UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934.**

For the quarterly period ended June 30, 2022

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934.**

For the transition period from _____ to _____.

Commission File No. 001-36276

ULTRAGENYX PHARMACEUTICAL INC.

(Exact name of registrant as specified in its charter)

Delaware	**27-2546083**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
60 Leveroni Court **Novato, California**	**94949**
(Address of principal executive offices)	*(Zip Code)*

(415) 483-8800
(Registrant's telephone number, including area code)

Not Applicable
(Former Name, Former Address and Former Fiscal Year, if Changed Since Last Report)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol	Name of each exchange on which registered
Common Stock, $0.001 par value	**RARE**	**The Nasdaq Global Select Market**

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. YES ☑ NO ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). YES ☑ NO ☐

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

Large accelerated filer	☑	Accelerated filer	☐
Non-accelerated filer	☐	Smaller reporting company	☐
		Emerging growth company	☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). YES ☐ NO ☑

As of July 25, 2022, the registrant had 70,036,868 shares of common stock issued and outstanding.

ULTRAGENYX PHARMACEUTICAL INC.

FORM 10-Q FOR THE QUARTER ENDED JUNE 30, 2022

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CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q (the Quarterly Report) contains forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this Quarterly Report are forward-looking statements. In some cases, you can identify forward-looking statements by words such as “anticipate,” “believe,” “contemplate,” “continue,” “could,” “estimate,” “expect,” “forecast,” “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 commercialization, marketing, and manufacturing capabilities and strategy;
- our expectations regarding the timing of clinical study commencements and reporting results from same;
- the timing and likelihood of regulatory approvals for our product candidates;
- the anticipated indications for our product candidates, if approved;
- the potential market opportunities for commercializing our products and product candidates;
- our expectations regarding the potential market size and the size of the patient populations for our products and product candidates, if approved for commercial use;
- estimates of our expenses, 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, products and product candidates and the integration and performance of any businesses we have acquired or may acquire;
- the initiation, timing, progress, and results of ongoing and future preclinical 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 products and product candidates;
- our ability to maintain and establish collaborations or strategic relationships or obtain additional funding;
- our ability to maintain and establish relationships with third parties, such as contract research organizations, contract manufacturing organizations, suppliers, and distributors;
- our financial performance and the expansion of our organization;
- the impact of the COVID-19 pandemic and related health measures on our business, financial condition and liquidity;
- our ability to obtain supply of our products and product candidates;
- the scalability and commercial viability of our manufacturing methods and processes;
- developments and projections relating to our competitors and our industry; and
- other risks and uncertainties, including those listed under Part II, Item 1A. Risk Factors.

Any forward-looking statements in this Quarterly Report 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 discussed under Part II, Item 1A. Risk Factors and elsewhere in this Quarterly Report. 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 Quarterly Report 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.

PART I. FINANCIAL INFORMATION

Item 1. ***Financial Statements***

ULTRAGENYX PHARMACEUTICAL INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(Unaudited)
(In thousands, except share amounts)

	June 30, 2022	December 31, 2021
Assets		
Current assets:		
Cash and cash equivalents	$ 159,868	$ 307,584
Marketable debt securities	382,927	432,612
Accounts receivable, net	37,207	28,432
Inventory	21,079	16,231
Prepaid expenses and other current assets	77,461	71,745
Total current assets	678,542	856,604
Property, plant, and equipment, net	207,381	141,247
Equity investments	15,412	34,925
Marketable debt securities	163,301	258,933
Right-of-use assets	31,245	34,936
Intangible assets, net	159,196	130,788
Goodwill	44,406	44,406
Other assets	21,144	20,558
Total assets	$ 1,320,627	$ 1,522,397
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 34,213	$ 17,138
Accrued liabilities	170,355	145,555
Contract liabilities	4,437	7,609
Lease liabilities	11,631	11,066
Total current liabilities	220,636	181,368
Contract liabilities	—	1,467
Lease liabilities	25,902	30,904
Deferred tax liabilities	33,306	33,306
Liability related to the sale of future royalties	358,942	351,786
Other liabilities	3,768	1,005
Total liabilities	642,554	599,836
Stockholders' equity:		
Preferred stock — 25,000,000 shares authorized; nil outstanding as of June 30, 2022 and December 31, 2021	—	—
Common stock — 250,000,000 shares authorized; 70,010,398 and 69,344,998 shares issued and outstanding as of June 30, 2022 and December 31, 2021, respectively	70	69
Additional paid-in capital	3,071,000	2,997,497
Accumulated other comprehensive loss	(8,914)	(1,404)
Accumulated deficit	(2,384,083)	(2,073,601)
Total stockholders' equity	678,073	922,561
Total liabilities and stockholders' equity	$ 1,320,627	$ 1,522,397

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(Unaudited)
(In thousands, except share and per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Revenues:				
Collaboration and license	$ 53,088	$ 63,940	$ 101,501	$ 142,950
Product sales	30,832	18,346	57,516	34,859
Non-cash collaboration royalty revenue	5,423	4,689	10,261	8,561
Total revenues	89,343	86,975	169,278	186,370
Operating expenses:				
Cost of sales	8,270	3,136	14,370	8,324
Research and development	154,529	113,205	297,684	260,723
Selling, general and administrative	68,137	53,410	135,449	106,668
Total operating expenses	230,936	169,751	447,503	375,715
Loss from operations	(141,593)	(82,776)	(278,225)	(189,345)
Interest income	899	441	1,393	1,080
Change in fair value of equity investments	(10,184)	(31,046)	(19,513)	(51,665)
Non-cash interest expense on liability related to the sale of future royalties	(6,052)	(8,517)	(12,636)	(16,935)
Other expense	(930)	(67)	(641)	(862)
Loss before income taxes	(157,860)	(121,965)	(309,622)	(257,727)
Provision for income taxes	(302)	(463)	(860)	(842)
Net loss	$ (158,162)	$ (122,428)	$ (310,482)	$ (258,569)
Net loss per share, basic and diluted	$ (2.26)	$ (1.81)	$ (4.45)	$ (3.84)
Weighted-average shares used in computing net loss per share, basic and diluted	69,925,358	67,607,752	69,722,141	67,356,443

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.
CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS
(Unaudited)
(In thousands)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Net loss	$ (158,162)	$ (122,428)	$ (310,482)	$ (258,569)
Other comprehensive income (loss):				
Foreign currency translation adjustments	(731)	103	(791)	(182)
Unrealized gain (loss) on available-for-sale securities	(1,629)	73	(6,719)	(275)
Other comprehensive income (loss):	(2,360)	176	(7,510)	(457)
Total comprehensive loss	$ (160,522)	$ (122,252)	$ (317,992)	$ (259,026)

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.
CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(Unaudited)
(In thousands, except share amounts)

