoterms 2010), as of the Effective Date all Ultragenyx Materials and such other physical materials that are under Ultragenyx's custody, control, ownership or influence that are necessary for Rentschler's performance of the Services including without limitation those materials identified in **Schedule One**. Ultragenyx shall at its own cost upon reasonable notice deliver to Rentschler further samples of such materials as are reasonably necessary and requested by Rentschler during the Term of this Agreement to facilitate Rentschler's proper performance of the Services. Ultragenyx shall inform Rentschler's incoming goods department (ware- neingang@rentschler.de) and the project manager of any delivery to be made according to this **Section 3.1** at least [***] Business Days before any such delivery to Rentschler is triggered. Rentschler acknowledges that the Ultragenyx Materials may have biological properties that are unpredictable and unknown at the time of transfer. However, Ultragenyx confirms that it has provided all safety

data and information available to Ultragenyx that are responsive to Rentschler's safety requirements pertaining to the Cell Line and the Ultragenyx Materials, as set forth on **Schedule One**. Ultragenyx represents and warrants that, to Ultragenyx's knowledge, the Ultragenyx Material will not be contaminated and will not have hazardous properties except as disclosed in such safety data and information from Ultragenyx, provided that Ultragenyx has tested the material in accordance with the corresponding provisions in **Schedule Two**. Additionally, Ultragenyx will provide Rentschler with any further data coming to Ultragenyx's knowledge regarding the safety of the Ultragenyx Materials.

3.2 Immediately upon supply of the Ultragenyx Materials to Rentschler, Rentschler shall inspect the Ultragenyx Materials in accordance with the Project Plan in order to determine whether the Ultragenyx Materials may be acceptable and presumably suited for the Process according to the criteria listed in **Schedule Two**. Except for Rentschler's negligent or willful breach of this Agreement, Rentschler shall not be liable for whatever reason for the unsuitability of the Ultragenyx Materials for the Process. If Rentschler determines that it will not be able to use the supplied Ultragenyx Materials for the Process in accordance with this Agreement, it shall inform Ultragenyx hereof without undue delay. In such event, Ultragenyx shall use all reasonable efforts to provide Rentschler with alternate, suitable Ultragenyx Material, and Rentschler shall use all reasonable efforts and will collaborate with Ultragenyx in order to select such other cell line or other material, as the case may be, or make such reasonable modifications and adjustments to procure alternate Ultragenyx Material as soon as possible. If Rentschler refuses the initial Cell Line and Ultragenyx is unable to provide an alternate, suitable Cell Line for the Process within a [***] week period, Ultragenyx shall have the right to terminate this Agreement in accordance with **Section 13.3** below. In the event of any delays caused by an unsuitable Ultragenyx Material and/or delayed receipt of Ultragenyx Material, Ultragenyx shall be liable for and shall bear any costs and consequences associated therewith and the timelines of the Project Plan shall be adjusted accordingly.

3.3 Ultragenyx shall make available to Rentschler as of the Effective Date in a useable and understandable format all Ultragenyx know-how and Confidential Information which is relevant to the Cell Line, Process, Product, Services and the Project, including without limitation full and complete details of Ultragenyx's laboratory scale process, that are necessary for Rentschler's performance of the Services.

3.4 When reasonably required by Rentschler or where stipulated in any Schedule, Ultragenyx shall, at its own cost and expense, supply to Rentschler suitably skilled, educated and technical employees or representatives with knowledge of the Project, Process, Cell Line, Product and Ultragenyx Materials for the purpose of facilitating and assisting the technology transfer of the Project technology to Rentschler to enable Rentschler to perform the Services hereunder. Ultragenyx shall ensure that such employees or representatives will (i) be subject to enforceable obligations of confidentiality preventing them from using any information of a confidential nature which they acquire during such visit; and (ii) obey the rules at Rentschler's facility at Laupheim with regard to health and safety, GMP and Ultragenyx confidentiality.

3.5 Ultragenyx hereby grants to Rentschler, for the Term of this Agreement and for the sole purpose of performing the Services in accordance with the Project Plan, a royalty free worldwide non-exclusive license in respect of all Ultragenyx Intellectual Property Rights which are required by Rentschler for the proper performance of the Services hereunder.

3.6 Rentschler shall be responsible for the storage, handling and processing of the Ultragenyx Materials in accordance with Ultragenyx's reasonable written instructions and the terms of the Quality Agreement. Rentschler shall use the Ultragenyx Materials solely as necessary to conduct the Services and shall not sell, transfer, lease, or license the Ultragenyx Materials to any third party except as expressly permitted under **Section 2.6**. Subject to **Sections 10.5** through **10.7,** Rentschler shall bear all risk of loss of the Ultragenyx Materials following delivery to Rentschler and shall replace at its cost any damaged or spoiled Ultragenyx Materials after delivery.

4 Deliverables; Supply Failure; Acceptance & Rejection

4.1 Rentschler will throughout the performance of the Services keep Ultragenyx reasonably and thoroughly informed of the progress of the Services.

4.2 Upon conclusion of the Services and upon receipt of full payment in respect of sums properly owed to Rentschler hereunder, Rentschler shall deliver to Ultragenyx a comprehensive documentation on the Services which shall comprise the results and reports generated pursuant to the Services.

4.3 Subject to **Section 2.2**, Rentschler will manufacture the Products for the use in clinical trials in accordance with GMP and the applicable Specifications according to **Schedule Three** and, if requested by Ultragenyx, package and label the Products in accordance with applicable regulatory requirements and subject to conditions to be agreed between the Parties, and deliver the Products EXW Laupheim (as defined by Incoterms 2010) in accordance with the estimated timelines in the Project Plan. Jointly with the delivery of the Products, Rentschler shall deliver to Ultragenyx the certificate of analysis, the certificate of conformity and such other documentation as is reasonably required to meet all applicable statutory and regulatory requirements.

4.4 If, following validation of the Product, Rentschler fails to deliver on a timely basis the full amount of Product ordered, Ultragenyx shall have the right to require Rentschler to cancel any part of such order with respect to which delivery has failed, provided that Rentschler may first attempt to remedy the failure in accordance with **Section 13.2.2**. The aforementioned remedy shall be Ultragenyx's sole remedy in case Rentschler successfully remedies such failure in accordance with **Section 13.2.2**. Further, should either Party perceive that a shortfall in delivery of Product by Rentschler is likely to occur for any reason, the Parties shall discuss appropriate steps to alleviate such a shortfall.

4.5 Following validation of the Product, Ultragenyx may reject any portion of any shipment of Product that does not conform to the Specifications. In order to reject delivery of an order of Product, Ultragenyx must give written notice to Rentschler of Ultragenyx's rejection of any delivery within [***] days after receipt of such delivery, which notice shall specify Ultragenyx's reason(s) for rejection. If no such notice of rejection is received within [***] days of delivery of the order, Ultragenyx shall be deemed to have accepted such delivery of Product; provided, however, that in the case of Product having defects that are not discoverable upon reasonable physical inspection and testing but are discovered at a later time (e.g., in the course or as a result of long-term stability studies) (**"Latent Defect(s)"**), Ultragenyx may reject such Product by giving written notice to Rentschler of Ultragenyx's rejection of such Product within [***] days after discovery of such Latent Defect(s). Product rejected by Ultragenyx will be returned to Rentschler at Rentschler's request and at Rentschler's expense. Whether or not Rentschler accepts Ultragenyx's basis for rejection, Rentschler will promptly initiate action to supply replacement Product at no additional cost after receiving Ultragenyx's rejection notice. The aforementioned remedy shall be Ultragenyx's sole remedy in case Rentschler promptly initiates such corrective action and successfully supplies replacement Product within a reasonable time. Within [***] days of receiving any notice of rejection from Ultragenyx, Rentschler will respond stating whether (i) it accepts the rejection or (ii) it disputes the rejection, in which case the Parties will refer such dispute to a mutually acceptable independent third party with the appropriate expertise to assess the conformity or non-conformity of the rejected Product to Specifications. In the event the Parties are unable to agree within [***] calendar days on the person of such third party, each Party may request the appointment of such third party by the President of the Zurich Chamber of Commerce. The costs of such expert opinion shall be borne equally by the Parties. Such independent third party shall test the applicable Product and shall determine whether such Product met or did not meet the applicable Specifications. The Parties agree that such third party's determination shall be final and binding upon the Parties. The Party against whom the independent third party rules shall bear the costs of testing by such independent third party. In case the independent third party determines that Product did meet the Specifications, Ultragenyx will reimburse Rentschler for any actual costs incurred by Rentschler in providing such replacement of Product.

5 Quarantine Shipment and Quarantine Production

5.1 Rentschler may initiate a Quarantine Shipment upon Ultragenyx's written request in order to provide that Ultragenyx receives the respective Products in a timely manner.

5.2 If Rentschler receives materials (especially active pharmaceutical ingredients) that are required for the performance of the Services and that were not released and/or were delivered without a complete certificate of release, the project leader will inform Ultragenyx hereof promptly. Upon written request by Ultragenyx, Rentschler will conduct a preliminary analysis (identity, sterility and BSE/TSE) of the materials and perform a Quarantine Production in order to ensure that the manufacturing process is not delayed.

5.3 Ultragenyx assumes all risks, responsibilities and costs associated with a Quarantine Shipment or Quarantine Production, except in case of Rentschler's gross negligence or willful misconduct.

6 Price and Payment Terms

6.1 Rentschler shall be paid the Price by Ultragenyx. The Price is exclusive of taxes, duties, levies, Value Added Tax, other fees of whatever nature imposed by or under the authority of any government or public authority (other than taxes on Rentschler's income), Capacity Fees or external costs (as indicated in **Schedule Two)**, raw material costs or other expenses set out herein that Rentschler incurs or charges to provide the Services and which Ultragenyx hereby agrees to pay. In respect of the external costs and raw material costs Rentschler has provided an estimate of those separate costs in **Schedule Two** but the final payment in respect of the same will be subject on the quantity used and any changes in market price during the Term. An administrative and handling fee, not to exceed [***] percent ([***]%), will be added to costs accrued in connection with material supply, but not to other external costs (e.g. bulk harvest safety testing, virus validation studies and analytics that are not performed by Rentschler). In each invoice, Rentschler shall itemize all external costs that exceed [***] Euros (€ [***]).

6.2 All prices are in Euro and all invoices will be raised in Euro and will be paid in Euro.

6.3 All payments shall be paid by Ultragenyx without any deductions or deferment and according to the payment schedule in **Schedule Two**. In the event that Ultragenyx disputes a portion of a particular invoice, Ultragenyx shall so notify Rentschler within [***] days after receiving such invoice. The Parties shall thereafter discuss the disputed portion of the invoice. If the disputed portion of the invoice is less than [***] percent ([***]%) of the total invoiced amount, then Ultragenyx shall pay the full invoiced amount and, if the Parties agree after discussion that such disputed amount was not properly owed to Rentschler, Rentschler shall credit Ultragenyx such disputed amount on the following invoice or if no following invoice is expected, shall promptly reimburse Ultragenyx the disputed amount. If the disputed portion of the invoice is equal to or greater than [***] percent ([***]%) of the total invoiced amount, then Ultragenyx shall not pay Rentschler such disputed portion of the invoice, but shall pay the undisputed portion, until the Parties have discussed and resolved the dispute. If the Parties agree after discussion that the disputed amount was properly owed to Rentschler,

Ultragenyx shall promptly pay Rentschler such amount. Ultragenyx shall have no right to retain undisputed sum(s) of such invoice and shall pay such sums in accordance with the terms of this **Section 6**.

6.4 Rentschler's hourly rates for the remuneration of Services as mentioned in this Agreement are:

Analytical Work—Development	€[***]
USP/DSP Work	€[***]
Project Management	€[***]
Regulatory Affairs and Quality Assurance	€[***]
Parenterals Leading	€[***]
Analytical Work—Routine	€[***]
Parenterals Production	€[***]
Services (Technical Staff and the like)	€[***]

Rentschler can adjust the hourly rates for the period of a further [***] months from the time of the start of the [***] month onwards in the same ratio by which the [***] has changed.

6.5 In the event of default of payment of an undisputed sum on the due date, Rentschler shall give Ultragenyx no less than [***] days written notice following which if such payment has not been received in full, Rentschler shall be entitled, at its own discretion and without any liability to Ultragenyx, to suspend the Services.

7 Steering Committee

7.1 The Parties shall—if not before—promptly after the Effective Date, form the Steering Committee which shall comprise a minimum of two (2) and an equal number of representatives ("**Representatives**") from each of Rentschler and Ultragenyx, and each Party shall notify the other of its elected Representatives. Each Representative shall carry an equal vote and proxy votes may be granted by Representatives to their fellow Representative(s) if they are unable to attend meetings. The Steering Committee will take action by unanimous consent of its Representatives.

7.2 Each Party shall be entitled to change their respective nominated Representatives at any time and shall promptly give written notice of the change to the other Party including the new contact details of the new Representative(s) in any event no less than [***] Business Days after the change has been implemented.

7.3 The quorum for the Steering Committee shall be a minimum of two (2) Representatives from each Party. The Steering Committee may meet in person or by telephone and advance written notice of not less than two (2) Business Days must be communicated to no less than two (2) Representatives of each Party should a meeting be requested.

7.4 The primary function of the Steering Committee is to ensure the ongoing communication between the Parties and discuss and resolve any issues arising under the Project. The Parties agree that their Representatives will endeavour to attend each meeting and both the Representatives and each Party shall discuss events in good faith with the aim of furthering and successfully concluding the Project. In addition to the primary function described above the Steering Committee shall also to take on the following responsibilities:

7.4.1 Discuss and seek resolution of issues around management of the Project;

7.4.2 Agree and monitor deadlines and milestones for the Project;

7.4.3 Agree and discuss amendments to the Specifications, including amendments due to comments or requirements from regulatory agencies, provided that in no event shall Rentschler implement any change to the Specifications without Ultragenyx's prior written consent and, in the event of a disagreement between Ultragenyx and Rentschler, and not withstanding anything to the contrary in **Section 7.6**, the Parties must jointly agree on changes to the Specifications;

7.4.4 Agree and discuss any changes to the Services, provided that any change to the Services must be evidenced by a signed Change Order in accordance with **Section 2.7**.

7.5 The Steering Committee shall meet at such times as the Steering Committee determines reasonably necessary to monitor the progress of the Services and issues arising therefrom. Each Party may call for an extraordinary meeting of the Steering Committee up to [***] times per calendar year with [***] day advance notice.

7.6 Should the Steering Committee be unable to reach agreement on any issue or should the Steering Committee not have met for a requested extraordinary meeting with the quorum according to **Section 7.3** even after the second call for such a meeting, the issues shall be referred to a personal face-to-face meeting between senior executives of Rentschler and Ultragenyx both of whom shall act in good faith and discuss the issues to seek a resolution amicably acceptable to both Parties and if resolved the resolution shall be binding and final. The meeting shall take place within [***] days of the date of the relevant referral. In case such persons cannot agree within further [***] days after such face-to-face meeting, then the following shall apply:

7.6.1 If the dispute is predominantly concerned with a scientific or technical issue then the entire dispute shall be referred to an independent third party expert (appointed jointly by the Parties who is an expert in the particular scientific or technical area at issue and who shall act as an expert and not an arbitrator). In the event the Parties are unable to agree within [***] calendar days on the person of such third party, each Party may request the

appointment of such third party by the President of the Zurich Chamber of Commerce. The costs of such expert opinion shall be borne equally by the Parties. The decision of the independent expert shall be given in writing and in English and considered final and binding on the Parties except if there has been a manifest error on the face of the decision whereupon the Parties may revert to their respective remedies under **Section 16.2**.

7.6.2 If the dispute is predominantly concerned with an issue other than scientific or technical then it shall be resolved in accordance with **Section 16.2** below.

8 Audits and Inspections

8.1 Audits

Ultragenyx shall be entitled to an initial [***]-day audit with one auditor or one team of Rentschler's production site and related facilities used for the Services and to a [***]-day audit of one auditor or one team per year with [***] months prior notice. Ultragenyx will have the option, at Ultragenyx's expense, to designate an independent third party, reasonably acceptable to Rentschler, to verify that all Services according to **Section 2.2** are in compliance with GMP and all Applicable Laws. Further, Ultragenyx may audit those parts of Rentschler's books and records which contain specific entries referring to Ultragenyx, if there is competent evidence that a material issue or a violation has occurred or will occur with respect to the U.S. Foreign Corrupt Practices Act of 1977 (as amended) (the "**FCPA**") or the U.S. Travel Act. For the avoidance of doubt, this does not entitle Ultragenyx to pre-trial discoveries in the sense of US procedural law. Any additional audit will be charged to Ultragenyx on a time and material basis depending on the scope of such audit. Notwithstanding the foregoing, if any such audit is a for-cause audit or follow up audit of an audit during which deficiencies or conditions requiring corrective action were identified, then the notice period shall be reduced to thirty days and Rentschler will not charge Ultragenyx for any costs or expenses (whether internal or external) related to such audit.

8.2 Inspections

In respect of GMP Services, Rentschler shall reasonably permit a governmental or regulatory authority body to enter those areas of Rentschler's premises concerned with the Services for the sole purpose of observing and inspecting the performance of the GMP Services and those records of Rentschler specific to the GMP Services where such entry is reasonably necessary or mandatory in order for Ultragenyx to maintain, approve, apply for or amend its regulatory application or approval in respect of the Product. Such inspections are subject to (i) the individuals representing such government or regulatory authority body obeying and adhering to the rules and regulations in place at Rentschler concerning health and safety, GMP and Ultragenyx confidentiality; and (ii) in the case of

inspections by a governmental or regulatory authority body other than the regional council Tübingen ("Regierungspräsidium Tübingen"), Rentschler being entitled to charge Ultragenyx for such inspections at Rentschler's then standard rates, provided that such charges shall not exceed [***] Euros (€ [***]) for an audit consisting of [***] auditors for [***] days duration.

9 Warranties ("Gewaehrleistungen")

9.1 Ultragenyx warrants ("gewährleistet") to Rentschler that:

9.1.1 it is legally incorporated and in good standing in its country of incorporation and that it has the right to enter into this Agreement.

9.1.2 it has the right to supply or license Rentschler with Ultragenyx Materials, Ultragenyx Intellectual Property Rights, and Confidential Information and, if necessary, has obtained for the benefit of Rentschler and advised Rentschler accordingly of all necessary license(s) as known to Ultragenyx' best knowledge as of the Effective Date to allow Rentschler to use the transferred Process, Ultragenyx Materials, Ultragenyx Intellectual Property and Confidential Information for the provision of the Services, development of the Process and manufacture of Product;

9.1.3 to its best knowledge as of the Effective Date, the Ultragenyx Materials are free of all contaminants including without limitation any virus, bacteria (other than the Cell Line itself), prions, or other vectors and will not pose a health hazard or biohazard;

9.1.4 where it has provided Rentschler with or directed or stipulated that Rentschler use the Cell Line, to Ultragenyx's best knowledge as of the Effective Date, Rentschler is lawfully entitled to use the Cell Line for the provision of the Services and manufacture of Product;

9.1.5 to its best knowledge as of the Effective Date, the use of the Cell Line, Ultragenyx know-how, Confidential Information, Ultragenyx Intellectual Property Rights and Ultragenyx Materials by Rentschler for the development of the Process and the manufacture of Products does not infringe any Intellectual Property Rights of a third party;

9.1.6 it will promptly inform Rentschler if it receives notice of any claim or potential claim relating to infringement or alleged infringement of any third party Intellectual Property Rights by virtue of Ultragenyx's or Rentschler's use of the Cell Line, Ultragenyx Information or Ultragenyx Materials or the manufacture of Product; and

9.1.7 to its best knowledge as of the Effective Date, the Ultragenyx Materials, Ultragenyx Intellectual Property Rights and Confidential Information transferred to Rentschler are all that is necessary for Rentschler to perform the Services (in addition to the Rentschler Intellectual Property Rights, Rentschler know-how and Rentschler's Confidential Information) and are suitable for the performance of the Services; and

9.1.8 it shall not use, or make available for use, Product for human consumption except in human clinical trials in accordance with GCP.

9.2 Rentschler warrants ("gewährleistet") to Ultragenyx that:

9.2.1 it is legally incorporated and in good standing in its country of incorporation and that it has the right to enter into this Agreement;

9.2.2 it has or will establish the necessary permits (including permit for work in a GMP environment), facilities and technically qualified employees that may be reasonably anticipated to be required for provision of the Services;

9.2.3 it shall diligently perform the Services in accordance with its obligations under **Section 2.1**;

9.2.4 to its best knowledge, the Rentschler Intellectual Property Rights and Rentschler know-how do not, on their own, infringe any Intellectual Property Rights of a third party;

9.2.5 to its best knowledge, the Rentschler Intellectual Property Rights and Rentschler know-how are all that is necessary for Rentschler to perform the Services (in addition to the Ultragenyx Intellectual Property Rights, Ultragenyx know-how and Ultragenyx's Confidential Information) and are suitable for the performance of the Services;

9.2.6 it will not knowingly infringe or misuse a third party's Intellectual Property Rights in its performance of the Services;

9.2.7 it will not misuse, sell or unlawfully disclose to a third party Ultragenyx Materials, Ultragenyx Intellectual Property Rights, Ultragenyx know-how and Confidential Information, nor assist another to do so;

9.2.8 it will use its reasonable endeavours to keep the Ultragenyx Materials and Cell Line safe and secure;

9.2.9 it will convey unencumbered title (save for any intellectual property rights which may exist) to Product to Ultragenyx upon delivery;

9.2.10 it will promptly inform Ultragenyx if it receives notice of any claim or potential claim relating to infringement or alleged infringement of any third party Intellectual Property Rights by virtue of its use of the Cell Line, Ultragenyx Information or Ultragenyx Materials or the manufacture of Product;

9.2.11 it will not with a corrupt intent directly or indirectly offer, promise, authorize, pay or give any money, favor, advantage, bribe, kickback, or anything else of value to any government official or entity or to any other individual or entity for purposes of obtaining, retaining, or directing business or any other improper advantage;

9.2.12 it has in good faith provided to Ultragenyx all documents and information of the character and type requested by Ultragenyx in writing in the course of Ultragenyx's due diligence review of Rentschler. To Rentschler's best knowledge, there are no documents or information of a character or type described in such request which have not been so provided by Rentschler; and

9.2.13 it shall respond to Ultragenyx's requests for information, to the extent reasonable and related to Ultragenyx's efforts to ensure compliance with the FCPA and any other applicable anti-corruption law.

9.3 In consideration of the express warranties set out above under this **Section 9**, to the maximum extent permitted by Applicable Laws and Swiss law (save for those express warranties set out above) neither Party makes or gives any other express or implied (whether by statute, custom or otherwise) warranties in relation to its respective obligations, duties or activities owed or performed under this Agreement and hereby excludes all such warranties.

9.4 If Ultragenyx finds any Service not to meet the warranties given by Rentschler in accordance with **Section 9.2.3**, Ultragenyx may upon notice to Rentschler require Rentschler to provide Services, which fully satisfy the given warranties of **Section 9.2.3** at Rentschler's sole risk and expense within the time periods stated in **Section 13.2.2** for curing the breach. In the event, however, such remedial Services do not meet the warranties of **Section 9.2.3** and Rentschler fails to rectify Ultragenyx' warranty claim within the time periods stated in **Section 13.2.2**, Ultragenyx may terminate this Agreement in accordance with **Section 13.2.2**, and/or claim damages and indemnification in accordance with **Section 10**, including the limitation of liability provisions stated in **Sections 10.5** through **10.7**.

10 Liability and Indemnifications

Indemnification

10.1 Ultragenyx shall indemnify and hold harmless Rentschler and each of its directors, officers, employees, shareholders, and Testing Laboratories (the **"Rentschler Parties"**) against any and all losses, demands, claims, liabilities, damages, costs and expenses (including but not limited to consequential losses and loss of profit, court costs and documented attorney's fees and expenses together with any applicable taxes thereon) brought or incurred by third parties to the extent resulting from the following:

10.1.1 any material unremedied breach by the Ultragenyx Parties of the warranties given pursuant to **Section 9.1**;

10.1.2 any infringement or alleged infringement or breach of any third party rights by any Party, including without limitation any intellectual or industrial property rights, patents, trademarks, copyright, know-how or confidential information, by use of the Ultragenyx Intellectual Property Rights and/or Ultragenyx know-how and/or Ultragenyx Confidential Information and/or Ultragenyx Materials in the performance of the Services;

10.1.3 any product liability claims relating to the Product or Ultragenyx Materials including any derivatives of the foregoing, conjugated form or formulation of the same to the extent such claim is based on the use of the Product following the manufacturer's release (except where such liability has arisen due solely to an act of at least gross negligence caused by Rentschler and is not covered under Ultragenyx's product liability insurance);

10.1.4 the negligence of Ultragenyx in relation to the use, processing, storage or sale of the Product or any derivative, conjugated form or formulation thereof; or

10.1.5 any negligence or willful misconduct by Ultragenyx designed and intended to cause detriment to Rentschler in performance of its obligations under this Agreement;

provided, however, that Ultragenyx shall have no obligation to indemnify Rentschler to the extent that any losses, demands, claims liabilities, damages, costs and expenses are caused by Rentschler's own negligence or willful misconduct in the performance of the Services or by Rentschler's at least negligent breach of this Agreement.

10.2 Rentschler shall indemnify and hold harmless Ultragenyx and each of its directors, officers and employees (the **"Ultragenyx Parties"**) against any and all losses, demands, claims, liabilities, damages, costs and expenses (including but not limited to reasonable court costs and documented attorney's fees and expenses together with any applicable taxes thereon) brought or incurred by third parties to the extent resulting from the following:

10.2.1 any material unremedied breach by the Rentschler Parties of the warranties given pursuant to Section 9.2;

10.2.2 any infringement or alleged infringement or breach of any third party rights by any Party, including without limitation any intellectual property rights, patents, trademarks, copyrights, know-how or confidential information, by use of the Rentschler Intellectual Property Rights and/or Rentschler know-how in the performance of the Services;

10.2.3 the negligence of Rentschler in relation to the use, processing, storage or sale of the Product or any derivative, conjugated form or formulation thereof; or

10.2.4 any negligence or willful misconduct by Rentschler designed and intended to cause detriment to Ultragenyx in performance of its obligations under this Agreement;

provided, however, that Rentschler shall have no obligation to indemnify Ultragenyx to the extent that any losses, demands, claims liabilities, damages, costs and expenses are caused by Ultragenyx's own negligence or willful misconduct in the performance of its rights obligations hereunder or by Ultragenyx's at least negligent breach of this Agreement.

Indemnification Procedure

10.3 The Party (the **"Indemnitee"**) that intends to claim indemnification under this Section 10 shall:

10.3.1 promptly, and in any event within [***] Business Days of it receiving notice of the claim, demand, threat or action, notify the other Party (the **"Indemnitor"**) in writing in general terms of any claim, demand, threat or action which has or has the potential to give rise to the Indemnitee seeking to rely on and claim the benefit of the indemnification together with notification of the Indemnitee's intention to rely on such indemnity, provided however, that failure to give such notice shall not relieve the Indemnitor of its indemnification obligations except and only to the extent such failure actually and materially prejudices the ability of the Indemnitor to defend against such claims;

10.3.2 not prejudice any defense to any claim or attempt to settle or compromise such claim;

10.3.3 subject to its other rights and obligations and compliance with the procedures set out in this **Section 10** permit the Indemnitor to have overall control of the conduct of the negotiations and the proceedings including any counterclaim;

10.3.4 cooperate as reasonably requested by the Indemnitor, at the Indemnitor's expense, in the conduct of such claim (and any counterclaim); and

10.3.5 have the right (at the Indemnitor's expense) to instruct independent counsel and participate in all proceedings and negotiations whether named or not as a party in the claim or proceedings.

10.4 The Indemnitor shall not settle or consent to an adverse judgment in any such claim, demand, action or other proceeding that adversely affects the rights or interests of any Indemnitee or imposes additional obligations (financial or otherwise) on such Indemnitee, without the prior express written consent of such Indemnitee (such consent to be at the Indemnitee's sole discretion).

Limitation of Liability

10.5 In no event shall either Party be liable to the other Party for incidental, indirect or consequential damages or loss of profit arising from or related to a breach of this Agreement. The foregoing disclaimer of damages shall not apply in the case of (i) a breach of **Section 11** (Confidentiality), (ii) personal injury or death, (iii) gross negligence or intentionally wrongful acts or omissions, or (iv) to third party claims subject to the indemnification provisions in **Section 10.1** and **Section 10.2**.

10.6 Rentschler's aggregate liability to Ultragenyx for any loss or damage suffered by Ultragenyx as a result of Rentschler's negligent or willful breach of this Agreement or of any other liability in respect to Rentschler's performance of the Services shall be except in cases of gross negligence or intentionally wrongful acts limited to

10.6.1 [***] percent ([***]%) of the [***] in case of negligence, or

10.6.2 the amount paid by Rentschler's general liability insurance, whichever amount is higher.

10.7 The Parties' liability and indemnification obligation as agreed to under this **Section 10** shall be the Parties' sole and exclusive liability and indemnification obligation towards each other and any further liability and indemnification obligation shall be explicitly excluded to the extent permitted by applicable law.

10.8 Insurance

Prior to the first commercial sale of a Product Ultragenyx shall obtain and shall thereafter maintain for [***] years after termination of this Agreement product liability insurance with a reputable and solvent insurance provider in an amount of at least $[***] per occurrence and in the aggregate. Such product liability insurance shall insure against all mandatory liability including liability for personal injury, physical injury and property damages. Further, Ultragenyx shall obtain any necessary patients insurance required to perform the clinical studies. Ultragenyx has obtained and will maintain during the Term of this Agreement general liability insurance of $[***] per occurrence and $[***] in the aggregate. Rentschler shall obtain and maintain during the Term of this Agreement and for [***] years thereafter-general liability insurance with a reputable and solvent insurance provider. Reference is made to Rentschler's current certificate of insurance attached hereto as **Schedule Four**. The Parties shall provide each other written proof of the existence of such insurance upon request.

11 Confidential Information

11.1 The Parties acknowledge that there may be disclosure of each other's Confidential Information to the other. In consideration of the disclosing Party making available its Confidential Information to the recipient Party, the recipient Party undertakes that it shall, and shall procure that each of its Permitted Recipients, shall:

11.1.1 treat and safeguard as private and confidential all the Confidential Information;

11.1.2 use the Confidential Information only for those purposes reasonably required or anticipated under this Agreement and without prejudice to the generality of the foregoing, not use any Confidential Information to obtain any commercial advantage over the disclosing Party;

11.1.3 ensure the proper and secure storage of all Confidential Information applying standards of care reasonably expected and no less stringent than standards applied to protection of recipient Party's own confidential information;

11.1.4 not at any time without the disclosing Party's prior written consent disclose or reveal, whether directly or indirectly any of the Confidential Information to any person whatsoever save its Permitted Recipients, and then on a limited need to know basis, who shall be informed by it of the confidential nature of the Confidential Information and of the confidentiality terms of this Agreement and for whom it hereby accepts full responsibility in the event that any such person shall breach the duty of confidence imposed upon them; and

11.1.5 not at any time have any discussion, correspondence or contact with any third party concerning the Confidential Information without the prior written consent of the disclosing Party.

11.2 The obligations in this Agreement regarding Confidential Information do not apply to the extent that the recipient Party can prove by written evidence that the respective Confidential Information:

11.2.1 is, at the time of its disclosure by the disclosing Party, wholly available to the public and could be obtained without reference to the Confidential Information by any person with no more than reasonable diligence;

11.2.2 becomes generally available to the public after such disclosure otherwise than by reason of a breach of any of the undertakings in this Agreement or any breaches of confidence by the recipient Party or its Permitted Recipients;

11.2.3 is, at the time of such disclosure, lawfully already within its possession;

11.2.4 is independently developed by the recipient Party without reference to the Confidential Information of the disclosing Party, as evidenced by records; or

11.2.5 was disclosed to an information technology service provider to implement, operate and/or maintain receiving Party's technical or software internal systems, provided that the information technology service provider is sworn to secrecy and confidentiality obligations at equal terms as stipulated in this Agreement.

In addition, each Party may disclose Confidential Information of the other Party to the extent such disclosure is required by law or by any stock exchange or other regulatory authority having jurisdiction (but, for the avoidance of doubt, only to that extent).

11.3 For the avoidance of doubt, no provision in this Agreement shall restrict each Party's right to disclose the existence of a business relationship between the Parties to potential customers.

11.4 Other than the limited and restricted rights of use set out in this **Section 11** nothing in this Agreement intends to or has the effect of granting any right, title, license or interest in or to the recipient Party in respect of the disclosing Party's Confidential Information.

11.5 If the recipient Party or any of its Permitted Recipients becomes compelled to disclose any Confidential Information in the circumstances described in **Section 11.2** of this Agreement or a breach or threatened breach of this **Section 11** occurs or becomes apparent or becomes aware of any misuse of the Confidential Information, the recipient Party shall inform the disclosing Party in writing of such obligation or fact as soon as possible after it is informed, or becomes aware, of it and if possible, before any Confidential Information is disclosed, so that (if the disclosing Party in its absolute discretion shall see fit) a protective order or other appropriate remedy may be sought. The recipient Party agrees to assist and co-operate (and shall procure that each of its Permitted Recipients shall, as appropriate, assist and co-operate) in any action which the disclosing Party may decide to take. The recipient Party shall notify the disclosing Party prior to each disclosure of Confidential Information if it is under any obligation which would or might compel it to disclose any Confidential Information and subsequent to such disclosure it shall not voluntarily assume any such obligation.

11.6 Except as otherwise provided for in this Agreement or otherwise required by law or administrative authorities, neither Ultragenyx nor Rentschler shall disclose any terms or conditions of this Agreement to any third party without the prior written consent of the other Party.

11.7 Upon termination of this Agreement or at the request of the disclosing Party, each Party shall promptly return to the other, at the other's request, any and all Confidential Information of the other (including copies of documents, computer records and records on all other media) then in its possession or under its control except that the receiving Party may retain one (1) copy of all such Confidential Information for legal archival purposes only. The receiving Party shall not be required to destroy any computer files stored securely by the receiving Party that are created during automatic system back-up.

11.8 The provisions of this **Section 11** shall survive termination or expiry of this Agreement by the shorter of (i) [***] years after first commercial launch of a Product or (ii) [***] years after termination or expiry of this Agreement.

11.9 For the purposes of this Section Confidential Information of Cellca GmbH shall be deemed to be Confidential Information of Rentschler.

12 Intellectual Property Rights

12.1 Rentschler and Ultragenyx agree that any manufacturing process, protocol, assay, and formulation developed in the course of Rentschler's performance of its obligation under this Agreement (together with all intellectual property rights therein) will be: (a) solely owned by Ultragenyx if solely related to Product, and Rentschler hereby assigns to Ultragenyx all of its rights and interest therein, provided that, in case of any invention made by an employee of Rentschler **("Inventor"),** Ultragenyx compensates Rentschler for any payments due to the Inventor according to the German Act on Employee Inventions ("Arbeitnehmererfindungsgesetz"); and (b) solely owned by Rentschler if generally applicable to the manufacture of other products in addition to Product, and Rentschler hereby grants to Ultragenyx a fully-paid royalty-free, worldwide, perpetual license thereunder, with the right to grant sublicenses in multiple tiers, for Ultragenyx to make, have made, use, sell, offer for sale and import Ultragenyx products, including Product and its derivatives and analogues. In addition, Rentschler hereby grants Ultragenyx a fully-paid, royalty-free, worldwide, perpetual license, with the right to grant sublicenses in multiple tiers, under other patents and know-how owned or in-licensed by Rentschler that cover technology incorporated into the Services provided by Rentschler under this Agreement, for Ultragenyx to make, have made, use, sell, offer for sale and import Ultragenyx products, including Product and its derivatives and analogues **("Rentschler's Background IP")**. Upon Ultragenyx's request, Rentschler shall transfer to Ultragenyx or its designee the know-how required for the exercise of the license granted to Ultragenyx hereunder regarding Rentschler's Background IP.

12.2 Ultragenyx hereby grants to Rentschler a license under the Ultragenyx Intellectual Property Rights as specified in **Section 3.5**. Other than this license, Rentschler shall not acquire any rights whatsoever in Ultragenyx Intellectual Property Rights and Rentschler acknowledges that these rights belong exclusively to Ultragenyx.

12.3 Each Party may file patent protection on any Intellectual Property Rights it owns or, as set forth above, subsequently owns under this Agreement and the other Party shall promptly upon request co-operate, at the requesting Party's reasonable expense, with any requests to assist or enable the Party's protection including but not limited to signing and delivering documents and other information necessary for the valid application and prosecution of any such patent.

13 Term and Termination

13.1 This Agreement shall commence on and become effective on the Effective Date provided that it has been lawfully executed by all Parties and will expire when the Services have been completed or terminated in accordance with this **Section 13** (the **"Term"**). In respect of any part(s) of the Services which have been performed before the Effective Date the Parties agree that the performance of those part(s) shall be deemed to have been performed during the Term and governed by the terms of this Agreement.

Events of Termination

13.2 Subject to force majeure and notwithstanding **Section 13.1**, either Party (**"Non-Defaulting Party"**) may terminate this Agreement before expiry of the Term with immediate effect upon written notice to the other Party (**"Defaulting Party"**) if:

13.2.1 the Defaulting Party fails to pay any sum payable under this Agreement thirty (30) days after a written demand issued after the original due date;

13.2.2 the Defaulting Party makes or has made a material misrepresentation or commits a material breach of its obligations under this Agreement and, if the breach is capable of remedy, fails to remedy it during the period of thirty (30) days starting on the date of receipt of notice from the Non-Defaulting Party generally identifying the breach and requiring it to be remedied. Notwithstanding the foregoing, in the event the alleged breach in question is not reasonably capable of cure within the foregoing thirty (30) day period, but is otherwise capable of being cured within an additional one hundred fifty (150) days, the Defaulting Party may submit a reasonable cure plan prior to the end of such initial thirty (30) day cure period, in which case, the Non-Defaulting Party shall not have the right to terminate under this **Section 13.2.2** with respect to such alleged breach for so long as the Defaulting Party is diligently implementing such cure plan, provided, however, that if the Defaulting Party fails to cure such breach (even if diligently implementing a cure plan) within one hundred eighty (180) days from the date of the first written notice identifying the material breach in reasonable detail, then the Non-Defaulting Party may terminate under this **Section 13.2.2** upon expiration of such one hundred eighty (180) day period.

13.2.3 (i) the Defaulting Party is deemed unable to pay its debts within the meaning of **Sections 17, 18** or **19** of the German Insolvency Act, or (ii) the assets of the Defaulting Party became part of insolvency proceedings, such right to terminate not existing if and as long as the (provisional) administrator continues business operations of the Defaulting Party and payments and counter payments are guaranteed for the time of administration, or (iii) an application to open insolvency proceedings at the Defaulting Party has been dismissed due to lack of assets; or (iv) any shareholders' meeting is convened for the dissolution of the Defaulting Party.

13.2.4 the Defaulting Party ceases to carry on its business for a period of no less than sixty (60) days;

13.2.5 the Defaulting Party suffers, or there occurs in relation to that Party, any event which in the reasonable opinion of the Non-Defaulting Party is analogous to any of the events mentioned in **Sections 13.2.1** to **13.2.4**;

13.3 Notwithstanding **Section 13.1** Rentschler and Ultragenyx may terminate this Agreement or any part of the Services on thirty (30) days advance written notice if the Steering Committee concludes that the Services cannot technically or scientifically or in any other way be delivered in accordance with this Agreement, and Ultragenyx may terminate this Agreement hereunder as set forth in **Section 3.2** above. In the event this Agreement is terminated in accordance with this **Section 13.3,** Ultragenyx shall pay to Rentschler the sums specified pursuant to **Section 13.5**.

13.4 Ultragenyx may terminate this Agreement at any time before completion of the Services by giving no less than sixty (60) days advance notice in writing to Rentschler. In the event Ultragenyx elects to terminate this Agreement in accordance with this **Section 13.4,** it shall pay to Rentschler the sums specified pursuant to **Section 13.5.**

Consequences of Termination

13.5 In the event that this Agreement is terminated pursuant to **Section 13.2** by Rentschler or based on the other extraordinary termination rights by either Party, neither Party shall incur any future liability towards the other Party other than:

13.5.1 in respect of any accrued rights;

13.5.2 the payment by Ultragenyx to Rentschler of sums due in respect to those Working Packages completed in accordance with this Agreement at the date of termination and a pro-rata Price for those Working Packages (fairly determined having regard to man hours, materials, profit element and uncancellable commitments incurred and unmitigated by Rentschler; provided that Rentschler shall use commercially reasonable efforts to mitigate any costs it may incur) which, at the date of termination, have not been completed but have been started and performed in accordance with this Agreement including all raw materials and other external costs incurred by Rentschler in performance of the Services; and,

13.5.3 in the event this Agreement is terminated pursuant to **Section 13.2** by Rentschler or this Agreement or individual Working Package(s) are terminated by Ultragenyx based on the other extraordinary termination rights, Ultragenyx shall pay to Rentschler, in addition to any other sums payable under this **Section 13.5**, the amounts stated in **Section 2.3** and **Section 2.4** as applicable. For the avoidance of doubt, any payments made under this **Section 13.5** shall not exceed the total Price of the Services plus actual costs incurred for raw materials and other external costs.

13.6 Termination of this Agreement for whatever reason shall not affect the accrued rights of either Rentschler or Ultragenyx arising under or out of this Agreement and all provisions which are expressed to survive this Agreement and the provisions of **Sections 9, 10, 11, 12, 13, and 15 through 18** shall survive termination or expiry and remain in full force and effect.

13.7 The rights and licenses and the like granted in accordance with this Agreement and any claims either Party might have (whether or not notified to the other Party prior to the Termination) against the other Party as well as the liability and insurance provision of this Agreement shall unless otherwise stated survive the termination of this Agreement.

13.8 Upon expiry of this Agreement or termination of this Agreement pursuant to **Section 13.2, Section 13.3** or **Section 13.4** by either Party, Rentschler shall without undue delay transfer to Ultragenyx all data and information necessary to exercise the manufacture (e.g., cell banks, fermentation processes, product identity assays, in-process-control assays, standard operating procedures), make available Process related media and feeds, and furnish all assistance and answer all questions received from Ultragenyx regarding the Process, in order to allow Ultragenyx or a third party designated by Ultragenyx to replicate the Process and take over the manufacturing of Products. The costs of such transfer and assistance shall be borne by Ultragenyx.

13.9 For clarity, in the event this Agreement is terminated by Ultragenyx pursuant to **Section 13.2**, Ultragenyx shall not owe Rentschler the amounts stated in **Section 2.3** and **Section 2.4**.

14 Force Majeure

14.1 Neither Party shall be held liable or responsible to the other Party nor be deemed to have defaulted under or breached this Agreement for failure or delay in fulfilling or performing any term of this Agreement or the Services to the extent, and for so long as, such failure or delay is caused by or results from causes beyond the reasonable control of such Party including but not limited to fires, earthquakes, floods, embargoes, wars, acts of war (whether war is declared or not), terrorist acts, insurrections, riots, civil commotion, strikes, lockouts or other labour disturbances, acts of God or other acts, omissions or delays in acting by any administrative authority or other party.

14.2 The Parties shall notify each other in writing of any force majeure event which prevents such Party from performing its obligations under this Agreement. In the event a force majeure situation continues for more than [***] months after notice is served, and is adversely effecting the performance of this Agreement, Ultragenyx will have the right, on [***] days advance written notice not to expire before the [***] month period to terminate this Agreement without any further liability towards Rentschler.

15 Period for Limitation of Ultragenyx Claims

If not explicitly otherwise provided for in this Agreement, but subject in all cases to Swiss law, claims by Ultragenyx in connection with this Agreement and/or the Services—regardless of their legal basis—are subject to a limitation period of [***] years after completion of the Services. The foregoing period of limitation shall not apply to any claim brought by Ultragenyx to the extent resulting from or relating to (1) Rentschler's breach of the warranty in **Section 9.2.11**, which instead shall be subject to a limitation period of [***] years after completion of the Services, or (2) Rentschler's gross negligence or willful misconduct, which shall [***].

16 Applicable Law and Dispute Resolution

16.1 This Agreement shall be governed by and construed in accordance with the laws of Switzerland, without regards to its conflicts of law principles requiring the application of laws of another jurisdiction.

16.2 Any dispute, controversy or claims arising out of or in connection with this Agreement, including disputes on its conclusion, binding effect, amendment and termination, shall be resolved exclusively by the International Court of Arbitration of the International Chamber of Commerce and shall be finally settled under the Rules of Arbitration of the International Chamber of Commerce by three arbitrators appointed in accordance with the said Rules. The seat of the arbitration shall be in Zurich, Switzerland. The arbitral proceedings shall be conducted in the English language.

17 Quality Agreement

17.1 Within [***] months of the Effective Date, the Parties will enter into a separate quality agreement (the **"Quality Agreement"**) with the Contract No: 0467A7F4-15C7.

17.2 The Quality Agreement and its annexes supplement this Agreement.

18 Miscellaneous

Amendment

18.1 No modification, extension or variation of this Agreement (or any document entered into pursuant to or in connection with this Agreement) shall be valid unless it is in writing and signed by or on behalf of each of the Parties to this Agreement. For the avoidance of doubt, no modification or variation of this Agreement shall be valid if made by e-mail.

18.2 Unless expressly so agreed, no modification or variation of this Agreement shall constitute or be construed as a general waiver of any provisions of this Agreement, nor shall it affect any rights, obligations or liabilities under this Agreement which have already accrued up to the date of such modification or waiver, and the rights and obligations of the Parties under this Agreement shall remain in full force and effect, except and only to the extent that they are so modified or varied.

Assignment

18.3 Except as provided in **Section 18.4**, Ultragenyx shall not without the prior written consent of Rentschler (such consent not to be unreasonably withheld) assign this Agreement or any rights and obligations under this Agreement. Each Party is entering into this Agreement as principal not agent, and may not enforce any of its rights under or in connection with this Agreement for the benefit of any other person. Any purported assignment in breach of this Section shall confer no rights on the purported assignee.

18.4 Ultragenyx shall be entitled upon giving written notice to Rentschler to assign its rights under this Agreement to any member of Ultragenyx's Group or to a third party which acquires all or substantially all assets relating to the Product. Any assignment made pursuant to this **Section 18.4** shall be subject to the following terms:

18.4.1 no such assignment shall relieve Ultragenyx of any of its accrued obligations under this Agreement; and

18.4.2 any such assignment shall be made on terms that the assignee acknowledges that Rentschler may continue to deal exclusively with Ultragenyx in respect of all matters relating to this Agreement at all times unless and until the assignee notifies Rentschler in writing that it is exercising its rights as assignee.

Entire Agreement

18.5 This Agreement, and the Schedules and documents referred to in it, constitutes the entire agreement and understanding of the Parties and supersedes any previous agreement between the Parties relating to the subject matter of this Agreement. In the event of any conflict or ambiguity between this Agreement, the Quality Agreement, or any Schedule, this Agreement will control.

Waiver

18.6 In no event will any delay, failure or omission (in whole or in part) in enforcing, exercising or pursuing any right, power, privilege, claim or remedy conferred by or arising under this Agreement or by law, be deemed to be or construed as a waiver of that or any other right, power, privilege, claim or remedy in respect of the circumstances in question, or operate so as to bar the enforcement of that, or any other right, power, privilege, claim or remedy, in any other instance at any time or times subsequently.

Severability

18.7 If any provision of this Agreement shall be found by any court or administrative body of competent jurisdiction to be invalid or unenforceable, such invalidity or unenforceability shall not affect the other provisions of this Agreement which shall remain in full force and effect. The Parties agree, in the circumstances referred to in this Section to attempt to substitute for any invalid or unenforceable provision a valid or enforceable provision which achieves to the greatest extent possible the same effect as would have been achieved by the invalid or unenforceable provision. The obligations of the Parties under any invalid or unenforceable provision of this Agreement shall be suspended whilst an attempt at such substitution is made.