	Common Stock		Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Income (Loss)	Deficit	Equity
Balance as of March 31, 2022	69,796,940	$ 70	$ 3,028,829	$ (6,554)	$ (2,225,921)	$ 796,424
Stock-based compensation	—	—	36,188	—	—	36,188
Issuance of common stock under equity plan awards, net of tax	213,458	—	5,983	—	—	5,983
Other comprehensive loss	—	—	—	(2,360)	—	(2,360)
Net loss	—	—	—	—	(158,162)	(158,162)
Balance as of June 30, 2022	70,010,398	$ 70	$ 3,071,000	$ (8,914)	$ (2,384,083)	$ 678,073

	Common Stock		Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Income (Loss)	Deficit	Equity
Balance as of December 31, 2021	69,344,998	$ 69	$ 2,997,497	$ (1,404)	$ (2,073,601)	$ 922,561
Stock-based compensation	—	—	65,766	—	—	65,766
Issuance of common stock under equity plan awards, net of tax	665,400	1	7,737	—	—	7,738
Other comprehensive loss	—	—	—	(7,510)	—	(7,510)
Net loss	—	—	—	—	(310,482)	(310,482)
Balance as of June 30, 2022	70,010,398	$ 70	$ 3,071,000	$ (8,914)	$ (2,384,083)	$ 678,073

	Common Stock		Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Income (Loss)	Deficit	Equity
Balance as of March 31, 2021	67,439,477	$ 67	$ 2,810,176	$ 56	$ (1,755,717)	$ 1,054,582
Stock-based compensation	—	—	27,099	—	—	27,099
Issuance of common stock under equity plan awards, net of tax	325,473	1	12,741	—	—	12,742
Other comprehensive income	—	—	—	176	—	176
Net loss	—	—	—	—	(122,428)	(122,428)
Balance as of June 30, 2021	67,764,950	$ 68	$ 2,850,016	$ 232	$ (1,878,145)	$ 972,171

	Common Stock		Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Income (Loss)	Deficit	Equity
Balance as of December 31, 2020	66,818,520	$ 67	$ 2,773,195	$ 689	$ (1,619,576)	$ 1,154,375
Stock-based compensation	—	—	51,319	—	—	51,319
Issuance of common stock under equity plan awards, net of tax	946,430	1	25,502	—	—	25,503
Other comprehensive loss	—	—	—	(457)	—	(457)
Net loss	—	—	—	—	(258,569)	(258,569)
Balance as of June 30, 2021	67,764,950	$ 68	$ 2,850,016	$ 232	$ (1,878,145)	$ 972,171

See accompanying notes.

ULTRAGENYX PHARMACEUTICAL INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(Unaudited)
(In thousands)

	Six Months Ended June 30,	
	2022	2021
Operating activities:		
Net loss	$ (310,482)	$ (258,569)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation	65,260	51,345
Amortization of premium on marketable debt securities, net	3,380	2,511
Depreciation and amortization	8,569	6,488
Change in fair value of equity investments	19,513	51,665
Non-cash collaboration royalty revenue	(10,261)	(8,561)
Non-cash interest expense on liability related to the sale of future royalties	12,636	16,935
Other	386	511
Changes in operating assets and liabilities:		
Accounts receivable	(8,902)	(1,669)
Inventory	(4,705)	(2,174)
Prepaid expenses and other assets	(6,793)	(9,002)
Accounts payable, accrued, and other liabilities	41,260	(11,526)
Contract liabilities	(4,639)	(62,656)
Net cash used in operating activities	(194,778)	(224,702)
Investing activities:		
Purchase of property, plant, and equipment	(63,107)	(36,485)
Purchase of marketable debt securities	(205,278)	(664,347)
Proceeds from sale of marketable debt securities	42,950	70,548
Proceeds from maturities of marketable debt securities	297,304	406,614
Payment for intangible asset	(30,000)	—
Other	(63)	—
Net cash provided by (used in) investing activities	41,806	(223,670)
Financing activities:		
Proceeds from the issuance of common stock from equity plan awards, net	7,738	25,503
Other	(289)	(226)
Net cash provided by financing activities	7,449	25,277
Effect of exchange rate changes on cash	(1,346)	(323)
Net decrease in cash, cash equivalents and restricted cash	(146,869)	(423,418)
Cash, cash equivalents and restricted cash at beginning of period	309,585	726,294
Cash, cash equivalents and restricted cash at end of period	$ 162,716	$ 302,876
Supplemental disclosures of non-cash information:		
Acquired lease liabilities arising from obtaining right-of-use assets	$ 1,036	$ 2,301
Non-cash interest expense on liability related to the sale of future royalties capitalized into ending property, plant and equipment	$ 4,781	$ —

See accompanying notes.

1. Organization

Ultragenyx Pharmaceutical Inc. (the Company) is a biopharmaceutical company incorporated in Delaware.

The Company is focused on the identification, acquisition, development, and commercialization of novel products for the treatment of serious rare and ultra-rare genetic diseases. The Company operates as one reportable segment. The Company has four commercially approved products.

Crysvita® (burosumab) is approved in the United States (U.S.) by the U.S. Food and Drug Administration (FDA) and in Canada for the treatment of X-linked hypophosphatemia (XLH) in adult and pediatric patients one year of age and older, and is approved in the European Union (EU) and the United Kingdom, for the treatment of XLH with radiographic evidence of bone disease in children one year of age and older, adolescents, and adults. In Brazil, Colombia, and Mexico, Crysvita is approved for treatment of XLH in adult and pediatric patients one year of age and older. Crysvita is also approved in the U.S. by the FDA for the treatment of fibroblast growth factor 23 (FGF23)-related hypophosphatemia in tumor-induced osteomalacia (TIO), associated with phosphaturic mesenchymal tumors that cannot be curatively resected or localized in adults and pediatric patients 2 years of age and older.

Mepsevii® (vestronidase alfa) is approved by the FDA as the first medicine for the treatment of children and adults with mucopolysaccharidosis VII (MPS VII), also known as Sly syndrome. In the European Union and the United Kingdom, Mepsevii is approved under exceptional circumstances for patients of all ages for the treatment of non-neurological manifestations of MPS VII. In Brazil, Mepsevii is approved for the treatment of MPS VII for patients of all ages.

Dojolvi® (triheptanoin) is approved in the U.S., Canada, and Brazil for the treatment of pediatric and adult patients severely affected by long-chain fatty acid oxidation disorders (LC-FAOD).

On January 7, 2022, the Company announced a collaboration with Regeneron Pharmaceuticals (Regeneron) to commercialize Evkeeza® (evinacumab) outside of the U.S. Evkeeza is approved in the U.S. and the European Economic Area (EEA) for the treatment of homozygous familial hypercholesterolemia (HoFH).