Notices

18.8 Any notice given or made under this Agreement shall be in writing and signed by or on behalf of the Party giving it and shall be served by hand, delivering it or sending it by prepaid recorded or special delivery post or prepaid international recorded airmail, to the address stated above or as notified in writing from time to time by the relevant Party to the other Party.

Counterparts; Interpretation; Independent Contractors

18.9 This Agreement may be executed in any number of counterparts and by the Parties to it on separate counterparts, each of which shall be an original, but all of which together shall constitute one and the same instrument. This Agreement is not effective until each Party has executed at least one counterpart.

18.10 This Agreement is in the English language only, which language shall be controlling in all respects, and all versions hereof in any other language shall be for accommodation only and shall not be binding on the Parties. All terms used in this Agreement shall be interpreted in accordance with the corresponding Swiss legal terms.

18.11 The Parties shall perform their obligations under this Agreement as independent contractors, and nothing contained in this Agreement shall be construed to be inconsistent with such relationship or status. This Agreement shall not constitute, create or in any way be interpreted as a joint venture or partnership of any kind.

Export Control

18.12 This Agreement is made subject to any restrictions concerning the export of products or technical information from the United States of America or other countries which may be imposed upon or related to Ultragenyx or Rentschler from time to time. Each Party agrees that it will not export, directly or indirectly, any technical information acquired from the other Party under this Agreement or any products using such technical information to a location or in a manner that at the time of export requires an export license or other governmental approval, without first obtaining the written consent to do so from the appropriate agency or other governmental entity.

THIS AGREEMENT has been executed by or on behalf of the Parties as of the date of the last signature shown below.

Signed on behalf of
Rentschler Biotechnologie GmbH

Date: August 31, 2012

Signature: /s/ Klaus Schoepe
Printed Name: Dr. Klaus Schoepe
Position: SVP Client Relations

Date: August 31, 2012

Signature: /s/ Thomas Sikloh
Printed Name: Thomas Sikloh
Position: SVP GMP-Operations

Signed on behalf of
Ultragenyx Pharmaceutical Inc.

Date: August 27, 2012

Signature: /s/ Thomas Kassberg
Printed Name: Thomas Kassberg
Position: Chief Business Officer

If second signature is required:

Date:

Signature:
Printed Name:
Position:

ULTRAGENYX PHARMACEUTICAL, INC.
2011 EQUITY INCENTIVE PLAN

ARTICLE 1
PURPOSE

1.1 General. The purpose of the Ultragenyx Pharmaceutical, Inc. 2011 Equity Incentive Plan (the "***Plan***") is to promote the success and enhance the value of Ultragenyx Pharmaceutical, Inc., Inc., a Delaware corporation (the "***Company***"), by linking the personal interests of the members of the Board, Employees and Consultants of the Company and any Parent or Subsidiary, to those of Company stockholders and by providing such individuals with an incentive for performance to generate returns to Company stockholders. The Plan is further intended to provide flexibility to the Company in its ability to motivate, attract, and retain the services of members of the Board, Employees and Consultants of the Company and any Parent or Subsidiary upon whose judgment, interest, and special effort the successful conduct of the Company's operation is largely dependent.

ARTICLE 2
DEFINITIONS AND CONSTRUCTION

2.1 Definitions. The following words and phrases shall have the following meanings:

(a) "***Administrator***" means the Board or a committee of the Board as described in Article 12.

(b) "***Award***" means an Option, a Restricted Stock award, a Stock Appreciation Right award, a Dividend Equivalents award, a Stock Payment award, or a Restricted Stock Unit award granted to a Participant pursuant to the Plan.

(c) "***Award Agreement***" means any written or electronic agreement, contract, or other instrument or document evidencing an Award.

(d) "***Board***" means the Board of Directors of the Company.

(e) "***Cause***," unless otherwise defined in an employment or services agreement between the Participant and the Company or any Parent or Subsidiary, means (i) a Participant's breach of any confidentiality or proprietary information agreement between the Participant and the Company or any Parent or Subsidiary; (ii) a Participant's conviction by, or entry of a plea of guilty or nolo contendere in, a court of competent and final jurisdiction for any crime involving moral turpitude or punishable by imprisonment in the jurisdiction involved; (iii) a Participant's commission of an act of fraud, whether prior to or subsequent to the date hereof upon the Company or any Parent or Subsidiary; (iv) a Participant's continuing repeated willful failure or refusal to perform his or her duties (including, without limitation, a Participant's inability to perform his or her duties as a result of chronic alcoholism or drug addiction and/or as a result of any failure to comply with any laws, rules or regulations of any governmental entity with respect to a Participant's employment by the Company or any Parent or Subsidiary); (v) a Participant's gross negligence, insubordination or material violation of any duty of loyalty to the Company or any Parent or Subsidiary or any other material misconduct on the part of a

Participant; (vi) a Participant's intentional commission of any act which he or she knows (or reasonably should know) is likely to be materially detrimental to the Company's or any Parent's or Subsidiary's business or goodwill; or (vii) a Participant's material breach of any other provision of any agreement between the Participant and the Company or any Parent or Subsidiary, provided that termination of a Participant's employment pursuant to this subsection (vii) shall not constitute valid termination for good cause unless such Participant shall have first received written notice from the Board or its designee stating with specificity the nature of such breach and affording the Participant at least fifteen days to correct the breach alleged.

The foregoing definition shall not in any way preclude or restrict the right of the Company or any successor or Parent or Subsidiary thereof to discharge or dismiss any Participant in the service of such entity for any other acts or omissions, but such other acts or omissions shall not be deemed, for purposes of this Plan, to constitute grounds for termination for Cause.

(f) "***Change in Control***" means and includes each of the following:

(i) the acquisition, directly or indirectly, by any "person" or "group" (as those terms are defined in Sections 3(a)(9), 13(d), and 14(d) of the Exchange Act and the rules thereunder) of "beneficial ownership" (as determined pursuant to Rule 13d-3 under the Exchange Act) of securities entitled to vote generally in the election of directors ("**voting securities**") of the Company that represent 50% or more of the combined voting power of the Company's then outstanding voting securities, other than:

(A) an acquisition by a trustee or other fiduciary holding securities under any employee benefit plan (or related trust) sponsored or maintained by the Company or any person controlled by the Company or by any employee benefit plan (or related trust) sponsored or maintained by the Company or any person controlled by the Company, or

(B) an acquisition of voting securities by the Company or a corporation owned, directly or indirectly, by the stockholders of the Company in substantially the same proportions as their ownership of the stock of the Company, or

(C) an acquisition of voting securities pursuant to a transaction described in subsection (iii) below that would not be a Change in Control under subsection (iii);

Notwithstanding the foregoing, the following event shall not constitute an "acquisition" by any person or group for purposes of this Section 2.1(e): an acquisition of the Company's securities by the Company which causes the Company's voting securities beneficially owned by a person or group to represent 50% or more of the combined voting power of the Company's then outstanding voting securities; *provided, however,* that if a person or group shall become the beneficial owner of 50% or more of the combined voting power of the Company's then outstanding voting securities by reason of share acquisitions by the Company as described above and shall, after such share acquisitions by the Company, become the beneficial owner of any additional voting securities of the Company, then such acquisition shall constitute a Change in Control; or

(ii) during any period of two consecutive years, individuals who, at the beginning of such period, constitute the Board together with any new director(s) (other than a director designated by a person who shall have entered into an agreement with the Company to effect a transaction described in subsections (a) or (c) of this Section 2.1(e)) whose election by the Board or nomination for election by the Company's stockholders was approved by a vote of at least two-thirds of the directors then still in office who either were directors at the beginning of the two year period or whose election or nomination for election was previously so approved, cease for any reason to constitute a majority thereof; or

(iii) the consummation by the Company (whether directly involving the Company or indirectly involving the Company through one or more intermediaries) of a merger, consolidation, reorganization, or business combination, a sale or other disposition of all or substantially all of the Company's assets, or the acquisition of assets or stock of another entity, in each case, other than a transaction

(A) which results in the Company's voting securities outstanding immediately before the transaction continuing to represent (either by remaining outstanding or by being converted into voting securities of the Company or the person that, as a result of the transaction, controls, directly or indirectly, the Company or owns, directly or indirectly, all or substantially all of the Company's assets or otherwise succeeds to the business of the Company (the Company or such person, the "***Successor Entity***")) directly or indirectly, at least 50% of the combined voting power of the Successor Entity's outstanding voting securities immediately after the transaction, and

(B) after which no person or group beneficially owns voting securities representing 50% or more of the combined voting power of the Successor Entity; *provided, however,* that no person or group shall be treated for purposes of this paragraph (iii) as beneficially owning 50% or more of combined voting power of the Successor Entity solely as a result of the voting power held in the Company prior to the consummation of the transaction; or

(iv) the Company's stockholders approve a liquidation or dissolution of the Company.

For purposes of subsection (i) above, the calculation of voting power shall be made as if the date of the acquisition were a record date for a vote of the Company's stockholders, and for purposes of subsection (iii) above, the calculation of voting power shall be made as if the date of the consummation of the transaction were a record date for a vote of the Company's stockholders.

Notwithstanding the foregoing, a transaction shall not constitute a "***Change of Control***" if: (i) its sole purpose is to change the state of the Company's incorporation; (ii) its sole purpose is to create a holding company that will be owned in substantially the same proportions by the persons who held the Company's securities immediately before such transaction; (iii) it

constitutes the Company's initial public offering of its securities; or (iv) it is a transaction effected primarily for the purpose of financing the Company with cash (as determined by the Administrator in its discretion and without regard to whether such transaction is effectuated by a merger, equity financing or otherwise).

The Administrator shall have full and final authority, which shall be exercised in its sole discretion, to determine conclusively whether a Change in Control of the Company has occurred pursuant to the above definition, and the date of the occurrence of such Change in Control and any incidental matters relating thereto.

(g) "***Code***" means the Internal Revenue Code of 1986, as amended from time to time, and the regulations issued thereunder.

(h) "***Committee***" means a committee of the Board described in Article 12.

(i) "***Consultant***" means any consultant or adviser if:

(i) The consultant or adviser renders bona fide services to the Company or any Parent or Subsidiary; and

(ii) The services rendered by the consultant or adviser are not in connection with the offer or sale of securities in a capital-raising transaction and do not directly or indirectly promote or maintain a market for the Company's securities.

(j) "***Disability***" means a permanent and total disability within the meaning of Section 22(e)(3) of the Code, as it may be amended from time to time.

(k) "***Dividend Equivalents***" means a right granted to a Participant pursuant to Article 8 to receive the equivalent value (in cash or Stock) of dividends paid on Stock.

(l) "***Eligible Individual***" means any person who is a member of the Board, a Consultant or an Employee, as determined by the Administrator.

(m) "***Employee***" means any officer or other employee (as defined in accordance with Section 3401(c) of the Code) of the Company or any Parent or Subsidiary.

(n) "***Equity Restructuring***" means a non-reciprocal transaction between the Company and its stockholders, such as a stock dividend, stock split, spin-off, rights offering or recapitalization through a large, nonrecurring cash dividend, that affects the shares of Stock (or other securities of the Company) or the share price of Stock (or other securities) and causes a change in the per share value of the Stock underlying outstanding Awards.

(o) "***Exchange Act***" means the Securities Exchange Act of 1934, as amended from time to time.

(p) "***Fair Market Value***" means, as of any date, the value of Stock determined as follows:

(i) If the Stock is listed on any established stock exchange or a national market system, including without limitation the Nasdaq National Market or The Nasdaq SmallCap Market of The Nasdaq Stock Market, its Fair Market Value shall be the closing sales price for such Stock as quoted on such exchange or system for the last market trading day prior to the date of determination for which a closing sales price is reported, as reported in *The Wall Street Journal* or such other source as the Administrator deems reliable;

(ii) If the Stock is regularly quoted by a recognized securities dealer but selling prices are not reported, its Fair Market Value shall be the mean of the closing bid and asked prices for the Stock on the date prior to the date of determination as reported in *The Wall Street Journal* or such other source as the Administrator deems reliable; or

(iii) In the absence of an established market for the Stock, the Fair Market Value thereof shall be determined in good faith by the Administrator.

(q) "***Good Reason***" means a Participant's voluntary resignation following any one or more of the following that is effected without the Participant's written consent: (i) a change in his or her position following the Change in Control that materially reduces his or her duties or responsibilities, (ii) a reduction in his or her base salary following a Change in Control, unless the base salaries of all similarly situated individuals are similarly reduced, or (iii) a relocation of such Participant's place of employment of more than fifty miles following a Change in Control. However, if the term or concept of "Good Reason" has been defined in an agreement between a Participant and the Company or any successor or parent or Subsidiary thereof, then "Good Reason" shall have the definition set forth in such agreement.

(r) "***Incentive Stock Option***" means an Option that is intended to be an incentive stock option and meets the requirements of Section 422 of the Code or any successor provision thereto.

(s) "***Misconduct***" means the commission of any commission of any act of fraud, embezzlement or dishonesty by the Participant, any unauthorized use or disclosure by such person of confidential information or trade secrets of the Company (or any Parent or Subsidiary), or any other intentional misconduct by such person adversely affecting the business or affairs of the Company (or any Parent or Subsidiary) in a material manner. The foregoing definition shall not in any way preclude or restrict the right of the Company (or any Parent or Subsidiary) to discharge or dismiss any Participant or other person in the service of the Company (or any Parent or Subsidiary) for any other acts or omissions, but such other acts or omissions shall not be deemed, for purposes of the Plan, to constitute grounds for termination for Misconduct.

(t) "***Non-Employee Director***" means a member of the Board who is not an Employee.

(u) "***Non-Qualified Stock Option***" means an Option that is not intended to be or otherwise does not qualify as an Incentive Stock Option.

(v) "***Option***" means a right granted to a Participant pursuant to Article 5 of the Plan to purchase a specified number of shares of Stock at a specified price during specified time periods. An Option may be either an Incentive Stock Option or a Non-Qualified Stock Option.

(w) "***Parent***" means any corporation in an unbroken chain of corporations ending with the Company if each of the corporations other than the Company then owns stock possessing 50% or more of the total combined voting power of all classes of stock in one of the other corporations in such chain at the relevant time, including after the Effective Date (as defined in Section 13.1).

(x) "***Participant***" means any Eligible Individual who, as a member of the Board, an Employee or a Consultant, has been granted an Award pursuant to the Plan.

(y) "***Plan***" means this Ultragenyx Pharmaceutical, Inc. 2011 Equity Incentive Plan, as it may be amended from time to time.

(z) "***Public Trading Date***" means the first date upon which the issuer is subject to the reporting requirements of Section 13 or 15(d)(2) of the Exchange Act.

(aa) "***Restricted Stock***" means Stock awarded to a Participant pursuant to Article 6 that is subject to certain restrictions and may be subject to risk of forfeiture or repurchase.

(bb) "***Restricted Stock Unit***" means a right to receive a share of Stock during specified time periods granted pursuant to Section 8.3.

(cc) "***Securities Act***" means the Securities Act of 1933, as amended from time to time.

(dd) "***Section 409A Award***" has the meaning set forth in Section 9.1.

(ee) "***Stock***" means the common stock of the Company and such other securities of the Company that may be substituted for Stock pursuant to Article 11.

(ff) "***Stock Appreciation Right***" or "***SAR***" means a right granted pursuant to Article 7 to receive a payment equal to the excess of the Fair Market Value of a specified number of shares of Stock on the date the SAR is exercised over the Fair Market Value of such number of shares of Stock on the date the SAR was granted as set forth in the applicable Award Agreement.

(gg) "***Stock Payment***" means (a) a payment in the form of shares of Stock, or (b) an option or other right to purchase shares of Stock, as part of any bonus, deferred compensation or other arrangement, made in lieu of all or any portion of the compensation, granted pursuant to Section 8.2.

(hh) "***Subsidiary***" means any corporation or other entity of which a majority of the outstanding voting stock or voting power is beneficially owned directly or indirectly by the Company at the relevant time, including after the Effective Date (as defined in Section 13.1).

(ii) "***Successor Entity***" has the meaning set forth in Section 2.1(f)(iii).

(jj) "***Termination of Consultancy***" means the time when the engagement of a Participant as a Consultant to the Company or a Parent or Subsidiary is terminated for any reason, with or without cause, including, but not by way of limitation, by resignation, discharge, death or retirement, but excluding terminations where there is a simultaneous commencement of employment with the Company or any Parent or Subsidiary. The Administrator, in its absolute discretion, shall determine the effect of all matters and questions relating to Termination of Consultancy, including, but not by way of limitation, the question of whether a Termination of Consultancy resulted from a discharge for good cause, and all questions of whether a particular leave of absence constitutes a Termination of Consultancy. Notwithstanding any other provision of the Plan, the Company or any Parent or Subsidiary has an absolute and unrestricted right to terminate a Consultant's service at any time for any reason whatsoever, with or without cause, except to the extent expressly provided otherwise in writing.

(kk) "***Termination of Directorship***" shall mean the time when a Participant who is a Non-Employee Director ceases to be a member of the Board for any reason, including, but not by way of limitation, a termination by resignation, failure to be elected, death or retirement. The Board, in its sole and absolute discretion, shall determine the effect of all matters and questions relating to Termination of Directorship with respect to Non- Employee Directors.

(ll) "***Termination of Employment***" shall mean the time when the employee-employer relationship between a Participant and the Company or any Parent or Subsidiary is terminated for any reason, with or without cause, including, but not by way of limitation, a termination by resignation, discharge, death, disability or retirement; but excluding: (a) terminations where there is a simultaneous reemployment or continuing employment of a Participant by the Company or any Parent or Subsidiary, (b) at the discretion of the Administrator, terminations which result in a temporary severance of the employee-employer relationship, and (c) terminations which are followed by the simultaneous establishment of a consulting relationship by the Company or a Parent or Subsidiary with the former employee. The Administrator, in its absolute discretion, shall determine the effect of all matters and questions relating to Termination of Employment, including, but not by way of limitation, the question of whether a Termination of Employment resulted from a discharge for good cause, and all questions of whether a particular leave of absence constitutes a Termination of Employment.

(ii) "***Termination of Service***" shall mean the last to occur of a Participant's Termination of Employment, Termination of Directorship or Termination of Consultancy. A Participant shall not be deemed to have a Termination of Service merely because of a change in the capacity in which the Participant renders service to the Company or any Parent or Subsidiary (i.e., a Participant who is an Employee becomes a Consultant) or a change in the entity for which the Participant renders such service (i.e., an Employee of the Company becomes an Employee of a Subsidiary), unless following such change in capacity or service the Participant is no longer serving as an Employee, Non-Employee Director or Consultant of the Company or any Parent or Subsidiary.

ARTICLE 3
SHARES SUBJECT TO THE PLAN

3.1 Number of Shares.

(a) Subject to Article 11, the aggregate number of shares of Stock which may be issued or transferred pursuant to Awards under the Plan shall be 7,455,988 shares.

(b) To the extent that an Award terminates, expires, or lapses for any reason, any shares of Stock subject to the Award shall again be available for the grant of an Award pursuant to the Plan. Additionally, any shares of Stock tendered or withheld to satisfy the grant or exercise price or tax withholding obligation pursuant to any Award shall again be available for the grant of an Award pursuant to the Plan. If shares of Stock issued pursuant to Awards are forfeited by a Participant or repurchased by the Company pursuant to Section 6.3 hereof, such shares of Stock shall become available for future grant under the Plan (unless the Plan has terminated). The payment of Dividend Equivalents in cash in conjunction with any outstanding Awards shall not be counted against the shares available for issuance under the Plan.

(c) Notwithstanding the provisions of this Section 3.1, no shares of Stock may again be optioned, granted or awarded if such action would cause an Incentive Stock Option to fail to qualify as an Incentive Stock Option under Section 422 of the Code.

3.2 Stock Distributed. Any Stock distributed pursuant to an Award may consist, in whole or in part, of authorized and unissued Stock, treasury Stock or, on and after the Public Trading Date, Stock purchased on the open market.

ARTICLE 4
ELIGIBILITY AND PARTICIPATION

4.1 Eligibility. Persons eligible to participate in this Plan include all Employees, Consultants and all members of the Board, as determined by the Administrator.

4.2 Actual Participation. Subject to the provisions of the Plan, the Administrator may, from time to time, select from among all Eligible Individuals those to whom Awards shall be granted and shall determine the nature and amount of each Award. No individual shall have any right to be granted an Award pursuant to this Plan.

4.3 Foreign Participants. Notwithstanding any provision of the Plan to the contrary, in order to comply with the laws in other countries in which the Company and its Parents or Subsidiaries operate or have Eligible Individuals, the Administrator, in its sole discretion, shall have the power and authority to: (i) determine which Parents or Subsidiaries shall be covered by the Plan; (ii) determine which Eligible Individuals outside the United States are eligible to participate in the Plan; (iii) modify the terms and conditions of any Award granted to Eligible Individuals outside the United States to comply with applicable foreign laws; (iv) establish subplans and modify exercise procedures and other terms and procedures, to the extent such actions may be necessary or advisable (any such subplans and/or modifications shall be attached to this Plan as appendices); *provided, however,* that no such subplans and/or modifications shall increase the share limitation contained in Section 3.1 of the Plan; and (v) take any action, before

or after an Award is made, that it deems advisable to obtain approval or comply with any necessary local governmental regulatory exemptions or approvals. Notwithstanding the foregoing, the Administrator may not take any actions hereunder, and no Awards shall be granted, that would violate the Exchange Act, the Code, any securities law or governing statute or any other applicable law.

ARTICLE 5
STOCK OPTIONS

5.1 General. The Administrator is authorized to grant Options to Eligible Individuals on the following terms and conditions:

(a) *Exercise Price*. The exercise price per share of Stock subject to an Option shall be determined by the Administrator and set forth in the Award Agreement; *provided* that the exercise price per share for any Option shall not be less than 100% of the Fair Market Value per share of the Stock on the date of the grant.

(b) *Time and Conditions of Exercise*. The Administrator shall determine the time or times at which an Option may be exercised in whole or in part; *provided* that the term of any Option granted under the Plan shall not exceed ten years. The Administrator shall also determine the performance or other conditions, if any, that must be satisfied before all or part of an Option may be exercised. The Administrator may extend the term of any outstanding Option in connection with any Termination of Employment, Termination of Directorship or Termination of Consultancy of the Participant holding such Option, or amend any other term or condition of such Option relating to such a Termination of Employment, Termination of Directorship or Termination of Consultancy.

(c) *Payment*. The Administrator shall determine the methods, terms and conditions by which the exercise price of an Option may be paid, and the form and manner of payment, including, without limitation, payment in the form of cash, a promissory note bearing interest at no less than such rate as shall then preclude the imputation of interest under the Code, shares of Stock previously owned by the Participant or otherwise issuable upon exercise of the Option, or other property acceptable to the Administrator and payment through the delivery of a notice that the Participant has placed a market sell order with a broker with respect to shares of Stock then issuable upon exercise of the Option, and that the broker has been directed to pay a sufficient portion of the net proceeds of the sale to the Company in satisfaction of the Option exercise price; *provided* that payment of such proceeds is then made to the Company upon settlement of such sale, and the methods by which shares of Stock shall be delivered or deemed to be delivered to Participants. Notwithstanding any other provision of the Plan to the contrary, no Participant who is a member of the Board or an “executive officer” of the Company within the meaning of Section 13(k) of the Exchange Act shall be permitted to pay the exercise price of an Option, or continue any extension of credit with respect to the exercise price of an Option with a loan from the Company or a loan arranged by the Company, in any method which would violate Section 13(k) of the Exchange Act.

(d) *Evidence of Grant*. All Options shall be evidenced by an Award Agreement between the Company and the Participant. The Award Agreement shall include such additional provisions as may be specified by the Administrator.

5.2 Incentive Stock Options. Incentive Stock Options may be granted only to employees (as defined in accordance with Section 3401(c) of the Code) of the Company or a Subsidiary which constitutes a "subsidiary corporation" of the Company within Section 424(f) of the Code or a Parent which constitutes a "parent corporation" of the Company within the meaning of Section 424(e) of the Code and the terms of any Incentive Stock Options granted pursuant to the Plan must comply with the following additional provisions of this Section 5.2 in addition to the requirements of Section 5.1:

(a) *Ten Percent Owners*. An Incentive Stock Option shall be granted to any individual who, at the date of grant, owns stock possessing more than ten percent of the total combined voting power of all classes of stock of the Company or any "subsidiary corporation" of the Company or "parent corporation" of the Company (each within the meaning of Section 424 of the Code) only if such Option is granted at an exercise price per share that is not less than 110% of the Fair Market Value per share of the Stock on the date of the grant and the Option is exercisable for no more than five years from the date of grant.

(b) *Transfer Restriction*. An Incentive Stock Option shall not be transferable by the Participant other than by will or by the laws of descent or distribution.

(c) *Right to Exercise*. During a Participant's lifetime, an Incentive Stock Option may be exercised only by the Participant.

(d) *Failure to Meet Requirements*. Any Option (or portion thereof) purported to be an Incentive Stock Option which, for any reason, fails to meet the requirements of Section 422 of the Code shall be considered a Non- Qualified Stock Option.

5.3 Early Exercisability. The Administrator may provide in the terms of a Participant's Award Agreement that the Participant may, at any time before the Participant's status as an Employee, member of the Board or Consultant terminates, exercise the Option(s) granted to such Participant in whole or in part prior to the full vesting of the Option(s); *provided, however,* shares of Stock acquired upon exercise of an Option which has not fully vested may be subject to any forfeiture, transfer or other restrictions as the Administrator may determine in its sole discretion.

5.4 Paperless Exercise. In the event that the Company establishes, for itself or using the services of a third party, an automated system for the exercise of Options, such as a system using an internet website or interactive voice response, then the paperless exercise of Options by a Participant may be permitted through the use of such an automated system.

ARTICLE 6
RESTRICTED STOCK AWARDS

6.1 Grant of Restricted Stock. The Administrator is authorized to make Awards of Restricted Stock to any Eligible Individual selected by the Administrator in such amounts and subject to such terms and conditions as determined by the Administrator. All Awards of Restricted Stock shall be evidenced by an Award Agreement.

6.2 Issuance and Restrictions. Restricted Stock shall be subject to such repurchase restrictions, forfeiture restrictions, restrictions on transferability and other restrictions as the Administrator may impose (including, without limitation, limitations on the right to vote Restricted Stock or the right to receive dividends on the Restricted Stock). These restrictions may lapse separately or in combination at such times, pursuant to such circumstances or in such installments or otherwise as the Administrator determines at the time of the grant of the Award or thereafter.

6.3 Repurchase or Forfeiture. Except as otherwise determined by the Administrator at the time of the grant of the Award or thereafter, upon a Participant's Termination of Service during the applicable restriction period, Restricted Stock that is at that time subject to restrictions shall be forfeited or subject to repurchase by the Company (or its assignee) under such terms as the Administrator shall determine; *provided, however,* that the Administrator may (a) provide in any Restricted Stock Award Agreement that restrictions or forfeiture conditions relating to Restricted Stock will be waived in whole or in part in the event of a Participant's Termination of Service, and (b) in other cases waive in whole or in part restrictions or forfeiture conditions relating to Restricted Stock.

6.4 Certificates for Restricted Stock. Restricted Stock granted pursuant to the Plan may be evidenced in such manner as the Administrator shall determine. If certificates representing shares of Restricted Stock are registered in the name of the Participant, certificates must bear an appropriate legend referring to the terms, conditions, and restrictions applicable to such Restricted Stock, and the Company may, at its discretion, retain physical possession of the certificate until such time as all applicable restrictions lapse or the Award Agreement may provide that the shares shall be held in escrow by an escrow agent designated by the Company.

ARTICLE 7
STOCK APPRECIATION RIGHTS

7.1 Grant of Stock Appreciation Rights. A Stock Appreciation Right may be granted to any Eligible Individual selected by the Administrator. A Stock Appreciation Right shall be subject to such terms and conditions not inconsistent with the Plan as the Administrator shall impose and shall be evidenced by an Award Agreement.

7.2 Terms of Stock Appreciation Rights.

(a) A Stock Appreciation Right shall have a term set by the Administrator. A Stock Appreciation Right shall be exercisable in such installments as the Administrator may determine. A Stock Appreciation Right shall cover such number of shares of Stock as the Administrator may determine. The exercise price per share of Stock subject to each Stock Appreciation Right shall be set by the Administrator.

(b) A Stock Appreciation Right shall entitle the Participant (or other person entitled to exercise the Stock Appreciation Right pursuant to the Plan) to exercise all or a specified portion of the Stock Appreciation Right (to the extent then exercisable pursuant to its

terms) and to receive from the Company an amount determined by multiplying (i) the amount (if any) by which the Fair Market Value of a share of Stock on the date of exercise of the Stock Appreciation Right exceeds the exercise price per share of the Stock Appreciation Right, by (ii) the number of shares of Stock with respect to which the Stock Appreciation Right shall have been exercised, subject to any limitations the Administrator may impose.

7.3 Payment and Limitations on Exercise.

(a) Subject to Sections 7.3(b) and (c), payment of the amounts determined under Section 7.2(b) above shall be in cash, in Stock (based on its Fair Market Value as of the date the Stock Appreciation Right is exercised) or a combination of both, as determined by the Administrator.

(b) To the extent payment for a Stock Appreciation Right is to be made in cash, the Award Agreement shall, to the extent necessary to comply with the requirements of Section 409A of the Code, specify the date of payment, which may be different than the date of exercise of the Stock Appreciation Right. If the date of payment for a Stock Appreciation Right is later than the date of exercise, the Award Agreement may specify that the Participant be entitled to earnings on such amount until paid.

(c) To the extent any payment under Section 7.2(b) is effected in Stock, it shall be made subject to satisfaction of all provisions of Article 5 above pertaining to Options.

ARTICLE 8
OTHER TYPES OF AWARDS

8.1 Dividend Equivalents. Any Eligible Individual selected by the Administrator may be granted Dividend Equivalents based on the dividends declared on the shares of Stock that are subject to any Award, to be credited as of dividend payment dates, during the period between the date the Award is granted and the date the Award is exercised, vests or expires, as determined by the Administrator. Such Dividend Equivalents shall be converted to cash or additional shares of Stock by such formula and at such time and subject to such limitations as may be determined by the Administrator.

8.2 Stock Payments. Any Eligible Individual selected by the Administrator may receive Stock Payments in the manner determined from time to time by the Administrator; *provided* that, unless otherwise determined by the Administrator, such Stock Payments shall be made in lieu of base salary, bonus or other cash compensation otherwise payable to such Eligible Individual. The number of shares shall be determined by the Administrator and may be based upon the Performance Goals or other specific performance goals determined appropriate by the Administrator.

8.3 Restricted Stock Units. The Administrator is authorized to make Awards of Restricted Stock Units to any Eligible Individual selected by the Administrator in such amounts and subject to such terms and conditions as determined by the Administrator. At the time of grant, the Administrator shall specify the date or dates on which the Restricted Stock Units shall become fully vested and nonforfeitable, and may specify such conditions to vesting as it deems appropriate. Alternatively, Restricted Stock Units may become fully vested and nonforfeitable

pursuant to the satisfaction of one or more Performance Goals or other specific performance goals as the Administrator determines to be appropriate at the time of the grant of the Restricted Stock Units or thereafter, in each case on a specified date or dates or over any period or periods determined by the Administrator. At the time of grant, the Administrator shall specify the maturity date applicable to each grant of Restricted Stock Units which shall be no earlier than the vesting date or dates of the Award and, to the extent permitted by the Administrator, may be determined at the election of the Eligible Individual to whom the Award is granted. On the maturity date, the Company shall transfer to the Participant one unrestricted, fully transferable share of Stock for each Restricted Stock Unit that is vested and scheduled to be distributed on such date and not previously forfeited. The Administrator shall specify the purchase price, if any, to be paid by the Participant to the Company for such shares of Stock.

8.4 Term. Except as otherwise provided herein, the term of any Award of Performance Shares, Dividend Equivalents, Stock Payments or Restricted Stock Units shall be set by the Administrator in its discretion.

8.5 Exercise or Purchase Price. The Administrator may establish the exercise or purchase price, if any, of any Award of Restricted Stock Units or Stock Payments; *provided, however,* that such price shall not be less than the par value of a share of Stock on the date of grant, unless otherwise permitted by applicable state law.

8.6 Form of Payment. Payments with respect to any Awards granted under Sections 8.1, 8.2 or 8.3 shall be made in cash, in Stock or a combination of both, as determined by the Administrator.

8.7 Award Agreement. All Awards under this Article 8 shall be subject to such additional terms and conditions as determined by the Administrator and shall be evidenced by a written Award Agreement.

ARTICLE 9
COMPLIANCE WITH SECTION 409A OF THE CODE

9.1 Awards subject to Code Section 409A. Any Award that constitutes, or provides for, a deferral of compensation subject to Section 409A of the Code (a "***Section 409A Award***") shall satisfy the requirements of Section 409A of the Code and this Article 9, to the extent applicable. The Award Agreement with respect to a Section 409A Award shall incorporate the terms and conditions required by Section 409A of the Code and this Article 9.

9.2 Distributions under a Section 409A Award.

(a) Subject to subsection (b), any shares of Stock or other property or amounts to be paid or distributed upon the grant, issuance, vesting, exercise or payment of a Section 409A Award shall be distributed in accordance with the requirements of Section 409A(a)(2) of the Code, and shall not be distributed earlier than:

(i) the Participant's separation from service, as determined by the Secretary of the Treasury;

(ii) the date the Participant becomes disabled;

(iii) the Participant's death;

(iv) a specified time (or pursuant to a fixed schedule) specified under the Award Agreement at the date of the deferral compensation;

(v) to the extent provided by the Secretary of the Treasury, a change in the ownership or effective control of the Company or a Parent or Subsidiary, or in the ownership of a substantial portion of the assets of the Company or a Parent or Subsidiary; or

(vi) the occurrence of an unforeseeable emergency with respect to the Participant.

(b) In the case of a Participant who is a "specified employee," the requirement of paragraph (a)(i) shall be met only if the distributions with respect to the Section 409A Award may not be made before the date which is six months after the Participant's separation from service (or, if earlier, the date of the Participant's death). For purposes of this subsection (b), a Participant shall be a "specified employee" if such Participant is a key employee (as defined in Section 416(i) of the Code without regard to paragraph (5) thereof) of a corporation any stock of which is publicly traded on an established securities market or otherwise, as determined under Section 409A(a)(2)(B)(i) of the Code and the Treasury Regulations thereunder.

(c) The requirement of paragraph (a)(vi) shall be met only if, as determined under Treasury Regulations under Section 409A(a)(2)(B)(ii) of the Code, the amounts distributed with respect to the unforeseeable emergency do not exceed the amounts necessary to satisfy such unforeseeable emergency plus amounts necessary to pay taxes reasonably anticipated as a result of the distribution, after taking into account the extent to which such unforeseeable emergency is or may be relieved through reimbursement or compensation by insurance or otherwise or by liquidation of the Participant's assets (to the extent the liquidation of such assets would not itself cause severe financial hardship).

(d) For purposes of this Section, the terms specified therein shall have the respective meanings ascribed thereto under Section 409A of the Code and the Treasury Regulations thereunder.

9.3 Prohibition on Acceleration of Benefits. The time or schedule of any distribution or payment of any shares of Stock or other property or amounts under a Section 409A Award shall not be accelerated, except as otherwise permitted under Section 409A(a)(3) of the Code and the Treasury Regulations thereunder.

9.4 Elections under Section 409A Awards.

(a) Any deferral election provided under or with respect to an Award to any Eligible Individual, or to the Participant holding a Section 409A Award, shall satisfy the requirements of Section 409A(a)(4)(B) of the Code, to the extent applicable, and, except as otherwise permitted under paragraph (i) or (ii) below, any such deferral election with respect to compensation for services performed during a taxable year shall be made not later than the close of the preceding taxable year, or at such other time as provided in Treasury Regulations.

(i) In the case of the first year in which an Eligible Individual or a Participant holding a Section 409A Award, becomes eligible to participate in the Plan, any such deferral election may be made with respect to services to be performed subsequent to the election with thirty days after the date the Eligible Individual, or the Participant holding a Section 409A Award, becomes eligible to participate in the Plan, as provided under Section 409A(a)(4)(B)(ii) of the Code.

(ii) In the case of any performance-based compensation based on services performed by an Eligible Individual, or the Participant holding a Section 409A Award, over a period of at least twelve months, any such deferral election may be made no later than six months before the end of the period, as provided under Section 409A(a)(4)(B)(iii) of the Code.

(b) In the event that a Section 409A Award permits, under a subsequent election by the Participant holding such Section 409A Award, a delay in a distribution or payment of any shares of Stock or other property or amounts under such Section 409A Award, or a change in the form of distribution or payment, such subsequent election shall satisfy the requirements of Section 409A(a)(4)(C) of the Code, and:

(i) such subsequent election may not take effect until at least twelve months after the date on which the election is made,

(ii) in the case such subsequent election relates to a distribution or payment not described in Section 9.2(a)(ii), (iii) or (vi), the first payment with respect to such election may be deferred for a period of not less than five years from the date such distribution or payment otherwise would have been made, and

(iii) in the case such subsequent election relates to a distribution or payment described in Section 9.2(a)(iv), such election may not be made less than twelve months prior to the date of the first scheduled distribution or payment under Section 9.2(a)(iv).

9.5 Compliance in Form and Operation. A Section 409A Award, and any election under or with respect to such Section 409A Award, shall comply in form and operation with the requirements of Section 409A of the Code and the Treasury Regulations thereunder.

ARTICLE 10
PROVISIONS APPLICABLE TO AWARDS

10.1 Stand-Alone and Tandem Awards. Awards granted pursuant to the Plan may, in the discretion of the Administrator, be granted either alone, in addition to, or in tandem with, any other Award granted pursuant to the Plan. Awards granted in addition to or in tandem with other Awards may be granted either at the same time as or at a different time from the grant of such other Awards.

10.2 Award Agreement. Awards under the Plan shall be evidenced by Award Agreements that set forth the terms, conditions and limitations for each Award which may include the term of an Award, the provisions applicable in the event of the Participant's Termination of Service, and the Company's authority to unilaterally or bilaterally amend, modify, suspend, cancel or rescind an Award.

10.3 Limits on Transfer.

(a) Except as otherwise provided by the Administrator pursuant to Section 10.3(b), no right or interest of a Participant in any Award may be pledged, encumbered, or hypothecated to or in favor of any party other than the Company or a Parent or Subsidiary, or shall be subject to any lien, obligation, or liability of such Participant to any other party other than the Company or a Parent or Subsidiary. Except as otherwise provided by the Administrator pursuant to Section 10.3(b), no Award shall be assigned, transferred, or otherwise disposed of by a Participant other than by will or the laws of descent and distribution, unless and until such Award has been exercised, or the shares underlying such Award have been issued, and all restrictions applicable to such shares have lapsed.

(b) Notwithstanding Section 10.3(a), the Administrator, in its sole discretion, may permit an Award (other than an Incentive Stock Option) to be transferred to, exercised by and paid to any one or more Permitted Transferees (as defined below), subject to the following terms and conditions: (i) an Award transferred to a Permitted Transferee shall not be assignable or transferable by the Permitted Transferee other than by will or the laws of descent and distribution; (ii) any Award which is transferred to a Permitted Transferee shall continue to be subject to all the terms and conditions of the Award as applicable to the original Participant (other than the ability to further transfer the Award); and (iii) the Participant and the Permitted Transferee shall execute any and all documents requested by the Administrator, including, without limitation documents to (A) confirm the status of the transferee as a Permitted Transferee, (B) satisfy any requirements for an exemption for the transfer under applicable federal and state securities laws and (C) evidence the transfer. For purposes of this Section 10.3(b), "***Permitted Transferee***" shall mean, with respect to a Participant, any child, stepchild, grandchild, parent, stepparent, grandparent, spouse, former spouse, sibling, niece, nephew, mother-in-law, father-in-law, son-in-law, daughter-in-law, brother-in-law, or sister-in-law, including adoptive relationships, any person sharing the Participant's household (other than a tenant or employee), a trust in which these persons (or the Participant) control the management of assets, and any other entity in which these persons (or the Participant) own more than fifty percent of the voting interests, or any other transferee specifically approved by the Administrator.

10.4 Beneficiaries. Notwithstanding Section 10.3, a Participant may, in the manner determined by the Administrator, designate a beneficiary to exercise the rights of the Participant and to receive any distribution with respect to any Award upon the Participant's death. A beneficiary, legal guardian, legal representative, or other person claiming any rights pursuant to the Plan is subject to all terms and conditions of the Plan and any Award Agreement applicable to the Participant, except to the extent the Plan and Award Agreement otherwise provide, and to any additional restrictions deemed necessary or appropriate by the Administrator. If the Participant is married and resides in a community property state, a designation of a person other

than the Participant's spouse as his or her beneficiary with respect to more than 50% of the Participant's interest in the Award shall not be effective without the prior written consent of the Participant's spouse. If no beneficiary has been designated or survives the Participant, payment shall be made to the person entitled thereto pursuant to the Participant's will or the laws of descent and distribution. Subject to the foregoing, a beneficiary designation may be changed or revoked by a Participant at any time provided the change or revocation is filed with the Administrator.

10.5 Stock Certificates; Book Entry Procedures.

(a) Notwithstanding anything herein to the contrary, the Company shall not be required to issue or deliver any certificates evidencing shares of Stock pursuant to the exercise or purchase of any Award, unless and until the Board has determined, with advice of counsel, that the issuance and delivery of such certificates is in compliance with all applicable laws, regulations of governmental authorities and, if applicable, the requirements of any exchange on which the shares of Stock are listed or traded. All Stock certificates delivered pursuant to the Plan are subject to any stop-transfer orders and other restrictions as the Administrator deems necessary or advisable to comply with federal, state, or foreign jurisdiction, securities or other laws, rules and regulations and the rules of any national securities exchange or automated quotation system on which the Stock is listed, quoted, or traded. The Administrator may place legends on any Stock certificate to reference restrictions applicable to the Stock. In addition to the terms and conditions provided herein, the Administrator may require that a Participant make such reasonable covenants, agreements, and representations as the Administrator, in its discretion, deems advisable in order to comply with any such laws, regulations, or requirements. The Administrator shall have the right to require any Participant to comply with any timing or other restrictions with respect to the settlement or exercise of any Award, including a window-period limitation, as may be imposed in the discretion of the Administrator.

(b) Notwithstanding any other provision of the Plan, unless otherwise determined by the Administrator or required by applicable law, rule or regulation, the Company shall not deliver to any Participant certificates evidencing shares of Stock issued in connection with any Award or exercise of any Award and instead such shares of Stock will be recorded in the books of the Company (or as applicable, its transfer agent or stock plan administrator).

ARTICLE 11
CHANGES IN CAPITAL STRUCTURE

11.1 Adjustments.

(a) In the event of any combination or exchange of shares, merger, consolidation, distribution of Company assets to stockholders (other than normal cash dividends), or any other corporate event affecting the Stock or the share price of the Stock, other than an Equity Restructuring, the Administrator shall make such proportionate adjustments to reflect such change with respect to (i) the aggregate number and type of shares that may be issued under the Plan (including, but not limited to, adjustments of the limitation in Section 3.1); (ii) the terms and conditions of any outstanding Awards (including, without limitation, any applicable performance targets or criteria with respect thereto); and (iii) the grant or exercise price per share for any outstanding Awards under the Plan.

(b) In the event of any transaction or event described in Section 11.1(a) or any unusual or nonrecurring transactions or events affecting the Company, any affiliate of the Company, or the financial statements of the Company or any affiliate (including without limitation any Change in Control), other than an Equity Restructuring, or of changes in applicable laws, regulations or accounting principles, and whenever the Administrator determines that such action is appropriate in order to prevent the dilution or enlargement of the benefits or potential benefits intended to be made available under the Plan or with respect to any Award under the Plan, to facilitate such transactions or events or to give effect to such changes in laws, regulations or principles, the Administrator, in its sole discretion and on such terms and conditions as it deems appropriate, either by amendment of the terms of any outstanding Awards or by action taken prior to the occurrence of such transaction or event and either automatically or upon the Participant's request, is hereby authorized to take any one or more of the following actions:

(i) To provide for either (A) termination of any such Award in exchange for an amount of cash and/or other property, if any, equal to the amount that would have been received upon the exercise of such Award or realization of the Participant's rights (and, for the avoidance of doubt, if as of the date of the occurrence of the transaction or event described in this Section 11.1(b) the Administrator determines in good faith that no amount would have been attained upon the exercise of such Award or realization of the Participant's rights, then such Award may be terminated by the Company without payment) or (B) the replacement of such Award with other rights or property selected by the Administrator in its sole discretion;

(ii) To provide that such Award be assumed by the successor or survivor entity, or a parent or subsidiary thereof, or shall be substituted for by similar options, rights or awards covering the stock of the successor or survivor entity, or a parent or subsidiary thereof, with appropriate adjustments as to the number and kind of shares and prices;

(iii) To make adjustments in the number and type of shares of Stock (or other securities or property) subject to outstanding Awards, and in the number and kind of outstanding Restricted Stock or Restricted Stock Units and/or in the terms and conditions of (including the grant or exercise price), and the criteria included in, outstanding options, rights and awards, and options, rights and awards which may be granted in the future;

(iv) To provide that such Award shall be exercisable or payable or fully vested with respect to all shares covered thereby, notwithstanding anything to the contrary in the Plan or the applicable Award Agreement; and

(v) To provide that the Award cannot vest, be exercised or become payable after such event.

(c) In connection with the occurrence of any Equity Restructuring, and notwithstanding anything to the contrary in Sections 11.1(a) and 11.1(b):

(i) The number and type of securities subject to each outstanding Award and the exercise price or grant price thereof, if applicable, will be proportionately adjusted by the Administrator as the Administrator deems appropriate to reflect such Equity Restructuring. The adjustments provided under this Section 11(c)(i) shall be nondiscretionary and shall be final and binding on the affected Participant and the Company.

(ii) The Administrator shall make such proportionate adjustments, if any, as the Administrator in its discretion may deem appropriate to reflect such Equity Restructuring with respect to the aggregate number and kind of shares that may be issued under the Plan (including, but not limited to, adjustments of the limitations in Article 3).

11.2 Acceleration Upon a Change in Control.

(a) Notwithstanding anything to the contrary contained in Section 11.1, and except as may otherwise be provided in any applicable Award Agreement or other written agreement entered into between the Company and a Participant, if a Change in Control occurs and a Participant's Awards are not continued, converted, assumed or replaced by (i) the Company or a Parent or Subsidiary, or (ii) a Successor Entity, such Awards shall become fully exercisable and/or payable, as applicable, and all forfeiture, repurchase and other restrictions on such Awards shall lapse immediately prior to such Change in Control. Upon, or in anticipation of, a Change in Control, the Administrator may cause any and all Awards outstanding hereunder to terminate at a specific time in the future, including without limitation, the date of such Change in Control, and shall give each Participant the right to exercise such Awards during a period of time as the Administrator, in its sole and absolute discretion, shall determine. The Administrator shall have sole discretion to determine whether an Award has been continued, converted, assumed or replaced in connection with a Change in Control.

(b) Except as otherwise provided in the Agreement evidencing the Award, any such Awards that are continued, converted, assumed, or replaced by (i) the Company or a Parent or Subsidiary of the Company, or (ii) a Successor Entity, in a Change in Control and do not otherwise accelerate at that time shall become fully exercisable and/or payable, as applicable, and all forfeiture, repurchase and other restrictions on such Awards shall lapse in the event that the Participant has a Termination of Employment, Termination of Directorship or Termination of Consultancy (i) in connection with the Change in Control or (ii) subsequently within twelve months following such Change in Control, unless such termination is by reason of the Participant's discharge by the Company or a Parent or Subsidiary or a Successor Entity for Cause or by reason of the Participant's voluntary resignation without Good Reason.

11.3 No Other Rights. Except as expressly provided in the Plan or pursuant to action of the Administrator under the Plan, no Participant shall have any rights by reason of any subdivision or consolidation of shares of stock of any class, the payment of any dividend, any increase or decrease in the number of shares of stock of any class or any dissolution, liquidation, merger, or consolidation of the Company or any other corporation. Except as expressly provided in the Plan or pursuant to action of the Administrator under the Plan, no issuance by the Company of shares of stock of any class, or securities convertible into shares of stock of any class, shall affect, and no adjustment by reason thereof shall be made with respect to, the number of shares of Stock subject to an Award or the grant or exercise price of any Award.