In addition to the approved products, the Company has the following ongoing clinical development programs:

- UX111 (formerly ABO-102) is an AAV9 gene therapy product candidate for the treatment of patients with Sanfilippo syndrome type A (MPS IIIA), a rare lysosomal storage disease. In May 2022, the Company announced an exclusive license agreement with Abeona Therapeutics Inc. (Abeona) for UX111 whereby the Company assumed responsibility for the UX111 program, as further described in Note 6;
- DTX401 is an adeno-associated virus 8 (AAV8) gene therapy product candidate for the treatment of patients with glycogen storage disease type Ia (GSDIa);
- DTX301 is an AAV8 gene therapy product candidate in development for the treatment of patients with ornithine transcarbamylase (OTC) deficiency, the most common urea cycle disorder;
- UX143 (setrusumab), which is subject to the Company's collaboration agreement with Mereo BioPharma 3 (Mereo), is a fully human monoclonal antibody that inhibits sclerostin, a protein that acts on a key bone-signaling pathway and inhibits the activity of bone-forming cells for the treatment of patients with osteogenesis imperfect (OI);
- GTX-102 is an antisense oligonucleotide (ASO), which the Company is developing through GeneTx Biotherapeutics LLC (GeneTx) for the treatment of Angelman syndrome, a debilitating and rare neurogenetic disorder caused by loss-of-function of the maternally inherited allele of the UBE3A gene. In July 2022, the Company executed its option to acquire GeneTx as further described in Note 12;
- UX701 is an adeno-associated virus 9, (AAV9) gene therapy designed to deliver stable expression of a truncated version of the ATP7B copper transporter following a single intravenous infusion to improve copper distribution and excretion from the body and reverse pathological findings of Wilson liver disease; and
- UX053 is a messenger RNA (mRNA) product candidate designed for the treatment of patients with Glycogen Storage Disease Type III (GSDIII), a disease caused by a glycogen debranching enzyme (AGL) deficiency that results in glycogen accumulation in the liver and muscle.

The Company has sustained operating losses and expects such annual losses to continue over the next several years. The Company's ultimate success depends on the outcome of its research and development and commercialization activities, for which it expects to incur additional losses in the future. Management recognizes that the Company will likely need to raise additional capital to fully implement its business plans. Through June 30, 2022, the Company has relied primarily on its sale of equity securities, its revenues from commercial products, its sale of future royalties, and strategic collaboration arrangements to finance its operations.

The Company will likely raise additional capital through the issuance of equity, borrowings, or strategic alliances with partner companies. However, if such financing is not available at adequate levels, the Company would need to reevaluate its operating plans.

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying unaudited Condensed Consolidated Financial Statements include the accounts of the Company and its wholly-owned subsidiaries and have been prepared in accordance with U.S. generally accepted accounting principles (GAAP) for interim financial information and in accordance with the instructions to Form 10-Q and Rule 10-01 of Regulation S-X. Accordingly, they do not include all of the information and footnotes required by GAAP for complete financial statements. The unaudited interim Condensed Consolidated Financial Statements have been prepared on the same basis as the annual financial statements. In the opinion of management, the accompanying unaudited Condensed Consolidated Financial Statements reflect all adjustments (consisting only of normal recurring adjustments) considered necessary for a fair presentation. These financial statements should be read in conjunction with the audited financial statements and notes thereto for the preceding fiscal year contained in the Company's Annual Report on Form 10-K filed on February 15, 2022 (Annual Report) with the U.S. Securities and Exchange Commission (the SEC).

The results of operations for the three and six months ended June 30, 2022 are not necessarily indicative of the results to be expected for the year ending December 31, 2022. The Condensed Consolidated Balance Sheet as of December 31, 2021 has been derived from audited financial statements at that date, but does not include all of the information required by GAAP for complete financial statements.

Use of Estimates

The accompanying Condensed Consolidated Financial Statements have been prepared in accordance with GAAP. The preparation of the Condensed Consolidated 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 Condensed Consolidated 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, income taxes, stock-based compensation, revenue recognition, and the liability related to the sale of future royalties. 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, Cash Equivalents and Restricted Cash

Restricted cash primarily consists of money market accounts used as collateral for the Company's obligations under its facility leases and the gene therapy building construction project. The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the Condensed Consolidated Balance Sheets that sum to the total of the same such amounts shown in the Condensed Consolidated Statement of Cash Flows (in thousands):

	June 30,	
	2022	2021
Cash and cash equivalents	$ 159,868	$ 290,875
Restricted cash included in prepaid expenses and other current assets	517	10,000
Restricted cash included in other assets	2,331	2,001
Total cash, cash equivalents, and restricted cash shown in the statements of cash flows	$ 162,716	$ 302,876

Credit Losses

The Company is exposed to credit losses primarily through receivables from customers and collaborators and through its available-for-sale debt securities. For trade receivables and other instruments, the Company uses a forward-looking expected loss

model that generally results in the earlier recognition of allowances for losses. For available-for-sale debt securities with unrealized losses, the losses are recognized as allowances rather than as reductions in the amortized cost of the securities.

The Company's expected loss allowance methodology for the receivables is developed using historical collection experience, current and future economic market conditions, a review of the current aging status and financial condition of the entities. Specific allowance amounts are established to record the appropriate allowance for customers that have a higher probability of default. Balances are written off when determined to be uncollectible. The Company's expected loss allowance methodology for the debt securities is developed by reviewing the extent of the unrealized loss, the size, term, geographical location, and industry of the issuer, the issuers' credit ratings and any changes in those ratings, as well as reviewing current and future economic market conditions and the issuers' current status and financial condition. There were no material credit losses recorded for receivables and available-for-sale debt securities for the three and six months ended June 30, 2022 and 2021.

Revenue Recognition

Collaboration and license revenue

The Company has certain license and collaboration agreements that are within the scope of Accounting Standards Codification (ASC) 808, *Collaborative Agreements*, which provides guidance on the presentation and disclosure of collaborative arrangements. Generally, the classification of the transactions under the collaborative arrangements is determined based on the nature of contractual terms of the arrangement, along with the nature of the operations of the participants. The Company records its share of collaboration revenue, net of transfer pricing related to net sales in the period in which such sales occur, if the Company is considered as an agent in the arrangement. The Company is considered an agent when the collaboration partner controls the product before transfer to the customers and has the ability to direct the use of and obtain substantially all of the remaining benefits from the product. Funding received related to research and development services and commercialization costs is generally classified as a reduction of research and development expenses and selling, general and administrative expenses, respectively, in the Condensed Consolidated Statement of Operations, because the provision of such services for collaborative partners are not considered to be part of the Company's ongoing major or central operations.

In order to record collaboration revenue, the Company utilizes certain information from its collaboration partners, including revenue from the sale of the product, associated reserves on revenue, and costs incurred for development and sales activities. For the periods covered in the financial statements presented, there have been no material changes to prior period estimates of revenues and expenses.

The Company sold the right to receive certain royalty payments from net sales of Crysvita in certain European territories to RPI Finance Trust (RPI), an affiliate of Royalty Pharma, as further described in Note 7. The Company records the royalty revenue from the net sales of Crysvita in the applicable European territories on a prospective basis as non-cash royalty revenue in the Condensed Consolidated Statements of Operations over the term of the arrangement.

The terms of the Company's collaboration and license agreements may contain multiple performance obligations, which may include licenses and research and development activities. The Company evaluates these agreements under ASC 606, *Revenue from Contracts with Customers* (ASC 606), to determine the distinct performance obligations. The Company analogizes to ASC 606 for the accounting for distinct performance obligations for which there is a customer relationship. Prior to recognizing revenue, the Company makes estimates of the transaction price, including variable consideration that is subject to a constraint. Amounts of variable consideration are included in the transaction price to the extent that it is probable that a significant reversal in the amount of cumulative revenue recognized will not occur and when the uncertainty associated with the variable consideration is subsequently resolved. Total consideration may include nonrefundable upfront license fees, payments for research and development activities, reimbursement of certain third-party costs, payments based upon the achievement of specified milestones, and royalty payments based on product sales derived from the collaboration.

If there are multiple distinct performance obligations, the Company allocates the transaction price to each distinct performance obligation based on its relative standalone selling price. The standalone selling price is generally determined based on the prices charged to customers or using expected cost-plus margin. The Company estimates the efforts needed to complete the performance obligations and recognizes revenue by measuring the progress towards complete satisfaction of the performance obligations using input measures.

Product sales

The Company sells its approved products through a limited number of distributors. Under ASC 606, revenue from product sales is recognized at the point in time when the delivery is made and when title and risk of loss transfers to these distributors. The Company also recognizes revenue from sales of certain products on a "named patient" basis, which are allowed in certain countries prior to the commercial approval of the product. Prior to recognizing revenue, the Company makes estimates of the transaction

price, including any variable consideration that is subject to a constraint. Amounts of variable consideration are included in the transaction price to the extent that it is probable that a significant reversal in the amount of cumulative revenue recognized will not occur and when the uncertainty associated with the variable consideration is subsequently resolved. Product sales are recorded net of estimated government-mandated rebates and chargebacks, estimated product returns, and other deductions.

Provisions for returns and other adjustments are provided for in the period the related revenue is recorded, as estimated by management. These reserves are based on estimates of the amounts earned or to be claimed on the related sales and are reviewed periodically and adjusted as necessary. The Company's estimates of government mandated rebates, chargebacks, estimated product returns, and other deductions depends on the identification of key customer contract terms and conditions, as well as estimates of sales volumes to different classes of payors. If actual results vary, the Company may need to adjust these estimates, which could have an effect on earnings in the period of the adjustment.

3. Financial Instruments

Financial assets and liabilities are recorded at fair value. The carrying amount of certain financial instruments, including cash and cash equivalents, accounts receivable, 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 determines the fair value of its equity investments in Arcturus Therapeutics Holdings Inc. (Arcturus) and Solid Biosciences Inc. (Solid) by using the quoted market prices, which are Level 1 fair value measurements.

The following tables set forth the fair value of the Company's financial assets remeasured on a recurring basis based on the three-tier fair value hierarchy (in thousands):

	June 30, 2022			
	Level 1	Level 2	Level 3	Total
Money market funds	$ 121,255	$ —	$ —	$ 121,255
Certificate of deposits and time deposits	—	23,907	—	23,907
Corporate bonds	—	326,733	—	326,733
Commercial paper	—	78,727	—	78,727
Asset-backed securities	—	10,351	—	10,351
U.S. Government Treasury and agency securities	7,181	93,581	—	100,762
Debt securities in government-sponsored entities	—	15,749	—	15,749
Investments in Arcturus and Solid common stock	12,687	—	—	12,687
Other	—	3,730	—	3,730
Total	$ 141,123	$ 552,778	$ —	$ 693,901

	December 31, 2021			
	Level 1	Level 2	Level 3	Total
Money market funds	$ 266,765	$ —	$ —	$ 266,765
Certificate of deposits and time deposits	—	16,000	—	16,000
Corporate bonds	—	349,691	—	349,691
Commercial paper	—	187,624	—	187,624
Asset-backed securities	—	41,245	—	41,245
U.S. Government Treasury and agency securities	—	87,435	—	87,435
Debt securities in government-sponsored entities	—	19,549	—	19,549
Investments in Arcturus and Solid common stock	32,200	—	—	32,200
Other	—	942	—	942
Total	$ 298,965	$ 702,486	$ —	$ 1,001,451

4. Balance Sheet Components

Cash Equivalents and Investments

The fair values of cash equivalents and investments classified as available-for-sale securities consisted of the following (in thousands):

	June 30, 2022			
		Gross Unrealized		
	Amortized Cost	Gains	Losses	Estimated Fair Value
Money market funds	$ 121,255	$ —	$ —	$ 121,255
Certificates of deposit and time deposits	23,907	—	—	23,907
Corporate bonds	333,287	—	(6,554)	326,733
Commercial paper	78,727	—	—	78,727
Asset-backed securities	10,372	—	(21)	10,351
U.S. Government Treasury and agency securities	101,892	36	(1,166)	100,762
Debt securities in government-sponsored entities	16,046	—	(297)	15,749
Total	$ 685,486	$ 36	$ (8,038)	$ 677,484

	December 31, 2021			
		Gross Unrealized		
	Amortized Cost	Gains	Losses	Estimated Fair Value
Money market funds	$ 266,765	$ —	$ —	$ 266,765
Certificates of deposit and time deposits	16,000	—	—	16,000
Corporate bonds	350,667	3	(979)	349,691
Commercial paper	187,624	—	—	187,624
Asset-backed securities	41,282	1	(38)	41,245
U.S. Government Treasury and agency securities	87,642	1	(208)	87,435
Debt securities in government-sponsored entities	19,612	—	(63)	19,549
Total	$ 969,592	$ 5	$ (1,288)	$ 968,309

At June 30, 2022, the remaining contractual maturities of available-for-sale securities were less than three years. There have been no significant realized gains or losses on available-for-sale securities for the three and six months ended June 30, 2022 and 2021. The unrealized losses on the Company's investments in marketable debt securities were caused by interest rate increases. All marketable securities with unrealized losses at June 30, 2022 have been in a loss position for less than twelve months except for two corporate bond securities with an aggregate fair value of $9.4 million and an aggregate unrealized loss of $0.2 million. The contractual terms of these investments do not permit the issuers to settle the securities at a price less than the par value. Accordingly, it is expected that the securities would not be settled at a price less than the amortized cost basis of these investments. The Company does not intend to sell the investments and it is not more likely than not that the Company will be required to sell the investments before recovery of their amortized cost basis.