ARTICLE 12
ADMINISTRATION

12.1 Administrator. The Plan shall be administered by the Board. The Board may delegate administration of the Plan to a Committee or Committees of one or more members of the Board, and the term "***Administrator***" shall apply to any person or persons who at the time have the authority to administer the Plan. If administration is delegated to a Committee, the Committee shall have, in connection with the administration of the Plan, the powers theretofore possessed by the Board, including the power to delegate to a subcommittee any of the administrative powers the Committee is authorized to exercise (and references in this Plan to the Board shall thereafter be to the Committee or subcommittee), subject, however, to such resolutions, not inconsistent with the provisions of the Plan, as may be adopted from time to time by the Board. Notwithstanding the foregoing, however, from and after the Public Trading Date, a Committee of the Board shall administer the Plan and such Committee shall consist solely of two or more members of the Board each of whom is an "outside director," within the meaning of Section 162(m) of the Code and a Non-Employee Director. Notwithstanding the foregoing: (a) the full Board, acting by a majority of its members in office, shall conduct the general administration of the Plan with respect to all Awards granted to Non-Employee Directors and for purposes of such Awards the term "***Administrator***" as used in this Plan shall be deemed to refer to the Board, and (b) the Board or the Committee may delegate its authority hereunder to the extent permitted by Section 12.5. In its sole discretion, the Board may at any time and from time to time exercise any and all rights and duties of the Administrator under the Plan except with respect to matters which, following the Public Trading Date, are required to be determined in the sole discretion of the Committee under Rule 16b-3 under the Exchange Act or Section 162(m) of the Code, or any regulations or rules issued thereunder. Committee members may resign at any time by delivering written notice to the Board. Vacancies in the Committee may only be filled by the Board.

12.2 Action by the Administrator. A majority of the members of the Administrator shall constitute a quorum. The acts of a majority of the members of the Administrator present at any meeting at which a quorum is present, and, subject to applicable law, acts approved in writing by a majority of the members of the Administrator in lieu of a meeting, shall be deemed the acts of the Administrator. Each member of the Administrator is entitled to, in good faith, rely or act upon any report or other information furnished to that member by any officer or other employee of the Company or any Parent or Subsidiary, the Company's independent certified public accountants, or any executive compensation consultant or other professional retained by the Company to assist in the administration of the Plan.

12.3 Authority of Administrator. Subject to any specific designation in the Plan, the Administrator has the exclusive power, authority and discretion to:

(a) Designate Eligible Individuals to receive Awards;

(b) Determine the type or types of Awards to be granted to each Eligible Individual;

(c) Determine the number of Awards to be granted and the number of shares of Stock to which an Award will relate;

(d) Determine the terms and conditions of any Award granted pursuant to the Plan, including, but not limited to, the exercise price, grant price, or purchase price, any reload provision, any restrictions or limitations on the Award, any schedule for lapse of forfeiture restrictions or restrictions on the exercisability of an Award, and accelerations or waivers thereof, any provisions related to non-competition and recapture of gain on an Award, based in each case on such considerations as the Administrator in its sole discretion determines;

(e) Determine whether, to what extent, and pursuant to what circumstances an Award may be settled in, or the exercise price of an Award may be paid in, cash, Stock, other Awards, or other property, or an Award may be canceled, forfeited, or surrendered;

(f) Prescribe the form of each Award Agreement, which need not be identical for each Participant;

(g) Decide all other matters that must be determined in connection with an Award;

(h) Establish, adopt, or revise any rules and regulations as it may deem necessary or advisable to administer the Plan;

(i) Interpret the terms of, and any matter arising pursuant to, the Plan or any Award Agreement; and

(j) Make all other decisions and determinations that may be required pursuant to the Plan or as the Administrator deems necessary or advisable to administer the Plan.

11.4 Decisions Binding. The Administrator's interpretation of the Plan, any Awards granted pursuant to the Plan, any Award Agreement and all decisions and determinations by the Administrator with respect to the Plan are final, binding, and conclusive on all parties.

11.5 Delegation of Authority. Within the scope of such authority, the Board or the Committee may delegate to a committee of one or more members of the Board or one or more officers of the Company the authority to grant or amend Awards to Participants other than Eligible Individuals who are either (a) "covered employees" at the time of recognition of income resulting from such Awards, and/or (b) persons with respect to whom the Company wishes to comply with Section 162(m) of the Code and/or (c) subject to Section 16 of the Exchange Act and/or (d) officers of the Company or members of the Board to whom authority to grant or amend Awards has been delegated pursuant to this Section 12.5. At all times, the delegate(s) appointed under this Section 12.5 shall serve in such capacity at the pleasure of the Board or the Committee.

ARTICLE 13
EFFECTIVE AND EXPIRATION DATE

13.1 Effective Date. The Plan will be effective on the date of the Board's initial adoption of the Plan (the "***Effective Date***"). The Plan will be submitted for the approval of the Company's stockholders within twelve months after the Effective Date. Awards may be granted or awarded prior to such stockholder approval, provided that such Awards shall not be exercisable, shall not vest and the restrictions thereon shall not lapse prior to the time when the Plan is approved by the stockholders, and provided further that if such approval has not been obtained at the end of said twelve-month period, all Awards previously granted or awarded under the Plan shall thereupon be canceled and become null and void.

13.2 Expiration Date. The Plan will expire on, and no Award may be granted pursuant to the Plan after, the tenth anniversary of the earlier of (i) the Effective Date or (ii) the date this Plan is approved by the Company's stockholders. Any Awards that are outstanding on the tenth anniversary of the Effective Date shall remain in force according to the terms of the Plan and the applicable Award Agreement.

ARTICLE 14
AMENDMENT, MODIFICATION, AND TERMINATION

14.1 Amendment, Modification, and Termination. The Board may terminate, amend or modify the Plan at any time and from time to time; *provided, however,* that to the extent necessary to comply with any applicable law, regulation, or stock exchange rule, the Company shall obtain stockholder approval of any Plan amendment in such a manner and to such a degree as required. The Administrator shall have the authority to effect, at any time and from time to time, with the consent of the affected Option holders, the cancellation of any or all outstanding Awards under the Plan and to grant in substitution therefor new Awards covering the same or different number of shares of Stock and with a different or no exercise price per share.

14.2 Awards Previously Granted. No termination, amendment, or modification of the Plan shall adversely affect in any material way any Award previously granted pursuant to the Plan without the prior written consent of the Participant.

ARTICLE 15
GENERAL PROVISIONS

15.1 No Rights to Awards. No Participant, Employee, or other person shall have any claim to be granted any Award pursuant to the Plan, and neither the Company nor the Administrator is obligated to treat Participants, Employees, and other persons uniformly.

15.2 No Stockholder Rights. Except as otherwise provided herein, a Participant shall have none of the rights of a stockholder with respect to shares of Stock covered by any Award until the Participant becomes the record owner of such shares of Stock.

15.3 Withholding. The Company or any Parent or Subsidiary shall have the authority and the right to deduct or withhold, or require a Participant to remit to the Company an amount sufficient to satisfy federal, state, local and foreign taxes (including the Participant's employment

tax obligations) required by law to be withheld with respect to any taxable event concerning a Participant arising as a result of this Plan. The Administrator may in its discretion and in satisfaction of the foregoing requirement allow a Participant to elect to have the Company or a Parent or Subsidiary, as applicable, withhold shares of Stock otherwise issuable under an Award (or allow the return of shares of Stock) having a Fair Market Value equal to the sums required to be withheld. Notwithstanding any other provision of the Plan, the number of shares of Stock which may be withheld with respect to the issuance, vesting, exercise or payment of any Award (or which may be repurchased from the Participant of such Award within six months (or such other period as may be determined by the Administrator) after such shares of Stock were acquired by the Participant from the Company) in order to satisfy the Participant's federal, state, local and foreign tax liabilities with respect to the issuance, vesting, exercise or payment of the Award shall be limited to the number of shares which have a Fair Market Value on the date of withholding or repurchase equal to the aggregate amount of such liabilities based on the minimum statutory withholding rates for federal, state, local and foreign income tax and employment tax purposes that are applicable to such supplemental taxable income.

15.4 No Right to Employment or Services. Nothing in the Plan or any Award Agreement shall interfere with or limit in any way the right of the Company or any Parent or Subsidiary to terminate any Participant's employment or services at any time, nor confer upon any Participant any right to continue in the employ or service of the Company or any Parent or Subsidiary.

15.5 Unfunded Status of Awards. The Plan is intended to be an "unfunded" plan for incentive compensation. With respect to any payments not yet made to a Participant pursuant to an Award, nothing contained in the Plan or any Award Agreement shall give the Participant any rights that are greater than those of a general creditor of the Company or any Parent or Subsidiary.

15.6 Indemnification. To the extent allowable pursuant to applicable law, the Administrator (and each member thereof) shall be indemnified and held harmless by the Company from any loss, cost, liability, or expense that may be imposed upon or reasonably incurred by such member in connection with or resulting from any claim, action, suit, or proceeding to which he or she may be a party or in which he or she may be involved by reason of any action or failure to act pursuant to the Plan and against and from any and all amounts paid by him or her in satisfaction of judgment in such action, suit, or proceeding against him or her; *provided* he or she gives the Company an opportunity, at its own expense, to handle and defend the same before he or she undertakes to handle and defend it on his or her own behalf. The foregoing right of indemnification shall not be exclusive of any other rights of indemnification to which such persons may be entitled pursuant to the Company's Certificate of Incorporation or Bylaws, as a matter of law, or otherwise, or any power that the Company may have to indemnify them or hold them harmless.

15.7 Relationship to Other Benefits. No payment pursuant to the Plan shall be taken into account in determining any benefits pursuant to any pension, retirement, savings, profit sharing, group insurance, welfare or other benefit plan of the Company or any Parent or Subsidiary except to the extent otherwise expressly provided in writing in such other plan or an agreement thereunder.

15.8 Expenses. The expenses of administering the Plan shall be borne by the Company and its Parents and Subsidiaries.

15.9 Titles and Headings. The titles and headings of the Sections in the Plan are for convenience of reference only and, in the event of any conflict, the text of the Plan, rather than such titles or headings, shall control.

15.10 Fractional Shares. No fractional shares of Stock shall be issued and the Administrator shall determine, in its discretion, whether cash shall be given in lieu of fractional shares or whether such fractional shares shall be eliminated by rounding up or down as appropriate.

15.11 Limitations Applicable to Section 16 Persons. Notwithstanding any other provision of the Plan, the Plan, and any Award granted or awarded to any Participant who is then subject to Section 16 of the Exchange Act, shall be subject to any additional limitations set forth in any applicable exemptive rule under Section 16 of the Exchange Act (including any amendment to Rule 16b-3 under the Exchange Act) that are requirements for the application of such exemptive rule. To the extent permitted by applicable law, the Plan and Awards granted or awarded hereunder shall be deemed amended to the extent necessary to conform to such applicable exemptive rule.

15.12 Government and Other Regulations. The obligation of the Company to make payment of awards in Stock or otherwise shall be subject to all applicable laws, rules, and regulations, and to such approvals by government agencies as may be required. The Company shall be under no obligation to register pursuant to the Securities Act any of the shares of Stock paid pursuant to the Plan. If the shares paid pursuant to the Plan may in certain circumstances be exempt from registration pursuant to the Securities Act, the Company may restrict the transfer of such shares in such manner as it deems advisable to ensure the availability of any such exemption.

15.13 Governing Law. The Plan and all Award Agreements shall be construed in accordance with and governed by the laws of the State of California, without regard to the conflicts of law principles thereof.

15.14 Compliance with California Securities Laws. Unless determined otherwise by the Administrator, prior to the Public Trading Date, this Plan is intended to comply with Section 25102(o) of the California Corporations Code and the regulations issued thereunder. Appendix I to the Plan sets forth the requirements under Section 25102(o) of the California Corporations Code and the regulations issued thereunder and is incorporated herein by reference. If any of the provisions contained in this Plan are inconsistent with such requirements or Appendix I, such provisions shall be deemed null and void. The invalidity of any provision of this Plan shall not affect the validity or enforceability of any other provision of this Plan, which shall remain in full force and effect.

15.15 Appendices. The Board may approve such supplements to, or amendments, or appendices to, the Plan as it may consider necessary or appropriate for purposes of compliance with applicable laws or otherwise and such supplements, amendments or appendices shall be considered a part of the Plan; *provided, however,* that no such supplements, amendments or appendices shall increase the share limitation contained in Section 3.1 of the Plan.

APPENDIX I TO

ULTRAGENYX PHARMACEUTICAL, INC.

2011 EQUITY INCENTIVE PLAN

California State Securities Law Compliance

Notwithstanding anything to the contrary contained in the Plan and except as otherwise determined by the Administrator, the provisions set forth in this Appendix shall apply to all Awards granted under the Ultragenyx Pharmaceutical, Inc. 2011 Equity Incentive Plan (the "***Plan***") prior to the Public Trading Date. This Appendix shall be of no further force and effect on or after the Public Trading Date. Definitions as set out in Article 2 of the Plan are applicable to this Appendix.

The purpose of this Appendix is to set forth those provisions of the Plan necessary to comply with Section 25102(o) of the California Corporations Code and the regulations issued thereunder. If any of the provisions contained in this Appendix are inconsistent with such requirements, such provisions shall be deemed null and void. The invalidity of any provision of this Appendix shall not affect the validity or enforceability of any other provision of this Appendix, which shall remain in full force and effect.

References to Articles and Sections set forth in this Appendix are to those Articles and Sections of the Plan.

1.1 Term of Awards. The term of each Award shall be no more than ten years from the date of grant thereof.

2.1 Award Exercise or Purchase Price. Except as provided in Article 11, the per share exercise or purchase price for the Stock to be issued upon exercise of an Award shall be such price as is determined by the Administrator, but in the case of an Award granted to a Participant who, at the time of grant of such Award, owns stock representing more than 10% of the voting power of all classes of stock of the Company or any parent (as defined in Section 175 of the California Corporations Code) or Subsidiary, the per share exercise or purchase price shall be no less than 110% of the Fair Market Value per share on the date of the grant (100% in the case of an Award other than an Option). Notwithstanding the foregoing, Awards may be granted with a per share exercise or purchase price other than as required above pursuant to a merger or other corporate transaction.

3.1 Exercisability. Except with regard to Awards granted to officers, members of the Board, managers or consultants, in no event shall an Award granted hereunder become vested and exercisable at a rate of less than 20% per year over five years from the date the Award is granted, subject to reasonable conditions, such as continuing to be a member of the Board, Employee or Consultant.

4.1 Exercisability Following Termination.

(a) Termination Other Than Death or Disability. If a Participant has a Termination of Service for any reason other than by reason of the Participant's Disability or death, such Participant may exercise his or her Award within such period of time as is specified in the Award Agreement to the extent that the Award is vested on the date of termination; *provided, however,* that prior to the Public Trading Date, such period of time shall not be less than thirty days (but in no event later than the expiration of the term of the Award as set forth in the Award Agreement). In the absence of a specified time in the Award Agreement, the Option shall remain exercisable for three months following the Participant's Termination of Service for any reason other than death or Disability.

(b) Death. If a Participant has a Termination of Service as a result of the Participant's death, the Award may be exercised within such period of time as is specified in the Award Agreement; *provided, however,* that prior to the Public Trading Date, such period of time shall not be less than six months (but in no event later than the expiration of the term of such Award as set forth in the Notice of Grant), by the Participant's estate or by a person who acquires the right to exercise the Award by bequest or inheritance, but only to the extent that the Award is vested on the date of death. In the absence of a specified time in the Award Agreement, the Award shall remain exercisable for twelve months following the Participant's Termination of Service for death.

(c) Disability of Participant. If a Participant has a Termination of Service as a result of the Participant's Disability, the Participant may exercise his or her Award within such period of time as is specified in the Award Agreement to the extent the Award is vested on the date of termination; *provided, however,* that prior to the Public Trading Date, such period of time shall not be less than six months (but in no event later than the expiration of the term of such Award as set forth in the Award Agreement). In the absence of a specified time in the Award Agreement, the Award shall remain exercisable for twelve months following the Participant's Termination of Service for Disability.

(d) Misconduct of Participant. If a Participant has a Termination of Service as a result of the Participant's Misconduct, the Award shall terminate immediately and cease to remain outstanding.

5.1 Repurchase Provisions. In the event the Administrator provides that the Company may repurchase Stock acquired upon exercise of an Award upon the occurrence of certain specified events, including, without limitation, a Participant's Termination of Service, divorce, bankruptcy or insolvency, then any such repurchase right shall be set forth in the applicable Award Agreement or in another agreement referred to in such agreement and, to the extent required by Section 260.140.41 and Section 260.140.42 of Title 10 of the California Code of Regulations (or any successor regulation), any such repurchase right set forth in an Award granted prior to the Public Trading Date to a person who is not an officer, member of the Board, manager or consultant shall be upon the following terms: (i) if the repurchase option gives the Company the right to repurchase the shares upon the Participant's Termination of Service at not less than the Fair Market Value of the shares to be purchased on the date of termination of employment or service, then (A) the right to repurchase shall be exercised for cash or

cancellation of purchase money indebtedness for the shares within ninety days of termination (or in the case of shares issued upon exercise of Awards after such date of termination, within ninety days after the date of the exercise) or such longer period as may be agreed to by the Administrator and the Participant and (B) the right terminates on the Public Trading Date; and (ii) if the repurchase option gives the Company the right to repurchase the Stock upon the Participant's Termination of Service at the original purchase price for such Stock, then (A) the right to repurchase at the original purchase price shall lapse at the rate of at least 20% of the shares per year over five (5) years from the date the Award is granted (without respect to the date the Award was exercised or became exercisable) and (B) the right to repurchase shall be exercised for cash or cancellation of purchase money indebtedness for the shares within ninety days of termination (or, in the case of shares issued upon exercise of Awards, after such date of termination, within ninety days after the date of the exercise) or such longer period as may be agreed to by the Company and the Participant.

6.1 Information Rights. Prior to the Public Trading Date and to the extent required by Section 260.140.46 of Title 10 of the California Code of Regulations, the Company shall provide to each Participant and to each individual who acquires Stock pursuant to the Plan, not less frequently than annually during the period such Participant has one or more Awards outstanding, and, in the case of an individual who acquires Stock pursuant to the Plan, during the period such individual owns such Stock, copies of annual financial statements. Notwithstanding the preceding sentence, the Company shall not be required to provide such statements to key employees whose duties in connection with the Company assure their access to equivalent information.

7.1 Transferability. Prior to the Public Trading Date, no Award shall be assigned, transferred, or otherwise disposed of by a Participant other than by will or the laws of descent and distribution or, with respect to Awards other than Incentive Stock Options, as permitted by Rule 701 of the Securities Act.

8.1 Limitation on Number of Shares. Prior to the Public Trading Date, at no time shall the total number of shares of Stock issuable upon exercise of all outstanding Options under the Plan and any shares of Stock provided for under any bonus or similar plan or agreement of the Company exceed 30% of the then-outstanding shares of Stock of the Company, as calculated pursuant to Section 260.140.45 of Title 10 of the California Code of Regulations (or any successor regulation), unless a percentage higher than 30% is approved by at least two-thirds of the outstanding securities of the Company entitled to vote. The number of shares of Stock which may be issued or transferred pursuant to Awards under the Plan shall be reduced to the extent necessary to comply with this provision.

ULTRAGENYX PHARMACEUTICAL, INC.

2011 EQUITY INCENTIVE PLAN STOCK OPTION GRANT NOTICE AND

STOCK OPTION AGREEMENT

Ultragenyx Pharmaceutical, Inc. (the "***Company***"), pursuant to its 2011 Equity Incentive Plan (the "***Plan***"), hereby grants to the holder listed below ("***Participant***"), an option to purchase the number of shares of the Company's Stock set forth below (the "***Option***"). This Option is subject to all of the terms and conditions as set forth herein and in the Stock Option Agreement attached hereto as Exhibit A (the "***Stock Option Agreement***") and the Plan, each of which are incorporated herein by reference. Unless otherwise defined herein, the terms defined in the Plan shall have the same defined meanings in this Grant Notice and the Stock Option Agreement.

Participant: _____

Grant Date: _____

Vesting Commencement Date: _____

Exercise Price per Share: $ _____

Total Exercise Price: $ _____

Total Number of Shares Subject to the Option: _____

Expiration Date: _____

Type of Option: ☐ Incentive Stock Option ☐ Non-Qualified Stock Option

Vesting Schedule: [To be specified in individual agreements.]

By his or her signature and the Company's signature below, Participant agrees to be bound by the terms and conditions of the Plan, the Stock Option Agreement and this Grant Notice. Participant has reviewed the Stock Option Agreement, the Plan and this Grant Notice in their entirety, has had an opportunity to obtain the advice of counsel prior to executing this Grant Notice and fully understands all provisions of this Grant Notice, the Stock Option Agreement and the Plan. Participant hereby agrees to accept as binding, conclusive and final all decisions or interpretations of the Administrator of the Plan upon any questions arising under the Plan or the Option.

ULTRAGENYX PHARMACEUTICAL, INC.

By: _____

Print Name:

Title:

Address:

PARTICIPANT:

By: _____

Print Name: _____

Address: _____

_____

EXHIBIT A

TO STOCK OPTION GRANT NOTICE

STOCK OPTION AGREEMENT

Pursuant to the Stock Option Grant Notice ("***Grant Notice***") to which this Stock Option Agreement (this "***Agreement***") is attached, Ultragenyx Pharmaceutical, Inc. (the "***Company***") has granted to Participant an option under the Company's 2011 Equity Incentive Plan (the "***Plan***") to purchase the number of shares of Stock indicated in the Grant Notice.

ARTICLE I
GENERAL

1.1 Defined Terms. Capitalized terms not specifically defined herein shall have the meanings specified in the Plan and the Grant Notice.

1.2 Incorporation of Terms of Plan. The Option is subject to the terms and conditions of the Plan which are incorporated herein by reference.

ARTICLE II
GRANT OF OPTION

2.1 Grant of Option. In consideration of Participant's past and/or continued employment with or service to the Company or a Parent or Subsidiary and for other good and valuable consideration, effective as of the Grant Date set forth in the Grant Notice (the "***Grant Date***"), the Company irrevocably grants to Participant the Option to purchase any part or all of an aggregate of the number of shares of Stock set forth in the Grant Notice, upon the terms and conditions set forth in the Plan and this Agreement. Unless designated as a Non-Qualified Stock Option in the Grant Notice, the Option shall be an Incentive Stock Option to the maximum extent permitted by law.

2.2 Exercise Price. The exercise price of the shares of Stock subject to the Option shall be as set forth in the Grant Notice, without commission or other charge; *provided, however,* that if this Option is designated as an Incentive Stock Option, the price per share of the shares subject to the Option shall not be less than the greater of (i) 100% of the Fair Market Value of a share of Stock on the Grant Date, or (ii) 110% of the Fair Market Value of a share of Stock on the Grant Date in the case of a Participant then owning (within the meaning of Section 424(d) of the Code) more than 10% of the total combined voting power of all classes of stock of the Company or any "subsidiary corporation" of the Company or any "parent corporation" of the Company (each within the meaning of Section 424 of the Code).

ARTICLE III
PERIOD OF EXERCISABILITY

3.1 Commencement of Exercisability.

(a) Subject to Sections 3.3 and 5.8, the Option shall become vested and exercisable in such amounts and at such times as are set forth in the Grant Notice.

(b) No portion of the Option which has not become vested and exercisable at the date of Participant's Termination of Service shall thereafter become vested and exercisable, except as may be otherwise provided by the Administrator or as set forth in a written agreement between the Company and Participant.

3.2 Duration of Exercisability. The installments provided for in the vesting schedule set forth in the Grant Notice are cumulative. Each such installment which becomes vested and exercisable pursuant to the vesting schedule set forth in the Grant Notice shall remain vested and exercisable until it becomes unexercisable under Section 3.3.

3.3 Expiration of Option. The Option may not be exercised to any extent by anyone after the first to occur of the following events:

(a) The expiration of ten years from the Grant Date;

(b) If this Option is designated as an Incentive Stock Option and Participant owned (within the meaning of Section 424(d) of the Code), at the time the Option was granted, more than 10% of the total combined voting power of all classes of stock of the Company or any "subsidiary corporation" of the Company or "parent corporation" of the Company (each within the meaning of Section 424 of the Code), the expiration of five years from the date the Option was granted; or

(c) The expiration of three months following the date of Participant's Termination of Service, unless such termination occurs by reason of Participant's death, Disability or Misconduct;

(d) The expiration of one year following the date of Participant's Termination of Service by reason of Participant's death or Disability; or

(e) The date of Participant's Termination of Service as a result of Participant's Misconduct.

Participant acknowledges that an Incentive Stock Option exercised more than three months after Participant's termination of status as an Employee, other than by reason of death or Disability, will be taxed as a Non-Qualified Stock Option.

3.4 Special Tax Consequences. Participant acknowledges that, to the extent that the aggregate Fair Market Value (determined as of the time the Option is granted) of all shares of Stock with respect to which Incentive Stock Options, including the Option, are first exercisable for the first time by Participant in any calendar year exceeds $100,000 (or such other limitation

as imposed by Section 422(d) of the Code), the Option and such other options shall be treated as not qualifying under Section 422 of the Code but rather shall be considered Non-Qualified Stock Options. Participant further acknowledges that the rule set forth in the preceding sentence shall be applied by taking Options and other "incentive stock options" into account in the order in which they were granted.

ARTICLE IV
EXERCISE OF OPTION

4.1 Person Eligible to Exercise. Except as provided in Sections 5.2(b) and 5.2(c), during the lifetime of Participant, only Participant may exercise the Option or any portion thereof. After the death of Participant, any exercisable portion of the Option may, prior to the time when the Option becomes unexercisable under Section 3.3, be exercised by Participant's personal representative or by any person empowered to do so under the deceased Participant's will or under the then applicable laws of descent and distribution.

4.2 Partial Exercise. Any exercisable portion of the Option or the entire Option, if then wholly exercisable, may be exercised in whole or in part at any time prior to the time when the Option or portion thereof becomes unexercisable under Section 3.3.

4.3 Manner of Exercise. The Option, or any exercisable portion thereof, may be exercised solely by delivery to the Secretary of the Company or the Secretary's office of all of the following prior to the time when the Option or such portion thereof becomes unexercisable under Section 3.3:

(a) An Exercise Notice in writing signed by Participant or any other person then entitled to exercise the Option or portion thereof, stating that the Option or portion thereof is thereby exercised, such notice complying with all applicable rules established by the Administrator. Such notice shall be substantially in the form attached as Exhibit B to the Grant Notice (or such other form as is prescribed by the Administrator); and

(b) Subject to Section 5.1(c) of the Plan:

(i) Full payment (in cash or by check) for the shares with respect to which the Option or portion thereof is exercised; or

(ii) With the consent of the Administrator, by delivery of a full recourse promissory note on such terms and conditions as may be approved by the Administrator; or

(iii) With the consent of the Administrator, by delivery of shares of Stock then issuable upon exercise of the Option having a Fair Market Value on the date of delivery equal to the aggregate exercise price of the Option or exercised portion thereof; or

(iv) On and after the Public Trading Date, such payment may be made, in whole or in part, through the delivery of shares of Stock which have been owned by Participant for at least six months, duly endorsed for transfer to the Company with a Fair Market Value on the date of delivery equal to the aggregate exercise price of the Option or exercised portion thereof; or

(v) On and after the Public Trading Date, through the delivery of a notice that Participant has placed a market sell order with a broker with respect to shares of Stock then issuable upon exercise of the Option, and that the broker has been directed to pay a sufficient portion of the net proceeds of the sale to the Company in satisfaction of the Option exercise price; *provided*, that payment of such proceeds is made to the Company upon settlement of such sale; or

(vi) Subject to any applicable laws, any combination of the consideration provided in the foregoing paragraphs (i), (ii) and (iii); and

(c) A bona fide written representation and agreement, in such form as is prescribed by the Administrator, signed by Participant or the other person then entitled to exercise such Option or portion thereof, stating that the shares of Stock are being acquired for Participant's own account, for investment and without any present intention of distributing or reselling said shares or any of them except as may be permitted under the Securities Act and then applicable rules and regulations thereunder, and that Participant or other person then entitled to exercise such Option or portion thereof will indemnify the Company against and hold it free and harmless from any loss, damage, expense or liability resulting to the Company if any sale or distribution of the shares by such person is contrary to the representation and agreement referred to above. The Administrator may, in its absolute discretion, take whatever additional actions it deems appropriate to ensure the observance and performance of such representation and agreement and to effect compliance with the Securities Act and any other federal or state securities laws or regulations. Without limiting the generality of the foregoing, the Administrator may require an opinion of counsel acceptable to it to the effect that any subsequent transfer of shares acquired on an Option exercise does not violate the Securities Act, and may issue stop-transfer orders covering such shares. Share certificates evidencing Stock issued on exercise of the Option shall bear an appropriate legend referring to the provisions of this subsection (c) and the agreements herein. The written representation and agreement referred to in the first sentence of this subsection (c) shall, however, not be required if the shares to be issued pursuant to such exercise have been registered under the Securities Act, and such registration is then effective in respect of such shares; and

(d) The receipt by the Company of full payment for such shares, including payment of any applicable withholding tax, which may be in the form of consideration used by Participant to pay for such shares under Section 4.3(b), subject to Section 15.3 of the Plan; and

(e) In the event the Option or portion thereof shall be exercised pursuant to Section 4.1 by any person or persons other than Participant, appropriate proof of the right of such person or persons to exercise the Option.

4.4 Conditions to Issuance of Stock Certificates. The shares of Stock deliverable upon the exercise of the Option, or any portion thereof, may be either previously authorized but unissued shares or issued shares which have then been reacquired by the Company. Such shares shall be fully paid and nonassessable. The Company shall not be required to issue or deliver any shares of Stock purchased upon the exercise of the Option or portion thereof prior to fulfillment of all of the following conditions:

(a) The admission of such shares to listing on all stock exchanges on which such Stock is then listed; and

(b) The completion of any registration or other qualification of such shares under any state or federal law or under rulings or regulations of the Securities and Exchange Commission or of any other governmental regulatory body, which the Administrator shall, in its absolute discretion, deem necessary or advisable; and

(c) The obtaining of any approval or other clearance from any state or federal governmental agency which the Administrator shall, in its absolute discretion, determine to be necessary or advisable; and

(d) The lapse of such reasonable period of time following the exercise of the Option as the Administrator may from time to time establish for reasons of administrative convenience; and

(e) The receipt by the Company of full payment for such shares, including payment of any applicable withholding tax, which may be in the form of consideration used by Participant to pay for such shares under Section 4.3(b), subject to Section 15.3 of the Plan.

4.5 Rights as Stockholder. The holder of the Option shall not be, nor have any of the rights or privileges of, a stockholder of the Company in respect of any shares purchasable upon the exercise of any part of the Option unless and until such shares shall have been issued by the Company to such holder.

ARTICLE V
OTHER PROVISIONS

5.1 Administration. The Administrator shall have the power to interpret the Plan and this Agreement and to adopt such rules for the administration, interpretation and application of the Plan as are consistent therewith and to interpret, amend or revoke any such rules. All actions taken and all interpretations and determinations made by the Administrator in good faith shall be final and binding upon Participant, the Company and all other interested persons. No member of the Administrator shall be personally liable for any action, determination or interpretation made in good faith with respect to the Plan, this Agreement or the Option. In its absolute discretion, the Board may at any time and from time to time exercise any and all rights and duties of the Administrator under the Plan and this Agreement.

5.2 Option Not Transferable.

(a) Subject to Section 5.2(b), the Option may not be sold, pledged, assigned or transferred in any manner other than by will or the laws of descent and distribution, unless and until the shares underlying the Option have been issued, and all restrictions applicable to such shares have lapsed. Neither the Option nor any interest or right therein shall be liable for the debts, contracts or engagements of Participant or his or her successors in interest or shall be

subject to disposition by transfer, alienation, anticipation, pledge, encumbrance, assignment or any other means whether such disposition be voluntary or involuntary or by operation of law by judgment, levy, attachment, garnishment or any other legal or equitable proceedings (including bankruptcy), and any attempted disposition thereof shall be null and void and of no effect, except to the extent that such disposition is permitted by the preceding sentence.

(b) Notwithstanding any other provision in this Agreement, with the consent of the Administrator and to the extent the Option is designated as a Non-Qualified Stock Option, the Option may be transferred to, exercised by and paid to one or more Permitted Transferees, subject to the terms and conditions set forth in Section 10.3 of the Plan.

(c) Unless transferred to a Permitted Transferee in accordance with Section 5.2(b), during the lifetime of Participant, only Participant may exercise the Option or any portion thereof. Subject to such conditions and procedures as the Administrator may require, a Permitted Transferee may exercise the Option or any portion thereof during Participant's lifetime. After the death of Participant, any exercisable portion of the Option may, prior to the time when the Option becomes unexercisable under Section 3.3, be exercised by Participant's personal representative or by any person empowered to do so under the deceased Participant's will or under the then applicable laws of descent and distribution.

5.3 Lock-Up Period. Participant hereby agrees that, if so requested by the Company or any representative of the underwriters (the "***Managing Underwriter***") in connection with any registration of the offering of any securities of the Company under the Securities Act, Participant shall not sell or otherwise transfer any shares of Stock or other securities of the Company during such period as may be requested in writing by the Managing Underwriter and agreed to in writing by the Company (which period shall not be longer than one hundred eighty days) (the "***Market Standoff Period***") following the effective date of a registration statement of the Company filed under the Securities Act; *provided, however,* that such restriction shall apply only to the first registration statement of the Company to become effective under the Securities Act that includes securities to be sold on behalf of the Company to the public in an underwritten public offering under the Securities Act.

5.4 Restrictive Legends and Stop-Transfer Orders.

(a) The share certificate or certificates evidencing the shares of Stock purchased hereunder shall be endorsed with any legends that may be required by state or federal securities laws.

(b) Participant agrees that, in order to ensure compliance with the restrictions referred to herein, the Company may issue appropriate "stop transfer" instructions to its transfer agent, if any, and that, if the Company transfers its own securities, it may make appropriate notations to the same effect in its own records.

(c) The Company shall not be required: (i) to transfer on its books any shares of Stock that have been sold or otherwise transferred in violation of any of the provisions of this Agreement, or (ii) to treat as owner of such shares of Stock or to accord the right to vote or pay dividends to any purchaser or other transferee to whom such shares shall have been so transferred.

5.5 Shares to Be Reserved. The Company shall at all times during the term of the Option reserve and keep available such number of shares of Stock as will be sufficient to satisfy the requirements of this Agreement.

5.6 Notices. Any notice to be given under the terms of this Agreement to the Company shall be addressed to the Company in care of the Secretary of the Company at the address given beneath the signature of the Company's authorized officer on the Grant Notice, and any notice to be given to Participant shall be addressed to Participant at the address given beneath Participant's signature on the Grant Notice. By a notice given pursuant to this Section 5.6, either party may hereafter designate a different address for notices to be given to that party. Any notice which is required to be given to Participant shall, if Participant is then deceased, be given to the person entitled to exercise his or her Option pursuant to Section 4.1 by written notice under this Section 5.6. Any notice shall be deemed duly given when sent via email or when sent by certified mail (return receipt requested) and deposited (with postage prepaid) in a post office or branch post office regularly maintained by the United States Postal Service.

5.7 Titles. Titles are provided herein for convenience only and are not to serve as a basis for interpretation or construction of this Agreement.

5.8 Stockholder Approval. The Plan will be submitted for approval by the Company's stockholders within twelve months after the date the Plan was initially adopted by the Board. The Option may not be exercised to any extent by anyone prior to the time when the Plan is approved by the stockholders, and if such approval has not been obtained by the end of said twelve month period, the Option shall thereupon be canceled and become null and void.

5.9 Governing Law; Severability. This Agreement shall be administered, interpreted and enforced under the laws of the State of California, without regard to the conflicts of law principles thereof. Should any provision of this Agreement be determined by a court of law to be illegal or unenforceable, the other provisions shall nevertheless remain effective and shall remain enforceable.

5.10 Conformity to Securities Laws. Participant acknowledges that the Plan is intended to conform to the extent necessary with all provisions of the Securities Act and the Exchange Act and any and all regulations and rules promulgated by the Securities and Exchange Commission thereunder, and state securities laws and regulations. Notwithstanding anything herein to the contrary, the Plan shall be administered, and the Option is granted and may be exercised, only in such a manner as to conform to such laws, rules and regulations. To the extent permitted by applicable law, the Plan and this Agreement shall be deemed amended to the extent necessary to conform to such laws, rules and regulations.

5.11 Amendments. This Agreement may not be modified, amended or terminated except by an instrument in writing, signed by Participant or such other person as may be permitted to exercise the Option pursuant to Section 4.1 and by a duly authorized representative of the Company.

5.12 No Employment Rights. If Participant is an Employee, nothing in the Plan or this Agreement shall confer upon Participant any right to continue in the employ of the Company or any Subsidiary or shall interfere with or restrict in any way the rights of the Company and its Subsidiaries, which are expressly reserved, to discharge Participant at any time for any reason whatsoever, with or without cause, except to the extent expressly provided otherwise in a written agreement between the Company and Participant.

5.13 Successors and Assigns. The Company may assign any of its rights under this Agreement to single or multiple assignees, and this Agreement shall inure to the benefit of the successors and assigns of the Company. Subject to the restrictions on transfer herein set forth, this Agreement shall be binding upon Participant and his or her heirs, executors, administrators, successors and assigns.

5.14 Notification of Disposition. If this Option is designated as an Incentive Stock Option, Participant shall give prompt notice to the Company of any disposition or other transfer of any shares of Stock acquired under this Agreement if such disposition or transfer is made (a) within two years from the Grant Date with respect to such shares or (b) within one year after the transfer of such shares to him. Such notice shall specify the date of such disposition or other transfer and the amount realized, in cash, other property, assumption of indebtedness or other consideration, by Participant in such disposition or other transfer.

5.15 Limitations Applicable to Section 16 Persons. Notwithstanding any other provision of the Plan or this Agreement, if Participant is subject to Section 16 of the Exchange Act, the Plan, the Option and this Agreement shall be subject to any additional limitations set forth in any applicable exemptive rule under Section 16 of the Exchange Act (including any amendment to Rule 16b-3 of the Exchange Act) that are requirements for the application of such exemptive rule. To the extent permitted by applicable law, this Agreement shall be deemed amended to the extent necessary to conform to such applicable exemptive rule.

5.16 Entire Agreement. The Plan and this Agreement (including all Exhibits hereto) constitute the entire agreement of the parties and supersede in their entirety all prior undertakings and agreements of the Company and Participant with respect to the subject matter hereof.

EXHIBIT B

TO STOCK OPTION GRANT NOTICE

FORM OF EXERCISE NOTICE

Effective as of today, ____, _____ the undersigned ("***Participant***") hereby elects to exercise _____ Participant's option to purchase shares of the Stock (the "***Shares***") of Ultragenyx Pharmaceutical, Inc. (the "***Company***") under and pursuant to the Ultragenyx Pharmaceutical, Inc. 2011 Equity Incentive Plan (the "***Plan***") and the Stock Option Grant Notice and Stock Option Agreement dated _____, ____ (the "***Option Agreement***"). Capitalized terms used herein without definition shall have the meanings given in the Option Agreement.

Grant Date:	_____
Number of Shares as to which Option is Exercised:	_____
Exercise Price per Share:	$ _____
Total Exercise Price:	$ _____
Certificate to the issued in the name of:	_____
Cash Payment delivered herewith:	$ _____ (Representing the full Exercise Price for the Shares, as well as any applicable withholding tax)

Type of Option: ☐ Incentive Stock Option ☐ Non-Qualified Stock Option

1. Representations of Participant. Participant acknowledges that Participant has received, read and understood the Plan and the Option Agreement. Participant agrees to abide by and be bound by their terms and conditions.

2. Rights as Stockholder. Until the Shares are issued (as evidenced by the appropriate entry on the books of the Company or of a duly authorized transfer agent of the Company), no right to vote or receive dividends or any other rights as a stockholder shall exist with respect to Shares subject to the Option, notwithstanding the exercise of the Option. No adjustment will be made for a dividend or other right for which the record date is prior to the date the Shares are issued, except as provided in Article 10 of the Plan.

Participant shall enjoy rights as a stockholder until such time as Participant disposes of the Shares or the Company and/or its assignee(s) exercises the Right of First Refusal hereunder. Upon such exercise, Participant shall have no further rights as a holder of the Shares so purchased except the right to receive payment for the Shares so purchased in accordance with the provisions of this Agreement, and Participant shall forthwith cause the certificate(s), if any issued, evidencing the Shares so purchased to be surrendered to the Company for transfer or cancellation.

3. Participant's Rights to Transfer Shares.

(a) Before any Shares held by Participant or any permitted transferee (each, a "***Holder***") may be sold, pledged, assigned, hypothecated, transferred, or otherwise disposed of (each, a "***Transfer***"), the Company or its assignee(s) shall have a right of first refusal to purchase the Shares proposed to be Transferred on the terms and conditions set forth in this Section (the "***Right of First Refusal***"). In the event that the Company's Bylaws contain a right of first refusal with respect to the Shares, such right of first refusal shall apply to the Shares to the extent such provisions are more restrictive than the Right of First Refusal set forth in this Section and the Right of First Refusal set forth in this Section shall not in any way restrict the operation of the Company's Bylaws.

(b) In the event any Holder desires to Transfer any Shares, the Holder shall deliver to the Company a written notice (the "***Notice***") stating: (i) the Holder's bona fide intention to sell or otherwise Transfer such Shares; (ii) the name of each proposed purchaser or other transferee ("***Proposed Transferee***"); (iii) the number of Shares to be Transferred to each Proposed Transferee; and (iv) the price for which the Holder proposes to Transfer the Shares (the "***Offered Price***"), and the Holder shall offer such Shares at the Offered Price to the Company or its assignee(s).

(c) Within twenty-five days after receipt of the Notice, the Company and/or its assignee(s) may elect in writing to purchase all, but not less than all, of the Shares proposed to be Transferred to any one or more of the Proposed Transferees by delivery of a written exercise notice to the Holder (a "***Company Notice***"). The purchase price will be determined in accordance with subsection (d) below.

(d) The purchase price ("***Purchase Price***") for the Shares repurchased under this Section shall be the Offered Price.

(e) Payment of the Purchase Price shall be made, at the option of the Company or its assignee(s), in cash (by check), by cancellation of all or a portion of any outstanding indebtedness of the Holder to the Company (or, in the case of repurchase by an assignee, to the assignee), or by any combination thereof within five days after delivery of the Company Notice or in the manner and at the times mutually agreed to by the Company and the Holder. Should the Offered Price specified in the Notice be payable in property other than cash, the Company shall have the right to pay the purchase price in the form of cash equal in amount to the value of such property. If the Holder and the Company cannot agree on such cash value within ten days after the Company's receipt of the Notice, the valuation shall be made by the Board. The payment of the purchase price shall then be held on the later of (i) five days following delivery of the Company Notice or (ii) five days after such valuation shall have been made.

(f) If all or a portion of the Shares proposed in the Notice to be Transferred are not purchased by the Company and/or its assignee(s) as provided in this Section, then the Holder may sell or otherwise Transfer such Shares to that Proposed Transferee at the Offered Price or at a higher price, provided that such sale or other Transfer is consummated within sixty days after the date of the Notice and provided further that any such sale or other Transfer is

effected in accordance with any applicable securities laws and the Proposed Transferee agrees in writing that the provisions of this Section shall continue to apply to the Shares in the hands of such Proposed Transferee. If the Shares described in the Notice are not Transferred to the Proposed Transferee within such sixty-day period, a new Notice shall be given to the Company, and the Company and/or its assignees shall again be offered the Right of First Refusal as provided herein before any Shares held by the Holder may be sold or otherwise Transferred.

(g) Anything to the contrary contained in this Section notwithstanding, the Transfer of any or all of the Shares during Participant's lifetime or upon Participant's death by will or intestacy to Participant's Immediate Family or a trust for the benefit of Participant's Immediate Family shall be exempt from the Right of First Refusal. As used herein, "***Immediate Family***" shall mean spouse, lineal descendant or antecedent, father, mother, brother or sister or stepchild (whether or not adopted). In such case, the transferee or other recipient shall receive and hold the Shares so Transferred subject to the provisions of this Section (including the Right of First Refusal) and the Restricted Stock Purchase Agreement, if applicable, and there shall be no further Transfer of such Shares except in accordance with the terms of this Section.

(h) The Right of First Refusal shall terminate as to all Shares upon the Public Trading Date.

(i) Any transfer or sale of the Shares is subject to restrictions on transfer imposed by any applicable state and federal securities laws. Any Transfer or attempted Transfer of any of the Shares not in accordance with the terms of this Agreement shall be void and the Company may enforce the terms of this Agreement by stop transfer instructions or similar actions by the Company and its agents or designees.

4. Tax Consultation. Participant understands that Participant may suffer adverse tax consequences as a result of Participant's purchase or disposition of the Shares. Participant represents that Participant has consulted with any tax consultants Participant deems advisable in connection with the purchase or disposition of the Shares and that Participant is not relying on the Company for any tax advice.

5. Restrictive Legends and Stop-Transfer Orders.

(a) Legends. Participant understands and agrees that the Company shall cause any certificates issued evidencing the Shares shall have the legends set forth below or legends substantially equivalent thereto, together with any other legends that may be required by state or federal securities laws:

THE SHARES REPRESENTED HEREBY HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED ("ACT"), NOR HAVE THEY BEEN REGISTERED OR QUALIFIED UNDER THE SECURITIES LAWS OF ANY STATE. NO TRANSFER OF SUCH SECURITIES WILL BE PERMITTED UNLESS A REGISTRATION STATEMENT UNDER THE ACT IS IN EFFECT AS TO SUCH TRANSFER, THE TRANSFER IS MADE IN ACCORDANCE WITH RULE 144 UNDER THE ACT, OR IN THE

OPINION OF COUNSEL (WHICH MAY BE COUNSEL FOR THE COMPANY) REGISTRATION UNDER THE ACT IS UNNECESSARY IN ORDER FOR SUCH TRANSFER TO COMPLY WITH THE ACT AND WITH APPLICABLE STATE SECURITIES LAWS.

THE SHARES REPRESENTED BY THIS CERTIFICATE MAY BE TRANSFERRED ONLY IN ACCORDANCE WITH THE TERMS OF AN AGREEMENT BETWEEN THE COMPANY AND THE STOCKHOLDER, A COPY OF WHICH IS ON FILE WITH THE SECRETARY OF THE COMPANY. SUCH TRANSFER RESTRICTIONS ARE BINDING ON TRANSFEREES OF THESE SHARES.

(b) Participant agrees that, in order to ensure compliance with the restrictions referred to herein, the Company may issue appropriate "stop transfer" instructions to its transfer agent, if any, and that, if the Company transfers its own securities, it may make appropriate notations to the same effect in its own records.

(c) The Company shall not be required (i) to transfer on its books any Shares that have been sold or otherwise transferred in violation of any of the provisions of this Agreement or (ii) to treat as owner of such Shares or to accord the right to vote or pay dividends to any purchaser or other transferee to whom such Shares shall have been so transferred.

6. Participant Representations. Participant hereby makes the following certifications and representations with respect to the Shares listed above:

(a) Participant is aware of the Company's business affairs and financial condition and has acquired sufficient information about the Company to reach an informed and knowledgeable decision to acquire the Shares. Participant is acquiring these Shares for investment for Participant's own account only and not with a view to, or for resale in connection with, any "distribution" thereof within the meaning of the Securities Act.