Inventory

Inventory consists of the following (in thousands):

	June 30, 2022	December 31, 2021
Work-in-process	$ 16,189	$ 10,504
Finished goods	4,890	5,727
Total inventory	$ 21,079	$ 16,231

Accrued Liabilities

Accrued liabilities consist of the following (in thousands):

	June 30, 2022	December 31, 2021
Research, clinical study, and manufacturing expenses	$ 67,743	$ 40,880
Payroll and related expenses	47,913	62,591
Other	54,699	42,084
Total accrued liabilities	$ 170,355	$ 145,555

5. Revenue

The following table disaggregates total revenues from customers (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Collaboration and license revenue:				
Crysvita collaboration revenue in profit-share territory	$ 51,609	$ 41,756	$ 96,773	$ 78,016
Crysvita royalty revenue in European territory	—	228	—	228
Daiichi Sankyo	1,479	21,956	4,728	64,706
Total collaboration and license revenue	53,088	63,940	101,501	142,950
Product sales:				
Crysvita	12,402	2,900	21,796	8,772
Mepsevii	4,933	5,399	9,794	9,006
Dojolvi	13,497	10,047	25,926	17,081
Total product sales	30,832	18,346	57,516	34,859
Crysvita non-cash collaboration royalty revenue	5,423	4,689	10,261	8,561
Total revenues	$ 89,343	$ 86,975	$ 169,278	$ 186,370

The following table disaggregates total revenues based on geographic location (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
U.S. and Canada	$ 67,328	$ 75,567	$ 129,245	$ 163,309
Europe	9,476	6,700	17,703	12,337
Latin America	12,539	4,708	22,330	10,724
Total revenues	$ 89,343	$ 86,975	$ 169,278	$ 186,370

The following table presents changes in the contract liabilities (in thousands):

	Six Months Ended June 30,	
	2022	2021
Balance of contract liabilities at beginning of period	$ 9,076	$ 66,568
Additions	89	2,050
Deductions	(4,728)	(64,706)
Balance of contract liabilities at end of period	$ 4,437	$ 3,912

See Note 6 for additional details on contract liabilities activities.

The Company's largest accounts receivable balance accounted for 54% and 71% of the total accounts receivable balance as of June 30, 2022 and December 31, 2021, respectively, and was due from a collaboration partner. The accounts receivable balance from a different customer accounted for 14% and nil of the total accounts receivable balance as of June 30, 2022 and December 31, 2021, respectively.

6. License and Research Agreements

Kyowa Kirin Collaboration and License Agreement

In August 2013, the Company entered into a collaboration and license agreement with Kyowa Kirin Co., Ltd. (KKC formerly Kyowa Hakko Kirin Co., Ltd. or KHK). Under the terms of this collaboration and license agreement, as amended, the Company and KKC collaborate on the development and commercialization of Crysvita in the field of orphan diseases in the U.S. and Canada, or the profit-share territory, and in the European Union, United Kingdom, and Switzerland, or the European territory, and the Company has the right to develop and commercialize such products in the field of orphan diseases in Mexico and Central and South America, or Latin America.

Development Activities

In the field of orphan diseases, and except for ongoing studies being conducted by KKC, the Company is the lead party for development activities in the profit-share territory and in the European territory until the applicable transition date. The Company shares the costs for development activities in the profit-share territory and the European territory conducted pursuant to the development plan before the applicable transition date equally with KKC. In April 2023, which is the transition date for the profit-share territory, KKC will become the lead party and be responsible for the costs of the development activities. However, the Company will continue to share the costs of the studies commenced prior to the applicable transition date equally with KKC.

The collaboration and license agreements are within the scope of ASC 808, which provides guidance on the presentation and disclosure of collaborative arrangements.

Collaboration revenue related to sales in profit-share territory

The Company and KKC share commercial responsibilities and profits in the profit-share territory until April 2023. Under the collaboration agreement, KKC manufactures and supplies Crysvita for commercial use in the profit-share territory and charges the Company the transfer price of 35% of net sales through December 31, 2022, and 30% thereafter. The remaining profit or loss after supply costs from commercializing products in the profit-share territory are shared between the Company and KKC on a 50/50 basis until April 2023. Thereafter, the Company will be entitled to receive a tiered double-digit revenue share from the mid-20% range up to 30%.

As KKC is the principal in the sale transaction with the customer, the Company recognizes a pro-rata share of collaboration revenue, net of transfer pricing, in the period the sale occurs. The Company concluded that its portion of KKC's sales in the profit-share territory is analogous to a royalty and therefore recorded its share as collaboration revenue, similar to a royalty.

In July 2022, the Company sold its right to receive 30% of the future royalty payments due to the Company based on net sales of Crysvita in the U.S. and Canada, subject to a cap, beginning from April 2023 to OCM LS23 Holdings LP, an investment vehicle for OMERS, one of Canada's largest defined benefit pension plans, as further described in Note 12.

Royalty revenue related to sales in European territory

KKC has the commercial responsibility for Crysvita in the European territory. In December 2019, the Company sold its right to receive royalty payments based on sales in the European territory to Royalty Pharma, effective January 1, 2020, as further described in Note 7. Prior to the Company's sale of the royalty, the Company received a royalty of up to 10% on net sales in the European

territory, which was recognized as the underlying sales occur. Beginning in 2020, the Company recorded the royalty revenue as non-cash royalty revenues.

The Company's share of collaboration and royalty revenue related to Crysvita was as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Company's share of revenue in profit-share territory	$ 51,609	$ 41,756	$ 96,773	$ 78,016
Royalty revenue in European territory	—	228	—	228
Non-cash royalty revenue in European territory	5,423	4,689	10,261	8,561
Total	$ 57,032	$ 46,673	$ 107,034	$ 86,805

Product revenue related to sales in other territories

The Company is responsible for commercializing Crysvita in Latin America and Turkey. The Company is considered the principal in these territories as the Company controls the product before it is transferred to the customer. Accordingly, the Company records revenue on a gross basis related to the sale of Crysvita once the product is delivered and the risk and title of the product is transferred to the distributor. The Company recorded product sales of $12.4 million and $21.8 million for the three and six months ended June 30, 2022, respectively, and $2.9 million and $8.8 million for the three and six months ended June 30, 2021, respectively, net of estimated product returns and other deductions. KKC has the option to assume responsibility for commercialization efforts in Turkey from the Company, after a certain minimum period.

Under the collaboration agreement, KKC manufactures and supplies Crysvita, which is purchased by the Company for sales in its territories and is based on 35% of the net sales through December 31, 2022 and 30% thereafter. The Company also pays to KKC a low single-digit royalty on net sales in Latin America.

Cost sharing payments

Under the collaboration agreement, KKC and the Company share certain development and commercialization costs. As a result, the Company was reimbursed for these costs and operating expenses were reduced as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Research and development	$ 4,321	$ 5,171	$ 8,940	$ 11,356
Selling, general and administrative	8,949	7,576	18,145	15,071
Total	$ 13,270	$ 12,747	$ 27,085	$ 26,427

Collaboration receivable and payable

The Company had accounts receivable from KKC in the amount of $20.1 million and $20.2 million from profit-share revenue and royalties and other receivables recorded in prepaid expenses and other current assets of $2.9 million and $16.0 million and accrued liabilities of $4.8 million and $2.3 million from commercial and development activity reimbursements, as of June 30, 2022 and December 31, 2021, respectively.

GeneTx

In August 2019, the Company entered into a Program Agreement and a Unitholder Option Agreement with GeneTx to collaborate on the development of GeneTx's GTX-102, an ASO for the treatment of Angelman syndrome.

Pursuant to the terms of the Unitholder Option Agreement, the Company made an upfront payment of $20.0 million for an exclusive option to acquire GeneTx, which was exercisable any time prior to 30 days following FDA acceptance of the IND for GTX-102. Pursuant to the agreement, upon acceptance of the IND, which occurred in January 2020, the Company elected to extend the option period by paying an option extension payment of $25.0 million (option extension premium) during the quarter ended March 31, 2020, which was recorded as an in-process research and development expense. In April 2022, the parties entered into an amendment to the Unitholder Option Agreement (the "Amendment") which provided the Company with an additional, earlier option to acquire GeneTx for the purchase price of $75.0 million based on the earlier of receipt of interim data in the Phase 1/2 study or a specified date (such option, the "Interim Option").

During the exclusive option period, GeneTx was responsible for conducting the program based on the development plan agreed upon between the parties and, subject to the terms in the Program Agreement, had the decision-making authority on all

matters in connection with the research, development, manufacturing and regulatory activities with respect to the Program. The Company provided support, at its discretion, including strategic guidance and clinical expertise.

In July 2022, the Company executed its option to acquire GeneTx as further described in Note 12.

Daichi Sankyo

In March 2020, the Company executed a License and Technology Access Agreement (the License Agreement) with Daiichi Sankyo Co., Ltd. (Daiichi Sankyo). Pursuant to the License Agreement, the Company granted Daiichi Sankyo a non-exclusive license to intellectual property, including know-how and patent applications, with respect to its Pinnacle PCL™ producer cell line platform (Pinnacle PCL Platform), and HEK293 transient transfection manufacturing technology platforms for AAV-based gene therapy products.

Under the terms of the License Agreement, Daiichi Sankyo made an upfront payment of $125.0 million and an additional $25.0 million upon completion of the technology transfer of the Pinnacle PCL Platform and HEK293 platform. Daiichi Sankyo reimbursed the Company for all costs associated with the transfer of the manufacturing technology and will pay single-digit royalties on net sales of products manufactured in either system.

The Company also entered into a Stock Purchase Agreement (SPA) with Daiichi Sankyo, pursuant to which Daiichi Sankyo purchased 1,243,913 shares of the Company's common stock in exchange for $75.0 million in cash during the first quarter of 2020. The fair market value of the common stock issued to Daiichi Sankyo was $55.3 million based on the stock price of $44.43 per share on the date of issuance, resulting in a $19.7 million premium on the SPA. Daiichi Sankyo is also subject to a three-year standstill and restrictions on sale of the shares (subject to customary exceptions or release).

In June 2020, the Company executed a subsequent license agreement (the Sublicense Agreement) with Daiichi Sankyo for transfer of certain technology in consideration for an upfront payment of $8.0 million and annual maintenance fees, milestone payments, and royalties on any net sales of products incorporating the licensed intellectual property.

The License Agreement, the Sublicense Agreement, and the SPA are being accounted for as one arrangement because they were entered into at or near the same time and negotiated in contemplation of one another. The Company evaluated the License Agreement and the Sublicense Agreement under ASC 606 and determined that the performance obligations under the agreements are (i) intellectual property with respect to its Pinnacle PCL Platform and HEK293 transient transfection manufacturing technology platform together with the initial technical assistance and technology transfer services, which was completed in the first quarter of 2022, and (ii) the transfer of any know-how and improvements after the completion of the initial technology transfer through the end of the three year technology transfer period ending March 2023.

As of June 30, 2022, the Company has determined that the total transaction price of the License Agreement was $183.4 million, which was comprised of the $19.7 million premium from the SPA, the $125.0 million upfront payment, the $25.0 million in unconstrained milestone payments, $8.0 million from the Sublicense Agreement, and the $5.7 million estimated reimbursement amount for delivering the license and technology services. Total revenue recognized under the license agreement through June 30, 2022 was $178.9 million.

The Company allocated the total transaction price to the two performance obligations on a relative stand-alone selling price basis. Revenue allocated to the intellectual property and the technology transfer services was recognized over an initial period which was completed during the first quarter of 2022, measuring the progress toward complete satisfaction of the individual performance obligation using an input measure. Revenue for know-how and improvements after the completion of technology transfer is being recognized on a straight-line basis over the remaining technology transfer period, which ends in March 2023, as it is expected that Daiichi Sankyo will receive and consume the benefits consistently throughout the period. Royalties from commercial sales will be accounted for as revenue upon achievement of such sales, assuming all other revenue recognition criteria are met.

The Company recognized $1.5 million and $4.7 million for the three and six months ended June 30, 2022, respectively, and $22.0 million and $64.7 million for the three and six months ended June 30, 2021, respectively, in revenue related to this arrangement. Accordingly, the Company recorded $4.4 million and $9.1 million of contract liabilities, net, as of June 30, 2022 and December 31, 2021, respectively. The Company recorded an accounts receivable related to the above agreements of nil and $0.1 million as of June 30, 2022 and December 31, 2021, respectively.

Mereo

In December 2020, the Company entered into a License and Collaboration Agreement with Mereo to collaborate on the development of setrusumab. Under the terms of the agreement, the Company will lead future global development of setrusumab in both pediatric and adult patients with OI. The Company was granted an exclusive license to develop and commercialize setrusumab in the U.S., Turkey, and the rest of the world, excluding the European Economic Area, United Kingdom, and Switzerland, or the Mereo territory, where Mereo retains commercial rights. Each party will be responsible for post-marketing commitments and commercial supply in their respective territories.

Upon closing of the transaction in January 2021, the Company made a payment of $50.0 million to Mereo and will be required to make payments of up to $254.0 million upon the achievement of certain clinical, regulatory, and commercial milestones. The Company will pay for all global development costs as well as tiered double-digit percentage royalties to Mereo on net sales in the U.S., Turkey, and the rest of the world (excluding the Mereo Territory), and Mereo will pay the Company a fixed double-digit percentage royalty on net sales in the Mereo Territory.

Although Mereo is a variable interest entity, the Company is not the primary beneficiary as it does not have the power to direct the activities that would most significantly impact the economic performance of Mereo. Prior to the achievement of certain development milestones, all consideration paid to Mereo represents rights to potential future benefits associated with Mereo’s in-process research and development activities, which have not reached technological feasibility and have no alternative future use. Accordingly, for the three months ended March 31, 2021, the Company recorded the upfront payment of $50.0 million as in-process research and development expense.