(b) Participant acknowledges and understands that the Shares constitute "restricted securities" under the Securities Act and have not been registered under the Securities Act in reliance upon a specific exemption therefrom, which exemption depends upon, among other things, the bona fide nature of Participant's investment intent as expressed herein. Participant understands that the Shares must be held indefinitely unless they are subsequently registered under the Securities Act or an exemption from such registration is available. Participant further acknowledges and understands that the Company is under no obligation to register the Shares. Participant understands that the certificate evidencing the Shares will be imprinted with a legend which prohibits the transfer of the Shares unless they are registered or such registration is not required in the opinion of counsel satisfactory to the Company and any other legend required under applicable state securities laws.

(c) Participant is familiar with the provisions of Rule 701 and Rule 144, each promulgated under the Securities Act, which, in substance, permit limited public resale of "restricted securities" acquired, directly or indirectly from the issuer thereof, in a non-public

offering subject to the satisfaction of certain conditions. Rule 701 provides that if the issuer qualifies under Rule 701 at the time of the grant of the Option to Participant, the exercise will be exempt from registration under the Securities Act. In the event the Company becomes subject to the reporting requirements of Section 13 or 15(d) of the Exchange Act, ninety days thereafter (or such longer period as any market stand-off agreement may require) the securities exempt under Rule 701 may be resold, subject to the satisfaction of certain of the conditions specified by Rule 144, including: (i) the resale being made through a broker in an unsolicited "broker's transaction" or in transactions directly with a market maker (as said term is defined under the Exchange Act); and, in the case of an affiliate, (ii) the availability of certain public information about the Company, (iii) the amount of securities being sold during any three month period not exceeding the limitations specified in Rule 144(e), and (iv) the timely filing of a Form 144, if applicable.

(d) In the event that the Company does not qualify under Rule 701 at the time of grant of the Option, then the securities may be resold in certain limited circumstances subject to the provisions of Rule 144, which requires the resale to occur not less than one year after the later of the date the securities were sold by the Company or the date the securities were sold by an affiliate of the Company, within the meaning of Rule 144; and, in the case of acquisition of the securities by an affiliate, or by a non-affiliate who subsequently holds the securities less than two years, the satisfaction of the conditions set forth in sections (i), (ii), (iii) and (iv) of paragraph (c) above.

(e) Participant further understands that in the event all of the applicable requirements of Rule 701 or 144 are not satisfied, registration under the Securities Act, compliance with Regulation A, or some other registration exemption will be required; and that, notwithstanding the fact that Rules 144 and 701 are not exclusive, the Staff of the Securities and Exchange Commission has expressed its opinion that persons proposing to sell private placement securities other than in a registered offering and otherwise than pursuant to Rules 144 or 701 will have a substantial burden of proof in establishing that an exemption from registration is available for such offers or sales, and that such persons and their respective brokers who participate in such transactions do so at their own risk. Participant understands that no assurances can be given that any such other registration exemption will be available in such event.

7. Successors and Assigns. The Company may assign any of its rights under this Agreement to single or multiple assignees, and this Agreement shall inure to the benefit of the successors and assigns of the Company. Subject to the restrictions on transfer herein set forth, this Agreement shall be binding upon Participant and his or her heirs, executors, administrators, successors and assigns.

8. Interpretation. Any dispute regarding the interpretation of this Agreement shall be submitted by Participant or by the Company forthwith to the Administrator, which shall review such dispute at its next regular meeting. The resolution of such a dispute by the Administrator shall be final and binding on the Company and on Participant.

9. Governing Law; Severability. This Agreement shall be governed by and construed in accordance with the laws of the State of California, excluding that body of law pertaining to conflicts of law. Should any provision of this Agreement be determined by a court of law to be illegal or unenforceable, the other provisions shall nevertheless remain effective and shall remain enforceable.

10. Notices. Any notice required or permitted hereunder shall be given in accordance with the provisions set forth in Section 5.6 of the Option Agreement.

11. Further Instruments. The parties agree to execute such further instruments and to take such further action as may be reasonably necessary to carry out the purposes and intent of this Agreement.

12. Entire Agreement. The Plan and Option Agreement are incorporated herein by reference. This Agreement, the Plan and the Option Agreement constitute the entire agreement of the parties and supersede in their entirety all prior undertakings and agreements of the Company and Participant with respect to the subject matter hereof.

ACCEPTED BY:
ULTRAGENYX PHARMACEUTICAL, INC.

By: _____

Print Name:

Title:

SUBMITTED BY
PARTICIPANT:

By: _____

Print Name: _____

Address: _____

_____

CONSENT OF SPOUSE

CONSENT OF SPOUSE

I, _____, spouse of _____, have read and approve the Option Agreement and this Exercise Notice between my spouse and Ultragenyx Pharmaceutical, Inc. In consideration of granting of the right to my spouse to purchase shares of Ultragenyx Pharmaceutical, Inc. set forth in the Option Agreement and this Exercise Notice, I hereby appoint my spouse as my attorney-in-fact in respect to the exercise of any rights under the Option Agreement and this Exercise Notice and agree to be bound by the provisions of the Plan, the Option Agreement and this Exercise Notice insofar as I may have any rights in said agreements or any shares issued pursuant thereto under the community property laws or similar laws relating to marital property in effect in the state of our residence as of the date of the signing of the foregoing Exercise Notice.

Dated: _____, _____

_____

Signature of Spouse

ULTRAGENYX PHARMACEUTICAL, INC.

2011 EQUITY INCENTIVE PLAN STOCK OPTION GRANT NOTICE AND

STOCK OPTION AGREEMENT

Ultragenyx Pharmaceutical, Inc. (the "***Company***"), pursuant to its 2011 Equity Incentive Plan (the "***Plan***"), hereby grants to the holder listed below ("***Participant***"), an option to purchase the number of shares of the Company's Stock set forth below (the "***Option***"). This Option is subject to all of the terms and conditions as set forth herein and in the Stock Option Agreement attached hereto as Exhibit A (the "***Stock Option Agreement***") and the Plan, each of which are incorporated herein by reference. Unless otherwise defined herein, the terms defined in the Plan shall have the same defined meanings in this Grant Notice and the Stock Option Agreement.

Participant: _____

Grant Date: _____

Vesting Commencement Date: _____

Exercise Price per Share: $ _____

Total Exercise Price: $ _____

Total Number of Shares Subject to the Option: _____

Expiration Date: _____

Type of Option: ☐ Incentive Stock Option ☐ Non-Qualified Stock Option

Exercise Schedule: ☒ Early Exercise Permitted

Vesting Schedule: This Option is exercisable immediately, in whole or in part, at such times as are established by the Administrator, conditioned upon Participant entering into a Restricted Stock Purchase Agreement with respect to any unvested shares of Stock. The shares subject to this Option shall vest and/or be released from the Company's Repurchase Option, as set forth in the Restricted Stock Purchase Agreement attached hereto as Exhibit C (the "***Restricted Stock Purchase Agreement***"), according to the following schedule:

[To be specified in individual agreements.]

By his or her signature and the Company's signature below, Participant agrees to be bound by the terms and conditions of the Plan, the Stock Option Agreement and this Grant Notice. Participant has reviewed the Stock Option Agreement, the Plan and this Grant Notice in their entirety, has had an opportunity to obtain the advice of counsel prior to executing this Grant Notice and fully understands all provisions of this Grant Notice, the Stock Option Agreement and the Plan. Participant hereby agrees to accept as binding, conclusive and final all decisions or interpretations of the Administrator of the Plan upon any questions arising under the Plan or the Option.

ULTRAGENYX PHARMACEUTICAL, INC.

By: _____

Print Name:

Title:

Address:

PARTICIPANT:

By: _____

Print Name: _____

Address: _____

EXHIBIT A

TO STOCK OPTION GRANT NOTICE

STOCK OPTION AGREEMENT

Pursuant to the Stock Option Grant Notice ("***Grant Notice***") to which this Stock Option Agreement (this "***Agreement***") is attached, Ultragenyx Pharmaceutical, Inc. (the "***Company***") has granted to Participant an option under the Company's 2011 Equity Incentive Plan (the "***Plan***") to purchase the number of shares of Stock indicated in the Grant Notice.

ARTICLE I
GENERAL

1.1 Defined Terms. Capitalized terms not specifically defined herein shall have the meanings specified in the Plan and the Grant Notice.

1.2 Incorporation of Terms of Plan. The Option is subject to the terms and conditions of the Plan which are incorporated herein by reference.

ARTICLE II
GRANT OF OPTION

2.1 Grant of Option. In consideration of Participant's past and/or continued employment with or service to the Company or a Parent or Subsidiary and for other good and valuable consideration, effective as of the Grant Date set forth in the Grant Notice (the "Grant *Date*"), the Company irrevocably grants to Participant the Option to purchase any part or all of an aggregate of the number of shares of Stock set forth in the Grant Notice, upon the terms and conditions set forth in the Plan and this Agreement. Unless designated as a Non-Qualified Stock Option in the Grant Notice, the Option shall be an Incentive Stock Option to the maximum extent permitted by law.

2.2 Exercise Price. The exercise price of the shares of Stock subject to the Option shall be as set forth in the Grant Notice, without commission or other charge; *provided, however,* that if this Option is designated as an Incentive Stock Option, the price per share of the shares subject to the Option shall not be less than the greater of (i) 100% of the Fair Market Value of a share of Stock on the Grant Date, or (ii) 110% of the Fair Market Value of a share of Stock on the Grant Date in the case of a Participant then owning (within the meaning of Section 424(d) of the Code) more than 10% of the total combined voting power of all classes of stock of the Company or any "subsidiary corporation" of the Company or any "parent corporation" of the Company (each within the meaning of Section 424 of the Code).

ARTICLE III
PERIOD OF EXERCISABILITY

3.1 Commencement of Exercisability.

(a) Subject to Sections 3.3 and 5.8, the Option shall become vested and exercisable in such amounts and at such times as are set forth in the Grant Notice. Alternatively, at the election of the Participant, this Option may be exercised in whole or in part at such times as are established by the Administrator as to shares of Stock which have not yet vested. Vested shares shall not be subject to the Company's Repurchase Option (as set forth in the Restricted Stock Purchase Agreement). As a condition to exercising this Option for unvested shares of Stock, the Participant shall execute the Restricted Stock Purchase Agreement.

(b) No portion of the Option which has not become vested and exercisable at the date of Participant's Termination of Service shall thereafter become vested and exercisable, except as may be otherwise provided by the Administrator or as set forth in a written agreement between the Company and Participant.

3.2 Duration of Exercisability. The installments provided for in the vesting schedule set forth in the Grant Notice are cumulative. Each such installment which becomes vested and exercisable pursuant to the vesting schedule set forth in the Grant Notice shall remain vested and exercisable until it becomes unexercisable under Section 3.3.

3.3 Expiration of Option. The Option may not be exercised to any extent by anyone after the first to occur of the following events:

(a) The expiration of ten years from the Grant Date;

(b) If this Option is designated as an Incentive Stock Option and Participant owned (within the meaning of Section 424(d) of the Code), at the time the Option was granted, more than 10% of the total combined voting power of all classes of stock of the Company or any "subsidiary corporation" of the Company or "parent corporation" of the Company (each within the meaning of Section 424 of the Code), the expiration of five years from the date the Option was granted; or

(c) The expiration of three months following the date of Participant's Termination of Service, unless such termination occurs by reason of Participant's death, Disability or Misconduct;

(d) The expiration of one year following the date of Participant's Termination of Service by reason of Participant's death or Disability; or

(e) The date of Participant's Termination of Service as a result of Participant's Misconduct.

Participant acknowledges that an Incentive Stock Option exercised more than three months after Participant's termination of status as an Employee, other than by reason of death or Disability, will be taxed as a Non-Qualified Stock Option.

3.4 Special Tax Consequences. Participant acknowledges that, to the extent that the aggregate Fair Market Value (determined as of the time the Option is granted) of all shares of Stock with respect to which Incentive Stock Options, including the Option, are first exercisable for the first time by Participant in any calendar year exceeds $100,000 (or such other limitation as imposed by Section 422(d) of the Code), the Option and such other options shall be treated as not qualifying under Section 422 of the Code but rather shall be considered Non-Qualified Stock Options. Participant further acknowledges that the rule set forth in the preceding sentence shall be applied by taking Options and other "incentive stock options" into account in the order in which they were granted.

ARTICLE IV
EXERCISE OF OPTION

4.1 Person Eligible to Exercise. Except as provided in Sections 5.2(b) and 5.2(c), during the lifetime of Participant, only Participant may exercise the Option or any portion thereof. After the death of Participant, any exercisable portion of the Option may, prior to the time when the Option becomes unexercisable under Section 3.3, be exercised by Participant's personal representative or by any person empowered to do so under the deceased Participant's will or under the then applicable laws of descent and distribution.

4.2 Partial Exercise. Any exercisable portion of the Option or the entire Option, if then wholly exercisable, may be exercised in whole or in part at any time prior to the time when the Option or portion thereof becomes unexercisable under Section 3.3.

4.3 Manner of Exercise. The Option, or any exercisable portion thereof, may be exercised solely by delivery to the Secretary of the Company or the Secretary's office of all of the following prior to the time when the Option or such portion thereof becomes unexercisable under Section 3.3:

(a) An Exercise Notice in writing signed by Participant or any other person then entitled to exercise the Option or portion thereof, stating that the Option or portion thereof is thereby exercised, such notice complying with all applicable rules established by the Administrator. Such notice shall be substantially in the form attached as Exhibit B to the Grant Notice (or such other form as is prescribed by the Administrator); and

(b) (b) A Restricted Stock Purchase Agreement, if applicable, substantially in the form attached as Exhibit C to the Grant Notice;

(c) Subject to Section 5.1(c) of the Plan:

(i) Full payment (in cash or by check) for the shares with respect to which the Option or portion thereof is exercised; or

(ii) With the consent of the Administrator, by delivery of a full recourse promissory note on such terms and conditions as may be approved by the Administrator; or

(iii) With the consent of the Administrator, by delivery of shares of Stock then issuable upon exercise of the Option having a Fair Market Value on the date of delivery equal to the aggregate exercise price of the Option or exercised portion thereof; or

(iv) On and after the Public Trading Date, such payment may be made, in whole or in part, through the delivery of shares of Stock which have been owned by Participant for at least six months, duly endorsed for transfer to the Company with a Fair Market Value on the date of delivery equal to the aggregate exercise price of the Option or exercised portion thereof; or

(v) On and after the Public Trading Date, through the delivery of a notice that Participant has placed a market sell order with a broker with respect to shares of Stock then issuable upon exercise of the Option, and that the broker has been directed to pay a sufficient portion of the net proceeds of the sale to the Company in satisfaction of the Option exercise price; provided, that payment of such proceeds is made to the Company upon settlement of such sale; or

(vi) Subject to any applicable laws, any combination of the consideration provided in the foregoing paragraphs (i), (ii) and (iii); and

(d) A bona fide written representation and agreement, in such form as is prescribed by the Administrator, signed by Participant or the other person then entitled to exercise such Option or portion thereof, stating that the shares of Stock are being acquired for Participant's own account, for investment and without any present intention of distributing or reselling said shares or any of them except as may be permitted under the Securities Act and then applicable rules and regulations thereunder, and that Participant or other person then entitled to exercise such Option or portion thereof will indemnify the Company against and hold it free and harmless from any loss, damage, expense or liability resulting to the Company if any sale or distribution of the shares by such person is contrary to the representation and agreement referred to above. The Administrator may, in its absolute discretion, take whatever additional actions it deems appropriate to ensure the observance and performance of such representation and agreement and to effect compliance with the Securities Act and any other federal or state securities laws or regulations. Without limiting the generality of the foregoing, the Administrator may require an opinion of counsel acceptable to it to the effect that any subsequent transfer of shares acquired on an Option exercise does not violate the Securities Act, and may issue stop-transfer orders covering such shares. Share certificates evidencing Stock issued on exercise of the Option shall bear an appropriate legend referring to the provisions of this subsection (d) and the agreements herein. The written representation and agreement referred to in the first sentence of this subsection (d) shall, however, not be required if the shares to be issued pursuant to such exercise have been registered under the Securities Act, and such registration is then effective in respect of such shares; and

(e) The receipt by the Company of full payment for such shares, including payment of any applicable withholding tax, which may be in the form of consideration used by Participant to pay for such shares under Section 4.3(b), subject to Section 15.3 of the Plan; and

(f) In the event the Option or portion thereof shall be exercised pursuant to Section 4.1 by any person or persons other than Participant, appropriate proof of the right of such person or persons to exercise the Option.

4.4 Conditions to Issuance of Stock Certificates. The shares of Stock deliverable upon the exercise of the Option, or any portion thereof, may be either previously authorized but unissued shares or issued shares which have then been reacquired by the Company. Such shares shall be fully paid and nonassessable. The Company shall not be required to issue or deliver any shares of Stock purchased upon the exercise of the Option or portion thereof prior to fulfillment of all of the following conditions:

(a) The admission of such shares to listing on all stock exchanges on which such Stock is then listed; and

(b) The completion of any registration or other qualification of such shares under any state or federal law or under rulings or regulations of the Securities and Exchange Commission or of any other governmental regulatory body, which the Administrator shall, in its absolute discretion, deem necessary or advisable; and

(c) The obtaining of any approval or other clearance from any state or federal governmental agency which the Administrator shall, in its absolute discretion, determine to be necessary or advisable; and

(d) The lapse of such reasonable period of time following the exercise of the Option as the Administrator may from time to time establish for reasons of administrative convenience; and

(e) The receipt by the Company of full payment for such shares, including payment of any applicable withholding tax, which may be in the form of consideration used by Participant to pay for such shares under Section 4.3(b), subject to Section 15.3 of the Plan.

4.5 Rights as Stockholder. The holder of the Option shall not be, nor have any of the rights or privileges of, a stockholder of the Company in respect of any shares purchasable upon the exercise of any part of the Option unless and until such shares shall have been issued by the Company to such holder.

ARTICLE V
OTHER PROVISIONS

5.1 Administration. The Administrator shall have the power to interpret the Plan and this Agreement and to adopt such rules for the administration, interpretation and application of the Plan as are consistent therewith and to interpret, amend or revoke any such rules. All actions taken and all interpretations and determinations made by the Administrator in good faith shall be final and binding upon Participant, the Company and all other interested persons. No member of the Administrator shall be personally liable for any action, determination or interpretation made in good faith with respect to the Plan, this Agreement or the Option. In its absolute discretion, the Board may at any time and from time to time exercise any and all rights and duties of the Administrator under the Plan and this Agreement.

5.2 Option Not Transferable.

(a) Subject to Section 5.2(b), the Option may not be sold, pledged, assigned or transferred in any manner other than by will or the laws of descent and distribution, unless and until the shares underlying the Option have been issued, and all restrictions applicable to such shares have lapsed. Neither the Option nor any interest or right therein shall be liable for the debts, contracts or engagements of Participant or his or her successors in interest or shall be subject to disposition by transfer, alienation, anticipation, pledge, encumbrance, assignment or any other means whether such disposition be voluntary or involuntary or by operation of law by judgment, levy, attachment, garnishment or any other legal or equitable proceedings (including bankruptcy), and any attempted disposition thereof shall be null and void and of no effect, except to the extent that such disposition is permitted by the preceding sentence.

(b) Notwithstanding any other provision in this Agreement, with the consent of the Administrator and to the extent the Option is designated as a Non-Qualified Stock Option, the Option may be transferred to, exercised by and paid to one or more Permitted Transferees, subject to the terms and conditions set forth in Section 10.3 of the Plan.

(c) Unless transferred to a Permitted Transferee in accordance with Section 5.2(b), during the lifetime of Participant, only Participant may exercise the Option or any portion thereof. Subject to such conditions and procedures as the Administrator may require, a Permitted Transferee may exercise the Option or any portion thereof during Participant's lifetime. After the death of Participant, any exercisable portion of the Option may, prior to the time when the Option becomes unexercisable under Section 3.3, be exercised by Participant's personal representative or by any person empowered to do so under the deceased Participant's will or under the then applicable laws of descent and distribution.

5.3 Lock-Up Period. Participant hereby agrees that, if so requested by the Company or any representative of the underwriters (the "***Managing Underwriter***") in connection with any registration of the offering of any securities of the Company under the Securities Act, Participant shall not sell or otherwise transfer any shares of Stock or other securities of the Company during such period as may be requested in writing by the Managing Underwriter and agreed to in writing by the Company (which period shall not be longer than one hundred eighty days) (the "***Market Standoff Period***") following the effective date of a registration statement of the Company filed under the Securities Act; *provided, however,* that such restriction shall apply only to the first registration statement of the Company to become effective under the Securities Act that includes securities to be sold on behalf of the Company to the public in an underwritten public offering under the Securities Act.

5.4 Restrictive Legends and Stop-Transfer Orders.

(a) The share certificate or certificates evidencing the shares of Stock purchased hereunder shall be endorsed with any legends that may be required by state or federal securities laws.

(b) Participant agrees that, in order to ensure compliance with the restrictions referred to herein, the Company may issue appropriate "stop transfer" instructions to its transfer agent, if any, and that, if the Company transfers its own securities, it may make appropriate notations to the same effect in its own records.

(c) The Company shall not be required: (i) to transfer on its books any shares of Stock that have been sold or otherwise transferred in violation of any of the provisions of this Agreement, or (ii) to treat as owner of such shares of Stock or to accord the right to vote or pay dividends to any purchaser or other transferee to whom such shares shall have been so transferred.

5.5 Shares to Be Reserved. The Company shall at all times during the term of the Option reserve and keep available such number of shares of Stock as will be sufficient to satisfy the requirements of this Agreement.

5.6 Notices. Any notice to be given under the terms of this Agreement to the Company shall be addressed to the Company in care of the Secretary of the Company at the address given beneath the signature of the Company's authorized officer on the Grant Notice, and any notice to be given to Participant shall be addressed to Participant at the address given beneath Participant's signature on the Grant Notice. By a notice given pursuant to this Section 5.6, either party may hereafter designate a different address for notices to be given to that party. Any notice which is required to be given to Participant shall, if Participant is then deceased, be given to the person entitled to exercise his or her Option pursuant to Section 4.1 by written notice under this Section 5.6. Any notice shall be deemed duly given when sent via email or when sent by certified mail (return receipt requested) and deposited (with postage prepaid) in a post office or branch post office regularly maintained by the United States Postal Service.

5.7 Titles. Titles are provided herein for convenience only and are not to serve as a basis for interpretation or construction of this Agreement.

5.8 Stockholder Approval. The Plan will be submitted for approval by the Company's stockholders within twelve months after the date the Plan was initially adopted by the Board. The Option may not be exercised to any extent by anyone prior to the time when the Plan is approved by the stockholders, and if such approval has not been obtained by the end of said twelve month period, the Option shall thereupon be canceled and become null and void.

5.9 Governing Law; Severability. This Agreement shall be administered, interpreted and enforced under the laws of the State of California, without regard to the conflicts of law principles thereof. Should any provision of this Agreement be determined by a court of law to be illegal or unenforceable, the other provisions shall nevertheless remain effective and shall remain enforceable.

5.10 Conformity to Securities Laws. Participant acknowledges that the Plan is intended to conform to the extent necessary with all provisions of the Securities Act and the Exchange Act and any and all regulations and rules promulgated by the Securities and Exchange Commission thereunder, and state securities laws and regulations. Notwithstanding anything herein to the contrary, the Plan shall be administered, and the Option is granted and may be exercised, only in such a manner as to conform to such laws, rules and regulations. To the extent permitted by applicable law, the Plan and this Agreement shall be deemed amended to the extent necessary to conform to such laws, rules and regulations.

5.11 Amendments. This Agreement may not be modified, amended or terminated except by an instrument in writing, signed by Participant or such other person as may be permitted to exercise the Option pursuant to Section 4.1 and by a duly authorized representative of the Company.

5.12 No Employment Rights. If Participant is an Employee, nothing in the Plan or this Agreement shall confer upon Participant any right to continue in the employ of the Company or any Subsidiary or shall interfere with or restrict in any way the rights of the Company and its Subsidiaries, which are expressly reserved, to discharge Participant at any time for any reason whatsoever, with or without cause, except to the extent expressly provided otherwise in a written agreement between the Company and Participant.

5.13 Successors and Assigns. The Company may assign any of its rights under this Agreement to single or multiple assignees, and this Agreement shall inure to the benefit of the successors and assigns of the Company. Subject to the restrictions on transfer herein set forth, this Agreement shall be binding upon Participant and his or her heirs, executors, administrators, successors and assigns.

5.14 Notification of Disposition. If this Option is designated as an Incentive Stock Option, Participant shall give prompt notice to the Company of any disposition or other transfer of any shares of Stock acquired under this Agreement if such disposition or transfer is made (a) within two years from the Grant Date with respect to such shares or (b) within one year after the transfer of such shares to him. Such notice shall specify the date of such disposition or other transfer and the amount realized, in cash, other property, assumption of indebtedness or other consideration, by Participant in such disposition or other transfer.

5.15 Limitations Applicable to Section 16 Persons. Notwithstanding any other provision of the Plan or this Agreement, if Participant is subject to Section 16 of the Exchange Act, the Plan, the Option, the Shares and this Agreement shall be subject to any additional limitations set forth in any applicable exemptive rule under Section 16 of the Exchange Act (including any amendment to Rule 16b-3 of the Exchange Act) that are requirements for the application of such exemptive rule. To the extent permitted by applicable law, this Agreement shall be deemed amended to the extent necessary to conform to such applicable exemptive rule.

5.16 Entire Agreement. The Plan and this Agreement (including all Exhibits hereto) constitute the entire agreement of the parties and supersede in their entirety all prior undertakings and agreements of the Company and Participant with respect to the subject matter hereof.

EXHIBIT B

TO STOCK OPTION GRANT NOTICE

FORM OF EXERCISE NOTICE

Effective as of today, ____, _____ the undersigned ("***Participant***") hereby elects to exercise Participant's option to purchase _____ shares of the Stock (the "***Shares***") of Ultragenyx Pharmaceutical, Inc. (the "***Company***") under and pursuant to the Ultragenyx Pharmaceutical, Inc. 2011 Equity Incentive Plan (the "***Plan***") and the Stock Option Grant Notice and Stock Option Agreement dated _____, ____ (the "***Option Agreement***"). Capitalized terms used herein without definition shall have the meanings given in the Option Agreement.

Grant Date: _____

Number of Shares as to which Option is Exercised: _____

Exercise Price per Share: $ _____

Total Exercise Price: $ _____

Certificate to the issued in the name of: _____

Cash Payment delivered herewith: $ _____ (Representing the full Exercise Price for the Shares, as well as any applicable withholding tax)

Type of Option: ☐ Incentive Stock Option ☐ Non-Qualified Stock Option

1. Representations of Participant. Participant acknowledges that Participant has received, read and understood the Plan and the Option Agreement. Participant agrees to abide by and be bound by their terms and conditions.

2. Rights as Stockholder. Until the Shares are issued (as evidenced by the appropriate entry on the books of the Company or of a duly authorized transfer agent of the Company), no right to vote or receive dividends or any other rights as a stockholder shall exist with respect to Shares subject to the Option, notwithstanding the exercise of the Option. No adjustment will be made for a dividend or other right for which the record date is prior to the date the Shares are issued, except as provided in Article 10 of the Plan.

Participant shall enjoy rights as a stockholder until such time as Participant disposes of the Shares or the Company and/or its assignee(s) exercises the Right of First Refusal hereunder. Upon such exercise, Participant shall have no further rights as a holder of the Shares so purchased except the right to receive payment for the Shares so purchased in accordance with the provisions of this Agreement, and Participant shall forthwith cause the certificate(s), if any issued, evidencing the Shares so purchased to be surrendered to the Company for transfer or cancellation.

3. Participant's Rights to Transfer Shares.

(a) Before any Shares held by Participant or any permitted transferee (each, a "***Holder***") may be sold, pledged, assigned, hypothecated, transferred, or otherwise disposed of (each, a "***Transfer***"), the Company or its assignee(s) shall have a right of first refusal to purchase the Shares proposed to be Transferred on the terms and conditions set forth in this Section (the "***Right of First Refusal***"). In the event that the Company's Bylaws contain a right of first refusal with respect to the Shares, such right of first refusal shall apply to the Shares to the extent such provisions are more restrictive than the Right of First Refusal set forth in this Section and the Right of First Refusal set forth in this Section shall not in any way restrict the operation of the Company's Bylaws.

(b) In the event any Holder desires to Transfer any Shares, the Holder shall deliver to the Company a written notice (the "***Notice***") stating: (i) the Holder's bona fide intention to sell or otherwise Transfer such Shares; (ii) the name of each proposed purchaser or other transferee ("***Proposed Transferee***"); (iii) the number of Shares to be Transferred to each Proposed Transferee; and (iv) the price for which the Holder proposes to Transfer the Shares (the "***Offered Price***"), and the Holder shall offer such Shares at the Offered Price to the Company or its assignee(s).

(c) Within twenty-five days after receipt of the Notice, the Company and/or its assignee(s) may elect in writing to purchase all, but not less than all, of the Shares proposed to be Transferred to any one or more of the Proposed Transferees by delivery of a written exercise notice to the Holder (a "***Company Notice***"). The purchase price will be determined in accordance with subsection (d) below.

(d) The purchase price ("***Purchase Price***") for the Shares repurchased under this Section shall be the Offered Price.

(e) Payment of the Purchase Price shall be made, at the option of the Company or its assignee(s), in cash (by check), by cancellation of all or a portion of any outstanding indebtedness of the Holder to the Company (or, in the case of repurchase by an assignee, to the assignee), or by any combination thereof within five days after delivery of the Company Notice or in the manner and at the times mutually agreed to by the Company and the Holder. Should the Offered Price specified in the Notice be payable in property other than cash, the Company shall have the right to pay the purchase price in the form of cash equal in amount to the value of such property. If the Holder and the Company cannot agree on such cash value within ten days after the Company's receipt of the Notice, the valuation shall be made by the Board. The payment of the purchase price shall then be held on the later of (i) five days following delivery of the Company Notice or (ii) five days after such valuation shall have been made.

(f) If all or a portion of the Shares proposed in the Notice to be Transferred are not purchased by the Company and/or its assignee(s) as provided in this Section, then the Holder may sell or otherwise Transfer such Shares to that Proposed Transferee at the Offered Price or at a higher price, provided that such sale or other Transfer is consummated within sixty days after the date of the Notice and provided further that any such sale or other Transfer is

effected in accordance with any applicable securities laws and the Proposed Transferee agrees in writing that the provisions of this Section shall continue to apply to the Shares in the hands of such Proposed Transferee. If the Shares described in the Notice are not Transferred to the Proposed Transferee within such sixty-day period, a new Notice shall be given to the Company, and the Company and/or its assignees shall again be offered the Right of First Refusal as provided herein before any Shares held by the Holder may be sold or otherwise Transferred.

(g) Anything to the contrary contained in this Section notwithstanding, the Transfer of any or all of the Shares during Participant's lifetime or upon Participant's death by will or intestacy to Participant's Immediate Family or a trust for the benefit of Participant's Immediate Family shall be exempt from the Right of First Refusal. As used herein, "***Immediate Family***" shall mean spouse, lineal descendant or antecedent, father, mother, brother or sister or stepchild (whether or not adopted). In such case, the transferee or other recipient shall receive and hold the Shares so Transferred subject to the provisions of this Section (including the Right of First Refusal) and the Restricted Stock Purchase Agreement, if applicable, and there shall be no further Transfer of such Shares except in accordance with the terms of this Section.

(h) The Right of First Refusal shall terminate as to all Shares upon the Public Trading Date.

(i) Any transfer or sale of the Shares is subject to restrictions on transfer imposed by any applicable state and federal securities laws. Any Transfer or attempted Transfer of any of the Shares not in accordance with the terms of this Agreement shall be void and the Company may enforce the terms of this Agreement by stop transfer instructions or similar actions by the Company and its agents or designees.

4. Tax Consultation. Participant understands that Participant may suffer adverse tax consequences as a result of Participant's purchase or disposition of the Shares. Participant represents that Participant has consulted with any tax consultants Participant deems advisable in connection with the purchase or disposition of the Shares and that Participant is not relying on the Company for any tax advice.

5. Restrictive Legends and Stop-Transfer Orders.

(a) Legends. Participant understands and agrees that the Company shall cause any certificates issued evidencing the Shares shall have the legends set forth below or legends substantially equivalent thereto, together with any other legends that may be required by state or federal securities laws:

THE SHARES REPRESENTED HEREBY HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933, AS AMENDED ("ACT"), NOR HAVE THEY BEEN REGISTERED OR QUALIFIED UNDER THE SECURITIES LAWS OF ANY STATE. NO TRANSFER OF SUCH SECURITIES WILL BE PERMITTED UNLESS A REGISTRATION STATEMENT UNDER THE ACT IS IN EFFECT AS TO SUCH TRANSFER, THE TRANSFER IS MADE IN ACCORDANCE WITH RULE 144 UNDER THE ACT, OR IN THE

OPINION OF COUNSEL (WHICH MAY BE COUNSEL FOR THE COMPANY) REGISTRATION UNDER THE ACT IS UNNECESSARY IN ORDER FOR SUCH TRANSFER TO COMPLY WITH THE ACT AND WITH APPLICABLE STATE SECURITIES LAWS.

THE SHARES REPRESENTED BY THIS CERTIFICATE MAY BE TRANSFERRED ONLY IN ACCORDANCE WITH THE TERMS OF AN AGREEMENT BETWEEN THE COMPANY AND THE STOCKHOLDER, A COPY OF WHICH IS ON FILE WITH THE SECRETARY OF THE COMPANY. SUCH TRANSFER RESTRICTIONS ARE BINDING ON TRANSFEREES OF THESE SHARES.

(b) Participant agrees that, in order to ensure compliance with the restrictions referred to herein, the Company may issue appropriate "stop transfer" instructions to its transfer agent, if any, and that, if the Company transfers its own securities, it may make appropriate notations to the same effect in its own records.

(c) The Company shall not be required (i) to transfer on its books any Shares that have been sold or otherwise transferred in violation of any of the provisions of this Agreement or (ii) to treat as owner of such Shares or to accord the right to vote or pay dividends to any purchaser or other transferee to whom such Shares shall have been so transferred.

6. Participant Representations. Participant hereby makes the following certifications and representations with respect to the Shares listed above:

(a) Participant is aware of the Company's business affairs and financial condition and has acquired sufficient information about the Company to reach an informed and knowledgeable decision to acquire the Shares. Participant is acquiring these Shares for investment for Participant's own account only and not with a view to, or for resale in connection with, any "distribution" thereof within the meaning of the Securities Act.

(b) Participant acknowledges and understands that the Shares constitute "restricted securities" under the Securities Act and have not been registered under the Securities Act in reliance upon a specific exemption therefrom, which exemption depends upon, among other things, the bona fide nature of Participant's investment intent as expressed herein. Participant understands that the Shares must be held indefinitely unless they are subsequently registered under the Securities Act or an exemption from such registration is available. Participant further acknowledges and understands that the Company is under no obligation to register the Shares. Participant understands that the certificate evidencing the Shares will be imprinted with a legend which prohibits the transfer of the Shares unless they are registered or such registration is not required in the opinion of counsel satisfactory to the Company and any other legend required under applicable state securities laws.

(c) Participant is familiar with the provisions of Rule 701 and Rule 144, each promulgated under the Securities Act, which, in substance, permit limited public resale of "restricted securities" acquired, directly or indirectly from the issuer thereof, in a non-public

offering subject to the satisfaction of certain conditions. Rule 701 provides that if the issuer qualifies under Rule 701 at the time of the grant of the Option to Participant, the exercise will be exempt from registration under the Securities Act. In the event the Company becomes subject to the reporting requirements of Section 13 or 15(d) of the Exchange Act, ninety days thereafter (or such longer period as any market stand-off agreement may require) the securities exempt under Rule 701 may be resold, subject to the satisfaction of certain of the conditions specified by Rule 144, including: (i) the resale being made through a broker in an unsolicited "broker's transaction" or in transactions directly with a market maker (as said term is defined under the Exchange Act); and, in the case of an affiliate, (ii) the availability of certain public information about the Company, (iii) the amount of securities being sold during any three month period not exceeding the limitations specified in Rule 144(e), and (iv) the timely filing of a Form 144, if applicable.

(d) In the event that the Company does not qualify under Rule 701 at the time of grant of the Option, then the securities may be resold in certain limited circumstances subject to the provisions of Rule 144, which requires the resale to occur not less than one year after the later of the date the securities were sold by the Company or the date the securities were sold by an affiliate of the Company, within the meaning of Rule 144; and, in the case of acquisition of the securities by an affiliate, or by a non-affiliate who subsequently holds the securities less than two years, the satisfaction of the conditions set forth in sections (i), (ii), (iii) and (iv) of paragraph (c) above.

(e) Participant further understands that in the event all of the applicable requirements of Rule 701 or 144 are not satisfied, registration under the Securities Act, compliance with Regulation A, or some other registration exemption will be required; and that, notwithstanding the fact that Rules 144 and 701 are not exclusive, the Staff of the Securities and Exchange Commission has expressed its opinion that persons proposing to sell private placement securities other than in a registered offering and otherwise than pursuant to Rules 144 or 701 will have a substantial burden of proof in establishing that an exemption from registration is available for such offers or sales, and that such persons and their respective brokers who participate in such transactions do so at their own risk. Participant understands that no assurances can be given that any such other registration exemption will be available in such event.

7. Successors and Assigns. The Company may assign any of its rights under this Agreement to single or multiple assignees, and this Agreement shall inure to the benefit of the successors and assigns of the Company. Subject to the restrictions on transfer herein set forth, this Agreement shall be binding upon Participant and his or her heirs, executors, administrators, successors and assigns.

8. Interpretation. Any dispute regarding the interpretation of this Agreement shall be submitted by Participant or by the Company forthwith to the Administrator, which shall review such dispute at its next regular meeting. The resolution of such a dispute by the Administrator shall be final and binding on the Company and on Participant.

9. Governing Law; Severability. This Agreement shall be governed by and construed in accordance with the laws of the State of California, excluding that body of law pertaining to conflicts of law. Should any provision of this Agreement be determined by a court of law to be illegal or unenforceable, the other provisions shall nevertheless remain effective and shall remain enforceable.

10. Notices. Any notice required or permitted hereunder shall be given in accordance with the provisions set forth in Section 5.6 of the Option Agreement.

11. Further Instruments. The parties agree to execute such further instruments and to take such further action as may be reasonably necessary to carry out the purposes and intent of this Agreement.

12. Entire Agreement. The Plan and Option Agreement are incorporated herein by reference. This Agreement, the Plan and the Option Agreement constitute the entire agreement of the parties and supersede in their entirety all prior undertakings and agreements of the Company and Participant with respect to the subject matter hereof.

ACCEPTED BY:
ULTRAGENYX PHARMACEUTICAL, INC.

By: _____

Print Name:

Title:

SUBMITTED BY
PARTICIPANT:

By: _____

Print Name: _____

Address: _____

_____

CONSENT OF SPOUSE

I, _____, spouse of _____, have read and approve the Option Agreement and this Exercise Notice between my spouse and Ultragenyx Pharmaceutical, Inc. In consideration of granting of the right to my spouse to purchase shares of Ultragenyx Pharmaceutical, Inc. set forth in the Option Agreement and this Exercise Notice, I hereby appoint my spouse as my attorney-in-fact in respect to the exercise of any rights under the Option Agreement and this Exercise Notice and agree to be bound by the provisions of the Plan, the Option Agreement and this Exercise Notice insofar as I may have any rights in said agreements or any shares issued pursuant thereto under the community property laws or similar laws relating to marital property in effect in the state of our residence as of the date of the signing of the foregoing Exercise Notice.

Dated: _____, _____

_____

Signature of Spouse

EXHIBIT C

TO STOCK OPTION GRANT NOTICE

RESTRICTED STOCK PURCHASE AGREEMENT

THIS RESTRICTED STOCK PURCHASE AGREEMENT is made between (the "***Purchaser***") and Ultragenyx Pharmaceutical, Inc. (the "Company"), as of _____, _____.

RECITALS

(1) Pursuant to the exercise of the Option granted to Purchaser under the Company's 2011 Equity Incentive Plan (the "***Plan***") and pursuant to the Stock Option Agreement (the "Option ***Agreement***") dated _____, _____, by and between the Company and Purchaser with respect to such grant, which Option Agreement is hereby incorporated by reference, Purchaser has elected to purchase of those shares which have not become vested under the vesting schedule set forth in the Option Agreement ("***Unvested Shares***"). The Unvested Shares and the shares subject to the Option Agreement which have become vested are sometimes collectively referred to herein as the "***Shares***".

(2) As required by the Option Agreement, as a condition to Purchaser's election to exercise the option, Purchaser must execute this Restricted Stock Purchase Agreement, which sets forth the rights and obligations of the parties with respect to Shares acquired upon exercise of the Option.

1. Repurchase Option.

(a) In the event of Purchaser's Termination of Service (as defined in the Option Agreement), for any reason, including for cause, death, Disability or Misconduct, the Company shall have the right and option to purchase from Purchaser, or Purchaser's personal representative, as the case may be, all of Purchaser's Unvested Shares as of the date of the Purchaser's Termination of Service at the exercise price paid by Purchaser for such Shares in connection with the exercise of the Option (the "***Repurchase Option***").

(b) The Company may exercise its Repurchase Option by delivering, personally or by registered mail, to Purchaser (or his or her transferee or legal representative, as the case may be), within ninety days of the date of the Purchaser's Termination of Service, a notice in writing indicating the Company's intention to exercise the Repurchase Option and setting forth a date for closing not later than thirty days from the mailing of such notice. The closing shall take place at the Company's office. At the closing, the holder of the certificates for the Unvested Shares being transferred shall deliver the stock certificate or certificates evidencing the Unvested Shares, and the Company shall deliver the purchase price therefor.

(c) At its option, the Company may elect to make payment for the Unvested Shares to a bank selected by the Company. The Company shall avail itself of this option by a notice in writing to Purchaser stating the name and address of the bank, date of closing, and waiving the closing at the Company's office.

(d) If the Company does not elect to exercise the Repurchase Option conferred above by giving the requisite notice within ninety days following the date of Purchaser's Termination of Service, the Repurchase Option shall terminate.

(e) 100% of the Unvested Shares shall initially be subject to the Repurchase Option. The Unvested Shares shall be released from the Repurchase Option in accordance with the Vesting Schedule set forth in the Option Agreement until all Shares are released from the Repurchase Option. Fractional Shares shall be rounded down to the nearest whole share.

2. Transferability of the Shares; Escrow.

(a) Purchaser hereby authorizes and directs the secretary of the Company, or such other person designated by the Company from time to time, to transfer the Unvested Shares as to which the Repurchase Option has been exercised from Purchaser to the Company.

(b) To insure the availability for delivery of Purchaser's Unvested Shares upon repurchase by the Company pursuant to the Repurchase Option under Section 1, Purchaser hereby appoints the assistant secretary, or any other person designated by the Company from time to time as escrow agent, as its attorney-in-fact to sell, assign and transfer unto the Company, such Unvested Shares, if any, repurchased by the Company pursuant to the Repurchase Option. If certificates for the Shares are issued, then Purchaser shall, upon execution of this Agreement, deliver and deposit with the assistant secretary of the Company, or such other person designated by the Company from time to time, any share certificate(s) issued representing the Unvested Shares, together with the stock assignment duly endorsed in blank, attached hereto as Exhibit C-1. The Unvested Shares and stock assignment shall be held by the assistant secretary in escrow, pursuant to the Joint Escrow Instructions of the Company and Purchaser attached as Exhibit C-2 hereto, until the Company exercises its Repurchase Option as provided in Section 1, until such Unvested Shares are vested, or until such time as this Agreement no longer is in effect. As a further condition to the Company's obligations under this Agreement, the spouse of Purchaser, if any, shall execute and deliver to the Company the Consent of Spouse set forth on the signature page hereto. Upon vesting of the Unvested Shares, the escrow agent shall promptly deliver to Purchaser the certificate or certificates representing such Shares in the escrow agent's possession belonging to Purchaser, and the escrow agent shall be discharged of all further obligations hereunder; *provided, however,* that the escrow agent shall nevertheless retain such certificate or certificates as escrow agent if so required pursuant to other restrictions imposed pursuant to this Agreement. If the Shares are held in book entry form, then such entry will reflect that the Shares are subject to the restrictions of this Agreement.

(c) The Company, or its designee, shall not be liable for any act it may do or omit to do with respect to holding the Shares in escrow and while acting in good faith and in the exercise of its judgment.

(d) Transfer or sale of the Shares is subject to restrictions on transfer imposed by any applicable state and federal securities laws. Any transferee shall hold such Shares subject to all the provisions hereof and the Exercise Notice executed by Purchaser with respect to any Unvested Shares purchased by Purchaser and shall acknowledge the same by signing a copy of this Agreement. Any transfer or attempted transfer of any of the Shares not in accordance with the terms of this Agreement shall be void and the Company may enforce the terms of this Agreement by stop transfer instructions or similar actions by the Company and its agents or designees.

3. Ownership, Voting Rights, Duties. This Agreement shall not affect in any way the ownership, voting rights or other rights or duties of Purchaser, except as specifically provided herein.

4. Legends. Any share certificate evidencing the Shares issued hereunder shall be endorsed with the following legend (in addition to any legend required under applicable securities laws):

> THE SHARES REPRESENTED BY THIS CERTIFICATE MAY BE TRANSFERRED ONLY IN ACCORDANCE WITH THE TERMS OF AN AGREEMENT BETWEEN THE COMPANY AND THE STOCKHOLDER, A COPY OF WHICH IS ON FILE WITH THE SECRETARY OF THE COMPANY.

5. Adjustment for Stock Split. In the event of any stock dividend, stock split, reverse stock split, recapitalization, combination, reclassification, or similar change in the capital structure of the Company, the Administrator shall make appropriate and equitable adjustments in the Unreleased Shares subject to the Repurchase Option and the number of Shares, consistent with any adjustment under Section 11.1 of the Plan. The provisions of this Agreement shall apply, to the full extent set forth herein with respect to the Shares, to any and all shares of capital stock or other securities or other property or cash which may be issued in respect of, in exchange for, or in substitution of the Shares, and shall be appropriately adjusted for any stock dividends, splits, reverse splits, combinations, recapitalizations and the like occurring after the date hereof.

6. Notices. Notices required hereunder shall be given in person or by registered mail to the address of Purchaser shown on the records of the Company, and to the Company at its principal executive office.

7. Survival of Terms. This Agreement shall apply to and bind Purchaser and the Company and their respective permitted assignees and transferees, heirs, legatees, executors, administrators and legal successors.

8. Section 83(b) Elections.

(a) Election for Unvested Shares Purchased Pursuant to a Non-Qualified Stock Option. Purchaser hereby acknowledges that he or she has been informed that, with respect to the exercise of a Non-Qualified Stock Option for Unvested Shares, that unless an election is filed by Purchaser with the Internal Revenue Service and, if necessary, the proper state taxing authorities, within thirty days of the purchase of the Shares, electing pursuant to Section 83(b) of the Code (and similar state tax provisions if applicable) to be taxed currently on any difference between the purchase price of the Shares and their Fair Market Value on the date of purchase, there will be a recognition of taxable income to the Purchaser, measured by the excess, if any, of the fair market value of the Shares, at the time the Company's Repurchase Option lapses over the purchase price for the Shares. Purchaser represents that Purchaser has consulted any tax consultant(s) Purchaser deems advisable in connection with the purchase of the Shares or the filing of the election under Section 83(b) and similar tax provisions.

(b) Election for Unvested Shares Purchased Pursuant to an Incentive Stock Option. Purchaser hereby acknowledges that he or she has been informed that, with respect to the exercise of an Incentive Stock Option for Unvested Shares, that unless an election is filed by Purchaser with the Internal Revenue Service and, if necessary, the proper state taxing authorities, within thirty days of the purchase of the Shares, electing pursuant to Section 83(b) of the Code (and similar state tax provisions if applicable) to be taxed currently on any difference between the purchase price of the Shares and their Fair Market Value on the date of purchase, there will be a recognition of income to the Purchaser, for alternative minimum tax purposes measured by the excess, if any, of the fair market value of the Shares at the time the Company's Repurchase Option lapses over the purchase price for the Shares. Purchaser further acknowledges that if an election is filed under Section 83(b) of the Code for the Unvested Shares and such shares are sold or transferred prior to the date two years following the Grant Date and one year following the purchase date of such shares, there will be a recognition of income to the Purchaser, for ordinary income, measured by the excess, if any, of the fair market value of the Shares at the time the Company's Repurchase Option lapses over the purchase price for the Shares. Purchaser represents that Purchaser has consulted any tax consultant(s) Purchaser deems advisable in connection with the purchase of the Shares or the filing of the election under Section 83(b) and similar tax provisions.