Regeneron

In January 2022, the Company announced a collaboration with Regeneron to commercialize Evkeeza for HoFH outside of the U.S. Evkeeza is approved in the U.S., where it is marketed by Regeneron, and in the EEA as a first-in-class therapy for use together with diet and other low-density lipoprotein-cholesterol- lowering therapies to treat adults and adolescents aged 12 years and older with HoFH. Pursuant to the terms of the agreement, the Company received the rights to develop, commercialize and distribute the product for HoFH in countries outside of the U.S. The Company may be obligated to pay up to $63.0 million in future milestone payments, contingent upon the achievement of certain regulatory and sales milestones. The Company will share in certain costs for global trials led by Regeneron and also has the right to opt into other potential indications, including an exclusive right to negotiate a separate agreement with Regeneron to collaborate on the development and commercialization outside of the U.S. of Regeneron's investigational antibody for the treatment of fibrodysplasia ossificans progressiva (FOP). The Company’s option to negotiate an agreement for the treatment of FOP expired in July 2022.

The collaboration agreement is within the scope of ASC 808 which provides guidance on the presentation and disclosure of collaborative arrangements. As the Company would be the principal in future sale transactions with the customer, the Company will recognize product sales and cost of sales in the period the related sale occurs and the related revenue recognition criteria are met. Under the collaboration agreement, Regeneron will supply the product and will charge the Company a transfer price from the low 20% range up to 40% on net sales, which will be recognized as cost of sales in the Company's Statement of Operations.

Upon closing of the transaction in January 2022, the Company paid Regeneron a $30.0 million upfront payment. As the upfront payment was related to the Company's usage of intellectual property related to Evkeeza for HoFH, the upfront payment was recorded on the Consolidated Balance Sheet as an intangible asset, which is being amortized over its useful life of 10.5 years.

The Company recorded costs of goods sold of $0.7 million and $1.4 million for the three and six months ended June 30, 2022, respectively, related to the amortization of the intangible asset. Further, the Company reimbursed Regeneron for certain development costs of $1.3 million and $2.0 million for the three and six months ended June 30, 2022, respectively. No sales of Evkeeza were recorded for the three and six months ended June 30, 2022.

Abeona

In May 2022, the Company announced an exclusive License Agreement for the AAV gene therapy for UX111 with Abeona for the treatment of MPS IIIA. Under the terms of the agreement, the Company assumed responsibility for the UX111 program and in return, Abeona is eligible to receive tiered royalties of up to 10% on net sales and commercial milestone payments of up to $30.0 million following regulatory approval of the product. Additionally, the Company entered into an Assignment and Assumption Agreement with Abeona to transfer and assign to the Company the exclusive license agreement between Nationwide Children’s Hospital (NCH) and Abeona for certain rights related to UX111. Under this agreement, NCH is eligible to receive from the Company up to $1.0 million in development and regulatory milestones as well as royalties in the low single-digits of net sales.

The Company is obligated to pay Abeona certain prior development costs and other transition costs related to UX111. Prior to product regulatory approval, all consideration paid to Abeona represents rights to potential future benefits associated with

Abeona's in-process research and development activities, which have not reached technological feasibility and have no alternative future use. Accordingly, the value of the acquired intellectual property rights and clinical inventory as well as prior development costs and transition costs amounting to $2.1 million were recorded as research and development expense.

Other Arrangements

The Company has also entered into several collaborations and/or licensing arrangements in prior periods. Except as disclosed above, there have not been material changes in these arrangements as disclosed in Note 7 to the Consolidated Financial Statements in the Annual Report.

Under the financial terms of these arrangements, the Company may be required to make payments upon achievement of developmental, regulatory, and commercial milestones, which could be significant. Future milestone payments, if any, will be reflected in the Condensed Consolidated Statements of Operations upon the occurrence of the contingent event. In addition, the Company may be required to pay royalties on future sales if products related to these arrangements are commercialized. The payment of these amounts, however, is contingent upon the occurrence of various future events, which have a high degree of uncertainty of occurrence.

As described in the Annual Report, in connection with the Company's collaborations with Solid Biosciences, Inc. (Solid) and Arcturus Therapeutic Holding, Inc. (Arcturus), the Company holds certain equity interests in each entity. The changes in the fair value of the Company's equity investments in the common stock of Solid and Arcturus were as follows (in thousands):

	Common stock
As of December 31, 2020	$ 154,756
Change in fair value	(42,713)
Sale of shares	(79,843)
December 31, 2021	32,200
Change in fair value	(19,513)
June 30, 2022	$ 12,687

7. Liability Related to the Sale of Future Royalties

In December 2019, the Company entered into a Royalty Purchase Agreement with RPI. Pursuant to the agreement, RPI paid $320.0 million to the Company in consideration for the right to receive royalty payments effective January 1, 2020, arising from the net sales of Crysvita in the European Union, the United Kingdom, and Switzerland under the terms of the Company's Collaboration and License Agreement with KKC dated August 29, 2013, as amended (KKC Collaboration Agreement). The agreement with RPI will automatically terminate, and the payment of royalties to RPI will cease, in the event aggregate royalty payments received by RPI are equal to or greater than $608.0 million prior to December 31, 2030, or in the event aggregate royalty payments received by RPI are less than $608.0 million prior to December 31, 2030, when aggregate royalty payments received by RPI are equal to $800.0 million.

Proceeds from the transaction were recorded as a liability (liability related to sale of future royalties on the Consolidated Balance Sheets). The Company amortizes $320.0 million, net of transaction cost of $5.8 million, using the effective interest method over the estimated life of the arrangement. In order to determine the amortization of the liability, the Company is required to estimate the total amount of future royalty payments to be received by the Company and paid to RPI, subject to the capped amount, over the life of the arrangement. The excess of future estimated royalty payments (subject to the capped amount), over the $314.2 million of net proceeds, is recorded as non-cash interest expense over the life of the arrangement. Consequently, the Company estimates an imputed interest on the unamortized portion of the liability and records interest expense relating to the transaction. The Company records the royalty revenue arising from the net sales of Crysvita in the applicable European territories as non-cash royalty revenue in the Consolidated Statements of Operations over the term of the arrangement.

The Company periodically assesses the expected royalty payments using a combination of historical results, internal projections and forecasts from external sources. To the extent such payments are greater or less than the Company's initial estimates or the timing of such payments is materially different than its original estimates, the Company will prospectively adjust the amortization of the liability and the effective interest rate. The Company's effective annual interest rate was approximately 9.6% as of June 30, 2022.