PURCHASER ACKNOWLEDGES THAT IT IS PURCHASER'S SOLE RESPONSIBILITY AND NOT THE COMPANY'S TO FILE TIMELY THE ELECTION UNDER SECTION 83(b), EVEN IF PURCHASER REQUESTS THE COMPANY OR ITS REPRESENTATIVE TO MAKE THIS FILING ON PURCHASER'S BEHALF.

9. Representations. Purchaser has reviewed with his or her own tax advisors the federal, state, local and foreign tax consequences of this investment and the transactions contemplated by this Agreement. Purchaser is relying solely on such advisors and not on any statements or representations of the Company or any of its agents. Purchaser understands that Purchaser (and not the Company) shall be responsible for his or her own tax liability that may arise as a result of this investment or the transactions contemplated by this Agreement.

10. Governing Law; Severability. This Agreement shall be governed by and construed in accordance with the laws of the State of California excluding that body of law pertaining to conflicts of law. Should any provision of this Agreement be determined by a court of law to be illegal or unenforceable, the other provisions shall nevertheless remain effective and shall remain enforceable.

Purchaser represents that he or she has read this Agreement and is familiar with its terms and provisions. Purchaser hereby agrees to accept as binding, conclusive and final all decisions or interpretations of the Administrator upon any questions arising under this Agreement.

(Signature Page Follows)

IN WITNESS WHEREOF, this Agreement is deemed made as of the date first set forth above.

ULTRAGENYX PHARMACEUTICAL, INC.

By: _____

Title: _____

PURCHASER

By: _____

Title: _____

Address:

_____

_____

CONSENT OF SPOUSE

I, _____, spouse of _____, have read and approve this Agreement between my spouse and Ultragenyx Pharmaceutical, Inc. In consideration of granting of the right to my spouse to purchase shares of Ultragenyx Pharmaceutical, Inc. set forth in the Agreement, I hereby appoint my spouse as my attorney-in-fact in respect to the exercise of any rights under the Agreement and agree to be bound by the provisions of the Plan, the Option Agreement and this Agreement insofar as I may have any rights in said agreements or any shares issued pursuant thereto under the community property laws or similar laws relating to marital property in effect in the state of our residence as of the date of the signing of the foregoing Agreement.

_____

Signature of Spouse

EXHIBIT C-1

TO RESTRICTED STOCK PURCHASE AGREEMENT

ASSIGNMENT SEPARATE FROM CERTIFICATE

FOR VALUE RECEIVED, the undersigned, _____, hereby sells, assigns and transfers unto Ultragenyx Pharmaceutical, Inc., a California corporation (the "***Company***"), _____ shares of the common stock of the Company standing in its name of the books of said corporation represented by Certificate No. _____ herewith and do hereby irrevocably constitute and appoint to _____ transfer the said stock on the books of the within named corporation with full power of substitution in the premises.

This Stock Assignment may be used only in accordance with the Restricted Stock Award Agreement between the Company and the undersigned dated , _____. _____

Dated: _____, _____

_____

Print: _____

Name:

INSTRUCTIONS: Please do not fill in the blanks other than the signature line. The purpose of this assignment is to enable the Company to exercise its "Repurchase Option," as set forth in the Restricted Stock Award Agreement, without requiring additional signatures on the part of Purchaser.

EXHIBIT C-2

TO RESTRICTED STOCK PURCHASE AGREEMENT

JOINT ESCROW INSTRUCTIONS

_____, ____

Secretary
Ultragenyx Pharmaceutical, Inc.
77 Digital Drive, Suite 210
Novato, CA 94949

As Escrow Agent for both Ultragenyx Pharmaceutical, Inc. (the "***Company***") and the undersigned purchaser of stock of the Company (the "***Purchaser***"), you are hereby authorized and directed to hold the documents delivered to you pursuant to the terms of that certain Restricted Stock Purchase Agreement ("***Agreement***") between the Company and the undersigned, in accordance with the following instructions:

1. In the event the Company and/or any assignee of the Company (referred to collectively for convenience herein as the "***Company***") exercises the Company's Repurchase Option set forth in the Agreement, the Company shall give to Purchaser and you a written notice specifying the number of shares of stock to be purchased, the purchase price, and the time for a closing hereunder at the principal office of the Company. Purchaser and the Company hereby irrevocably authorize and direct you to close the transaction contemplated by such notice in accordance with the terms of said notice.

2. At the closing, you are directed (a) to date the stock assignments necessary for the transfer in question, (b) to fill in the number of shares being transferred, and (c) to deliver the same, together with the certificate evidencing the shares of stock to be transferred, to the Company or its assignee, against the simultaneous delivery to you of the purchase price (by cash, a check, or a combination thereof) for the number of shares of stock being purchased pursuant to the exercise of the Company's Repurchase Option.

3. Purchaser irrevocably authorizes the Company to deposit with you any certificates evidencing shares of stock to be held by you hereunder and any additions and substitutions to said shares as defined in the Agreement. Purchaser does hereby irrevocably constitute and appoint you as Purchaser's attorney-in-fact and agent for the term of this escrow to execute, with respect to such securities, all documents necessary or appropriate to make such securities negotiable and to complete any transaction herein contemplated, including but not limited to the filing with any applicable state blue sky authority of any required applications for consent to, or notice of transfer of, the securities. Subject to the provisions of this paragraph 3, Purchaser shall exercise all rights and privileges of a stockholder of the Company while the stock is held by you.

4. Upon written request of Purchaser, but no more than once per calendar year, unless the Company's Repurchase Option has been exercised, you will deliver to Purchaser a certificate or certificates representing the number of shares of stock as are not then subject to the Company's Repurchase Option. Within one hundred twenty days after the date of the Purchaser's Termination of Service, you will deliver to Purchaser a certificate or certificates representing the aggregate number of shares held or issued pursuant to the Agreement and not purchased by the Company or its assignees pursuant to exercise of the Company's Repurchase Option.

5. If at the time of termination of this escrow you should have in your possession any documents, securities, or other property belonging to Purchaser, you shall deliver all of the same to Purchaser and shall be discharged of all further obligations hereunder.

6. Your duties hereunder may be altered, amended, modified or revoked only by a writing signed by all of the parties hereto.

7. You shall be obligated only for the performance of such duties as are specifically set forth herein and may rely and shall be protected in relying or refraining from acting on any instrument reasonably believed by you to be genuine and to have been signed or presented by the proper party or parties. You shall not be personally liable for any act you may do or omit to do hereunder as Escrow Agent or as attorney-in-fact for Purchaser while acting in good faith, and any act done or omitted by you pursuant to the advice of your own attorneys shall be conclusive evidence of such good faith.

8. You are hereby expressly authorized to disregard any and all warnings given by any of the parties hereto or by any other person or corporation, excepting only orders or process of courts of law and are hereby expressly authorized to comply with and obey orders, judgments or decrees of any court. In case you obey or comply with any such order, judgment or decree, you shall not be liable to any of the parties hereto or to any other person, firm or corporation by reason of such compliance, notwithstanding any such order, judgment or decree being subsequently reversed, modified, annulled, set aside, vacated or found to have been entered without jurisdiction.

9. You shall not be liable in any respect on account of the identity, authorities or rights of the parties executing or delivering or purporting to execute or deliver the Agreement or any documents or papers deposited or called for hereunder.

10. You shall not be liable for the expiration of any rights under any applicable state, federal or local statute of limitations or similar statute or regulation with respect to these Joint Escrow Instructions or any documents deposited with you.

11. You shall be entitled to employ such legal counsel and other experts as you may deem necessary properly to advise you in connection with your obligations hereunder, may rely upon the advice of such counsel, and may pay such counsel reasonable compensation therefore.

12. Your responsibilities as Escrow Agent hereunder shall terminate if you shall cease to be an officer or agent of the Company or if you shall resign by written notice to each party. In the event of any such termination, the Company shall appoint a successor Escrow Agent.

13. If you reasonably require other or further instruments in connection with these Joint Escrow Instructions or obligations in respect hereto, the necessary parties hereto shall join in furnishing such instruments.

14. It is understood and agreed that should any dispute arise with respect to the delivery and/or ownership or right of possession of the securities held by you hereunder, you are authorized and directed to retain in your possession without liability to anyone all or any part of said securities until such disputes shall have been settled either by mutual written agreement of the parties concerned or by a final order, decree or judgment of a court of competent jurisdiction after the time for appeal has expired and no appeal has been perfected, but you shall be under no duty whatsoever to institute or defend any such proceedings.

15. Any notice required or permitted hereunder shall be given in writing and shall be deemed effectively given upon personal delivery or upon deposit in the United States Post Office, by registered or certified mail with postage and fees prepaid, addressed to each of the other parties thereunto entitled at such addresses as a party may designate by written notice to each of the other parties hereto.

16. By signing these Joint Escrow Instructions, you become a party hereto only for the purpose of said Joint Escrow Instructions; you do not become a party to the Agreement.

17. This instrument shall be binding upon and inure to the benefit of the parties hereto, and their respective successors and permitted assigns.

18. These Joint Escrow Instructions shall be governed by, and construed and enforced in accordance with, the laws of the State of California, excluding that body of law pertaining to conflicts of law.

(Signature Page Follows)

IN WITNESS WHEREOF, these Joint Escrow Instructions shall be effective as of the date first set forth above.

Very truly yours,

ULTRAGENYX PHARMACEUTICAL INC.

By: _____
Name:
Title:

Address:

PARTICIPANT

_____

Print _____

Name: _____

Address: _____

_____

ESCROW AGENT:

By: _____

Address:

Exhibit 10.12

ULTRAGENYX PHARMACEUTICAL INC.

AMENDMENT TO THE

2011 EQUITY INCENTIVE PLAN

This AMENDMENT (this "***Amendment***") to the 2011 Equity Incentive Plan (the "***Plan***"), of Ultragenyx Pharmaceutical Inc. (the "***Company***"), is being adopted by the Board of Directors, by unanimous written consent in lieu of a special meeting dated as of December 14, 2012, and by the stockholders of the Company, by written consent dated as of December 14, 2012, such amendment to be effective contingent upon the closing of the sale and issuance of shares of Series B Preferred Stock pursuant to that certain Series B Preferred Stock Purchase Agreement, dated as of December 18, 2012, by and between the Company and the investors named therein. The Plan is hereby amended as follows:

1. Section 3.1(a) of the Plan is hereby amended in its entirety as follows: "Subject to Article 11, the aggregate number of shares of Stock which may be issued or transferred pursuant to Awards under the Plan shall be 10,469,827 Shares."

Except to the extent amended hereby, all of the terms, provisions and conditions set forth in the Plan are hereby ratified and confirmed and shall remain in full force and effect. The Plan and this Amendment shall be read and construed together as a single instrument.

[*End of document*]

EXECUTIVE EMPLOYMENT AGREEMENT

This Executive Employment Agreement (the "**Agreement**"), dated June 15, 2011, is between ULTRAGENYX PHARMACEUTICAL INC. (the "**Company**") and EMIL D. KAKKIS, M.D., PH.D. ("**Executive**").

I. POSITION AND RESPONSIBILITIES

A. Position. Executive is employed by the Company to render services to the Company in the position of President and Chief Executive Officer. Executive shall perform such duties and responsibilities as are normally related to such position in accordance with the standards of the industry and any additional duties now or hereafter assigned to Executive by the Board of Directors of the Company (the "**Board**"). Executive shall abide by the rules, regulations, and practices as adopted or modified from time to time in the Company's sole discretion. Executive shall report to the Board.

B. Other Activities. Except upon the prior written consent of the Company duly authorized by the Board, Executive will not, during the term of this Agreement, (i) accept any other employment, or (ii) engage, directly or indirectly, in any other business activity (whether or not pursued for pecuniary advantage). Executive will devote his best efforts and substantially all of his business time and attention to the business of the Company. Notwithstanding the foregoing, the Company agrees that Executive may continue his present involvement with the not-for-profit activities of the Kakkis Everylife Foundation, provided that (a) such activities do not interfere with the performance of Executive's duties and responsibilities to the Company or create a conflict of interest with the Company, and (b) Executive will not utilize any Company employees or personnel, Company premises or any Company funds or resources for such activities.

C. No Conflict. Executive represents and warrants that Executive's execution of this Agreement, employment with the Company, and the performance of Executive's proposed duties under this Agreement shall not violate any obligations Executive may have to any other employer, person or entity, including any obligations with respect to proprietary or confidential information of any other person or entity.

II. COMPENSATION AND BENEFITS

A. Base Salary. In consideration of the services to be rendered under this Agreement, the Company shall pay Executive a salary at the rate of Three Hundred Thousand Dollars ($300,000) per year ("**Base Salary**"). The Base Salary shall be paid in accordance with the Company's regularly established payroll practice. Executive's Base Salary will be reviewed from time to time in accordance with the established procedures of the Company for adjusting salaries for similarly situated employees and may be adjusted in the sole discretion of the Board or the compensation committee thereof.

B. Stock Compensation. Executive may also be eligible to receive grants of options to purchase shares of common stock of the Company or other equity compensation in accordance with the terms of the Company's 2011 Stock Compensation Plan (the "**Stock Compensation Plan**"). Any such grants shall be in the sole discretion of the Company's Board of Directors or

the compensation committee thereof. Executive's entitlement to any receive any options or other equity compensation that may be approved is conditioned upon Executive's signing of the appropriate stock compensation agreement and is subject to its terms and the terms of the Stock Compensation Plan under which the options or other equity compensation are granted, including vesting requirements.

C. Benefits. Executive shall be eligible to participate in the benefits made generally available by the Company to similarly-situated executives, in accordance with the benefit plans established by the Company, and as may be amended from time to time in the Company's sole discretion.

D. Expenses. The Company shall reimburse Executive for reasonable business expenses incurred in the performance of Executive's duties hereunder in accordance with the Company's expense reimbursement guidelines.

III. AT-WILL EMPLOYMENT; TERMINATION BY COMPANY

A. At-Will Termination by Company. Executive's employment with the Company shall be "at-will" at all times. The Company, by due authorization of the Board, may terminate Executive's employment with the Company at any time, without any advance notice, for any reason or no reason at all, notwithstanding anything to the contrary contained in or arising from any statements, policies or practices of the Company relating to the employment, discipline or termination of its employees. Upon and after such termination, all obligations of the Company under this Agreement shall cease, except as otherwise provided herein.

B. Severance. Except in situations where the employment of Executive is terminated For Cause, By Death or By Disability (as defined in Section IV below), in the event that the Company terminates Executive's employment at any time, Executive will be eligible to receive an amount equal to six (6) months of Executive's then-current Base Salary, payable in the form of salary continuation ("**Severance**"). Such Severance shall be reduced by any remuneration paid to Executive because of Executive's employment or self-employment during the severance period, and Executive shall promptly report all such remuneration to the Company in writing. Executive's eligibility for the foregoing Severance is conditioned on Executive having first signed a release agreement in the form attached as Exhibit A and such release becoming effective. Executive shall not be entitled to any Severance if Executive's employment is terminated For Cause, By Death or By Disability (as described in Section IV below) or if Executive's employment is terminated by Executive.

IV. OTHER TERMINATIONS BY COMPANY

A. Termination for Cause. For purposes of this Agreement, "**For Cause**" shall mean: (i) Executive commits a felony or any crime involving dishonesty, breach of trust, or physical harm to any person; (ii) Executive willfully engages in conduct that is in bad faith and materially injurious to the Company, including but not limited to, misappropriation of trade secrets, fraud or embezzlement; (iii) Executive commits a material breach of this Agreement, which breach is not cured within ten (10) days after written notice to Executive from the Company; or (iv) Executive willfully refuses to implement or follow a lawful policy or directive

of the Company, which breach is not cured within ten (10) days after written notice to Executive from the Company. The Company may terminate Executive's employment For Cause at any time, without any advance notice. The Company shall pay to Executive all compensation to which Executive is entitled up through the date of termination, subject to any other rights or remedies of the Company under law; and thereafter all obligations of the Company under this Agreement shall cease.

B. By Death. Executive's employment shall terminate automatically upon Executive's death (such termination, "**By Death**"). The Company shall pay to Executive's beneficiaries or estate, as appropriate, any compensation then due and owing. Thereafter all obligations of the Company under this Agreement shall cease. Nothing in this Section shall affect any entitlement of Executive's heirs or devisees to the benefits of any life insurance plan or other applicable benefits.

C. By Disability. If Executive becomes eligible for the Company's long term disability benefits or if, in the sole opinion of the Company, Executive is unable to carry out the responsibilities and functions of the position held by Executive by reason of any physical or mental impairment for more than ninety consecutive days or more than one hundred and twenty days in any twelve-month period, then, to the extent permitted by law, the Company may terminate Executive's employment (such termination, "**By Disability**"). The Company shall pay to Executive all compensation to which Executive is entitled up through the date of termination, and thereafter all obligations of the Company under this Agreement shall cease. Nothing in this Section shall affect Executive's rights under any disability plan in which Executive is a participant.

V. TERMINATION BY EXECUTIVE

A. At-Will Termination by Executive. Executive may terminate employment with the Company at any time for any reason or no reason at all, upon four (4) weeks' advance written notice. During such notice period Executive shall continue to diligently perform all of Executive's duties hereunder. The Company shall have the option, in its sole discretion, to make Executive's termination effective at any time prior to the end of such notice period as long as the Company pays Executive all compensation to which Executive is entitled up through the last day of employment. Thereafter all obligations of the Company shall cease.

B. Termination for Good Reason After Change of Control. Executive's termination shall be for "**Good Reason**" if Executive provides written notice to the Company of the Good Reason within six months of the event constituting Good Reason and provides the Company with a period of twenty days to cure the Good Reason and the Company fails to cure the Good Reason within that period. For purposes of this Agreement, "Good Reason" shall mean any of the following events if (i) the event is effected by the Company without the consent of Executive and (ii) such event occurs after a Change in Control (as hereinafter defined): (A) a change in Executive's position with Employer which materially reduces Executive's level of responsibility; (B) a material reduction in Executive's Base Salary, except for reductions that are comparable to reductions generally applicable to similarly situated executives of the Company; or (C) a relocation of Executive's principal place of employment by more than fifty miles. In such event Executive may terminate his employment for Good Reason, in which case Executive

will be eligible to receive an amount equal to twelve (12) months of Executive's then-current Base Salary payable in the form of salary continuation. Executive's eligibility for severance is conditioned on Executive having first signed a release agreement in the form attached as Exhibit A. Such Severance shall be reduced by any remuneration paid to Executive because of Executive's employment or self-employment during the severance period, and Executive shall promptly report all such remuneration to the Company or its successor in writing. Thereafter all obligations of the Company or its successor under this Agreement shall cease.

C. "Change of Control." For purposes of this Agreement, "**Change of Control**" shall mean a change in ownership or control of the Company effected through a merger, consolidation or acquisition by any person or related group of persons (other than an acquisition by the Company or by a Company-sponsored employee benefit plan or by a person or persons that directly or indirectly controls, is controlled by, or is under common control with, the Company) of beneficial ownership (within the meaning of Rule 13d-3 of the Securities Exchange Act of 1934) of securities possessing more than fifty percent of the total combined voting power of the outstanding securities of the Company.

VI. TERMINATION OBLIGATIONS

A. Return of Property. Executive agrees that all property (including without limitation all equipment, tangible proprietary information, documents, records, notes, contracts and computer-generated materials) furnished to or created or prepared by Executive incident to Executive's employment belongs to the Company and shall be promptly returned to the Company upon termination of Executive's employment.

B. Resignation and Cooperation. Upon termination of Executive's employment, Executive shall be deemed to have resigned from all offices and directorships then held with the Company. Following any termination of employment, Executive shall cooperate with the Company in the winding up of pending work on behalf of the Company and the orderly transfer of work to other employees. Executive shall also cooperate with the Company in the defense of any action brought by any third party against the Company that relates to Executive's employment by the Company.

VII. COMPANY REPURCHASE OPTION UPON TERMINATION OF EMPLOYMENT

Executive and his wife shall sign and be bound by the Founder's Stock Repurchase Agreement, which is attached as Exhibit C.

VIII. INVENTIONS AND PROPRIETARY INFORMATION; PROHIBITION ON THIRD PARTY INFORMATION

A. Proprietary Information Agreement. Executive agrees to sign and be bound by the terms of the Company's Confidential Information and Inventions Assignment Agreement, which is attached as Exhibit B ("**Proprietary Information Agreement**").

IX. AMENDMENTS; WAIVERS; REMEDIES

This Agreement may not be amended or waived except by a writing signed by Executive and by a duly Board-authorized representative of the Company other than Executive. Failure to exercise any right under this Agreement shall not constitute a waiver of such right. Any waiver of any breach of this Agreement shall not operate as a waiver of any subsequent breaches. All rights or remedies specified for a party herein shall be cumulative and in addition to all other rights and remedies of the party hereunder or under applicable law.

X. ASSIGNMENT; BINDING EFFECT

A. Assignment. The performance of Executive is personal hereunder, and Executive agrees that Executive shall have no right to assign and shall not assign or purport to assign any rights or obligations under this Agreement. This Agreement may be assigned or transferred by the Company; and nothing in this Agreement shall prevent the consolidation, merger or sale of the Company or a sale of any or all or substantially all of its assets.

B. Binding Effect. Subject to the foregoing restriction on assignment by Executive, this Agreement shall inure to the benefit of and be binding upon each of the parties; the affiliates, officers, directors, agents, successors and assigns of the Company; and the heirs, devisees, spouses, legal representatives and successors of Executive.

XI. NOTICES

All notices or other communications required or permitted hereunder shall be made in writing and shall be deemed to have been duly given if delivered: (a) by hand; (b) by a nationally recognized overnight courier service; or (c) by United States first class registered or certified mail, return receipt requested, to the principal address of the other party, as set forth below. The date of notice shall be deemed to be the earlier of (i) actual receipt of notice by any permitted means, or (ii) five business days following dispatch by overnight delivery service or the United States Mail. Executive shall be obligated to notify the Company in writing of any change in Executive's address. Notice of change of address shall be effective only when done in accordance with this paragraph.

Company's Notice Address:

Ultragenyx Pharmaceuticals Inc.
77 Digital Drive, Suite 210
Novato, CA 94949
Attn: Legal

Executive's Notice Address:

XII. SEVERABILITY

If any provision of this Agreement shall be held by a court to be invalid, unenforceable, or void, such provision shall be enforced to the fullest extent permitted by law, and the remainder of this Agreement shall remain in full force and effect. In the event that the time period or scope of any provision is declared by a court of competent jurisdiction to exceed the maximum time period or scope that such court deems enforceable, then such court shall reduce the time period or scope to the maximum time period or scope permitted by law.

XIII. TAXES

All amounts paid under this Agreement shall be paid less all applicable state and federal tax withholdings (if any) and any other withholdings required by any applicable jurisdiction or authorized by Executive. Notwithstanding any other provision of this Agreement whatsoever, the Company, in its sole discretion, shall have the right to provide for the application and effects of Section 409A of the Code (relating to deferred compensation arrangements) and any related administrative guidance issued by the Internal Revenue Service. The Company shall have the authority to delay the payment of any amounts under this Agreement to the extent it deems necessary or appropriate to comply with Section 409A(a)(2)(B)(i) of the Code (relating to payments made to certain "key employees" of publicly-traded companies); in such event, the payment(s) at issue may not be made before the date which is six (6) months after the date of Executive's separation from service, or, if earlier, the date of death.

XIV. GOVERNING LAW

This Agreement shall be governed by and construed in accordance with the laws of the State of California.

XV. INTERPRETATION

This Agreement shall be construed as a whole, according to its fair meaning, and not in favor of or against any party. Sections and section headings contained in this Agreement are for reference purposes only, and shall not affect in any manner the meaning or interpretation of this Agreement. Whenever the context requires, references to the singular shall include the plural and the plural the singular.

XVI. OBLIGATIONS SURVIVE TERMINATION OF EMPLOYMENT

Executive agrees that any and all of Executive's obligations under this agreement, including but not limited to Exhibits B, shall survive the termination of employment and the termination of this Agreement.

XVII. COUNTERPARTS

This Agreement may be executed in any number of counterparts, each of which shall be deemed an original of this Agreement, but all of which together shall constitute one and the same instrument.

XVIII. AUTHORITY

Each party represents and warrants that such party has the right, power and authority to enter into and execute this Agreement and to perform and discharge all of the obligations hereunder; and that this Agreement constitutes the valid and legally binding agreement and obligation of such party and is enforceable in accordance with its terms.

XIX. ENTIRE AGREEMENT

This Agreement is intended to be the final, complete, and exclusive statement of the terms of Executive's employment by the Company and may not be contradicted by evidence of any prior or contemporaneous statements or agreements, except for agreements specifically referenced herein (including the Confidentiality and Inventions Assignment Agreement attached as Exhibit B). To the extent that the practices, policies or procedures of the Company, now or in the future, apply to Executive and are inconsistent with the terms of this Agreement, the provisions of this Agreement shall control. Any subsequent change in Executive's duties, position, or compensation will not affect the validity or scope of this Agreement.

XX. EXECUTIVE ACKNOWLEDGEMENT

EXECUTIVE ACKNOWLEDGES EXECUTIVE HAS HAD THE OPPORTUNITY TO CONSULT LEGAL COUNSEL CONCERNING THIS AGREEMENT, THAT EXECUTIVE HAS READ AND UNDERSTANDS THE AGREEMENT, THAT EXECUTIVE IS FULLY AWARE OF ITS LEGAL EFFECT, AND THAT EXECUTIVE HAS ENTERED INTO IT FREELY BASED ON EXECUTIVE'S OWN JUDGMENT AND NOT ON ANY REPRESENTATIONS OR PROMISES OTHER THAN THOSE CONTAINED IN THIS AGREEMENT.

IN WITNESS WHEREOF, the parties have duly executed this Agreement as of the date first written above.

UGENYX PHARMACEUTICAL INC.	EMIL D. KAKKIS, M.D., PH.D.
/s/ Emil Kakkis	/s/ Emil Kakkis
Signature	Signature
Title CEO	
Date 6/14/2011	Date 6/14/2011

Exhibit A

General Release of Claims

EMIL D. KAKKIS ("You") and ULTRAGENYX PHARMACEUTICAL INC. (the "Company") have agreed to enter into this General Release of Claims ("Release") on the following terms:

In exchange for the severance benefits set forth in your Executive Employment Agreement dated April _, 2011 (the "Agreement"), you and your representatives completely release the Company, its affiliated, related, parent or subsidiary corporations, and its and their present and former directors, officers, and employees (the "Released Parties") from all claims of any kind, known and unknown, which you may now have or have ever had against any of them, or arising out of your relationship with any of them, including all claims arising from your employment or the termination of your employment, whether based on contract, tort, statute, local ordinance, regulation or any comparable law in any jurisdiction ("Released Claims"). By way of example and not in limitation, the Released Claims shall include any claims arising under Title VII of the Civil Rights Act of 1964, the Americans with Disabilities Act, the Worker Adjustment and Retraining Notification Act, the Age Discrimination in Employment Act, and the California Fair Employment and Housing Act, or any other comparable state or local law, as well as any claims asserting wrongful termination, breach of contract, breach of the covenant of good faith and fair dealing, negligent or intentional misrepresentation, and defamation and any claims for attorneys' fees. The parties intend for this release to be enforced to the fullest extent permitted by law. You understand that you are not waiving any right or claim that cannot be waived as a matter of law, such as workers' compensation or unemployment insurance benefits.

You further agree that because this Release Certificate specifically covers known and unknown claims, you waive your rights under Section 1542 of the California Civil Code or under any other comparable law of another jurisdiction that limits a general release to claims that are known to exist at the date of this release. Section 1542 of the California Civil Code states as follows: "A general release does not extend to claims which the creditor does not know or suspect to exist in his favor at the time of executing the release, which if known by him must have materially affected his settlement with the debtor."

You agree not to file or initiate any lawsuit concerning the Released Claims. You understand that this paragraph does not prevent you from filing a charge with or participating in an investigation by a governmental administrative agency; provided, however, that you hereby waive any right to receive any monetary award resulting from such a charge or investigation.

You acknowledge that the release of claims under the Age Discrimination in Employment Act ("ADEA") is subject to special waiver protection. Therefore, you acknowledge the following: (a) you have had 21 days to consider this Release (but may sign it at any time beforehand if you so desire); (b) you can consult an attorney in doing so; (c) you can revoke this Release within seven (7) days of signing it by sending a certified letter to that effect to [**name and address**]; and that (d) this Release shall not become effective or enforceable and no severance benefits shall be provided until the 7-day revocation period has expired.

The parties agree that this Release and the Agreement contain all of their agreements and understandings with respect to their subject matter, and may not be contradicted by evidence of any prior or contemporaneous agreement, except to the extent that the provisions of any such agreement have been expressly referred to in this Release or the Agreement as having continued effect. It is agreed that this Release shall be governed by the laws of the State of California. If any provision of this Release or its application to any person, place, or circumstance is held by a court of competent jurisdiction to be invalid, unenforceable, or void, the remainder of this Release and such provision as applied to other person, places, and circumstances will remain in full force and effect.

Please note that this Release may not be signed before the last day of your employment with the Company, and that your eligibility for benefits is conditioned upon meeting the terms set forth in the Agreement.

EMIL D. KAKKIS	[Name of Company Signatory] ULTRAGENYX PHARMACEUTICAL INC.
Date: _____	Date: _____

Exhibit B

Confidential Information and Invention Assignment Agreement

ULTRAGENYX PHARMACEUTICAL INC.

CONFIDENTIAL INFORMATION AND INVENTION ASSIGNMENT AGREEMENT

Employee Name: Emil Kakkis

Effective Date: June 15, 2011

As a condition of my employment being continued by Ultragenyx Pharmaceutical Inc., a Delaware corporation, or any of its current or future subsidiaries, affiliates, successors or assigns, (collectively, "Ultragenyx") and in consideration of my employment with Ultragenyx and my receipt of the compensation now and hereafter paid to me by Ultragenyx.

1. **Relationship.**

This Agreement will apply to my employment relationship with Ultragenyx and any of its predecessors in interest, including without limitation Ultragenyx Pharmaceutical, Inc., a California corporation (collectively, the "Company"). If that relationship ends and the Company, within a year thereafter, either re-employs me or engages me as a consultant, I agree that this Agreement will also apply to such later employment or consulting relationship, unless the Company and I otherwise agree in writing. Any such employment or consulting relationship between the Company and me, whether commenced prior to, upon or after the date of this Agreement, is referred to herein as the "**Relationship**."

2. **Confidential Information**.

(a) **Protection of Information.** I agree, at all times during the term of the Relationship and thereafter, to hold in strictest confidence, and not to use, except for the benefit of the Company to the extent necessary to perform my obligations to the Company under the Relationship, and not to disclose to any person, firm, corporation or other entity, without written authorization from the Company in each instance, any Confidential Information (as defined below) that I obtain, access or create during the term of the Relationship, whether or not during working hours, until such Confidential Information becomes publicly and widely known and made generally available through no wrongful act of mine or of others who were under confidentiality obligations as to the item or items involved.

(b) **Confidential Information.** I understand that "Confidential Information" means (i) all information, irrespective of form, and all physical materials concerning the Company's business, technology, business relationships or financial affairs not generally known or available outside the Company and (ii) all information, irrespective of form, and all physical material entrusted to the Company in confidence by third parties. By way of illustration, and not limitation, Confidential Information includes, without limitation: (i) Company Inventions (as defined below); (ii) Company's databases, all data and information incorporated in such databases and all schema for organizing such database; (iii) operational and technological information, including plans, specifications, manuals, forms, templates, designs, methods,

procedures, formulas, discoveries, improvements, concepts and ideas, technical data, trade secrets, know-how, research, product or service ideas or plans, software (including, without limitation, software codes and designs), developments, inventions, laboratory notebooks, processes, formulas, techniques, biological materials, mask works, engineering designs and drawings, hardware configuration information, (iv) marketing information, including strategies, methods, customer identities or other information about customers, prospect identities or other information about prospects, or market analyses or projections, lists of, or information relating to, suppliers and customers (including, but not limited to, customers of the Company on whom I called or with whom I became acquainted during the Relationship), price lists, pricing methodologies, cost data, market share data, marketing plans, licenses, contract information, (v) business plans, financial forecasts, historical financial data, budgets or other business information, financial information, including cost and performance data, debt arrangements, equity structure, investors and holdings, purchasing and sales data and price lists (vi) corporate information, including plans, strategies, methods, policies, resolutions, negotiations or litigation; (vii) personnel information, including personnel lists, reporting or organizational structure, resumes, personnel data, compensation structure, performance evaluations and termination arrangements or documents, in each of the foregoing cases as disclosed and/or otherwise made available to me by the Company either directly or indirectly, whether in writing, electronically, orally, or by observation.

(c) **Third Party Information.** My agreements in this Section 2 are intended to be for the benefit of the Company and any third party that has entrusted information or physical material to the Company in confidence.

(d) **Other Rights.** This Agreement is intended to supplement, and not to supersede, any rights the Company may have in law or equity with respect to the protection of trade secrets or confidential or proprietary information.

3. **Ownership of Inventions.**

(a) **Inventions Retained and Licensed.** I have attached hereto, as Exhibit A, a complete list describing with particularity all Inventions (as defined below) that, as of the Effective Date, belong solely to me or belong to me jointly with others, and that relate in any way to any of the Company's proposed businesses, products or research and development, and which are not assigned to the Company hereunder; or, if no such list is attached, I represent that there are no such Inventions at the time of signing this Agreement.

(b) **Use or Incorporation of Inventions.** If in the course of the Relationship, I use or incorporate into a product, process or machine any Invention not covered by Section 3(d) of this Agreement in which I have an interest, I will promptly so inform the Company. Whether or not I give such notice, I hereby irrevocably grant to the Company a nonexclusive, fully paid-up, royalty-free, assumable, perpetual, worldwide license, with right to transfer and to sublicense, to practice and exploit such Invention and to make, have made, copy, modify, make derivative works of, use, sell, import, and otherwise distribute under all applicable intellectual properties without restriction of any kind.

(c) **Inventions.** I understand that "Inventions" means discoveries, developments, concepts, designs, ideas, know how, improvements, inventions, trade secrets and/or original works of authorship, whether or not patentable, copyrightable or otherwise legally protectable. I understand this includes, but is not limited to, any new product, machine, article of manufacture, biological material, method, procedure, process, technique, use, equipment, device, apparatus, system, compound, formulation, composition of matter, design or configuration of any kind, or any improvement thereon. I understand that "Company Inventions" means any and all Inventions that I may solely or jointly author, discover, develop, conceive, or reduce to practice during the period of the Relationship, except as otherwise provided in Section 3(g) below.

(d) **Assignment of Company Inventions.** I agree that I will promptly make full written disclosure to the Company, will hold in trust for the sole right and benefit of the Company, and hereby assign to the Company, or its designee, all my right, title and interest throughout the world in and to any and all Company Inventions. I further acknowledge that all Company Inventions that are made by me (solely or jointly with others) within the scope of and during the period of the Relationship are "works made for hire" (to the greatest extent permitted by applicable law) and are compensated by my compensation (equity vesting and/or salary). I hereby waive and irrevocably quitclaim to the Company or its designee any and all claims, of any nature whatsoever, that I now have or may hereafter have for infringement of any and all Company Inventions.

(e) **Maintenance of Records.** I agree to keep and maintain adequate and current written records of all Company Inventions made by me (solely or jointly with others) during the term of the Relationship. The records may be in the form of notes, sketches, drawings, flow charts, electronic data or recordings, laboratory notebooks, or any other format. The records will be available to and remain the sole property of the Company at all times. I agree not to remove such records from the Company's place of business except as expressly permitted by Company policy which may, from time to time, be revised at the sole election of the Company for the purpose of furthering the Company's business. I agree to deliver all such records (including any copies thereof) to the Company at the time of termination of the Relationship as provided for in Sections 4 and 5.

(f) **Patent and Copyright Rights.** I agree to assist the Company, or its designee, at its expense, in every proper way to secure the Company's, or its designee's, rights in the Company Inventions and any copyrights, patents, trademarks, mask work rights, moral rights, or other intellectual property rights relating thereto in any and all countries, including the disclosure to the Company or its designee of all pertinent information and data with respect thereto, the execution of all applications, specifications, oaths, assignments, recordations, and all other instruments which the Company or its designee shall deem necessary in order to apply for, obtain, maintain and transfer such rights, or if not transferable, waive such rights, and in order to assign and convey to the Company or its designee, and any successors, assigns and nominees the sole and exclusive right, title and interest in and to such Company Inventions, and any copyrights, patents, mask work rights or other intellectual property rights relating thereto. I further agree that my obligation to execute or cause to be executed, when it is in my power to do so, any such instrument or papers shall continue during and at all times after the end of the Relationship and until the expiration of the last such intellectual property right to expire in any country of the world. I hereby irrevocably designate and appoint the Company and its duly

authorized officers and agents as my agent and attorney-in-fact, to act for and in my behalf and stead to execute and file any such applications and to do all other lawfully permitted acts to further the application for, prosecution, issuance, maintenance or transfer of letters of patents, copyright, mask work and other registrations related to such Company Inventions. This power of attorney is coupled with an interest and shall not be affected by my subsequent incapacity.

(g) **Exception to Assignments.** I understand that the Company Inventions will not include, and the provisions of this Agreement requiring assignment of inventions to the Company do not apply to, any invention which qualifies fully for exclusion under the provisions of applicable state law, if any, attached hereto as Exhibit B.

4. **Company Property; Returning Company Documents.** I acknowledge and agree that I have no expectation of privacy with respect to the Company's telecommunications, networking or information processing systems (including, without limitation, files, e-mail messages, and voice messages). The Company has no rights to access personal or private telecommunications, networking, or information processing systems (including, without limitation, files, e-mail messages, and voice messages). I further agree that any property situated on the Company's premises and owned by the Company, including disks and other storage media, filing cabinets or other work areas, is subject to inspection by Company personnel at any time with or without notice. I agree that, at the time of termination of the Relationship, I will deliver to the Company (and will not keep in my possession, recreate or deliver to anyone else) any and all devices, records, data, reports, proposals, lists, correspondence, specifications, drawings, blueprints, sketches, laboratory notebooks, materials, flow charts, equipment, other documents or property, or reproductions of any of the aforementioned items developed by me pursuant to the Relationship or otherwise belonging to the Company, its successors or assigns.

5. **Termination Certification.** In the event of the termination of the Relationship, I agree to sign and deliver the "Termination Certification" attached hereto as Exhibit C; however, my failure to sign and deliver the Termination Certification shall in no way diminish my continuing obligations under this Agreement.

6. **Solicitation of Employees, Consultants and Other Parties.** I agree that during the term of the Relationship, and for a period of twelve (12) months immediately following the termination of the Relationship for any reason, whether with or without cause, I shall not either directly or indirectly solicit, induce, recruit or encourage any of the Company's employees to terminate their relationship with the Company, or attempt to solicit, induce, recruit, encourage or take away employees of the Company, either for myself or for any other person or entity. I will also not hire Consultants of the Company if this would adversely interfere with the consultant's work for the Company. Further, during the Relationship and at any time following the termination of the Relationship for any reason, whether with or without cause, I shall not use any Confidential Information of the Company to negatively influence any of the Company's clients or customers from purchasing Company products or services or to solicit or influence or attempt to influence any client, customer or other person either directly or indirectly, to direct any purchase of products and/or services to any person, firm, corporation, institution or other entity in competition with the business of the Company.

7. **At-Will Relationship.** I understand and acknowledge that, except as may be otherwise explicitly provided in a separate written agreement between the Company and me, my Relationship with the Company is and shall continue to be at-will, as defined under applicable law, meaning that either I or the Company may terminate the Relationship at any time for any reason or no reason, without further obligation or liability, other than those provisions of this Agreement that explicitly survive the termination of the Relationship.

8. **Representations and Covenants.**

(a) **Facilitation of Agreement.** I agree to execute promptly, both during and after the end of the Relationship, any proper oath, and to verify any proper document, required to carry out the terms of this Agreement, upon the Company's written request to do so.

(b) **No Conflicts.** I represent that my performance of all the terms of this Agreement does not and will not breach any agreement I have entered into, or will enter into, with any third party, including without limitation any agreement to keep in confidence proprietary information or materials acquired by me in confidence or in trust prior to or during the Relationship. I will not disclose to the Company or use any inventions, confidential or non-public proprietary information or material belonging to any previous client, employer or any other party. I will not induce the Company to use any inventions, confidential or non-public proprietary information, or material belonging to any previous client, employer or any other party. I acknowledge and agree that I have listed on Exhibit A all agreements (e.g., non-competition agreements, non-solicitation of customers agreements, non-solicitation of employees agreements, confidentiality agreements, inventions agreements, etc.), if any, with a current or former client, employer, or any other person or entity, that may restrict my ability to accept employment with the Company or my ability to recruit or engage customers or service providers on behalf of the Company, or otherwise relate to or restrict my ability to perform my duties for the Company or any obligation I may have to the Company. I agree not to enter into any written or oral agreement that conflicts with the provisions of this Agreement.

(c) **Voluntary Execution.** I certify and acknowledge that I have carefully read all of the provisions of this Agreement, that I understand and have voluntarily accepted such provisions, and that I will fully and faithfully comply with such provisions.

9. **General Provisions.**

(a) **Governing Law.** The validity, interpretation, construction and performance of this Agreement shall be governed by the laws of the State of California, without giving effect to the principles of conflict of laws.

(b) **Entire Agreement.** This Agreement sets forth the entire agreement and understanding between the Company and me relating to this subject matter and merges all prior discussions between us. No amendment to this Agreement will be effective unless in writing signed by both parties to this Agreement. The Company shall not be deemed hereby to have waived any rights or remedies it may have in law or equity, nor to have given any authorizations or waived any of its rights under this Agreement, unless, and only to the extent, it does so by a specific writing signed by a duly authorized officer of the Company, it being understood that,

even if I am an officer of the Company, I will not have authority to give any such authorizations or waivers for the Company under this Agreement without specific approval by the Board of Directors. Any subsequent change or changes in my duties, obligations, rights or compensation will not affect the validity or scope of this Agreement.

(c) **Severability.** If one or more of the provisions in this Agreement are deemed void or unenforceable to any extent in any context, such provisions shall nevertheless be enforced to the fullest extent allowed by law in that and other contexts, and the validity and force of the remainder of this Agreement shall not be affected.

(d) **Successors and Assigns.** This Agreement will be binding upon my heirs, executors, administrators and other legal representatives, and my successors and assigns, and will be for the benefit of the Company, its successors, and its assigns.

(e) **Arbitration.** I acknowledge and agree that to the fullest extent allowed by law, and except as set forth in the last paragraph of this section (e), any controversy or claim arising out of or relating to my employment or the termination of my employment as against the Company or any of its agents or employees, and as against me, shall be finally settled by binding arbitration, employing a neutral arbitrator, and administered by the American Arbitration Association ("AAA") under its National Rules for the Resolution of Employment Disputes. Such claims shall include, but are not limited to, any claims under (as amended) Title VII of the Civil Rights Act of 1964, the Civil Rights Act of 1866, the Civil Rights Act of 1991, the Age Discrimination in Employment Act, the Rehabilitation Act of 1973, the Americans with Disabilities Act, the Family and Medical Leave Act of 1993, the Employee Retirement Income Security Act of 1974, and any other federal, state or local statute, regulation or common law doctrine, including contract or tort, regarding employment discrimination, the terms and conditions of employment or termination of employment. Prior to invoking arbitration, I am required to try and resolve the matter through direct discussion with the board of directors of the Company.

The arbitration will be conducted in the city with an AAA office nearest to where I am or was last employed. The parties are each waiving their rights to trial by jury, in exchange for arbitration. Judgment upon any award rendered in an arbitration proceeding may be entered in any court having jurisdiction of the matter. Any controversy or claim subject to arbitration by either me or the Company shall be deemed waived, and shall be forever barred, if arbitration is not initiated within two (2) years after the later of (a) the date the controversy or claim first arose or (b) the date the claim is discovered, or if statutory rights are involved, within the time limit established by the applicable statute of limitations. To the extent statutory claims are asserted, the parties will have the same statutory remedies in arbitration as to those statutory claims as they would otherwise have had if such a claim had been filed in a court of law, including, where authorized by statute, compensatory and punitive damages, injunctive relief and attorneys' fees. The Company will pay all costs of the AAA to administer the arbitration and the costs for the arbitrator. The prevailing party in the arbitration shall be entitled to recover its attorneys' fees.

In any arbitration commenced pursuant to this agreement, depositions may be taken and discovery obtained as provided in the Federal Rules of Civil Procedure, subject to limitation by the arbitrator to a reasonable amount necessary for both parties to be able to present their claims and defenses. Any award by the arbitrator(s) shall be accompanied by a statement of the factual and legal bases for the award.

This agreement to arbitrate shall not apply to claims for workers' compensation or unemployment compensation or to claims for temporary or preliminary injunctive relief arising out of or related to misappropriation of trade secrets or confidential information, unfair competition or breach of any non-competition or non-solicitation agreement between me and the Company.

(f) **ADVICE OF COUNSEL.** I ACKNOWLEDGE THAT, IN EXECUTING THIS AGREEMENT, I HAVE HAD THE OPPORTUNITY TO SEEK THE ADVICE OF INDEPENDENT LEGAL COUNSEL, AND I HAVE READ AND UNDERSTOOD ALL OF THE TERMS AND PROVISIONS OF THIS AGREEMENT. THIS AGREEMENT SHALL NOT BE CONSTRUED AGAINST ANY PARTY BY REASON OF THE DRAFTING OR PREPARATION HEREOF.

[SIGNATURE PAGE FOLLOWS]

The parties have executed this Agreement on the respective dates set forth below, to be effective as of the Effective Date first above written.

COMPANY:

Ultragenyx Pharmaceutical, Inc.

/s/ Emil Kakkis

Name: Emil D. Kakkis, M.D., Ph.D.
Title: CEO & President
Date: June 15, 2011

Address:

EMPLOYEE:

Emil D. Kakkis, M.D., Ph.D., an Individual

/s/ Emil Kakkis

(Signature)
Date: June 15, 2011

Address:

EXHIBIT A

LIST OF PRIOR INVENTIONS
AND ORIGINAL WORKS OF AUTHORSHIP
EXCLUDED UNDER SECTION 2(A)

Title	Date	Identifying Number or Brief Description

☒ No inventions, improvements, or original works of authorship *(per 3(a) that relate to the Company's businesses EDK)*

☐ Additional sheets attached

Signature of Employee: /s/ Emil D. Kakkis

Print Name of Employee: Emil D. Kakkis

Date: June 15, 2011

EXHIBIT B

Section 2870 of the California Labor Code is as follows:

(a) Any provision in an employment agreement which provides that an employee shall assign, or offer to assign, any of his or her rights in an invention to his or her employer shall not apply to an invention that the employee developed entirely on his or her own time without using the employer's equipment, supplies, facilities, or trade secret information except for those inventions that either:

(1) Relate at the time of conception or reduction to practice of the invention to the employer's business, or actual or demonstrably anticipated research or development of the employer; or

(2) Result from any work performed by the employee for the employer.

(b) To the extent a provision in an employment agreement purports to require an employee to assign an invention otherwise excluded from being required to be assigned under subdivision (a), the provision is against the public policy of this state and is unenforceable.

EXHIBIT C

TERMINATION CERTIFICATION

This is to certify that I do not have in my possession, nor have I failed to return, any devices, records, data, reports, proposals, lists, correspondence, specifications, drawings, blueprints, sketches, laboratory notebooks, flow charts, materials, equipment, other documents or property, or copies or reproductions of any aforementioned items belonging to Ultragenyx Pharmaceutical, Inc., a California corporation, its subsidiaries, affiliates, successors or assigns (collectively, the "Company").

I further certify that I have complied with all the terms of the Company's Confidential Information and Invention Assignment Agreement signed by me, including the reporting of any Inventions (as defined therein), conceived or made by me (solely or jointly with others) covered by that agreement.

I further agree that, in compliance with the Confidential Information and Invention Assignment Agreement, I will preserve as confidential all trade secrets, confidential knowledge, data or other proprietary information relating to products, processes, know-how, designs, formulas, developmental or experimental work, computer programs, data bases, other original works of authorship, customer lists, business plans, financial information or other subject matter pertaining to any business of the Company or any of its employees, clients, consultants or licensees.

I further agree that for twelve (12) months from the date of this Certification, I shall not either directly or indirectly solicit, induce, recruit or encourage any of the Company's employees or consultants to terminate their relationship with the Company, or attempt to solicit, induce, recruit, encourage or take away employees of the Company, either for myself or for any other person or entity. I will also not hire Consultants of the Company if this would adversely interfere with the consultant's work for the Company. Further, I shall not at any time use any Confidential Information of the Company to negatively influence any of the Company's clients or customers from purchasing Company products or services or to solicit or influence or attempt to influence any client, customer or other person either directly or indirectly, to direct any purchase of products and/or services to any person, firm, corporation, institution or other entity in competition with the business of the Company.

Date:

_____

(Employee's Signature)

_____

(Print Employee's Name)

Exhibit C

Founders' Stock Repurchase Agreement

FOUNDERS' STOCK REPURCHASE AGREEMENT

This Agreement is made as of the 16th day of June 2011, by and between ULTRAGENYX PHARMACEUTICAL INC., (the "**Company**") and the Emil Kakkis and Jenny Soriano Living Trust dated June 18, 2009 ("**Founders**").

WHEREAS, Founders jointly own 8,000,000 shares of the Company's common stock (the "**Shares**"), and

WHEREAS, the Company proposes to enter into a Series A Preferred Stock Purchase Agreement (the "**Series A Purchase Agreement**") with certain investors (the "**Investors**"), and

WHEREAS, Founders, as the principal shareholders of the Company, expect to derive benefit from the Investors' investment in the Company,

WHEREAS, Emil D. Kakkis, M.D., Ph.D. ("**Executive**"), and the Company are entering into an Executive Employment Agreement to which this Agreement is Attached as Exhibit C, and capitalized terms not otherwise defined herein shall have the meanings given in the Employment Agreement, and

WHEREAS, execution of this Agreement, by the Company and Founders, is a condition to the Investors' obligation to buy stock under the Series A Purchase Agreement; and Founders are willing to enter into this Agreement;

NOW THEREFORE, in consideration of the mutual covenants and representations herein set forth, and in consideration of the Investors' agreement to purchase Series A Preferred Stock pursuant to the Series A Purchase Agreement, the parties agree that the Company shall have the following repurchase right with respect to the Shares, including with respect to Shares acquired by Executive prior to his employment with the Company:

1. Repurchase Option. In the event that: (i) Executive's employment or consulting relationship with the Company is terminated by the Company or its successor for Cause, or (ii) Executive voluntarily terminates his employment or consulting relationship with the Company or its successor for other than Good Reason, the Company shall have the irrevocable, exclusive option, exercisable for 90 days (the "**Repurchase Period**") from the date upon which the Executive shall so cease to be employed or continues to be a consultant (as reasonably fixed and determined by the Company) (the "**Termination Date**") to purchase from Founders up to 8,000,000 of the Shares at a price (the "**Option Price**") of $0.01 per share (such number and such price being subject to equitable adjustment for any stock split, stock dividend, combination of shares or the like and based upon common stock or common stock equivalents), other than any of such Shares with respect to which this repurchase option (the "**Repurchase Option**") has lapsed as described in Section 2.

2. Lapsing of the Repurchase Option. On the anniversary of the date of this Agreement, the Company's Repurchase Option shall lapse with respect to 50% of the Shares. After the first anniversary of the date of this Agreement, the Company's Repurchase Option shall lapse with respect to an additional 12.5% of the Shares at the end of each 90 day period thereafter; provided, however, that the Company's Repurchase Option shall cease to lapse with

respect to any additional shares after the Termination Date. The Company's Repurchase Option shall lapse with respect to 100% percent of the Shares if: (i) Executive's employment with the Company is terminated (a) by the Company or its successor without Cause or (b) By Death or Disability, or (ii) Executive voluntarily terminates his employment with the Company or its successor for Good Reason.

3. Procedure. If the Company desires to exercise its Repurchase Option, it shall notify the Founders (the "**Repurchase Notice**"), stating the number of Shares the Company is electing to purchase and the Option Price, prior to the expiration of the Repurchase Period. The sale shall be effect at the offices of the Company on 7th day following the date of the Repurchase Notice (if such day is a not a business day then on the next succeeding business day) by Escrow Agent's delivery to the Company of a certificate or certificates evidencing the shares to be purchased by it, duly endorsed for transfer to the Company, against payment to Founders by the Company of the Option Price for each such Share.

4. Restriction on Transfer. None of the Shares subject to the Repurchase Option or any beneficial interest therein shall be transferred, encumbered or otherwise disposed of in any way until the lapsing of the Company's Repurchase Option on such Shares in accordance with the provisions of this Agreement.

5. Legends. (a) All certificates representing any of the Shares subject to the provisions of this Agreement shall have endorsed thereon the following legends:

THE SHARES REPRESENTED BY THIS CERTIFICATE HAVE BEEN ACQUIRED FOR INVESTMENT AND NOT WITH A VIEW TO, OR IN CONNECTION WITH, THE SALE OR DISTRIBUTION THEREOF. THESE SECURITIES HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933. THEY MAY NOT BE SOLD, OFFERED FOR SALE, PLEDGED OR HYPOTHECATED IN THE ABSENCE OF AN EFFECTIVE REGISTRATION STATEMENT AS TO THE SECURITIES UNDER SAID ACT OR AN OPINION OF COUNSEL SATISFACTORY TO THE COMPANY THAT SUCH REGISTRATION IS NOT REQUIRED.

THE SHARES REPRESENTED BY THIS CERTIFICATE ARE SUBJECT TO CERTAIN RESTRICTIONS UPON TRANSFER AND RIGHTS OF REPURCHASE AS SET FORTH IN AN AGREEMENT BETWEEN THE COMPANY AND THE REGISTERED HOLDER, A COPY OF WHICH IS ON FILE AT THE PRINCIPAL OFFICE OF THE COMPANY. SUCH TRANSFER RESTRICTIONS ARE BINDING ON TRANSFEREES OF THESE SHARES. COPIES OF THE AGREEMENT COVERING THE PURCHASE OF THESE SHARES AND RESTRICTING THEIR TRANSFER MAY BE OBTAINED AT NO COST BY WRITTEN REQUEST MADE BY THE HOLDER OF RECORD OF THIS CERTIFICATE TO THE SECRETARY OF THE COMPANY AT THE PRINCIPAL EXECUTIVE OFFICES OF THE COMPANY.

(b) The certificates for Shares shall also bear the following legend and any other legends required by applicable state corporate or securities laws:

THE SHARES REPRESENTED BY THIS CERTIFICATE ARE SUBJECT TO A RIGHT OF FIRST REFUSAL OPTION AS SET FORTH IN THE COMMON STOCK PURCHASE AGREEMENT BETWEEN THE HOLDER AND THE COMPANY.

6. Escrow. As security for the faithful performance of the terms of this Agreement and to insure the availability for delivery of the Shares upon exercise of the Repurchase Option herein provided for, Founders agree to deliver to and deposit with the Secretary of the Company, or such other person designated by the Company, as escrow agent in this transaction ("**Escrow Agent**"), two stock assignments duly endorsed (with date and number of shares blank) in substantially the form attached hereto as Exhibit A, together with the certificate or certificates evidencing the Shares; said documents are to be held by the Escrow Agent and delivered by said Escrow Agent pursuant to the Joint Escrow Instructions of the Company and Founders set forth in Exhibit B attached hereto and incorporated by this reference, which instructions shall also be delivered to the Escrow Agent at the closing hereunder.

7. Miscellaneous.

(a) Subject to the provisions and limitations hereof, Founders may, during the term of this Agreement, exercise all rights and privileges of a shareholder of the Company with respect to the Shares deposited in said escrow.

(b) The parties agree to execute such further instruments and to take such further action as may reasonably be necessary to carry out the intent of this Agreement.

(c) Any notice required or permitted hereunder shall be given in writing and shall be deemed to have been duly given if delivered: (a) by hand; (b) by a nationally recognized overnight courier service; or (c) by United States first class registered or certified mail, return receipt requested, addressed to Founders at the address shown in the Company's employment records for the Executive and to the Company at the address of its principal corporate offices (attention: President) or at such other address as such party may designate by ten days' advance written notice to the other party hereto.

(d) Subject to the terms and conditions of this Agreement, the Company may assign its rights and delegate its duties under this Agreement, including paragraphs 1 and 3 hereof. This Agreement shall inure to the benefit of the successors and assigns of the Company and, subject to the restrictions on transfer herein set forth, be binding upon the Founders, their heirs, executors, administrators, successors and assigns.

(e) Nothing in this Agreement shall affect in any manner whatsoever the right or power of the Company, or a parent or subsidiary of the Company, to terminate Executive's employment, for any reason, with or without cause.

(f) This Agreement shall be construed as a whole, according to its fair meaning, and not in favor of or against any party. Sections and section headings contained in this Agreement are for reference purposes only, and shall not affect in any manner the meaning or interpretation of this Agreement. Whenever the context requires, references to the singular shall include the plural and the plural the singular.

(g) Each party represents and warrants that such party has the right, power and authority to enter into and execute this Agreement and to perform and discharge all of the obligations hereunder; and that this Agreement constitutes the valid and legally binding agreement and obligation of such party and is enforceable in accordance with its terms.

(h) This Agreement may be executed in any number of counterparts, each of which shall be deemed an original of this Agreement, but all of which together shall constitute one and the same instrument.

8. Governing Law. This Agreement shall be governed by, and shall be construed and enforced in accordance with the laws of the State of California without giving effect to the conflicts of laws principles thereof.

IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the day and year first above written.

COMPANY:

ULTRAGENYX PHARMACEUTICAL INC.

By: /s/ Emil Kakkis
(Signature)

Name: Emil Kakkis
Title: CEO

Fax: (415) 884-0562

FOUNDERS:

THE EMIL KAKKIS AND JENNY SORIANO LIVING TRUST DATED JUNE 18, 2009

By: /s/ Emil Kakkis
Name: Emil D. Kakkis, M.D., Ph.D.
Title: Trustee

By: /s/ Jenny Soriano
Name: Jenny Soriano
Title: Trustee
Fax: (415) 898-8457

EXHIBIT A

ASSIGNMENT SEPARATE FROM CERTIFICATE

FOR VALUE RECEIVED and pursuant to that certain Founders' Stock Repurchase Agreement dated as of June 16, 2011(the "Agreement") the Emil Kakkis and Jenny Soriano Living Trust, dated June 18, 2009 hereby sells, assigns and transfers unto _____ (_____) shares of the Common Stock of Ultragenyx Pharmaceutical Inc. standing in the undersigned's name on the books of said corporation represented by certificate no. _____ herewith, and does hereby irrevocably constitute and appoint _____ attorney to transfer the said stock on the books of the said corporation with full power of substitution in the premises. THIS ASSIGNMENT MAY ONLY BE USED AS AUTHORIZED BY THE AGREEMENT AND THE EXHIBITS THERETO.

Dated: _____

The Emil Kakkis and Jenny Soriano Living Trust, dated June 18, 2009

By: /s/ Emil D. Kakkis
Emil D. Kakkis, Trustee

By: /s/ Jenny Soriano
Jenny Soriano, Trustee

Instruction: Please do not fill in any blanks other than the signature line. The purpose of this assignment is to enable the Corporation to exercise its "Repurchase Option" as set forth in the Agreement without requiring additional signatures on the part of the Founders.

EXHIBIT B

JOINT ESCROW INSTRUCTIONS

June 16, 2011

Secretary
Ultragenyx Pharmaceutical Inc.
77 Digital Drive, Suite 210
Novato, CA 94949

RE: Joint Escrow Instructions

Dear Sir or Madam:

As Escrow Agent for both Ultragenyx Pharmaceutical Inc., ("Company"), and the Emil D. Kakkis and Jenny Soriano Living Trust dated June 18, 2009 (the "Founders"), you are hereby authorized and directed to hold the documents delivered to you pursuant to the terms of that certain Founders' Stock Repurchase Agreement ("Agreement") between the Company and the undersigned, to which a copy of these Joint Escrow Instructions is attached as Exhibit B, in accordance with the following instructions:

1. In the event the Company and/or any assignee of the Company (referred to collectively for convenience herein as the "Company") exercises the Repurchase Option set forth in the Agreement, the Company shall give to Founders and you a written notice specifying the number of shares of stock to be purchased, the purchase price, and the time for a closing hereunder at the principal office of the Company. Founders and the Company hereby irrevocably authorize and direct you to close the transaction contemplated by such notice in accordance with the terms of said notice.

2. At the closing, you are directed (a) to date the stock assignments necessary for the transfer in question, (b) to fill in the number of shares being transferred, and (c) to deliver same, together with the certificate evidencing the shares of stock to be transferred, to the Company or its assignee, in accordance with the Agreement, against the simultaneous delivery to you of the purchase price (by check) for the number of shares of stock being purchased pursuant to the exercise of the Repurchase Option.

3. Each Founder irrevocably authorizes the Company to deposit with you any certificates evidencing shares of stock to be held by you hereunder and any additions and substitutions to said shares as defined in the Agreement. Each Founder does hereby irrevocably constitute and appoint you as their attorney-in-fact and agent for the term of this escrow to execute with respect to such securities all documents necessary or appropriate to make such securities negotiable and to complete any transaction herein contemplated. Subject to the provisions of this paragraph 3, Founders shall exercise all rights and privileges of a shareholder of the Company while the stock is held by you.

4. Upon written request of a Founder, but no more than once per calendar year, unless the Repurchase Option has been exercised, you will deliver to Founders a certificate or certificates representing so many shares of stock as are not then subject to the Repurchase Option. Within 100 days after cessation of Emil D. Kakkis' continuous employment by the Company or consulting services to the Company, or any parent or subsidiary of the Company, you will deliver to Founders a certificate or certificates representing the aggregate number of shares sold and issued pursuant to the Agreement and not purchased by the Company or its assignees pursuant to exercise of the Repurchase Option.

5. If at the time of termination of this escrow you should have in your possession any documents, securities, or other property belonging to the Founders, you shall deliver all of same to Founder and shall be discharged of all further obligations hereunder.

6. Your duties hereunder may be altered, amended, modified or revoked only by a writing signed by all of the parties hereto.

7. You shall be obligated only for the performance of such duties as are specifically set forth herein and may rely and shall be protected in relying or refraining from acting on any instrument reasonably believed by you to be genuine and to have been signed or presented by the proper party or parties. You shall not be personally liable for any act you may do or omit to do hereunder as Escrow Agent or as attorney-in-fact for a Founder while acting in good faith, and any act done or omitted by you pursuant to the advice of your own or the Company's attorneys shall be conclusive evidence of such good faith.

8. You shall not be liable in any respect on account of the identity, authorities or rights of the parties executing or delivering or purporting to execute or deliver the Agreement or any documents or papers deposited or called for hereunder.

9. You shall not be liable for the expiration of any rights under the Statute of Limitations with respect to these Joint Escrow Instructions or any documents deposited with you.

10. You shall be entitled to employ such legal counsel and other experts as you may deem necessary properly to advise you in connection with your obligations hereunder, may rely upon the advice of such counsel, and may pay such counsel reasonable compensation therefor. The Company will reimburse you for your reasonable legal fees and expenses.

11. Your responsibilities as Escrow Agent hereunder shall terminate if you shall cease to be an officer of the Company or if you shall resign by written notice to each party. In the event of any such termination, the Company shall appoint a successor Escrow Agent.

12. If you reasonably require other or further instruments in connection with these Joint Escrow Instructions or obligations in respect hereto, the necessary parties hereto shall join in furnishing such instruments.

13. It is understood and agreed that should any dispute arise with respect to the delivery and/or ownership or right of possession of the securities held by you hereunder, you are authorized and directed to retain in your possession without liability to anyone all or any part of said securities until such disputes shall have been settled either by mutual written agreement of the parties concerned or by a final order, decree or judgment of a court of competent jurisdiction after the time for appeal has expired and no appeal has been perfected, but you shall be under no duty whatsoever to institute or defend any such proceedings.

14. All notices or other communications required or permitted hereunder shall be made in writing and shall be deemed to have been duly given if delivered: (a) by hand; (b) by a nationally recognized overnight courier service; or (c) by United States first class registered or certified mail, return receipt requested, to the principal address of the other party, as set forth below. The date of notice shall be deemed to be the earlier of (i) actual receipt of notice by any permitted means, or (ii) five business days following dispatch by overnight delivery service or the United States Mail. Each party shall be obligated to notify the Company in writing of any change in address. Notice of change of address shall be effective only when done in accordance with this paragraph.

Company's Notice Address:

Ultragenyx Pharmaceuticals Inc.
77 Digital Drive, Suite 210
Novato, CA 94949
Attn: President

Founders' Notice Address:

The Emil Kakkis and Jenny Soriano Living Trust, dated June 18, 2009

Escrow Agent's Notice Address:

Ultragenyx Pharmaceuticals Inc.
77 Digital Drive, Suite 210
Novato, CA 94949
Attn: Secretary

15. By signing these Joint Escrow Instructions, you become a party hereto only for the purpose of said Joint Escrow instructions; you do not become a party to the Agreement.

16. This instrument shall be binding upon and inure to the benefit of the parties hereto, and their respective successors and permitted assigns.

[Signature Page to Follow]

Very truly yours,

COMPANY:

Ultragenyx Pharmaceutical Inc.

By: /s/ Emil Kakkis
Name: Emil Kakkis
Its: CEO

FOUNDERS:

The Emil Kakkis and Jenny Soriano Living Trust, dated June 18, 2009

/s/ Emil Kakkis
Emil D. Kakkis, Trustee

/s/ Jenny Soriano
Jenny Soriano, Trustee

ESCROW AGENT:

_____, Secretary
Name:

Exhibit 10.19

ultragenyx
pharmaceutical

77 Digital Drive, Suite 210
Novato, California 94949
Tel: 415-884-2800

October 31, 2011

Thomas R. Kassberg

Re: Offer of Employment

Dear Tom,

On behalf of Ultragenyx Pharmaceutical, Inc. (the "Company"), I am pleased to present to you an offer of full-time employment as Chief Business Officer and Senior Vice President. The Company's Board of Directors (the "Board") and I are excited about the important contributions you can make by joining the Ultragenyx management team and are confident that you will play a key role in our company's growth and success.

In your role as Chief Business Officer and Senior Vice President, you will report directly to the CEO and be a member of the Senior Management Team. In this role, you will have the key responsibility of identifying opportunities and developing partnerships that grow and expand Ultragenyx as a top tier rare disease company. Your role will also include developing the strategic direction of the company with the CEO and developing the company as a business. You will participate in Board meetings, and present and discuss business development opportunities and corporate strategy with the Board and CEO.

Your duties will include, but are not limited to, developing, identifying and executing long-term partnerships and licensing opportunities and evaluating new opportunities for their scientific and strategic fit for Ultragenyx. As we discussed during your interviews, in addition to your business development functions, you will take on additional responsibilities, including but not limited to Human Resources, Legal, and Intellectual Property. Additional responsibilities will be considered and will be mutually agreed upon by you and the CEO after your start date.

Compensation

In the regular exempt position of Chief Business Officer and Senior Vice President, the Company shall pay you as compensation for your services an initial base salary at a gross annual rate of $275,000 (the "Base Salary"). Such Base Salary shall be payable in accordance with the Company's standard payroll procedures and subject to standard payroll deductions and withholdings. The Board or any Compensation Committee of the Board shall review your Base Salary at least annually.

In addition to your Base Salary, upon the adoption of a written bonus plan for the Company, you will be eligible to receive an annual bonus of up to 30% of your Base Salary based upon your performance during the previous year as evaluated by the CEO in consultation with the Board against goals mutually agreed upon between you and the Company and based on the Company's overall performance.

Stock Option

Subject to the approval of the Board, you will also receive an option to purchase an aggregate of 635,000 shares of the Company's Common Stock (the "Option") pursuant to the Company's stock incentive plan (the "Plan"). The exercise price of the Option will be equal to the fair market value per share of Common Stock as determined by the Board in good faith on the date of grant.

The Option will vest and become exercisable as follows: 1/4th of the shares initially subject to the Option shall vest and become exercisable on the first (1st) anniversary of the first day of your employment with the Company, and thereafter 1/48th of the shares initially subject to the Option shall vest and become exercisable each month until the Option is fully vested, in each case subject to your continued employment by the Company (or its subsidiaries). The Option shall be governed by the Company's standard form of stock option agreement and the Plan.

Notwithstanding the foregoing, in the event that (i) the Company consummates a Deemed Liquidation Event (as defined in the Company's Amended and Restated Certificate of Incorporation, as amended from time to time) on or after June 16, 2013, (ii) on the date such Deemed Liquidation Event is consummated you are employed by the Company (or its subsidiaries) and (iii) within 12 months after the date such Deemed Liquidation Event is consummated your employment by the Company (or its successor or subsidiaries) is terminated without Cause (as defined below) or you resign such employment due to a Constructive Termination (as defined below), then provided such termination constitutes a "separation from service" (as defined under Treasury Regulation Section 1.409A-1(h), without regard to any alternative definition thereunder, a "Separation from Service"), in addition to the severance benefits set forth below, (A) the vesting of the Option shall accelerate with respect to 50% of the then-unvested shares then subject to the Option, and (B) the vesting of any equity in the Company granted to you in connection with your employment by the Company other than the Option shall accelerate with respect to 100% of the then-unvested shares that comprise such other equity.

Benefits

You shall be eligible to participate in all of the employee benefits and benefit plans that the Company generally makes available to its full-time regular employees, subject to the terms and conditions of such benefits and benefit plans. At this time, these include medical, dental and vision insurance coverage. Coverage for these benefits begins on the 1st day of the month following your date of hire. Detailed information about the benefits presently available will be provided to you on your first day of employment.

You will be entitled to accrue and use Paid Time Off (PTO) in accordance with the terms of the Company's PTO policy. You will accrue PTO at the rate of 160 hours per year.

"At Will" Employment

Employment with the Company is "at-will". This means that it is not for any specified period of time and can be terminated by you or by the Company at any time, with or without advance notice, and for any or no particular reason or Cause. It also means that your job duties, title, responsibilities, reporting level, compensation and benefits, as well as the Company's personnel policies and procedures, may be changed at any time, with or without notice in the sole discretion of the Company. This "at-will" nature of your employment shall remain unchanged during your tenure as an employee, and can only be changed by an express written agreement that is signed by you and by the Company's CEO.

Severance

If, at any time, your employment with the Company or its successor is terminated without Cause, or you resign your employment due to a Constructive Termination, then provided such termination constitutes a Separation from Service, the Company shall: (i) extend the exercise period applicable to the Option (and to any other options to purchase the Company's Common Stock you then hold) such that you will have until the date that is twelve (12) months after the date of your Separation from Service to exercise any of the vested shares subject to the Option (but in no event will the exercise period be extended until later than the date of expiration of the term of the Option as set forth in the agreement evidencing such Option); and (ii) the Company shall pay you, as severance, the equivalent of six (6) months of your Base Salary in effect as of the date of your Separation from Service, subject to standard payroll deductions and withholdings (the "Severance Amount"). The Severance Amount will be paid in installments in the form of continuation of your Base Salary payments, paid on the Company's regular payroll dates, commencing on the Company's first regular payroll date that follows the 60th day after such Separation from Service, and shall be for all accrued Base Salary for the 60-day period plus the period from the 60th day until the regular payroll date and the remainder of the Base Salary continuation payments shall thereafter be made on the Company's regular payroll dates.

Notwithstanding anything herein to the contrary, the receipt of any of the severance or acceleration benefits described in this letter will be subject to and conditioned upon: (i) your signing and not revoking a separation agreement and release of claims in a form reasonably satisfactory to the Company (the "Separation Agreement") within the time period specified therein, but in any event no later than sixty (60) days following your Separation from Service; and (ii) your continued compliance with the terms of this letter, the Separation Agreement, the enclosed Confidential Information and Invention Assignment Agreement (including without limitation, your not using or disclosing any confidential or proprietary information of the Company), and any other agreement entered into between you and the Company. No severance benefits of any kind will be paid or provided, and no acceleration of vesting shall be effective, until the Separation Agreement becomes effective. You shall also resign from all positions and terminate any relationships as an employee, advisor, officer or director with the Company and any of its affiliates, each effective on the date of termination.

Additionally, and for the avoidance of doubt, in the event that the Company terminates your employment for Cause, or you resign your employment for any reason other than due to a Constructive Termination, or your employment terminates upon your death or disability, you will no longer vest in the Option (or any other equity) and you will not be entitled to any severance benefits described herein.

For purposes of this offer letter, “Cause” means any of the following: (i) your gross negligence in carrying out, or material failure to carry out, your duties for the Company (including without limitation, your failure to cooperate in any Company investigation), after notice from the Board and a reasonable opportunity to cure (if deemed curable); (ii) any breach of your fiduciary duties to the Company, after notice from the Board and a reasonable opportunity to cure (if deemed curable); (iii) conviction of, or plea of guilty or no contest to, any felony; (iv) any act of fraud or embezzlement by you with respect to your obligations or otherwise relating to the business of the Company; (v) your material violation of any Company policy; (vi) your material breach of any agreement entered into between you and the Company; or (vii) your unauthorized use or disclosure of confidential information or trade secrets of the Company or its affiliates.

For the purposes of this letter, “Constructive Termination” means the occurrence of any of the following events without your written consent: (i) a material reduction or change in your job duties, responsibilities and requirements from your job duties, responsibilities and requirements immediately prior to such reduction or change, taking into account the differences in job title and duties that are normally occasioned by reason of an acquisition of one company by another; (ii) a material reduction of your Base Salary (other than an equal, across-the-board reduction in the compensation of all similarly-situated employees of the Company or the surviving entity that is approved by the board of directors); or (iii) a requirement that you relocate to a principal office that increases your one-way commute by more than 50 miles relative to your immediately preceding principal office. Notwithstanding the foregoing, none of the foregoing events or conditions will constitute Constructive Termination unless: (x) you provide the Company with written objection (or notice) to the event or condition within 30 days following the occurrence thereof, (y) the Company does not reverse or otherwise cure the event or condition within 30 days of receiving that written objection, and (z) you resign your employment within 30 days following the expiration of that cure period.

Compliance with Section 409A

It is intended that all of the severance benefits and other payments payable under this letter satisfy, to the greatest extent possible, the exemptions from the application of Code Section 409A provided under Treasury Regulations 1.409A-1(b)(4), 1.409A-1(b)(5) and 1.409A-1(b)(9), and this letter agreement will be construed to the greatest extent possible as consistent with those provisions, and to the extent not so exempt, this letter (and any definitions hereunder) will be construed in a manner that complies with Section 409A. Notwithstanding any provision to the contrary in this letter, if you are deemed by the Company at the time of your Separation from Service to be a “specified employee” for purposes of Code Section 409A(a)(2)(B)(i), and if any of the payments upon Separation from Service set forth herein and/or under any other agreement with the Company are deemed to be “deferred compensation”, then to the extent delayed commencement of any portion of such payments is required in order to avoid a prohibited distribution under Code Section 409A(a)(2)(B)(i) and the related adverse taxation under Section 409A,

such payments shall not be provided to you prior to the earliest of (i) the expiration of the six-month period measured from the date of your Separation from Service with the Company, (ii) the date of your death, or (iii) such earlier date as permitted under Section 409A without the imposition of adverse taxation. Upon the first business day following the expiration of such applicable Code Section 409A(a)(2)(B)(i) period, all payments deferred pursuant to this paragraph shall be paid in a lump sum to you, and any remaining payments due shall be paid as otherwise provided herein or in the applicable agreement. No interest shall be due on any amounts so deferred.

Compliance with Company Policies

As an employee of the Company, you will be expected to comply with the Company's personnel and other policies, including but not limited to, the Company's policies prohibiting discrimination and unlawful harassment, conflicts of interest and violation of applicable laws in the course of performing services to the Company. During your orientation, you will be provided with the Company's policy and procedures.

Full-time Services to the Company

The Company requires that, as a full-time employee, you devote your full business time, attention, skills and efforts to the tasks and duties of your position as assigned by the Company. However, the Company will not preclude you from providing services to others, so long as such services would not be to the benefit of a competitor of the Company and will not otherwise interfere with your ability to satisfactorily fulfill your job responsibilities to the Company. If you wish to perform services (for any or no form of compensation) to any other person or business entity while employed by the Company, please contact the CEO and discuss your plans in advance of providing such services so that no problem later arises that could have been avoided from the outset. Any other such services must be approved by the CEO and Board.

Conditions

This offer, and any employment pursuant to this offer, is conditioned upon the following:

- Your ability to provide satisfactory documentary proof of your identity and right to work in the United States of America on your first day of employment. Enclosed is the INS Form I-9, Employment Eligibility Verification, the second page of which includes a description of acceptable documentary proof.
- Your signed agreement to, and ongoing compliance with, the terms of the enclosed Confidential Information and Invention Assignment Agreement without modification.
- Your consent (by your signature below) to, and results satisfactory to the Company of, reference and background checks. Until you have been informed in writing by the Company that such checks have been completed and the results satisfactory to the Company, you should defer reliance on this offer.
- Your return to me of the enclosed copy of this letter, after being signed by you without modification, no later than November 3, 2011, after which time this offer will expire.

No Conflicting Obligations

By signing and accepting this offer, you represent and warrant that: (i) you are not subject to any pre-existing contractual or other legal obligation with any person, company or business enterprise which may be an impediment to or is inconsistent with your employment with, or your providing services to, the Company; (ii) you have not and shall not bring onto Company premises, or use or disclose in the course of your employment with the Company, any confidential or proprietary information or trade secrets of another person, company or business enterprise; (iii) you have returned all property and confidential information belonging to any prior employer; and (iv) you are not relying on any representations, promises or agreements not expressly contained in this letter.

Choice of Law and Severability

This letter shall be interpreted in accordance with the laws of the State of California without giving effect to provisions governing the choice of law. If any provision of this letter becomes or is deemed invalid, illegal or unenforceable in any applicable jurisdiction by reason of the scope, extent or duration of its coverage, then such provision shall be deemed amended to the minimum extent necessary to conform to applicable law so as to be valid and enforceable or, if such provision cannot be so amended without materially altering the intention of the parties, then such provision shall be stricken and the remainder of this letter shall continue in full force and effect. If any provision of this letter is rendered illegal by any present or future statute, law, ordinance or regulation (collectively, the "Law") then that provision shall be curtailed or limited only to the minimum extent necessary to bring the provision into compliance with the Law. All the other terms and provisions of this letter shall continue in full force and effect without impairment or limitation.

Entire Agreement

If you accept this offer, and the conditions of this offer are satisfied, this letter and the written agreements referenced in this letter shall constitute the complete agreement between you and the Company with respect to the initial terms and conditions of your employment. Any representations, promises or agreements, whether written or oral, not contained in this letter or contrary to those contained in this letter, that may have been made to you are expressly cancelled and replaced by this offer letter. Except as otherwise specified in this letter or in the written agreements referenced in this letter, the terms and conditions of your employment pursuant to this letter may not be changed, except by a writing issued by the CEO with approval by the Board.

We look forward to you accepting this offer and a mutually rewarding relationship. As with all important decisions, you should make a decision concerning this offer based on your own investigation and judgment concerning the Company and its prospects, independent of the opinions and perspectives that may have been shared with you by any Company employee.

If you accept this offer, please date and sign below, on the enclosed copy of this letter and return it to me no later than November 3, 2011. Please retain the original of this letter for your records. You should bring your INS Form 1-9 required identification and proof of authorization to work with you on your first day of employment.

We look forward to working with you on developing treatment for many rare genetic diseases and hope you find your employment at Ultragenyx Pharmaceutical Inc. a rewarding experience. If you have any questions regarding this offer letter, please feel free to contact me at (415) 884-2800.

Warm Regards,

/s/ Emil D. Kakkis
Emil D. Kakkis, M.D., Ph.D.
Chief Executive Officer

I accept the above offer:

Signature: /s/ Thomas Kassberg Dated: October 31, 2011
Print Name: Thomas Kassberg

Exhibit 10.20

60 Leveroni Court
Novato, California 94949
p: 415.884.2800

March 12, 2012

Shalini Sharp

Re: Offer of Employment

Dear Shalini,

On behalf of Ultragenyx Pharmaceutical Inc. (the "Company"), I am pleased to present to you an offer of full-time employment as Chief Financial Officer and Senior Vice President, Finance. The Company's Board of Directors (the "Board") and I are excited about the important contributions you can make by joining the Ultragenyx management team and are confident that you will play a key role in our company's growth and success. This letter will supercede the offer letter dated March 8, 2012.

In your role as Chief Financial Officer and Senior Vice President, Finance you will report directly to me and be a member of the Senior Management Team. In this role, you will have the key responsibility of leading the corporate finance function including strategic financial planning, accounting, budgeting and forecasting, project-specific financial analysis, general risk analysis, and financings, both private and ultimately public. As for all functions at Ultragenyx, the Finance function is expected to work in an integrated fashion with business development, program development, technical operations, and commercial operations to drive the growth and success of Ultragenyx. You will also be responsible for creating and implementing the financial strategy and infrastructure required to support the company's growth and for building a long-term financial model for the development and commercialization of the company's products based on the goal of treating rare and ultra-rare diseases.

Compensation

In the regular exempt position of Chief Financial Officer and Senior Vice President, Finance, the Company shall pay you as compensation for your services an initial base salary at a gross annual rate of $270,000 (the "Base Salary"). Such Base Salary shall be payable in accordance with the Company's standard payroll procedures and subject to standard payroll deductions and withholdings. The Board or any Compensation Committee of the Board shall review your Base Salary at least annually.

In addition to your Base Salary, upon the adoption of a written bonus plan for the Company, you will be eligible to receive an annual bonus of up to 30% of your Base Salary based upon your performance during the previous year as evaluated by the CEO in consultation with the Board against goals mutually agreed upon between you and the Company and based on the Company's overall performance.

Stock Option

Subject to the approval of the Board, you will also receive an option to purchase an aggregate of 600,000 shares (approximately 1.2% of the fully diluted, outstanding stock at the time of the second closing in June 2012) of the Company's Common Stock (the "Option") pursuant to the Company's stock incentive plan (the "Plan"). The exercise price of the Option will be equal to the fair market value per share of Common Stock as determined by the Board in good faith on the date of grant. As of the date of this letter, the current strike price for options is set at $0.10 per share. However, this price can change if events occur that affect the value of the company leading to a new valuation analysis.

The Option will vest and become exercisable as follows: 1/4th of the shares initially subject to the Option shall vest and become exercisable on the first (1st) anniversary of the first day of your employment with the Company, and thereafter 1/48th of the shares initially subject to the Option shall vest and become exercisable each month until the Option is fully vested, in each case subject to your continued employment by the Company (or its subsidiaries). The Option shall be governed by the Company's standard form of stock option agreement and the Plan.

Notwithstanding the foregoing, in the event that (i) the Company consummates a Deemed Liquidation Event (as defined in the Company's Amended and Restated Certificate of Incorporation, as amended from time to time) on or after June 16, 2013, (ii) on the date such Deemed Liquidation Event is consummated you are employed by the Company (or its subsidiaries) and (iii) within 12 months after the date such Deemed Liquidation Event is consummated your employment by the Company (or its successor or subsidiaries) is terminated without Cause (as defined below) or you resign such employment due to a Constructive Termination (as defined below), then provided such termination constitutes a "separation from service" (as defined under Treasury Regulation Section 1.409A-1(h), without regard to any alternative definition thereunder, a "Separation from Service"), in addition to the severance benefits set forth below, the vesting of the Option shall accelerate with respect to 50% of the then-unvested shares then subject to the Option. This acceleration provision also shall apply to any equity you are issued in the future.

Benefits

You shall be eligible to participate in all of the employee benefits and benefit plans that the Company generally makes available to its full-time regular employees, subject to the terms and conditions of such benefits and benefit plans. At this time, these include medical, dental vision and life insurance coverage. Coverage for these benefits begins on the 1st day of the month following your date of hire. Detailed information about the benefits presently available will be provided to you on your first day of employment.

You will be entitled to accrue and use Paid Time Off (PTO) at the rate of 120 hours per year, up to an accrual cap of 210 hours, in accordance with the terms of the Company's PTO policy.

"At Will" Employment

Employment with the Company is "at-will". This means that it is not for any specified period of time and can be terminated by you or by the Company at any time, with or without advance notice, and for any or no particular reason or Cause. It also means that your job duties, title, responsibilities, reporting level, compensation and benefits, as well as the Company's personnel policies and procedures, may be changed at any time, with or without notice in the sole discretion of the Company. This "at-will" nature of your employment shall remain unchanged during your tenure as an employee, and can only be changed by an express written agreement that is signed by you and by the Company's CEO.

Severance

If, at any time, your employment with the Company or its successor is terminated without Cause, or you resign your employment due to a Constructive Termination, then provided such termination constitutes a Separation from Service, the Company shall: (i) extend the exercise period applicable to the Option (and to any other options to purchase the Company's Common Stock you then hold) such that you will have until the date that is twelve (12) months after the date of your Separation from Service to exercise any of the vested shares subject to the Option (but in no event will the exercise period be extended until later than the date of expiration of the term of the Option as set forth in the agreement evidencing such Option); and (ii) the Company shall pay you, as severance, (a) following your date of hire and prior to the 12-month anniversary of your employment, the equivalent of twelve (12) months of Base Salary, or (b) following the 12-month anniversary of your employment, the equivalent of six (6) months of Base Salary in effect as of the date of your Separation from Service, and subject to standard payroll deductions and withholdings (the "Severance Amount"). The Severance Amount will be paid in installments in the form of continuation of your Base Salary payments, paid on the Company's regular payroll dates, commencing on the Company's first regular payroll date that follows the 60th day after such Separation from Service, and shall be for all accrued Base Salary for the 60-day period plus the period from the 60th day until the regular payroll date and the remainder of the Base Salary continuation payments shall thereafter be made on the Company's regular payroll dates.

Notwithstanding anything herein to the contrary, the receipt of any of the severance or acceleration benefits described in this letter will be subject to and conditioned upon: (i) your signing and not revoking a separation agreement and release of claims in a form reasonably satisfactory to the Company (the "Separation Agreement") within the time period specified therein, but in any event no later than sixty (60) days following your Separation from Service; and (ii) your continued compliance with the terms of this letter, the Separation Agreement, the enclosed Confidential Information and Invention Assignment Agreement (including without limitation, your not using or disclosing any confidential or proprietary information of the Company), and any other agreement entered into between you and the Company. No severance

benefits of any kind will be paid or provided, and no acceleration of vesting shall be effective, until the Separation Agreement becomes effective, You shall also resign from all positions and terminate any relationships as an employee, advisor, officer or director with the Company and any of its affiliates, each effective on the date of termination.

Additionally, and for the avoidance of doubt, in the event that the Company terminates your employment for Cause, or you resign your employment for any reason other than due to a Constructive Termination, or your employment terminates upon your death or disability, you will no longer vest in the Option (or any other equity) and you will not be entitled to any severance benefits described herein.

For purposes of this offer letter, “Cause” means any of the following: (i) your gross negligence in carrying out, or material failure to carry out, your duties for the Company (including without limitation, your failure to cooperate in any Company investigation), after notice from the Board and a reasonable opportunity to cure (if deemed curable); (ii) any breach of your fiduciary duties to the Company, after notice from the Board and a reasonable opportunity to cure (if deemed curable); (iii) conviction of, or plea of guilty or no contest to, any felony; (iv) any act of fraud or embezzlement by you with respect to your obligations or otherwise relating to the business of the Company; (v) your material violation of any Company policy; (vi) your material breach of any agreement entered into between you and the Company; or (vii) your unauthorized use or disclosure of confidential information or trade secrets of the Company or its affiliates.

For the purposes of this letter, “Constructive Termination” means the occurrence of any of the following events without your written consent: (i) a material reduction or change in your job duties, responsibilities and requirements from your job duties, responsibilities and requirements immediately prior to such reduction or change, taking into account the differences in job title and duties that are normally occasioned by reason of an acquisition of one company by another; (ii) a material reduction of your Base Salary (other than an equal, across-the-board reduction in the compensation of all similarly- situated employees of the Company or the surviving entity that is approved by the board of directors); or (iii) a requirement that you relocate to a principal office that increases your one-way commute by more than 50 miles relative to your immediately preceding principal office. Notwithstanding the foregoing, none of the foregoing events or conditions will constitute Constructive Termination unless: (x) you provide the Company with written objection (or notice) to the event or condition within 30 days following the occurrence thereof, (y) the Company does not reverse or otherwise cure the event or condition within 30 days of receiving that written objection, and (z) you resign your employment within 30 days following the expiration of that cure period.

Compliance with Section 409A

It is intended that all of the severance benefits and other payments payable under this letter satisfy, to the greatest extent possible, the exemptions from the application of Code Section 409A provided under Treasury Regulations 1.409A-1(b)(4), 1.409A-1(b)(5) and 1.409A-1(b)(9), and this letter agreement will be construed to the greatest extent possible as consistent with those provisions, and to the extent not so exempt, this letter (and any definitions hereunder) will be construed in a manner that complies with Section 409A. Notwithstanding any provision to the contrary in this letter, if you are deemed by the Company at the time of your Separation from

Service to be a "specified employee" for purposes of Code Section 409A(a)(2)(B)(i), and if any of the payments upon Separation from Service set forth herein and/or under any other agreement with the Company are deemed to be "deferred compensation", then to the extent delayed commencement of any portion of such payments is required in order to avoid a prohibited distribution under Code Section 409A(a)(2)(B)(i) and the related adverse taxation under Section 409A, such payments shall not be provided to you prior to the earliest of (i) the expiration of the six-month period measured from the date of your Separation from Service with the Company, (ii) the date of your death, or (iii) such earlier date as permitted under Section 409A without the imposition of adverse taxation. Upon the first business day following the expiration of such applicable Code Section 409A(a)(2)(B)(i) period, all payments deferred pursuant to this paragraph shall be paid in a lump sum to you, and any remaining payments due shall be paid as otherwise provided herein or in the applicable agreement. No interest shall be due on any amounts so deferred.

Compliance with Company Policies

As an employee of the Company, you will be expected to comply with the Company's personnel and other policies, including but not limited to, the Company's policies prohibiting discrimination and unlawful harassment, conflicts of interest and violation of applicable laws in the course of performing services to the Company. During your orientation, you will be provided with the Company's policy and procedures.

Full-time Services to the Company

The Company requires that, as a full-time employee, you devote your full business time, attention, skills and efforts to the tasks and duties of your position as assigned by the Company. However, the Company will not preclude you from providing services to others, so long as such services would not be to the benefit of a competitor of the Company and will not otherwise interfere with your ability to satisfactorily fulfill your job responsibilities to the Company. If you wish to perform services (for any or no form of compensation) to any other person or business entity while employed by the Company, please contact the CEO and discuss your plans in advance of providing such services so that no problem later arises that could have been avoided from the outset. Any other such services must be approved by the CEO and Board.

Conditions

This offer, and any employment pursuant to this offer, is conditioned upon the following:

- Your ability to provide satisfactory documentary proof of your identity and right to work in the United States of America on your first day of employment. Enclosed is the INS Form 1-9, Employment Eligibility Verification, the second page of which includes a description of acceptable documentary proof.
- Your signed agreement to, and ongoing compliance with, the terms of the enclosed Confidential Information and Invention Assignment Agreement without modification.
- Your consent (by your signature below) to, and results satisfactory to the Company of, reference and background checks. Until you have been informed in writing by the Company that such checks have been completed and the results satisfactory to the Company, you should defer reliance on this offer.

- Your return to me of the enclosed copy of this letter, after being signed by you without modification.

No Conflicting Obligations

By signing and accepting this offer, you represent and warrant that: (i) you are not subject to any pre-existing contractual or other legal obligation with any person, company or business enterprise which may be an impediment to or is inconsistent with your employment with, or your providing services to, the Company; (ii) you have not and shall not bring onto Company premises, or use or disclose in the course of your employment with the Company, any confidential or proprietary information or trade secrets of another person, company or business enterprise; (iii) you have returned all property and confidential information belonging to any prior employer; and (iv) you are not relying on any representations, promises or agreements not expressly contained in this letter.

Choice of Law and Severability

This letter shall be interpreted in accordance with the laws of the State of California without giving effect to provisions governing the choice of law. If any provision of this letter becomes or is deemed invalid, illegal or unenforceable in any applicable jurisdiction by reason of the scope, extent or duration of its coverage, then such provision shall be deemed amended to the minimum extent necessary to conform to applicable law so as to be valid and enforceable or, if such provision cannot be so amended without materially altering the intention of the parties, then such provision shall be stricken and the remainder of this letter shall continue in full force and effect. If any provision of this letter is rendered illegal by any present or future statute, law, ordinance or regulation (collectively, the "Law") then that provision shall be curtailed or limited only to the minimum extent necessary to bring the provision into compliance with the Law. All the other terms and provisions of this letter shall continue in full force and effect without impairment or limitation.

Entire Agreement

If you accept this offer, and the conditions of this offer are satisfied, this letter and the written agreements referenced in this letter shall constitute the complete agreement between you and the Company with respect to the initial terms and conditions of your employment. Any representations, promises or agreements, whether written or oral, not contained in this letter or contrary to those contained in this letter, that may have been made to you are expressly cancelled and replaced by this offer letter. Except as otherwise specified in this letter or in the written agreements referenced in this letter, the terms and conditions of your employment pursuant to this letter may not be changed, except by a writing issued by the CEO with approval by the Board.

We look forward to you accepting this offer and a mutually rewarding relationship. As with all important decisions, you should make a decision concerning this offer based on your own investigation and judgment concerning the Company and its prospects, independent of the

opinions and perspectives that may have been shared with you by any Company employee. If you accept this offer, please date and sign below, on the enclosed copy of this letter and return it to me no later than March 14, 2012. Please retain the original of this letter for your records. You should bring your INS Form 1-9 required identification and proof of authorization to work with you on your first day of employment.

We look forward to working with you on developing treatment for many rare genetic diseases and hope you find your employment at Ultragenyx Pharmaceutical a rewarding experience. If you have any questions regarding this offer letter, please feel free to contact me at (415) 483-8800.

Warm Regards,

/s/ Emil D. Kakkis, M.D., Ph.D.
Emil D. Kakkis, M.D., Ph.D.
Chief Executive Officer

I accept the above offer:

Signature: /s/ Shalini Sharp Dated: March 13, 2012
Print Name: Shalini Sharp

LAKEPOINT BUSINESS PARK
STANDARD LEASE
BASIC LEASE INFORMATION

1.) Date: 7/5/11

2.) Landlord: CONDIOTTI ENTERPRISES, INC.

3.) Tenant: Ultragenyx Pharmaceutical, Inc., a Delaware corporation
Address for Tenant Notices: 60 Leveroni Court, Suite 200, Novato, Ca. 94949

Phone: FAX:

4.) Tenant Contact Party: Dr. Emil Kakkis, President and Chief Executive Officer

5.) Premises: Entire second floor of 60 Leveroni Court, Novato, Ca. 94949

Office: 19,916sf* **Warehouse:** -0- **Other:** -0- **Total:** 19,916sf
*Inclusive of 10% load factor

6.) Term: Five years.

7.) Estimated Commencement Date: 8/01/2011

8.) Base Term Expiration Date: 7/31/2016

9.) Initial Base Monthly Rent: $23,106.00

10.) Security Deposit: Letter of Credit as detailed in Addendum 1, Section 45.

11.) Use: General office use.

12.) Exhibits:
"A" The Building
"B" The Premises
"C" Declaration of Covenants and Protective Standards
"D" Insurance Endorsement Format
"E" Estoppel Format
"F" SNDA
"G" Space Plans

13.) Addenda:
Addendum 1, Sections 44 through 52.

In the event of conflict between any Basic Lease Information and the Lease, the former shall control.

__*(JW)*__ Lessor

__*(EK)*__ Lessee

LAKEPOINT BUSINESS PARK
STANDARD LEASE

For and in consideration of the rents, covenants, conditions and agreements hereinafter reserved and contained, by Lessee, to be paid, kept, observed and performed, Condiotti Enterprises, Inc. ("Lessor") leases unto Ultragenyx Pharmaceutical, Inc., a Delaware corporation ("Lessee") and Lessee hires from Lessor the premises described in the Basic Lease Information (the Premises) together with appurtenances, situated in Building "L" at 60 Leveroni Court, located in Lakepoint Business Park (the Project), in the city of Novato, county of Marin, state of California.

Said hiring and letting is upon each and every of the following terms and conditions:

1. BACKGROUND:

The Lessor holds a fee simple interest in the Project and represents and warrants that Lessor has right, title and authority to enter into this agreement.

2. LESSEE AUTHORITY:

If Lessee is a corporation, trust or general or limited partnership, or sole proprietor, each individual executing this Lease on behalf of such entity represents and warrants that he and/or she is duly authorized to execute and deliver this Lease on behalf of said entity. Lessee shall, prior to the execution of this Lease, deliver satisfactory evidence to Lessor of such authority. Failure to comply with this requirement within the time stated shall constitute a breach of the Lease.

3. TERM:

The term of this Lease shall be for a period of 5 (five) years, beginning upon the earlier of (a) one hundred and twenty days after full Lease execution , or (b) receipt of a Certificate of Occupancy from the City of Novato.

Lessor shall keep Lessee informed of any changes in the Commencement Date.

If Lessor shall not have tendered possession of the Premises to Lessee such that the occupancy date thereof would be more than one hundred, eighty (180) days after the commencement date set forth in paragraph "a" above, Lessee may at Lessee's option by notice in writing to Lessor, cancel this Lease, in which event Lessor and Lessee shall be discharged from all obligations hereunder. In the event this Lease is cancelled by Lessee due to Lessor's failure to diligently pursue work on the Premises to be performed by it, Lessor shall be discharged from all obligations under this Lease and shall not be liable for any other costs or damages which Lessee may suffer, except for return of advance rent payments and security deposits made by Lessee.

4. SECURITY DEPOSIT:

Lessee shall deposit with Lessor upon execution hereof the sum indicated on the Basic Lease Information and as further detailed in Addendum 1, Section 45 as security for Lessee's full and faithful performance of Lessee's obligations hereunder. Such deposit does not constitute an advance payment of last months' rent. If Lessee fails to pay rent or other charges due hereunder or otherwise defaults with respect to any provision of this Lease, Lessor may use, apply or retain all or any portion of such deposit for payment of any rent or other charge in default, or for the payment of any other sum to which Lessor may become obligated by reason of Lessee's default, or to compensate Lessor for any loss or damage which Lessor may suffer thereby. Should Lessor so use, apply or retain all or any portion of such deposit, Lessee shall, within five (5) days after written demand therefore, deposit certified funds with Lessor in an amount sufficient to restore such deposit to the full amount herein stated, and Lessee's failure to do so shall be a material breach of this Lease.

Lessor shall not be required or in any way obligated to keep such deposit separate from its general accounts. No trust relationship is created herein between Lessor and Lessee with respect to such deposit. If Lessee performs all of Lessee's obligations hereunder, such deposit, or so much thereof as has not therefore been used, applied or retained by Lessor, shall be returned, without payment of interest or other increment for its use, to Lessee within twenty-one (21) days of the later of Lease expiration or Lessee's vacating the Premises and return of keys to Lessor.

5. USE:

The Premises are hereby leased to Lessee upon the express condition that Lessee shall use said Premises ONLY for the use indicated in the Basic Lease Information.

Lessee agrees that utility and/or fire sprinkler riser closets are considered “Common Area” and are not for use by Lessee as storage, or for any other use (except as may be appropriate for telecommunications equipment, when approved in advance in writing by Lessor). Lessee shall at all times maintain clear access to the utility and/or fire sprinkler riser closets.

Lessee agrees that Lessee’s business shall be established and conducted through the term hereof in a first class manner, which shall include acquisition and maintenance of any and all licenses, permits, etc. which are required by any governmental agency having jurisdiction over Lessee and/or Lessee’s business. Lessee will not use the demised Premises for, or carry on or permit upon said Premises any offensive, noisy, dangerous or unlawful trade, business, manufacture or occupation or any nuisance or violation of public policy, nor permit any auction sale to be held or conducted on or about said Premises. Lessee shall not commit, or suffer to be committed, any waste upon the Premises. Lessee shall not do or suffer anything to be done upon said Premises which will cause structural injury to said Premises or the Building of which same form a part; that said Premises will not be overloaded and that no machinery, apparatus or other appliance shall be used or operated in or upon the Premises which will in any manner injure, vibrate or shake said Premises or the Building of which it is a part. No use will be made of the Premises which will in any way impair the efficient operation of the sprinkler system within the Building containing the Premises. Lessee will not vacate or abandon said Premises during the term hereof unless pursuant to an assignment of this Lease or subletting of the Premises approved by Lessor in the manner described elsewhere in this Lease; and that without the advance written permission of Lessor no musical instrument of any sort, or any noise-making device will be operated or allowed upon said Premises for the purpose of attracting trade or otherwise.

Lessee further agrees not to use or permit the use of the Premises or any part thereof for any purpose prohibited by law or which will increase the existing rate of insurance upon the Building in which the Premises may be located, or cause a cancellation of any insurance policy covering said Building or any part thereof. If any act or use of Premises on the part of Lessee shall cause, directly or indirectly, any increase of Lessor’s insurance premiums said additional premiums shall be paid by Lessee to Lessor upon demand. No such payment by Lessee shall limit Lessor in the exercise of any other rights or remedies, or constitute a waiver of Lessor’s right to require Lessee to discontinue such act or use.

No use shall be made or permitted to be made of the Premises or any part thereof, and no act done therein, which may unreasonably disturb the quiet enjoyment of any other tenant in the Project or the Building of which the Premises are a part.

Lessee, at Lessee’s sole cost and expense, agrees to do all things necessary to:

1. maintain the Premises in a clean, neat, sanitary and safe manner;
2. to repair and maintain the interior of the Premises forming all or part of the Building in compliance and conformity with all municipal, state, federal laws and ordinances, and with the requirements of any other governmental board or authority, present or future, in any way relating to the condition, use or occupancy of the Premises throughout the entire term of this Lease and to the perfect exoneration from liability of Lessor,
3. or if due to Lessee’s specific use of the Premises any governmental authority requires alterations, Lessee shall make such alterations at its sole cost and expense.

The judgment of any court or the admission of Lessee in any action or proceeding against Lessee, whether Lessor be a party thereto or not, that Lessee has violated any such law, ordinance, requirement or order in the use of the Premises, shall be conclusive of that fact between Lessor and Lessee.

Lessee shall keep at the Premises, and maintain in good working condition, a sufficient number of fire extinguishers as determined by the Novato Fire Department or other agency of authority in this regard.

Lessee shall not, without prior written consent from Lessor, use any common area of the Project or Building(s) for any purpose not specifically granted in this Lease and Attachments (if any).

6. ACCEPTANCE OF PREMISES BY LESSEE:

Lessee has examined the Premises as of the date of this Lease and acknowledges and covenants to Lessor that the same is in the condition represented by Lessor and fit and suitable for the use and purpose demised. Lessee hereby accepts the Premises “AS IS”, subject to the provisions of Addendum 1, Section 48, and shall indemnify and defend Lessor from and against any claim or action that may arise from any defective or allegedly defective condition existing in or with respect to the Premises.

7. RENT:

a. Starting with the commencement date of this Lease, Lessee shall pay to Lessor the Base Monthly Rent as indicated in the Basic Lease Information. The Base Monthly Rent shall be paid on the first (1st) day of each month during the term thereof without notice, demand or any offset, and shall be paid in lawful money of the

United States. Lessee shall pay to Lessor one month's Base Monthly Rent upon execution of this Lease, which shall be applied to the first (1st) month rent due hereunder.

b. If the commencement date does not fall on the first (1st) of the month, or if Lessee with Lessor's consent occupies the Premises prior to the commencement date, Lessee shall pay to Lessor rent for the first month of the term, prorated at 1/30th of the Basic Monthly Rent thereof per day. In this case, Lessee's anniversary date shall be considered to be the first (1st) day of the month immediately following occupancy.

c. The Basic Monthly Rent hereunder shall be increased during the term of the Lease as hereinafter provided, but in no event shall any adjustment to rent result in a reduction below the initial Basic Monthly Rent or any subsequently determined rent, whichever is higher.

d. Rent shall be payable to Lessor at the address listed on the Basic Lease Information or to such other person(s) or at such other place(s) as Lessor may designate in writing.

8. COST OF LIVING ADJUSTMENT:

On each and every anniversary following the commencement of this Lease, the Basic Monthly Rent shall be adjusted for the ensuing twelve (12) months by the proportion that the Consumer Price Index All Items, All Urban Consumers in the San Francisco Bay Area, of the U.S. Department of Labor, Bureau of Labor Statistics, then most recently published, bears to the Consumer Price Index All Items, All Urban Consumers in the San Francisco Bay Area most recently published prior to the previous anniversary. Should the U.S. Department of Labor discontinue its computation and publication of said Consumer Price Index or the publication thereof should be delayed so as to prevent its use hereunder at the times required, there shall be substituted therefore by Lessor such other index or method of ascertaining changes in the price levels as, in the opinion of the Lessor, most closely resembles the Consumer Price Index and method of arriving at the index figure by said Bureau. The annual increase shall not be less than three percent (3%) nor more than six percent (6%).

9. OPERATING EXPENSES:

Lessee shall pay to Lessor in addition to the Basic Monthly Rent during the term hereof, Lessee's share of all Operating Expenses, as hereinafter defined, during each month of the term of this Lease. "Lessee's share" is defined, for the purposes of this Lease, as the percentage determined by division of Lessee's Premises square footage divided by the rentable square footage of the Project. As improvements, additions and deletions may affect the Project square footage, and/or Lessee may add space, this percentage may change from time to time. "Operating Expenses" is defined, for the purposes of this Lease, as all costs incurred by Lessor, including, but not limited to:

a. the operation, repair and maintenance in neat, clean, good order and condition, of the common areas, including, but not limited to parking areas, loading and unloading areas, trash enclosures, roadways, sidewalks, parkways, driveways, landscaped areas, striping, irrigation systems, common area lighting, fences and gates;

b. Fire detection systems, including sprinkler maintenance, monitoring and repair;

c. Project management fees;

d. The cost of premiums for liability, property and business income insurance policies elsewhere described in this Lease to be responsibility of Lessor and any deductible portion of an insured loss covered by said policies;

e. Utility services to the Common Areas;

f. Capital expenditures such as roofing, HVAC replacement, and parking lot resurfacing, etc. pro-rated over their useful life as reasonably determined by Lessor;

g. The pro-rata portion of a maintenance contract for the HVAC,

h. All costs relating to Lessor's employees for the Project;

i. Other costs connected with the operation and maintenance of the Project.

The inclusion of the improvements, facilities and services set forth above shall not be deemed to impose an obligation upon Lessor to either have or provide said improvements, facilities or services unless the Project already has the same, Lessor already provides the services, or Lessor has agreed elsewhere in this Lease to provide the same or some of them.

Lessee's share of Operating Expenses shall be payable by Lessee within five (5) days after a statement of expenses is presented to Lessee by Lessor, which shall include copies of billing documents applicable to Operating Expenses except when such expenses have been amortized over more than one month and copies have been previously provided. At Lessor's option, however, an amount may be estimated by Lessor from time to time of Lessee's share of Operating Expenses, which estimate shall be due within five (5) days after such estimate is presented to Lessee by Lessor. Lessor shall prepare and present to Lessee an actual statement of expenses as soon as is

practical after such estimate has been presented, and shall credit Lessee any over-charge or bill any short-charge as may be actually due.

10. UTILITIES:

Lessee shall pay for all water, gas, heat, light, power, telephone and other utilities and services supplied to the Premises, together with any taxes thereon. If any such services are not separately metered to Lessee, Lessee shall pay a reasonable proportion, to be determined by Lessor, of all charges jointly metered with other premises. Lessor's reasonable determination thereof, in good faith, shall be conclusive. Lessor shall have exclusive right of selection of power provider.

11. INSURANCE:

a.) Liability Insurance: Lessee shall, at Lessee's expense, obtain and keep in force throughout the term of this Lease, beginning upon Lessee taking possession of the Premises or the Commencement Date, whichever occurs first, and for a period of not less than two years following the expiration of this Lease, a policy or policies of General Liability Insurance insuring Lessor and Lessee against any liability arising out of the ownership, use, occupancy or maintenance of the Premises and all areas appurtenant thereto. Such insurance shall provide bodily injury, property damage, personal injury, advertising injury, contractual liability and products-completed operations coverage and shall have combined single limits in an amount not less than $2,000,000 per occurrence or offense. The policy or policies shall provide fire legal liability coverage with limits of at least $1,000,000.00. The policy or policies shall also insure the risks assumed by Lessee of the indemnity provisions of Section 15 of this Lease. The limits of said insurance shall not, however, limit or decrease the liability of Lessee hereunder.

b.) Additional Insured/Primary Insurance: Lessee will insure that the policy or policies required by subparagraph 11a name Lessor as an additional insured and further provide that such insurance shall be primary to, and non-contributory with, any and all insurance maintained by Lessor. Lessee shall provide Lessor with an endorsement to the policy or policies required by subparagraph 11a, substantially identical to Exhibit "D" to this Lease.

c.) Property Insurance: Lessee shall obtain and keep in force during the term of this Lease a policy or policies of insurance covering loss or damage to its fixtures, equipment, tenant improvements, inventory and other contents stored in the Premises, whether the property of Lessee or anyone else, in the amount of the full replacement value thereof, as the same may exist from time to time. Such policy or policies shall contain a waiver of subrogation provision specifying that the insurer(s) waives any right of recovery against Lessor to recover any payments made under the policy or policies.

d.) Insurance Policies: Insurance required hereunder shall be in companies holding a "General Policyholders Rating" of at least "A", or such other rating as may be required by a lender having a lien on the Premises, as set forth in the most current issue of "Best's Insurance Guide". The insuring party shall deliver to the other party copies of policies of such insurance or certificates evidencing the existence and amounts of such insurance with loss payable clauses as required by this Paragraph. No such policy shall be cancelable or subject to reduction of coverage or other modification except after thirty (30) days prior written notice to Lessor. If Lessee is the insuring party, Lessee shall, at least thirty (30) days prior to the expiration of such policies, furnish Lessor with renewals or binders thereof, or Lessor may, at Lessor's sole option and discretion, order such insurance and charge the cost thereof to Lessee, which amount shall be payable by Lessee immediately upon demand.

e.) Waiver of Claims and Right of Subrogation: Lessor shall not be liable to Lessee, nor shall Lessor be liable to any person claiming under Lessee by lease, license, assignment or subrogation by operation of law or contract, or upon the Premises with the express, implied or constructive consent of Lessee, or for any loss of, or damage to or the destruction of the Premises, or some part or parts thereof, or any goods, furniture, fixtures or equipment thereon, for the interruption of any business, or for any injury to the person (including wrongful death) or property of the Lessee or to any such persons claiming under Lessee, resulting from any cause whatsoever, including without limitation the sole negligence of Lessor, any broken pipe, water seepage, blocked drain, defective wiring, interruption in power, malfunction of any air conditioning, plumbing system, sewer, storm drain or the like.

f.) Fire and Extended Coverage Insurance: At all times from and after the date of commencement of this Lease, Lessor shall keep the Building of which the Premises are a part, but not the contents of the Premises or any property of Lessee or others, insured for the mutual benefit of Lessor and Lessee against (i) loss or damage by fire and such other risks as may be included in a standard form of Extended Coverage Insurance from time to time available, in amounts sufficient to prevent Lessor or Lessee from becoming a co-insurer within the terms of the applicable policies and, in any event, in an amount not less than one hundred percent (I00%) of the then full replacement value of the Building, together with an addendum thereto providing for six (6) months rental income coverage payable to Lessor, (ii) loss or damage from leakage of sprinkler systems now or hereafter installed in the Building by Lessor in such amount as Lessor shall reasonably establish, and (iii) loss or damage

resulting from explosion of steam boiler, air conditioning equipment, pressure vessels or similar apparatus, now or hereinafter installed in or on the Building by Lessor, in such amount as Lessor shall reasonably establish.

g.) Notice by Lessee: Lessee agrees to give prompt written notice to Lessor with respect to all events occurring upon the Premises which may be the subject of claims on insurance policies, whether policies are being maintained by Lessor or Lessee.

12. TAXES:

a.) **Definition of "Real Property Tax**": As used herein the term "real property tax" shall include any form of real estate tax or assessment, general, special, ordinary or extraordinary, and any license, fee, commercial rental tax, improvement bond or bonds, levy or tax (other than inheritance, personal income or estate taxes) imposed on the Premises by any authority having the direct or indirect power to tax including any city, state or federal government or any school, agricultural, sanitary, fire, street, drainage, lighting or other improvement district thereof, as against any legal or equitable interest of Lessor in the Premises or in the real property of which the Premises are a part, as against Lessor's right to rent or other income therefrom, and as against Lessor's business of leasing the Premises. The term "real property tax" shall also include any tax, fee, levy, assessment or charge (i) in substitution of, partially or totally, any tax, fee, levy, assessment or charge hereinabove included within the definition of "real property tax", or (ii) the nature of which was hereinabove included within the definition of "real property tax", or (iii) which is imposed for a service or right not charged prior to June 1, 1978, or, if previously charged, has been increased since June 1, 1978, or (iv) which is imposed as a result of transfer, either partial or total, of Lessor's interest in the Premises or which is added to a tax or charge hereinabove included within the definition of "real property tax" by reason of such transfer, or (v) which is imposed by reason of this transaction, any modification or changes hereto, or any transfers thereof.

There shall be excluded from real property taxes all income taxes, environmental assessments, charges or liens arising in connection with the remediation of Hazardous Materials (as defined in Section 39 below) from the Project or any portion thereof, the causation of which arose prior to the delivery of the Premises to Lessee, or to the extent caused by Lessor; costs or fees (other than real property taxes) attributable to Lessor's transfer of ownership of the Project or any interest of Lessor therein, capital stock, inheritance, estate, gift, or any other taxes imposed upon or measured by Lessor's gross income or profits unless the same is specifically included within the definition of real property taxes above or otherwise shall be imposed in lieu of real estate taxes or other ad valorem taxes. For example, assessment district fees would be included in real property taxes.

b.) **Joint Assessment:** If the Premises are not separately assessed, Lessee's liability shall be an equitable proportion of the real property taxes for all of the land and improvements included within the tax parcel assessed plus any property taxes assessed as a result of tenant improvements, such proportion to be determined by Lessor from such information as may be reasonably available. Lessor's determination thereof, in good faith, shall be conclusive. Lessor is not obligated to pro-rate the recovery of such taxes. Lessee shall pay such proportion to Lessor within five (5) days of receipt of Lessor's written notice thereof.

c.) **Personal Property Taxes:** Lessee shall pay prior to delinquency all taxes assessed against and levied upon trade fixtures, furnishings, equipment and all other personal property of Lessee contained in the Premises or elsewhere as it may affect the Project. When possible, Lessee shall cause said trade fixtures, furnishings, equipment and all other personal property to be assessed and billed separately from the real property of the Lessor. If any of Lessee's personal property shall be assessed with Lessor's real property, Lessee shall pay the taxes attributable to Lessee within five (5) days of receipt of written notice thereof from Lessor or taxing entity.

13. REPAIRS:

a.) **Repairs by Lessee:** Lessee agrees at its sole cost and expense to maintain, repair and keep the interior of the Premises and all building systems within and serving the Premises though not necessarily located within the Premises forming a part of the Building, and all appurtenances (including without limitation wiring, plumbing, sewage system, heating and air cooling installation, all glazing and doors in or bordering the Premises and any store front) in good condition and repair during the term of this Lease, excepting only the foundations and structural portions of the Premises, damage thereto by fire, earthquake, civil insurrection and act of God or the elements. Upon request from Lessor, Lessee shall provide Lessor with evidence satisfactory to Lessor of Lessee's maintenance contracts and invoices of repair. In the event Lessee fails to make the repairs required of Lessee within thirty (30) days from delivery of Lessor's notice to Lessee to make such repairs, Lessor, in addition to all other remedies available hereunder or by law, and without waiving any said alternative remedies, may make same and

Lessee agrees to repay Lessor the cost thereof, plus ten percent (10%) of costs for Lessor's administration expenses, within five (5) days of delivery of Lessor's demand thereof. Failure by Lessee to pay same shall carry with it the same consequences as failure to pay any installment of rent. To the extent Lessee is responsible for such maintenance, repair and replacement, Lessee agrees during the full term of this Lease, at its sole cost and expense, to make all repairs and replacements of whatever kind or nature, to either the interior or exterior of the Premises, rendered necessary by reason of any act or omission of Lessee or its agents, servants or employees. Lessee waives all rights to make repairs at the expense of Lessor as provided for in any statute or law in effect at the time of the execution of this Lease or any amendment thereof or any other statute or law which may be hereafter enacted during the term of this Lease, or sooner termination hereof, and to surrender unto Lessor the Premises in the same condition as received, ordinary wear and tear and damage by fire, earthquake, civil insurrection, act of God or the elements alone excepted.

b.) **Repairs by Lessor:** Lessor agrees, after receipt of written notice of the necessity therefore, and should same not be caused by Lessee or by reason of Lessee's occupancy, or its misuse, negligence, or alterations to the Premises to initiate necessary repairs to the fire sprinkler system, foundations and other structural portions of the Premises which could be a part of the Building, the common areas, the roof, exterior walls, parking areas, landscaping, wiring to the Building, plumbing and sewage system exterior to the Premises, within thirty (30) days or as soon as is reasonably practical.

14. ALTERATIONS; LIENS; PERMITS:

Lessee agrees not to make any structural alterations of, changes in or additions to the Premises. Lessee agrees not to make any non-structural alterations of, changes in or additions to the Premises without the prior written consent of Lessor. Lessee shall be required to submit plans, specifications and a budget, as well as the name, address and license number of the general contractor performing the work, along with the application for Lessor's consent. Lessee agrees that all non-structural alterations, changes, additions and improvements, including fixtures, made in, to or on the Premises, except unattached movable business fixtures, shall be the property of Lessor at termination of Lease and shall remain upon and be surrendered with the Premises. Should Lessor so desire, Lessee shall restore the Premises or such part or parts thereof by the end of the term of this Lease at Lessee's sole cost and expense. Lessee agrees that if any such alterations, changes, additions or improvements are approved by Lessor, Lessee shall take all steps necessary to assure the completion of same in a good and workmanlike manner and in compliance with applicable code, and shall, at Lessee's sole cost and expense, secure any permits or licenses which may be required by government agency. Lessee agrees that no such alterations, changes, additions or improvements will commence until seven (7) days after Lessee's receipt of written consent of Lessor as required by this Paragraph in order that Lessor may post appropriate notices to avoid liability on account thereof. Lessee agrees to indemnify and save harmless Lessor from all liens, claims or demands arising out of any work performed, materials furnished or obligations incurred by or for Lessee upon the Premises during the entire term of this Lease, and agrees not to suffer any such lien or other lien to be created.

15. HOLD HARMLESS:

This Lease is made upon the express condition that Lessee agrees to indemnify, keep, save and hold Lessor free and harmless from all liability, penalties, losses, damages, costs, expenses, causes of action, claims and/or judgments arising by reason of any injury or damage to any person or persons, including without limitation, Lessee, its invitees, servants, agents and employees, or property of any kind whatsoever and to whomsoever belonging, including, without limitation, Lessee, its invitees, servants', agents' and employees', from any cause or causes whatsoever, excepting the sole negligence of Lessor, while in, upon, or in any way connected with said demised Premises or the Building or Project of which the Premises are a part, or its appurtenances, or the sidewalks or other areas adjacent thereto, during the term of this Lease or any occupancy hereunder. Lessee hereby covenants and agrees to defend, indemnify, protect and save Lessor harmless from all liability, loss, costs and obligations on account of or arising out of any such claims, injuries or losses, however occurring, excepting those that are a result of Lessor's sole negligence.

In the event of any breach by Lessee of any of the requirements of Paragraph 11 Insurance, then Lessee's indemnity obligations shall be extended to include any benefits to which Lessor would have been entitled under the policy or policies described in Paragraph 11 Insurance, and Lessee shall be obligated to provide Lessor with all of the benefits that would have been furnished under such policy or policies, and Lessee shall assume all of the obligations of an insurer under such policy or policies in addition to any other remedy available to Lessor, either provided in this Lease or by law.

Lessee, as a material part of the consideration to be rendered to Lessor, hereby waives all claims against Lessor for damages to goods, wares and merchandise in, upon or about the Premises and for injuries to Lessee, his agents or

third persons in or about the Premises from any cause arising at any time including, without limiting the generality of the foregoing, damages arising from the sole negligence of Lessor, from acts or omissions of other tenants of the Building or Project of which the Premises are a part and from failure of Lessor to make repairs.

16. ENTRY AND INSPECTION:

Lessee agrees that Lessor and his agents may enter upon the Premises to inspect same, to submit them to a prospective purchaser, lender or lessee or to make any changes, alterations or repairs which Lessor shall consider necessary for the protection, improvement or preservation thereof, or of the Building in which the Premises are situated, or to make changes in the plumbing, wiring, meters or other equipment, fixtures or appurtenances of the Building, or to post any notice provided for by law, or otherwise to protect any and all rights of Lessor. Lessor shall provide Lessee reasonable notice of intent to enter, except in case of emergency. Lessor shall have the right to erect and maintain all necessary or proper scaffolding or other structures for the making of such changes, alterations or repairs (provided the entrance to the Premises shall not be blocked thereby and that such work shall be completed with diligence and dispatch) and there shall be no liability against Lessor for damages thereby sustained by Lessee, nor shall Lessee be entitled to any abatement of rent by reason of the exercise by Lessor of any such rights herein reserved. Nothing herein contained shall be construed to obligate Lessor to make any changes, alterations or repairs. Lessee further agrees that at any time after six (6) months prior to the termination of this Lease, Lessor may place thereon “To Let” or “To Lease” signs.

17. EASEMENTS:

Lessor reserves to itself the right, from time to time, to grant such easements, rights and dedications that Lessor deems necessary or desirable and to cause the recordation of parcel maps and restrictions, so long as such easements, rights, dedications, maps and restrictions do not unreasonably interfere with the use of the Premises by Lessee. Lessee shall sign any of the aforementioned documents upon request of Lessor and failure to do so in a timely manner shall constitute a material breach of this Lease.

18. ESTOPPELS:

Lessee agrees, at any time and from time to time within seven (7) days of written request from Lessor to execute, acknowledge and deliver to Lessor a statement in writing certifying that this Lease is unmodified and in full force and effect (or, if there have been modifications, that the same is in full force and effect as modified, and stating the modifications) and the dates to which rent and other charges have been paid in advance, if any, and any other matters reasonably requested by Lessor, it being intended that any such statement delivered pursuant to this Paragraph may be relied upon by any present or perspective purchasers, mortgagee(s) or assignee(s) of any mortgagee of the Premises. Failure of Lessee to comply with any and all provisions of this Paragraph shall constitute a breach of the Lease.

19. MULTIPLE TENANT BUILDING:

In the event that the Premises are a part of a larger building or group of buildings, Lessee agrees that it will abide by, keep and observe all reasonable rules and regulations which Lessor may make from time to time, including but not limited to the management, safety, operation, care and cleanliness of the Building and grounds, the parking of the vehicles and the preservation of good order therein as well as for the convenience of other occupants and tenants of the Project. Violation of any such rules and regulations shall be deemed a breach of this Lease by Lessee.

20. SIGNS:

Lessee agrees not to inscribe, paint or affix any signs, advertisements, placards or awnings on the exterior or roof of the Premises or upon the entrance doors, windows, monuments, or the sidewalk on or adjacent to the Premises without the written consent of Lessor first obtained. Any signs so placed on the Premises shall be so placed upon the understanding and agreement that Lessee will remove same at the termination of tenancy herein created and repair any damage or injury to the Premises caused thereby, and if not so removed by Lessee, then Lessor may have same so removed at Lessee’s expense. Lessee shall not be allowed to use the name of the Project, or words to such effect in connection with any business carried on in the Premises (except as the address of the Lessee) without the written consent of Lessor. Lessor reserves the right to change the name and title of the Project and Building at any time during the term of said Lease. Lessee hereby expressly agrees to such change at the option of Lessor and waives any and all damage occasioned thereby.

21. ABANDONMENT:

If Lessee should abandon or surrender Premises or be dispossessed by process of law, in addition to all other remedies of Lessor, Lessor, at its option, may deem that any personal property belonging to Lessee left on the Premises is

abandoned and/or Lessor may at once enter upon the Premises and remove therefrom any and all equipment, fixtures and merchandise therein and may sell same at public or private sale at such price and upon such terms as Lessor may determine, without notice to or demand upon Lessee. Out of the proceeds of such sale Lessor may reimburse itself for the expense of such taking, removal and sale and for any indebtedness of Lessee to Lessor and the surplus, if any, shall be accounted for to Lessee at Lessee's last known address. Abandonment of the Premises shall be deemed a breach of the Lease.

22. DESTRUCTION; RENEWAL:

A. In the event of damage or destruction to the Premises during the term hereof which Lessor is obligated to repair by the terms of this Lease, Lessor shall forthwith repair the same, provided such repairs can be made within ninety (90) days under the laws and regulations of State, Federal, County or Municipal authorities, but such destruction shall in nowise annul or void this Lease, except that Lessee shall be entitled to a proportionate reduction of rent to be based upon the extent to which the making of such repairs shall interfere with the business carried on by Lessee in the Premises. If such repairs cannot be made within ninety (90) days, Lessor may, at its option, make same within a reasonable time, in which event this Lease shall continue in full force and effect and the monthly rent shall be proportionately reduced as aforesaid in this Paragraph provided. In the event that Lessor does not so elect to make such repairs which cannot be made within one hundred eighty (180) days, this Lease may be terminated at the option of either party. In the event that the Building of which the Premises are a part is damaged or destroyed to the extent of one-third (33.33%) or more of the replacement cost thereof, Lessor may elect to terminate this Lease, whether the Premises be injured or not.

B. Notwithstanding anything herein to the contrary, if, at any time during the term hereof, any governmental agency having jurisdiction over the Premises of Building(s) of which the Premises are a part, shall require the making of any repairs, improvements or alterations to said Building(s) or Premises, and Lessor determines to demolish said Building(s) or Premises rather than to make said repairs, improvements or alterations, or allow same to be made, Lessor, upon written notice to Lessee as soon as is practical, shall have the right to terminate this Lease. Upon the date specified in such notice, this Lease shall terminate and Lessor shall have no further liability to Lessee except that Lessor shall refund to Lessee any unearned rents and shall return the balance of security deposit after such offsets as may be provided in this Lease. In the event Lessor had heretofore given written consent to any leasehold improvements upon the Premises made and paid for by Lessee, and had agreed, in writing, as to the cost thereof, Lessor shall pay to Lessee upon such termination the unamortized portion of such cost as the number of full calendar months remaining in the original term of this Lease bears to the total number of calendar months is said original term.

23. CONDEMNATION:

If any part of the Premises or of the Building of which same are a part (even if no part of the Premises be taken) be condemned for a public or quasi-public use by right of eminent domain, with or without litigation, or transferred by agreement in connection with such public or quasi-public use, this Lease, as to the part so taken, shall terminate as of the date title shall vest in the condemnor, and the rent payable hereunder shall be adjusted so that the Lessee shall be required to pay for the remainder of the term only such portion of rent as the value of the part remaining after condemnation bears to the value of the entire Premises at the date of condemnation. In such event Lessor shall have the option to terminate this Lease as of the date when title to the part so condemned vests in the condemnor. If more than one-third (33.33%) of the Premises is taken, Lessee at Lessee's option to be exercised in writing within ten (10) days after Lessor shall have given written notice to Lessee of such taking (or in absence of such notice, within ten (10) days after the condemning authority shall have taken possession or title), terminate this Lease as of the date the condemning authority takes such possession or title.

All compensation awarded upon such condemnation or taking shall belong and be paid to Lessor and Lessee shall have no claim thereto, and Lessee hereby irrevocably assigns and transfers to Lessor any right to compensation or damages to which Lessee may become entitled during the term hereof by reason of the condemnation of all or part of the Premises.

24. SALE OF PREMISES:

In the event of a sale or conveyance by Lessor of the Premises, the same shall operate to release Lessor from any future liability upon any of the covenants or conditions, express or implied, herein contained in favor of Lessee, and in such event Lessee agrees to look solely to the responsibility of the successor in interest of Lessor. If any security be given by Lessee to secure faithful performance of Lessee's covenants in this Lease or any rent shall have been prepaid, Lessor may transfer same as such to the purchaser of the reversion thereon and thereupon Lessor shall be discharged from any further liability in reference thereto.

25. COSTS OF SUIT:

Lessee agrees that if Lessor is involuntarily made a party defendant to any litigation concerning this Lease or the Premises by reason of any act or omission of Lessee and not because of any act or omission of Lessor, then Lessee shall hold Lessor harmless from all liability by reason thereof, including reasonable attorney's fees incurred by Lessor in such litigation and all court costs. If legal action be brought by either party hereto for the unlawful detainer of the Premises, for the recovery of any rent due under the provisions of this Lease, or due to any breach of any term, covenant, condition or provision thereof, the party prevailing in said action (Lessor or Lessee as the case may be) shall be entitled to recover from the party not prevailing court costs and a reasonable attorney's fee which shall be fixed by the Judge of the Court. The entry of a dismissal without prejudice shall not create a prevailing party.

If any action be instituted by Lessor to enforce any provision of this Lease and/or collect any sum due hereunder, Lessor shall recover from Lessee all costs connected with such enforcement and/or collection.

26. SECURITY MEASURES:

Lessee hereby acknowledges that the rent payable to Lessor hereunder does not include the cost of guard service or other security measures, and that Lessor has no obligation whatsoever to provide it. Lessee assumes all responsibility for the protection of Lessee, its agents and invitees, the Premises and any property on the Premises from acts of third parties.

27. PERFORMANCE UNDER PROTEST:

If at any time a dispute shall arise as to any amount or sum of money to be paid by one party to the other under the provisions hereof, the party against whom the obligation to pay the money is asserted shall have the right to make payment "under protest" and such payment shall not be regarded as a voluntary payment, and there shall survive the right on the part of said party to institute suit for recovery of such sum. If it shall be adjudged that there was no legal obligation on the part of said party to pay such sum or any part thereof, said party shall be entitled to recover such sum or so much thereof as it was not legally required to pay under the provisions of this Lease.

28. ASSIGNMENT AND SUBLETTING:

a. **Lessor's Consent Required:** Lessee shall not voluntarily or by operation of law assign, transfer, mortgage, sublet or otherwise transfer or encumber all or any part of Lessee's interest in this Lease or in the Premises without Lessor's prior written consent, which consent shall not be unreasonably withheld or conditioned. Lessee shall provide Lessor with documentation including, but not limited to the sublease or assignment document, the legal name of the proposed subtenant/assignee, financial statements for the proposed subtenant/assignee, proposed use of the premises, the type of business to be conducted by proposed subtenant/assignee, and any other documentation required by Lessor to make a reasonable determination. (For the purposes of this section 28, <u>if the combined vacancy in 52, 55, 60 and 68 Leveroni exceeds 25%</u>, it shall be deemed unreasonable for a tenant or approved subtenant or assignee to sublet or assign the Premises at less than the adjusted Base Rent payable under this Lease.) Lessor shall respond to Lessee's request for consent hereunder in a timely manner. Any attempted assignment, transfer, mortgage, encumbrance or subletting without Lessor's written consent shall be void and shall constitute a breach of this Lease. <u>In no event shall Lessee have the right to sublease beyond Lessee's lease term.</u>

b. **No Release of Lessee:** Regardless of Lessor's consent, no subletting or assignment shall release Lessee of Lessee's obligation or alter the primary liability of Lessee to pay the rent and to perform all other obligations to be performed by Lessee hereunder. The acceptance of rent by Lessor from any other person or entity shall not be deemed a waiver by Lessor of any provision hereunder. Consent to one assignment or subletting shall not be deemed consent to any subsequent assignment or subletting. In the event of default by any assignee of Lessee or any successor of Lessee in the performance of any of the terms hereof, Lessor may proceed directly against Lessee without the necessity of exhausting remedies against said assignee or sub-tenant. Lessor may or may not consent to subsequent assignments or subletting of this Lease, or amendments or modifications to this Lease with assignees of Lessee, with or without notifying Lessee, or any successors of Lessee, and without obtaining its or their consent thereto and such action shall not relieve Lessee of liability under this Lease.

c. **Attorney's Fees:** In the event Lessee shall assign or sublet the Premises or request consent of Lessor to any assignment or subletting, or if Lessee shall request the consent of Lessor for any act Lessee proposes to do, then Lessee shall pay Lessor's attorney's fees and any other costs incurred in connection therewith.

29. CONFLICT:

Any conflict between the printed provisions of this Lease and the typewritten or handwritten provisions shall be controlled by the typewritten or handwritten provisions, where both parties have initialed such provisions.

30. SUBORDINATION:

a. This Lease, at Lessor's option, shall be subordinate to any ground lease, mortgage, deed of trust, or any other hypothecation or security now or hereafter placed upon the real property of which the Premises are a part and to any and all advances made on the security thereof and to all renewals, modifications, consolidations, replacements and extensions thereof. If any mortgagee, trustee or ground Lessor shall elect to have this Lease prior to the lien of its mortgage, deed of trust or ground lease, and shall give written notice thereof to Lessee, this Lease shall be deemed prior to such mortgage, deed of trust or ground lease on the date of recording thereof.

b. Lessee agrees to execute any documents required to effectuate an attornment, a subordination, or to make this Lease prior to the lien of any mortgage, deed of trust or ground lease, as the case may be. Lessee's failure to execute such documents within seven (7) days after written demand shall constitute a default by Lessee hereunder.

31. SURRENDER OF LEASE:

No act or conduct of Lessor, whether consisting of the acceptance of the keys to the Premises, or otherwise, shall be deemed to be or constitute an acceptance of the surrender of the Premises by Lessee prior to the expiration of the term hereof, and such acceptance by Lessor of surrender by Lessee shall only flow from and must be evidenced by a written acknowledgement of acceptance of surrender by Lessor. The voluntary or other surrender of this Lease by Lessee, or a mutual cancellation thereof, shall not work a merger and shall, at the option of the Lessor, terminate all or any existing subleases, or subtenancies or may, at the option of the Lessor, operate as an assignment to it of any or all such subleases, or subtenancies.

32. HOLDING OVER:

Should Lessee hold over the term hereby created with written consent of Lessor, Lessee shall become a tenant from month to month at one hundred twenty-five percent (125%) of the rent payable hereunder for the prior six (6) months, and otherwise upon the covenants and conditions in this Lease contained, and shall continue to be such tenant until thirty (30) days after either party hereto serves upon the other written notice of intent to terminate such monthly tenancy. Should such termination occur on any day other than the last day of the month, any unearned prepaid rent shall be prorated based on a 30 day month and shall be credited to Lessee's account as additional security deposit, to be disbursed according to the provisions of Paragraph 4.

33. NOTICES:

It is agreed between the parties hereto that any notice required hereunder or by law to be served upon either of the parties shall be in writing and shall be delivered personally upon the other or sent by first class mail, postage prepaid, addressed to the addresses listed in the Basic Lease Information or such other address as may be from time to time furnished in writing by Lessor to Lessee or by Lessee to Lessor, each of the parties waiving personal or any other service than as in this Paragraph provided for. Notice by first class mail shall be deemed to be communicated the second business day from the time of mailing. Notice by any other means shall be effective upon delivery, or refusal of delivery.

34. DEFAULTS; REMEDIES:

a. **Defaults:** The occurrence of any one or more of the following events shall constitute a default and breach of this Lease by Lessee:

i. The vacating or abandonment of the Premises by Lessee or assignee or sublessee as applicable.

ii. The failure of Lessee to make any payment of rent or any other payment required to be made by Lessee hereunder, as and when due.

iii. The failure by Lessee to observe or perform any conditions, covenants or provisions of this Lease to be observed or performed by Lessee, other than described in Section 34a(ii) above, and where such failure shall continue for a period of seven (7) days after written notice thereof from Lessor to Lessee provided, however, that if the nature of Lessee's default is such that more than seven (7) days are reasonably required for its cure, then Lessee shall not be deemed to be in default if Lessee commenced such cure within said seven (7) day period and thereafter diligently prosecutes such cure to completion.

iv. The making by Lessee of any general arrangement or assignment for the benefit of creditors;

v. The appointment of a trustee or receiver to take possession of substantially all of Lessee's assets located at the Premises or of Lessee's interest in this Lease, where possession is not restored to Lessee within thirty (30) days; or

vi. The attachment, execution or other judicial seizure of substantially all of Lessee's assets located at the Premises or of Lessee's interest in this Lease where such seizure is not discharged within thirty (30) days. Provided, however, in the event that any provision in this Paragraph is contrary to any applicable law, such provision shall be of no force or effect.

vii. The discovery by Lessor that any financial information given to Lessor by Lessee, any assignee of Lessee, any subtenant of Lessee, any successor in interest of Lessee or any guarantor of Lessee's obligation hereunder, and any of them, was materially false.

b. **Remedies:** In the event of any such default or breach by Lessee, Lessor may at any time thereafter, by written notice or demand and without limiting Lessor in the exercise of any right or remedy which Lessor may have by reason of such default or breach:

i. Terminate Lessee's right to possession of the Premises by any lawful means, in which case this Lease shall terminate and Lessee shall immediately surrender possession of the Premises to Lessor. In such event Lessor shall be entitled to recover from Lessee all damages incurred by Lessor by reason of Lessee's default including, but not limited to, the cost of recovering possession of the Premises, attorney's fees, any real estate commission actually paid, the balance remaining of unamortized tenant improvements, if any, the worth at the time of award by the court having jurisdiction thereof of the unpaid rent which had been earned at the time of termination; the worth at the time of award of the amount by which the unpaid rent would have been earned after termination until the time of award exceeds the amount of such rental loss that the Lessee proves could have been reasonably avoided; the worth at the time of award of the amount by which the unpaid rent for the balance of the term after the time of award exceeds the amount of such rental loss that the Lessee proves could be reasonably avoided; and any other amount necessary to compensate Lessor for all the detriment proximately caused by Lessee's failure to perform his obligations under the Lease or which in the ordinary course of things would be likely to result therefrom.

ii. Maintain Lessee's right to possession in which case this Lease shall continue in effect whether or not Lessee shall have abandoned the Premises. In such event Lessor shall be entitled to enforce all of Lessor's rights and remedies under this Lease, including the right to recover rent as it becomes due hereunder.

iii. Pursue any other remedy now or hereafter available to Lessor under the laws or judicial decisions of the state wherein the Premises are located. Unpaid installments of rent and other monetary obligations of Lessee under the terms of this Lease shall bear interest from the date due at the maximum rate then allowable by law.

c. **Default by Lessor:** Lessor shall not be in default unless Lessor fails to perform obligations required of Lessor within a reasonable time, after written notice by Lessee to Lessor specifying wherein Lessor has failed to perform such obligations provided, however, that if the nature of Lessor's obligations is such that more than sixty (60) days are required for performance then Lessor shall not be in default if Lessor commences performance within such sixty (60) day period and thereafter diligently pursues the same to completion.

d. **Late Charges:** Lessee hereby acknowledges that late payment by Lessee to Lessor of rent and/or other sums due hereunder will cause Lessor to incur costs not contemplated by this Lease, the exact amount of which will be extremely difficult to ascertain. Such costs include, but are not limited to, processing and accounting charges, and late charges which may be imposed on Lessor by terms of any mortgage or trust deed covering the premises. Accordingly, if any installment of rent or any other sum due from Lessee shall not be received by Lessor (or Lessor's designee, if applicable) within five (5) days after such amount shall be due, then, without any requirement for notice to Lessee, Lessee shall pay to Lessor a late charge equal to six percent (6%) of such overdue amount, plus accrued interest at the maximum amount allowable by law. The parties agree that such late charge represents a fair and reasonable estimate of the costs Lessor will incur by reason of late payment by Lessee. Acceptance of such late charge by Lessor shall in no event constitute a waiver of Lessee's default with respect to such overdue amount, nor prevent Lessor from exercising any of the other rights and remedies hereunder. In the event that a late charge is payable hereunder, whether or not collected, for three (3) installments of rent or other sum, then rent shall automatically become due and payable quarterly in advance, rather than monthly, notwithstanding Paragraph 8 or any other provision of this Lease to the contrary.

e. **Impounds:** In the event that a late charge is payable hereunder, whether or not collected, for three (3) installments of rent or any other sum due hereunder, Lessee shall pay to Lessor, if Lessor shall so request, in addition to any other payments required under this Lease, a monthly advance installment, due on the first (1st) day of each month, as estimated by Lessor for the real property tax, insurance and operating expenses on the Premises which are payable by Lessee under the terms of this Lease. Such fund shall be established to insure

payment when due, before delinquency, of any or all such taxes, premiums and expenses. If the amounts paid to Lessor by Lessee under the provisions of this Paragraph are insufficient to discharge the obligations of Lessee to pay such taxes, premiums and expenses as the same shall become due, Lessee shall pay to Lessor, upon Lessor's demand, such additional sums as necessary to pay such obligations. All monies paid to Lessor under this Paragraph may be intermingled with other monies of Lessor and shall not bear interest. In the event of a default in the obligations of Lessee to perform under this Lease, then any balance remaining from the funds paid to Lessor under the provisions of this Paragraph may, at the option of Lessor, be applied to the payment of any monetary default of Lessee in lieu of being applied to the payment of taxes, premiums and/or expenses.

35. CUMULATIVE REMEDIES; NON-WAIVER:

The receipt by Lessor of any rent or payment with or without knowledge of the breach of any covenant hereof shall not be deemed a waiver of any such breach and no waiver by Lessor of any sum due hereunder or any provision hereof shall be deemed to have been made unless expressed in writing and signed by Lessor. No delay or omission in the exercise of any right or remedy accruing to Lessor upon any breach by Lessee under this Lease shall impair such right or remedy or be construed as a waiver of any such breach theretofore or hereafter occurring. The waiver by Lessor of any breach of any term, covenant or condition herein contained shall not be deemed to be a waiver of any subsequent breach of the same or any other term, covenant or condition herein contained. All rights, powers, options or remedies afforded to Lessor either hereunder or by law shall be cumulative and not alternative and the exercise of one right, power, option or remedy shall not bar other rights, powers, options or remedies allowed herein or by law.

36. ADDITIONAL RENT:

Any monetary obligations of Lessee to Lessor under the terms of this Lease shall be deemed to be rent. All amounts due Lessor are payable in lawful money of the United States of America. Any amount due to Lessor, if not paid when due, shall bear interest from the date due until paid at the maximum amount allowable by law. Payment of interest shall not excuse or cure any default hereunder by Lessee.

37. INCORPORATION OF PRIOR AGREEMENTS; AMENDMENTS:

This Lease contains all agreements of the parties hereto with respect to any matter mentioned herein. No prior agreement or understanding pertaining to any such matter shall be effective. This Lease may be modified in writing only, signed by the parties in interest at the time of the modification. Except as otherwise stated in this Lease, Lessee hereby acknowledges that neither the Lessor nor any employees or agents of any said persons has made any oral or written warranties or representations relative to the condition or use by Lessee of the Premises and Lessee acknowledges that Lessee assumes all responsibility regarding the Occupational Safety Health Act, the legal use and adaptability of the Premises and the compliance thereof with all applicable laws and regulations in effect during the term of this Lease except as otherwise specifically state in this Lease.

38. COMPLIANCE WITH APPLICABLE LAWS:

Lessee shall use the Premises in accordance with all applicable laws and government regulations. If any law or government regulation (such as, but not limited to, the Americans With Disabilities Act of 1990) should require renovation or alteration of the Premises, Lessee shall be responsible for such alteration or renovation and shall pay all costs and expenses required to alter or renovate the Premises to conform to any such law or governmental regulation. If any law or governmental regulation (such as, but not limited to, the Americans With Disabilities Act of 1990) should require the renovation or alteration of the Common Area(s), Lessor shall be responsible for such renovation or alteration and shall pay all costs and expenses required to alter or renovate the Common Area(s) to conform to any such law or governmental regulation. All such costs and expenses required to alter or renovate the Common Area(s) to conform to any such law or governmental regulation shall be included in the Operating Expenses as provided in Paragraph 9 of this Lease. Lessee shall indemnify and defend Lessor against any loss suffered or liability incurred by Lessor, and all actions, suits, damages or claims brought against Lessor as a result of Lessee's failure to use the Premises in accordance with any such law or governmental regulation.

39. ENVIRONMENTAL MATTERS:

A. (i) The term "Hazardous Materials" as used herein shall mean any petroleum products, asbestos, polychlorinated biphenyls, P.C.B.'s, chemicals, compounds, materials, mixtures or substances that are now or hereinafter defined, listed in or otherwise classified as a "hazardous substance", "hazardous material", "hazardous waste", "extremely hazardous waste", "infectious waste", "toxic substance", "toxic pollutant", or any other formulation intended to define, list or classify substances by reason of deleterious properties such as ignitability, corrosivity, reactivity, carcinogenicity or toxicity pursuant to any federal, state or local environmental law, regulation, ordinance, resolution, order or decree relating to industrial hygiene,

environmental protection or the use, analysis, generation, manufacture, storage, release, disposal or transportation of the same ("Hazardous Materials Laws").

(ii) Lessee shall not use, manufacture, store, release, dispose or transport any Hazardous Materials in, on, under or about the Premises, the Building or the Project without giving prior written notice to Lessor and obtaining Lessor's prior written consent, which consent Lessor may withhold in its sole discretion. Lessee shall, at its own expense, procure, maintain in effect and comply with all conditions of any and all permits, licenses and other governmental and regulatory approvals required in connection with Lessee's generation, use, storage, disposal and transportation of Hazardous Materials. Except as discharged into the sanitary sewer in strict accordance and conformity with all applicable Hazardous Materials laws, Lessee shall cause any and all Hazardous Materials to be removed from the Premises and transported solely by duly licensed haulers to duly licensed facilities for final disposal of such materials and wastes. Regardless whether permitted under the Hazardous Materials laws, Lessee shall not maintain in, on, under or about the Premises, the Building or the Project any above or below ground storage tanks, clarifiers or sumps nor shall any wells for the monitoring of ground water, soils or subsoils be allowed.

(iii) Lessee shall immediately notify Lessor in writing of any enforcement, cleanup, removal or other governmental or regulatory action instituted, completed or threatened pursuant to any Hazardous Materials law; any claim made or threatened by any person or entity against Lessee or the Premises, Building and/or Project relating to damage, contribution, cost, recovery, compensation, loss or injury resulting from or claimed to result from any Hazardous Materials; any reports, information, inquiries or demands made, ordered or received by or on behalf of Lessee which arise out of or in connection with the existence or potential existence of any Hazardous Materials in, on, under or about the Premises, the Building or the Project, including, without limitation, any complaints, notices, warnings, asserted violations, or mandatory or voluntary informational filings with any governmental agency in connection therewith, and immediately supply Lessor with copies thereof.

B. Lessee shall indemnify, defend (by counsel reasonably acceptable to Lessor), protect, and hold Lessor, and each of Lessor's partners, officers, directors, partners, employees, affiliates, joint venturers, members, trustees, owners, shareholders, principals, agents, representatives, attorneys, successors and assigns free and harmless from and against any and all claims, liabilities, damages, fines, penalties, forfeitures, losses, cleanup and remediation costs or expenses (including attorney fees) or death of or injury to any person or damage to any property whatsoever, arising from or caused in whole or in part, directly or indirectly by Lessee's use, analysis, generation, manufacture, storage, release, disposal or transportation of Hazardous Materials by Lessee, Lessee's agents, employees, contractors, licensees or invitees to, in, on, under, about or from the Premises, the Building or the Project or Lessee's failure to comply with any Hazardous Materials law. Lessee's obligations hereunder shall include, without limitation, and whether foreseeable or unforeseeable, all costs of any required or necessary repair, cleanup, detoxification or decontamination of the Premises, the Building or the Project, and the preparation and implementation of any closure, remedial action or other required plans in connection therewith, and shall survive the expiration or earlier termination of this Lease.

C. Lessor shall have the right to enter the Premises during regular business hours upon reasonable prior notice at all times for the purposes of ascertaining compliance by Lessee with all applicable Hazardous Materials laws, provided, however, that in the instance of an emergency Lessor's entry onto the Premises shall not be restricted to regular business hours nor shall notice be required.

D. Lessor shall have the option to declare a default of this Lease for the release or discharge of Hazardous Materials by Lessee, Lessee's employees, agents, contractors or invitees on the Premises, Building or Project or in violation of law or in deviation from prescribed procedures in Lessee's use or storage of Hazardous Materials. If Lessee fails to comply with any of the provisions under this entire Paragraph 39, Lessor shall have the right, but not the obligation, to remove or otherwise cleanup any Hazardous Materials from the Premises, the Building or the Project. In such case, the costs of any Hazardous Materials investigation, removal or other cleanup (including, without limitation, transportation, storage, disposal, attorney fees and costs) will be deemed additional rent due under this Lease, whether or not a court has ordered the cleanup, and will become due and payable on demand by Lessor.

E. Lessor shall indemnify, protect, and hold Lessee free and harmless from and against any and all claims, liabilities, damages, fines, penalties, forfeitures, losses, cleanup and remediation costs or expenses (including reasonable attorney fees) or death or injury to any person or damage to any property whatsoever arising from or caused by any Hazardous Materials existing on, under, in or about the Project or any portion thereof (including, without limitation, the Building and the Premises) on or prior to the Commencement Date. Lessor's obligations hereunder shall include, without limitation, all costs of any required or necessary repair, cleanup, detoxification or decontamination of the Premises, the Building or the Project, and the preparation and implementation of any closure, remedial action or other required plans in connection therewith.

40. MISCELLANEOUS:

a. Lessee and Lessee's Guarantor, if any, agree to deliver to Lessor, within thirty (30) days from written request therefore (but not more frequently than once each calendar year) complete financial statements prepared and certified by a Public Accountant or Certified Public Accountant showing the true and accurate net worth of Lessee and said Guarantor, if any, as of the close of Lessee's and said Guarantor's most recent accounting period.

b. In case there is more than one Lessee, the obligations of Lessee executing this Lease shall be joint and several. The words "Lessor" and "Lessee" as used herein shall include the plural as well as the singular. The covenants and agreements contained herein shall be binding upon and enforceable by the parties hereto and their respective heirs, executors, administrators, successors and assigns, subject to the restrictions herein imposed on assignment by Lessee.

c. Time is of the essence of this Lease and each and every covenant, condition and provision herein contained.

d. The paragraph headings are inserted only as a matter of convenience and for reference, and in no way define, limit or describe the scope or intent of this agreement or any provision thereof or in any way affect this agreement.

e. In the event there is a Guarantor of this Lease, said Guarantor shall have the same obligations as Lessee under this Lease.

41. PARKING:

Lessee shall be entitled to up to three (3) unassigned and undesignated onsite parking spaces per one thousand (1,000) square feet of office space, and one (1) unassigned and undesignated space per fifteen hundred (1,500) square feet of warehouse space covered by this Lease.

42. ADDENDA:

Attached hereto is an addendum or addenda as indicated on the Basic Lease Information which constitute(s) a part of this Lease: Sections 44 through 52.

43. ENTIRE AGREEMENT:

Except as noted in Section 42 above, this Lease includes the entire agreement between the parties and may neither be added to nor amended without the signature of all parties.

IN WITNESS WHEREOF, the parties hereto have subscribed their names, and if corporations, executed this Lease by officers thereunto duly authorized by resolution of said corporations, in duplicate, the day and year stated below.

Lessee		Lessor	
/s/ Emil Kakkis		/s/ J. Wang	7/5/2011
By: Ultragenyx Pharmaceutical, Inc.		By: J. Wang, V.P. Condiotti Enterprises, Inc.	Date
CEO	7/1/2011		
Title	Date		
By: Ultragenyx Pharmaceutical, Inc.			
Title	Date		

Exhibit 10.23

License and Services Agreement

This License and Services Agreement ("Agreement") is entered into on September 24, 2010 by and between The Buck Institute for Age Research, an independent non-profit research organization ("Buck") with facilities at 8001 Redwood Blvd., Novato, CA 94945 ("Facility") and Ultragenyx Pharmaceutical Inc., a California corporation, located at 77 Digital Drive, Suite 210, Novato, CA 94949 ("Ultragenyx").

Recitals

A. Ultragenyx is a biopharmaceutical company developing novel therapeutics for innovative diseases and it has an interest in expanding its research and early development capabilities;

B. Buck is an expanding biomedical research institute and has a full laboratory facility and set of core services, including, but not limited to a Microscopy and Imaging Core, Genetics Core and an AALAC accredited Vivarium. Its campus is reflected in Exhibit "A" attached hereto and incorporated herein by reference (the "Facility");

C. Ultragenyx wishes to procure access at the Facility to certain laboratory space in the Hillblom Center, Building A of the Facility, First Floor, approximating 500 square feet, as more particularly shown on the attached Exhibit "B" (herein the "Licensed Lab Space") in order to conduct research and facilitate its therapeutic development programs; and

D. Subject to the terms and conditions described below, Buck is willing to license the Licensed Lab Space to Ultragenyx and provide access to its Facility for these purposes.

For valuable consideration, acknowledged as received the parties agree as follows:

Agreement

1. Recitals. The Recitals are incorporated into this Agreement by reference.

2. Term. The term of this Agreement ("Term") shall be for two (2) years from the Effective Date, except that (i) either party may terminate this Agreement upon one year's prior notice without cause and purely out of convenience of such party; and (ii) either party may terminate this Agreement for cause upon a breach as specified below.

3. The Facility. The Facility contains various publicly accessible areas, certain private areas and certain areas where access is limited to persons with security clearances. The public areas include a parking area, an exterior entry way, as well as lobbies and publicly accessible interior entry space and hallways, etc. ("Public Areas"). The Facility also contains interior areas that are unavailable to the general public without security clearances or separate permission from Buck ("Controlled Areas"). The Controlled Areas include interior stairways, hallways, restrooms, laboratory space and conference rooms; these are herein referred to as "Amenities."

Ultragenyx proposes to pursue laboratory research within the Licensed Lab Space and place up to four staff/employees ("Agents") at the Facility to conduct research for Ultragenyx, and these Agents will require access to the following (i) parking at the Facility parking lot; (ii) the Licensed Lab Space; and (iii) the Amenities, to the extent these are available. Should additional laboratory space be utilized by Ultragenyx at the Facility, this Agreement will be amended in writing to reflect the change, and Ultragenyx shall pay Buck such sums for such additional space and use as the parties may from time to time determine.

4. Grant of License for Use of Facility

(a) Subject to the payment of the License Fees described in Section 6, below, and to the terms and conditions of this Agreement, for the duration of the Term, Buck hereby grants to Ultragenyx a non-exclusive license to access and use the parking, the Public Areas; the Amenities; and the Licensed Lab Space for up to four Agents. The license so granted includes a non-exclusive license to use the equipment identified in Exhibit C ("Shared Equipment"). Use of the Shared Equipment is on a non-exclusive basis.

These licenses are granted for the sole purpose of permitting Agents to conduct laboratory research and analysis on Ultragenyx projects at the Facility during regular working hours (i.e. Monday through Friday from 8am to 6pm). These licenses (i) do not give Ultragenyx any property rights whatsoever in or to the Facility or any of Buck's furniture, fixtures or equipment or its other personal property within the Facility; (ii) are conditioned on Ultragenyx's compliance with the Protocols and Procedures identified in Section 5 below; (iii) are terminable upon a breach, and (iv) will automatically end and be of no further force or effect upon the expiration or earlier termination of this Agreement.

(b) Regardless of any contrary provision in this Agreement, (i) the Licensed Lab Space is within an open laboratory area and as such there is no physical barrier or security associated with such Licensed Lab Space, (ii) Buck's maintenance staff will have regular access to the Licensed Lab Space to perform housekeeping and maintenance functions; (iii) Buck does not warrant or guaranty that the Licensed Lab Space will not be intruded upon from time to time by others at the Facility; and (iv) Buck staff may at any time enter the licensed areas in case on any emergency or to perform maintenance and repairs to the Facility. These intrusions into the Licensed Lab Space shall not be a default by Buck under this Agreement, nor shall they justify any change in the License Fee payable by Ultragenyx to Buck.

(c) The expiration or termination of the licenses granted hereunder will not in any manner affect any obligations of the parties that accrue prior to the date of such expiration or termination, including, e.g. indemnity obligations.

5. Protocols and Procedures.

(a) This Agreement anticipates that Ultragenyx, through its Agents, will access various areas of the Facility, and will undertake numerous laboratory projects within the licensed areas. All such uses of the Facility, and all such actions and proceedings, are subject to Buck's protocols and procedures, as these may be amended from time to time ("Protocols and Procedures"). Such Protocols and Procedures include compliance with the following, without limitation:

- Security controls for access to Controlled Areas including the Licensed Lab Space (these security measures can include magnetic cards, passwords, biometric recognition systems, etc.);

- Life safety protocols;
- Protocols for the handling of any hazardous and waste materials, including meeting all local, state and federal compliance standards as well as all standards to maintain the Facility in compliance with its accreditation; and
- Technical protocols and procedures for undertaking any laboratory work done at the Facility.
- All general environmental, health and safety procedures

(b) In the course of its activities, Ultragenyx and its Agents must conduct all of its laboratory work in compliance with good scientific procedures and strive to avoid cross-contamination. It may not at any time cause a nuisance condition at the Facility, nor shall it engage in any use or activities that would jeopardize the Facility's accreditation or breach the Protocols and Procedures. Ultragenyx shall advise Buck of the personnel it intends to locate at the Facility and provide that persons identification details in advance so that appropriate clearances can be implemented in an orderly way before such person is granted access to Controlled Areas.

(c) Ultragenyx shall require its Agents to undergo such training or briefing through Buck personnel and standards as is reasonable to ensure its Agents at the Facility are aware of and will abide by the Protocols and Procedures (as these may be amended by Buck from time to time). All of the costs of Ultragenyx's Agents and other staff shall be the sole responsibility of Ultragenyx, and Buck shall have no liability whatsoever for any costs associated with Ultragenyx's personnel at the Facility; provided, however, that Buck shall provide Ultragenyx personnel with such on-site training and oversight of the Protocols and Procedures as it would for its own staff; this will be done in coordination with other on-site training so as to minimize any additional training costs for Buck. Ultragenyx warrants that its personnel will abide by the Protocols and Procedures (as amended from time to time) and that it will hold harmless and indemnify Buck and the Buck Parties (as defined in Para, 8, below) against any claim, liability, loss, cost (including e.g. court costs and attorneys fees) arising from any failure by Ultragenyx to do so.

6. License Fee. The fees payable by Ultragenyx to Buck for the use of the Licensed Lab Space and incidental use of the Facility are as follows: For providing the Licensed Lab Space, inclusive of all costs of associated with utilities, the heating, ventilation, janitorial and general maintenance services for such space, Ultragenyx will pay an annual fee of $33,600, payable in equal monthly installments of $2,800.00 on the first day of each month during the Term. This sum shall be prorated for any partial months of the Term.

7. Insurance Coverages and Liability.

(a) Ultragenyx shall obtain such insurance coverage as is specified in Exhibit "D" attached hereto. Proof of insurance shall be provided to Buck by appropriate means evidencing each of the required coverages; it shall be received by Buck not less than 7 days prior to Ultragenyx's use of the Facility.

(b) Ultragenyx will exercise its best good faith efforts to ensure that all insurance required of Ultragenyx hereunder will include a waiver of subrogation to prevent claims against Buck and the Buck Parties. It is intended by the parties that Buck and the Buck Parties shall have no liability whatsoever to Ultragenyx arising out of any damage or loss caused to property of Ultragenyx whether or not the loss was caused by the negligence of Buck or its agents, or is caused due to the condition of the Facility. No such losses shall be recoverable from Buck except to the extent of any insurance coverage required to be provided under this Section 7. Furthermore, Ultragenyx waives any rights against Buck and the Buck Parties for losses or damage arising from a cause that is to be insured against by Ultragenyx under this Agreement, even if such loss or damage is caused by the negligence of Buck or a Buck Party.

(c) Buck may require additional insurance coverage from time to time to the extent this is required in the industry to address risks associated with the use of the Facility.

8. Indemnity.

(a) Ultragenyx agrees to indemnify, hold harmless, and defend Buck and the Buck Parties (as defined below) against any and all claims, actions, proceedings, liability, loss, damage, cost or expense (including reasonable attorney's fees and expenses and cost of investigation) ("Claim") with respect to any matter arising from, resulting from, or connected to this Agreement or the use of the Facility by Ultragenyx and its Agents, and such defense and indemnity shall include and extend, without limitation, to: Claims caused by or allegedly caused by the acts or omissions of Buck or the Buck Parties, unless these are shown to be due to their gross negligence of willful misconduct; and the research, development, manufacture, use or commercialization of products by or emanating from Ultragenyx, including all product liability or other claims for injury or death arising from the sale or use of products sold by or on behalf of Ultragenyx, regardless of the theory under which such claims are brought. Ultragenyx's indemnification obligations shall extend to Buck, its subsidiaries and affiliates and their respective officers, employees, Trustees, donors, volunteers, researchers, independent contractors, veterinary and medical doctors, agents, vendors, IACUC and directors (herein defined as "Buck Parties").

(b) Buck agrees to indemnify, hold harmless and defend Ultragenyx and the Ultragenyx Parties (as defined below) against any and all claims, actions, proceedings, liability, loss, damage, cost or expense (including reasonable attorney's fees and expenses and cost of investigation) by a third party with respect to any matter arising from, resulting from, or connected to Buck's gross negligence or willful misconduct. Buck's obligations under this paragraph shall extend to Ultragenyx, its subsidiaries and affiliates and their respective officers, employees, directors, and consultants (herein defined as "Ultragenyx Parties").

9. Confidentiality. Subject to the provisions of this Para. 9, each party will maintain as confidential all information of the other party that is identified as "confidential" or "proprietary" by the other party (hereinafter referred to as "Confidential Information"). Without otherwise limiting the definition of Confidential Information, "Confidential Information" as that term is used in this Agreement excludes information that the receiving party proves: (a) is available to the public through no fault of the receiving party or its representatives; (b) is already in the possession of the receiving party without restriction and prior to any disclosure under this Agreement; (c) is or has been lawfully disclosed to the receiving party by a third party without obligation of confidentiality upon the receiving party; or (d) was or is developed independently by the receiving party without access to the information disclosed hereunder.

Subject to the obligations imposed by law or any legal proceedings, each of the parties agree that any Confidential Information obtained by them shall be used solely in connection with the purposes of this Agreement, and shall not be disclosed, discussed or distributed to any third party except as stated herein. The receiving party may not reproduce any Confidential Information received other than in connection with the purposes of this Agreement. All such Confidential Information will be treated by the receiving party as confidential for five years after the termination of this Agreement.

10. Limitation of Liability. THE PARTIES EXPRESSLY AGREE AND UNDERSTAND THAT NEITHER PARTY SHALL HAVE ANY LIABILITY WHATSOEVER FOR ANY INCIDENTAL, INDIRECT, CONSEQUENTIAL, OR SPECIAL DAMAGES (INCLUDING, BUT NOT LIMITED TO, LOSS OF PROFITS) OF ANY KIND RESULTING FROM OR IN ANY WAY RELATED TO THIS AGREEMENT OR THE TERMINATION OF THIS AGREEMENT, EVEN IF INFORMED IN ADVANCE OF THE POSSIBILITY OF SUCH DAMAGES. PROVIDED, HOWEVER, THAT THIS CLAUSE SHALL NOT IN ANY MANNER LIMIT THE EXTENT OF ANY INDEMNITY OBLIGATION FOR THIRD PARTY CLAIMS, AND DOES NOT EXTEND TO A VIOLATION OF THE CALIFORNIA TRADE SECRETS ACT.

11. Default and Breach. It is a breach of this Agreement if a party fails to perform any obligation hereunder, and then fails to cure such default within the applicable cure period noted below, if any:

(a) The failure to pay any monetary obligation due under this Agreement, followed by a failure to cure such default within five (5) days of notice of the default;

(b) There are more than three breaches of the Protocols and Procedures in any 90 day period by one or more Agents;

(c) The failure to perform any other obligations required under this Agreement followed by a failure to cure such default within thirty (30) days of notice of the default;

(d) Any assignment of the Agreement without consent where consent is required.

12. Miscellaneous. Neither party shall be liable for any failure of performance beyond its reasonable control. This Agreement constitutes the entire agreement between the parties relative to Ultragenyx's use of the Facility and supersedes all other proposals, understandings or agreements. This Agreement may be amended or terminated only by a written agreement between the parties. The invalidity, illegality or unenforceability of any provision of this Agreement shall not affect the remainder of the Agreement, and this Agreement shall be construed and modified without such provision. This Agreement shall be governed by the laws of California, without regard for conflicts of laws principles, and Ultragenyx consents to California as the proper venue for settling all disputes and controversies. Nothing in this Agreement shall be deemed to create a relationship of employment or agency or to constitute the parties as partners or joint venturers. This Agreement may not be assigned without the prior written consent of the non-assigning party, except upon the sale of its business interests in the subject matter of this Agreement. In such case either party may freely assign without the prior written consent of the other. This Agreement shall become binding when signed by the Ultragenyx and executed by an authorized officer of Buck. In the event of any dispute with respect to this Agreement, the prevailing party shall be entitled to reasonable attorneys' fees and other costs and expenses incurred in resolving such dispute.

13. Notices. Unless otherwise specifically provided, all notices required or permitted by this Agreement shall be in writing and may be delivered by facsimile or email (via PDF or other means); by personal delivery, or by first class prepaid certified mail, return receipt requested, to the following addresses:

If to Ultragenyx:

Ultragenyx Pharmaceuticals Inc.
Attention: Emil Kakkis, MD, PhD, CEO
77 Digital Drive, Suite 210, Novato, CA 94949

Fax: 415.884.0562
Email: cmcgilbra@ultragenyx.com

If to Buck:

Buck Institute For Age Research
Attention: Remy Gross
8001 Redwood Blvd.
Novato, CA 94945
Fax: 415-493-2248
Email: rgross@buckinstitute.org

The parties may alter such addresses by appropriate notices consistent with this subsection. Any notice shall be deemed to have been received as follows: (1) if by personal delivery, upon receipt; (2) if by certified mail, five (5) business days after mailing at the U.S. post office by the party serving notice; and (3) if by email or facsimile, on the business day following electronic confirmation that the notice has been received at the facsimile number of email address provided.

ULTRAGENYX WARRANTS AND ACKNOWLEDGES THAT AN AUTHORIZED OFFICIAL HAS READ THIS AGREEMENT, UNDERSTANDS IT AND AGREES TO BE BOUND BY ITS TERMS AND CONDITIONS.

BUCK INSTITUTE FOR AGE RESEARCH

By: /s/ Ralph O'Rear
Name: Ralph O'Rear
Title: VP, Facilities and Planning
Date: 9/23/2010

ULTRAGENYX PHARMACEUTICALS INC.

By: /s/ Emil Kakkis
Name: Emil Kakkis
Title: CEO
Date: 9/24/2010

Exhibit 10.24

AMENDMENT NO. 1 TO LICENSE AND SERVICES AGREEMENT

This AMENDMENT NO. 1 TO LICENSE AND SERVICES AGREEMENT (herein referred to as "**Amendment No. 1**") is made and entered into as of this September 4th, 2012 (the "**Amendment No. 1 Effective Date**"), by and between Ultragenyx Pharmaceutical, Inc. (herein referred to as "**Ultragenyx**"), a California Corporation, and The Buck Institute for Research on Aging, an independent non-profit research organization organized under the laws of California ("**Buck**"), each herein referred to individually as "**Party**" and collectively as "**Parties**".

WHEREAS, Ultragenyx and Buck are Parties to the Agreement (as defined below), pursuant to which Ultragenyx has rights to use certain Buck facilities, specifically laboratory and write-up space; and

WHEREAS, the Parties now desire to amend the Agreement to provide for Ultragenyx's use of a newer, larger laboratory, storage, write-up area and potential expansion laboratory space.

NOW, THEREFORE, the Parties agree as follows:

1. This Amendment No. 1 shall serve as an amendment to that certain License and Services Agreement (the "**Agreement**"), effective September 24, 2010, by and between Buck and Ultragenyx. Except as expressly modified hereby, the Agreement shall continue in full force according to its terms. Capitalized terms not otherwise defined in this Amendment No. 1 shall have the meaning ascribed to such term in the Agreement.

2. Recital B of the Agreement is hereby deleted and replaced in its entirety with

   B. Buck is an expanding biomedical research institute dedicated to research and education on the biology of aging and age-related disease and has a full laboratory facility and set of core services, including, but not limited to a Microscopy and Imaging Core, Genetics Core and an AALAC accredited Vivarium (the "Facility");

3. Recital C of the Agreement shall be deleted in its entirety and replaced with the following:

   C. Ultragenyx wishes to procure access at the Facility to certain laboratory space in Building A of the Facility, Fourth Floor, as shown on the attached Exhibit "A" as "Licensed Lab Space" and also have a right of first refusal to lease the "Expansion Space" also shown in Exhibit "A" in order to conduct research and facilitate its therapeutic development programs; and

4. Section 2 of the Agreement shall be deleted in its entirety and replaced with the following:

   "Term. The term of this Agreement ("Term") shall be for two (2) years from the Amendment No. 1 Effective Date, except that (i) either party may terminate this Agreement upon one year's prior written notice without cause and purely out of convenience of such party; and (ii) either party may terminate this Agreement for cause

upon a breach as specified below. Within ninety (90) days prior to the expiry of the Term, Ultragenyx may extend the Term by an additional twelve (12) months (the "Option") by sending written notice to Buck. Ultragenyx may exercise the Option for up to three (3) consecutive years."

5. The first sentence of the second paragraph of Section 3 is hereby deleted and replaced in its entirety with the following:

"Ultragenyx proposed to pursue laboratory research within the Licensed Lab Space and place four (or more, as approved and trained by Buck in environmental safety, fire codes and provisioned with Facility-access key cards) staff/employees ("Agents") at the Facility to conduct research for Ultragenyx, and these Agents will require access to the following (i) parking at the Facility parking lot; (ii) the Licensed Lab Space; and (iii) the Amenities, to the extent these are available."

6. The first sentence of the first paragraph of Section 4(a) is hereby deleted and replaced in its entirety with the following:

"Subject to the payment of the License Fees described in Section 6, below, and to the terms and conditions of this Agreement, for the duration of the Term, Buck hereby grants to Ultragenyx a non-exclusive license to access and use the parking; the Public Areas; the Amenities; and the Licensed Lab Space for its Agents."

7. The following shall be added as Section 4(d):

"(d) Ultragenyx shall have the option at any time during the Term to license all or part of the Expansion Space on the same terms and conditions as contained in this Agreement (and coterminous with the Term) except at a fee to be negotiated, on a per square foot basis no greater than five percent (5%) higher than the fee paid at the time Ultragenyx exercises the option (the "Option"); provided that the space in the Expansion Space that is occupied at the Amendment No. 1 Effective Date (i.e., the stem cell training area) shall be included in the Expansion Space only if vacated by the current occupants at the time of exercise of the Option. Ultragenyx may exercise the Option by providing Buck ninety (90) days prior written notice of its intent to license the Expansion Space or part thereof. The parties shall enter into an amendment to this Agreement or new agreement, as appropriate, to reflect the license of the Expansion Space on such terms within such ninety day period. If during the Term and prior to the exercise of the Option by Ultragenyx, a potential tenant, other than the Buck itself, ("Potential Tenant") expresses definitive written interest in licensing or leasing part or all of the Expansion Space, Buck will send written notice to Ultragenyx. Ultragenyx shall have sixty (60) days after receipt of such notice to exercise the Option. If Ultragenyx does not exercise the Option within such 60 day period, the Option will be considered terminated and Ultragenyx will have no further contractual right to the Expansion Space."

8. In Section 6 of the Agreement, the numbers $33,600 and $2,800.00 are hereby deleted and replaced with the numbers $60,000 and $5,000.00, respectively.

9. The following shall be added at the end of the Section 6 of the Agreement:

   "This License Fee will remain in effect for the first twelve months following the Amendment No. 1 Effective Date and shall be subject to an increase based on multiplying the percent change of the Producer Pricing Index from the previous twelve month period to the License Fee charged in the preceding year, but in no case shall the License Fee be lower than at the stated first year rate. Such increase will take effect and be adjusted immediately on the first and subsequent anniversaries of the Amendment No. 1 Effective Date."

10. **Exhibit A** of the Agreement is hereby deleted and replaced in its entirety with Attachment No. 1 hereto.

11. This Amendment No. 1 shall inure to the benefit of and be binding upon the Parties hereto and their respective heirs, successors, trustees, transferees and assigns.

12. This Amendment No. 1 may be executed in any number of counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

IN WITNESS WHEREOF, the Parties hereto, intending to be legally bound hereby, have caused this Amendment No. 1 to be executed and delivered by their proper and duly authorized officers effective on the Amendment No. 1 Effective Date.

ULTRAGENYX, INC.

BY: /S/ SHALINI SHARP
PRINTED NAME: SHALINI SHARP
TITLE: CFO

BUCK INSTITUTE FOR RESEARCH ON AGING

BY: /S/ REMY GROSS, III
PRINTED NAME: REMY GROSS, III
TITLE: VICE PRESIDENT, BUSINESS DEVELOPMENT

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the reference to our firm under the caption “Experts” and to the use of our report dated October 3, 2013, in the Registration Statement (Form S-1) and related Prospectus of Ultragenyx Pharmaceutical Inc. (a development stage Company) for the registration of its common stock.

/s/ Ernst & Young LLP

Redwood City, California
November 8, 2013

Exhibit 99.1

CONSENT TO BE NAMED DIRECTOR

Pursuant to Rule 438 under the Securities Act of 1933, as amended, I hereby consent to being named in the Registration Statement on Form S-1 (File No. 333-), together with any and all amendments or supplements thereto, of Ultragenyx Pharmaceutical Inc., a Delaware corporation (the "Company"), as a person who has agreed to serve as a director of the Company upon closing of the offering and the inclusion of my biographical information in the Registration Statement. I also consent to the filing of this consent as an exhibit to the Registration Statement.

Dated: November 6, 2013

/s/ Matthew Fust

Matthew Fust
Print Name: Matthew Fust

November 8, 2013

Lisa M. Kahle
T+1 (415) 315-6320
F+1 (415) 315-6033
lisa.kahle@ropesgray.com

Via EDGAR and Federal Express

Jeffrey P. Riedler
Assistant Director
U.S. Securities and Exchange Commission
100 F Street, N.E.
Washington, D.C. 20549

Re: Ultragenyx Pharmaceutical Inc.
Confidential Draft Registration Statement on Form S-1
Submitted October 4, 2013
CIK No. 0001515673

Dear Mr. Riedler:

On behalf of Ultragenyx Pharmaceutical Inc. ("***Ultragenyx***" or the "***Company***"), we are responding to the staff of the Securities and Exchange Commission's (the "***Staff***") letter dated October 31, 2013 (the "***Comment Letter***"), relating to the above-referenced Confidential Draft Registration Statement on Form S-1 (the "***Draft Registration Statement***"). In response to the comments set forth in the Comment Letter, the Draft Registration Statement has been amended and Ultragenyx is hereby filing its Registration Statement on Form S-1 ("***Filing No. 1***") with this response letter. For your convenience, we have repeated the Staff's comments below in bold face type before each of our responses below. The numbered paragraphs of this letter correspond to the numbered paragraphs of the Comment Letter.

General

1. **If our comments are applicable to portions of the filings that we have not cited as examples, please make the appropriate changes elsewhere in the filing in accordance with our comments.**

   The Company respectfully acknowledges the Staff's comment.

2. **Please submit all exhibits as soon as practicable. We may have further comments upon examination of these exhibits.**

   The Company respectfully acknowledges the Staff's comment.

3. **Please supplementally provide us with copies of all written communications, as defined in Rule 405 under the Securities Act, that you, or anyone authorized to do so on your behalf, present to potential investors in reliance on Section 5(d) of the Securities Act, whether or not they retain copies of the communications. Similarly, please supplementally provide us with any research reports about you that are published or distributed in reliance upon Section 2(a)(3) of the Securities Act of 1933 added by Section 105(a) of the Jumpstart Our Business Startups Act by any broker or dealer that is participating or will participate in your offering.**

   The Company respectfully acknowledges the Staff's comment and will, under separate supplemental cover, provide copies of all written communications, if any, presented to potential investors in reliance on Section 5(d) of the Securities Act, as well as research reports published or distributed in reliance on Section 2(a)(3) of the Securities Act, if any.

4. **We note that you submitted a confidential treatment request on October 4, 2013. We will provide any comments in relation to any such confidential treatment request and the related disclosure in a separate comment letter.**

   The Company respectfully acknowledges the Staff's comment.

Risk Factors
Risks Related to Our Financial Condition and Capital Requirements
"We are a development-stage company…" page 10

5. **Please expand this risk factor or include a standalone risk factor that discusses the risks associated with the fact that the potential markets in which your product candidates may ultimately receive regulatory approval are very small, such that even with a significant market share and acceptance, your products may not become profitable.**

   The Company respectfully acknowledges the Staff's comment and has revised pages 10 and 23 of Filing No. 1.

"Even if this offering is successful, we expect that we will need to raise additional funding…"

6. **Please revise this risk factor and the related discussion on page 65 to clarify whether your existing funds that you expect to be sufficient to fund your operations for the next 12 months include the proceeds from this offering.**

The Company respectfully acknowledges the Staff's comment and has revised pages 12 and 66 of Filing No. 1.

"We are subject to a multitude of manufacturing risks…" page 20

7. **To the extent you have experienced any significant costs, delays or other difficulties to date as a result of contamination, equipment failure, or similar events, please revise this risk factor to include a brief description of those costs, delays or difficulties.**

The Company respectfully acknowledges the Staff's comment and has revised page 20 of Filing No. 1.

"We may be involved in lawsuits to protect or enforce our patents…" page 32
"We may be subject to claims that our employees…" page 32
"We may be subject to claims challenging the inventorship of our patents…" page 33

8. **Please revise the text, and, if applicable, the headings of these risk factors to clarify the extent to which you are currently a party to or involved in the type of lawsuits or claims described.**

The Company respectfully acknowledges the Staff's comment and has revised page 32 of Filing No. 1.

Management's Discussion and Analysis of Financial Condition and Results of Operations
Critical Accounting Policies and Significant Judgments and Estimates
Stock-based Compensation, pages 57-61

9. **On pages 57 through 61 of your discussion about the fair value of stock option grants, you indicate that you utilized and assumed an annual volatility rate based on "comparable publicly traded biopharmaceutical companies." Please tell us the name of these companies and explain to us why you deemed them to be comparable to you. In your response, for each of these companies, tell us the following information at your valuation dates:**

- annual revenues;
- annual product revenues;
- net income/loss;
- assets;

- equity;
- number of products in development and their stages of development;
- number of marketed products;
- market capitalization; and
- volatility.

The Company respectfully advises the Staff that the Company considered various factors in evaluating which comparable publicly traded biopharmaceutical companies should be used in calculating its annual volatility rates. In evaluating similarity of the companies, the Company considered factors such as the industry, stage of life and program cycle, size and financial leverage. The peer group of publicly traded companies in the valuation reports is comprised of companies that focus primarily on developing biopharmaceutical treatments for rare, or orphan, diseases, which is consistent with the focus of the Company. Orphan disease companies generally have several unique characteristics, such as: the numbers of patients targeted for treatment are small; because pricing for these therapies can be relatively high, gross margins can therefore also be relatively high; research and development costs and general and administrative costs to support the products tend to be lower than those of traditional pharmaceutical products; and competition is typically limited.

The Company periodically reevaluates the list of comparable publicly traded biopharmaceutical companies and updates the list as deemed necessary, taking into consideration the development stage or unique characteristics of the companies based on the information available at the time. The Company's peer group of publicly traded companies consisted of nine companies for the June 30, 2012 valuation report; nine companies for the December 31, 2012 valuation report; 10 companies for the June 30, 2013 valuation report; and 11 companies for the September 30, 2013 valuation report. The selected peer guideline public companies changed as of December 31, 2012 in order to include companies that were expected to generate product revenues while excluding very large public companies that the Company determined were no longer comparable companies based on size and program cycle. The number of selected peer guideline public companies has increased as of June 30, 2013 and September 30, 2013 in order to include companies that have filed for an initial public offering in the last year and had a meaningful trading history.

Several companies selected for the Company's analysis are revenue-generating and are therefore at a more advanced development stage than the Company. These include BioMarin, Swedish Orphan Biovitrum, and ViroPharma. It was necessary to include

such companies in the analysis in order to generate a meaningful basis for the calculation of various multiples. The Company also included some companies with no marketed products, which are at a similar development stage, such as Sarepta and Amicus.

Comparable Companies	Valuation Report				Comment
	6/12	12/12	6/13	9/13	
Alexion Pharmaceuticals, Inc.	X				**Deleted 12/12** - large public company determined to be no longer comparable based on size and program cycle
Alnylam Pharmaceuticals, Inc.	X	X	X	X	Has orphan drug programs
Amicus Therapeutics, Inc.	X	X	X	X	Has orphan drug programs
BioMarin Pharmaceutical, Inc.	X	X	X	X	Has orphan drug programs
Corcept Therapeutics, Inc.	X				**Deleted 12/12–**due to limited revenues from Korlym tablets
Hyperion Therapeutics, Inc.				X	**Added 9/13 –** company went public in July 2012
Intermune, Inc.			X	X	**Added 6/13 –** began selling Esbriet in Germany and other EU countries in 2012
Isis Pharmaceuticals, Inc.	X	X	X	X	Has orphan drug programs
Protalix Biotherapeutics, Inc.		X	X	X	**Added 12/12–** began generating sufficient revenues in 2012 and has orphan drug program
Raptor Pharmaceuticals, Inc.	X	X	X	X	Included due to focus on rare diseases
Sarepta Pharmaceuticals, Inc.	X	X	X	X	Has orphan drug programs
Shire plc	X				**Deleted 12/12** - very large public company determined to be no longer comparable based on size and program cycle
Swedish Orphan Biovitrum AB		X	X	X	**Added 12/12–** Has orphan drug programs
Viropharma, Inc.		X	X	X	**Added 12/12–** Has orphan drug programs

The average volatility for the comparable publicly traded biopharmaceutical companies over various time periods ranged from 38% to 104% and the aggregate average across the peer group was 56%. Because the Company is smaller and less established than some of the larger companies included in the analysis, it used a higher volatility than the aggregate average across the peer group, which was closer to the volatility of the smaller companies in the analysis. The comparable publicly traded biopharmaceutical companies and the information requested by the Staff is set forth in the table below as of September 30, 2013 (or June 30, 2013 if September results have not yet been reported):

Company Name	LTM Revenue	LTM Product Revenues	LTM Net Income/ (Loss)	Assets	Equity	Number of Products in Development						Market Cap	Average Volatility
						Marketed	Filed	Phase 3	Phase 2	Phase 1	Pre-clinical		
Alexion	1,430	1,430	353	3,050	2,355	1	—	—	1	3	—	22,716	37%
Alnylam	53	—	(109)	463	308	—	—	—	2	4	5	4,030	54%
Amicus	—	N/A	(59)	82	40	—	—	1	1	—	2	115	86%
BioMarin	525	525	(120)	1,602	1,276	4	1	2	1	2	—	10,111	38%
Corcept	6	6	(43)	80	40	1	—	1	1	—	Multiple	173	64%
Hyperion	8	—	3	134	116	2	—	—	1	—	—	525	53%
InterMune	41	53	(207)	451	95	1	—	—	—	—	1	1,260	86%
Isis	113	N/A	(52)	789	401	2	—	2	14	3	9	4,329	57%
Protalix	12	N/A	(27)	62	(12)	1	—	—	1	2	3	424	46%
Raptor	N/A	N/A	(42)	68	20	1	—	1	2	—	2	890	47%
Sarepta	22	—	(173)	184	89	—	—	—	1	2	3	1,584	104%
Shire	4,874	4,639	643	8,388	4,169	27	—	2	5	1	—	22,193	26%
Swedish	295	152	(31)	978	717	5	—	3	—	—	—	2,643	46%
ViroPharma	430	430	(72)	1,113	728	4	1	—	4	1	1	2,572	46%
												Mean	56%
			LTM – last twelve months									Median	50%

10. **Please provide in your filing containing the IPO price range, a discussion of each significant factor contributing to the difference between the fair value as of the date of each grant and the estimated IPO price range. Please reconcile and explain the differences between the mid-point of your estimated offering price range and the fair values included in your analysis.**

    The Company respectfully acknowledges the Staff's comment and will, in a future filing of the registration statement containing the estimated IPO price range, provide a discussion of each significant factor contributing to the difference between the fair value as of the date of each grant and the estimated IPO price range and will reconcile and explain the differences between the mid-point of the estimated offering price range and the fair values included in the Company's analysis.

11. **Please confirm that you have not issued any additional equity issuances including stock options, warrants, convertible preferred stock and debt since the latest balance sheet date or provide additional disclosure through the date of effectiveness.**

    The Company respectfully acknowledges the Staff's comment and has revised pages 60 and 62 of Filing No. 1 to reflect the grant of stock options on November 1, 2013.

Results of Operations
Research and Development Expenses, pages 62 and 63

12. **Please expand your tables of Research & Development costs by project to include a column for total Research & Development expenses incurred for each project since inception.**

    The Company respectfully acknowledges the Staff's comment and has revised pages 63 and 64 of Filing No. 1.

Business
Product Candidates
Biologics product candidates – rhPPCA, page 75

13. **Please expand your discussion in this section to disclose your plans to continue preclinical development of rhPPCA.**

    The Company respectfully acknowledges the Staff's comment and has revised page 77 of Filing No. 1.

Small-molecule product candidates
Triheptanoin for the treatment of LC-FAOD – Triheptanoin background and clinical development, page 78

14. Please expand your discussion on page 78 to provide a brief description of the serious adverse events and identify those that were considered related to triheptanoin treatment.

The Company respectfully acknowledges the Staff's comment and has revised page 80 of Filing No. 1.

Triheptanoin for the treatment of Glut 1 DS, page 80

15. Please revise your disclosure to explain what you mean by an "adaptive Phase 2 study."

The Company respectfully acknowledges the Staff's comment and has revised page 82 of Filing No. 1.

SA-ER for the treatment of HIBM
SA-ER background and clinical development, page 83

16. Please expand your disclosure to describe briefly the nature of the debate in the literature regarding HIBM and how any alternative evidence or theory may affect the development of your product candidate.

The Company respectfully acknowledges the Staff's comment and has revised page 85 of Filing No. 1.

17. You state that improvement demonstrated in your Phase 2 study was statistically significant or trended towards statistical significance. Please explain what you mean by trended towards statistical significance. In addition, please expand your discussion to identify the p-values and to explain what the p-values measure.

The Company respectfully acknowledges the Staff's comment and has revised pages 85 and 86 of Filing No. 1. Additionally, the 24-week data from the Phase 2 study of SA-ER results came from an interim analysis of the trial, and detailed results were not disclosed, as the final top-line 48-week results are anticipated later in 2013, which results will be disclosed in the prospectus to the extent available. A detailed

presentation or publication of the 24- and 48-week data is planned for 2014, as noted in Filing No. 1.

License Agreements
Kyowa Hakko Kirin, page 85

18. Please expand your description of your agreement with KHK:

- **to describe the applicable transition date; and**
- **to disclose the range of royalties to which you may be entitled, expressed as a percentage within ten percent.**

The Company respectfully acknowledges the Staff's comment and has revised pages 87 and 88 of Filing No. 1.

Intellectual Property, page 90

19. Please revise your discussion of your patents to specify the expiration dates for your material issued patents rather than grouping such dates with the estimated expiration dates of pending patent applications.

The Company respectfully acknowledges the Staff's comment and has revised page 92 of Filing No. 1.

Manufacturing, page 91

20. Please expand your descriptions of your agreements with Rentschler and Cremer Oleo to describe the duration and termination provisions of such agreements.

The Company respectfully acknowledges the Staff's comment and has revised pages 93 and 94 of Filing No. 1.

Management
Non-Employee Directors, page 106

21. Please specify the directorships held by Mr. Aliski, including any held during the past five years pursuant to Item 401(e)(2) of Regulation S-K.

The Company respectfully acknowledges the Staff's comment and informs the Staff that no directorships held by Mr. Aliski, including any held during the past five years are required to be disclosed pursuant to Item 401(e)(2) of Regulation S-K.

Description of Capital Stock, page 132

22. We note your disclosure entitled "Exclusive Jurisdiction of Certain Actions" on page 132. Several lawsuits are currently challenging the validity of choice of forum provisions in certificates of incorporation. Please disclose that although you have included a choice of forum clause in your amended and restated certification of incorporation, it is possible that a court could rule that such provision is inapplicable or unenforceable.

The Company respectfully acknowledges the Staff's comment and has revised page 135 of Filing No. 1.

Financial Statements
Note 12, Stock-Based Awards, page F-25

23. Tell us why expected volatility declined to 67% in 2012 from 75% in 2011 and then increased to 75% in 2013 (page F-41).

The Company respectfully advises the Staff that, in accordance with the Interpretive Response Question Six of SAB Topic 14D1, the Company considered the individual volatilities of the comparable companies as discussed in the response to comment number 9 above in establishing the expected volatility for its stock-based awards. The Company considered the mean volatility as well as the range of volatilities for the comparable companies included in the analysis. After consideration, the Company used an estimated volatility of 75% for the year ended December 31, 2011 and 67% for the year ended December 31, 2012. For the six months ended June 30, 2013, the Company used an estimated volatility of 75%, and has used this same rate for the nine months ended September 30, 2013. The volatilities used by the Company in calculating its stock-based compensation were generally higher than the mean volatilities for the comparable companies as the Company believes that given its early stage of development, it would have a higher volatility than the comparable public companies.

The mean volatility for the comparable companies during the year ended December 31, 2012 was lower than the mean volatility for 2011 and the periods to date in 2013. The Company did not adjust the mean in 2012, which was approximately 64%, as much as it did in the other periods. As such, the Company respectfully advises the Staff that it chose to use 67% as the expected volatility for the 2012 period. The Company also advises the Staff that if it had used 75% as the expected volatility rather than 67% in 2012, the impact to the financial statements would not have been material. The impact

to stock-based compensation expense would have been an increase of $20,000, which would be recognized to expense over the requisite service period of the awards, and a $4,000 increase to the expense recorded for the year ended December 31, 2012.

Item 16. Exhibits and financial statements schedules.
(a) Exhibits, page II-5

24. Please file a copy of the form of lock-up agreement as an exhibit to your registration statement.

The Company respectfully acknowledges the Staff's comment and confirms for the Staff that it will include the form of lock-up agreement as an exhibit to the Underwriting Agreement in a future filing of the registration statement to be filed as Exhibit 1.1 to the registration statement.

* * * * *

We thank you in advance for your consideration of this response. If you have any questions, please do not hesitate to contact me.

Very truly yours,

/s/ Lisa M. Kahle

Lisa M. Kahle

LMK: lcv

cc: Shalini Sharp, Ultragenyx Pharmaceutical, Inc.
Ryan A. Murr, Ropes & Gray LLP
B. Shayne Kennedy, Latham & Watkins LLP