There are a number of factors that could materially affect the amount and timing of royalty payments from KKC in the applicable European territories, most of which are not within the Company's control. Such factors include, but are not limited to, the success of KKC's sales and promotion of Crysvita, changing standards of care, delays or disruptions related to the COVID-19 pandemic, the introduction of competing products, pricing for reimbursement in various European territories, manufacturing or

other delays, intellectual property matters, adverse events that result in governmental health authority imposed restrictions on the use of Crysvita, significant changes in foreign exchange rates as the royalty payments are made in U.S. dollars (USD) while significant portions of the underlying European sales of Crysvita are made in currencies other than USD, and other events or circumstances that could result in reduced royalty payments from European sales of Crysvita, all of which would result in a reduction of non-cash royalty revenue and the non-cash interest expense over the life of the arrangement. Conversely, if sales of Crysvita in Europe are more than expected, the non-cash royalty revenue and the non-cash interest expense recorded by the Company would be greater over the term of the arrangement.

The following table shows the activity within the liability account (in thousands):

	Liability related to the sale of future royalties
December 31, 2020	$ 335,665
Non-cash collaboration royalty revenue	(17,951)
Non-cash interest expense	34,072
December 31, 2021	351,786
Non-cash collaboration royalty revenue	(10,261)
Non-cash interest expense	17,417
June 30, 2022	$ 358,942

8. Stock-Based Awards

The 2014 Incentive Plan (the 2014 Plan) provides for automatic annual increases in shares available for grant, beginning on January 1, 2015 through January 1, 2024. As of June 30, 2022, there were 1,972,064 shares reserved under the 2014 Plan for the future issuance of equity awards, 4,640,287 shares reserved for the 2014 Employee Stock Purchase Plan, and 403,963 shares reserved for the Employment Inducement Plan.

The table below sets forth the stock-based compensation expense for the periods presented (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Cost of sales	$ 330	$ 223	$ 505	$ 602
Research and development	20,430	15,094	37,337	28,583
Selling, general and administrative	15,105	11,825	27,410	22,255
Total stock-based compensation expense	$ 35,865	$ 27,142	$ 65,252	$ 51,440

9. Net Loss Per Share

Basic net loss per share has been computed by dividing the net loss by the weighted-average number of shares of common stock outstanding during the period. Diluted net loss per share is calculated by dividing net loss by the weighted-average number of shares of common stock and potential dilutive securities outstanding during the period.

The following weighted-average outstanding common stock equivalents were excluded from the computation of diluted net loss per share for the periods presented because including them would have been antidilutive:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	2021	2022	2021
Options to purchase common stock, restricted stock units, and performance stock units	11,982,625	8,491,447	10,680,380	8,287,698
Employee stock purchase plan	13,880	8,621	6,978	4,334
	11,996,505	8,500,068	10,687,358	8,292,032

10. Equity Transactions

In May 2021, the Company entered into an Open Market Sale Agreement with Jefferies LLC, (Jefferies), pursuant to which the Company may offer and sell shares of the Company's common stock having an aggregate offering proceeds up to $350.0 million, from time to time, in at-the-market (ATM) offerings through Jefferies. As of June 30, 2022, the Company has sold 1,050,372 shares under the arrangement resulting in net proceeds of approximately $78.9 million. No shares were sold under the arrangement for the three and six months ended June 30, 2022.

11. Accumulated Other Comprehensive Loss

Total accumulated other comprehensive loss consisted of the following (in thousands):

	June 30, 2022	December 31, 2021
Foreign currency translation adjustments	$ (912)	$ (121)
Unrealized loss on available-for-sale securities	(8,002)	(1,283)
Total accumulated other comprehensive loss	$ (8,914)	$ (1,404)

12. Subsequent events

Acquisition of GeneTx

In July 2022, the Company exercised its option to acquire GeneTx and entered into a Unit Purchase Agreement (the Purchase Agreement) pursuant to which the Company purchased all the outstanding units of GeneTx. In accordance with the terms of the Purchase Agreement, the Company paid $75.0 million in cash, subject to customary adjustments for working capital and transaction expenses. The Company is also required to make milestone payments of up to $115.0 million based on certain regulatory milestone events, including a milestone payment in an amount up to $30.0 million upon achievement of the earlier of initiation of a Phase 3 clinical study or product approvals in Canada and the U.K, and certain commercial milestone payments of up to $75.0 million based on specified worldwide net product sales, if such milestones are achieved. In addition, the Company is required to pay tiered royalties ranging from a mid-single to low double-digit percentage based on the Company's worldwide annual net sales of GTX-102 (as a single agent or in a combination product) and tiered royalties ranging from mid to high single-digit percentage based on the Company's worldwide annual net sales of products other than GTX-102 that are covered by certain of GeneTx's patents or are developed with, manufactured using, or practice GeneTx know-how. The Company is also required to pay 30% of the cash value of consideration received upon the sale by the Company of a priority review voucher (PRV) awarded by the FDA in connection with a rare pediatric product application for GTX-102 or $25.0 million if the Company chooses to retain the PRV.

Sale of Future Royalties to OMERS

In July 2022, the Company entered into a Royalty Purchase Agreement with OCM LS23 Holdings LP, an investment vehicle for OMERS. Pursuant to the agreement, OMERS paid $500.0 million to the Company in consideration for the right to receive 30% of the future royalty payments due to the Company from KKC based on net sales of Crysvita in the U.S. and Canada under the terms of the KKC Collaboration Agreement. The calculation of royalty payments to OMERS will be based on net sales of Crysvita beginning in April 2023 and will expire upon the earlier of the date on which aggregate payments received by OMERS equals $725.0 million or the date the final royalty payment is made to the Company under the KKC Collaboration Agreement.

Item 2. ***Management's Discussion and Analysis of Financial Condition and Results of Operations***

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with the accompanying unaudited Condensed Consolidated Financial Statements and related notes in Item 1 and with the audited Consolidated Financial Statements and the related notes included in our Annual Report on Form 10-K for the year ended December 31, 2021 ("Annual Report").

Overview

Ultragenyx Pharmaceutical Inc. (we or the Company) is a biopharmaceutical company focused on the identification, acquisition, development, and commercialization of novel products for the treatment of serious rare and ultra-rare genetic diseases. We target diseases for which the unmet medical need is high, the biology for treatment is clear, and for which there are typically no approved therapies treating the underlying disease. 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.

Approved Therapies and Clinical Product Candidates

Our current approved therapies and clinical-stage pipeline consist of four product categories: biologics, small molecules, gene therapy, and nucleic acid product candidates. See section entitled "Recent Program Updates" below for a description of recent updates to certain of our approved therapies and clinical-stage pipeline products.

Our biologic products include approved therapies Crysvita® (burosumab), Mepsevii® (vestronidase alfa), and Evkeeza® (evinacumab) and UX143 in clinical development:

- Crysvita is an antibody administered via subcutaneous injection that targets fibroblast growth factor 23 (FGF23), developed for the treatment of XLH, a rare, hereditary, progressive and lifelong musculoskeletal disorder characterized by renal phosphate wasting caused by excess FGF23 production. There are approximately 48,000 patients with XLH in the developed world, including approximately 36,000 adults and 12,000 children. Crysvita is the only approved treatment that addresses the underlying cause of XLH. Crysvita is approved in the U.S. and Canada for the treatment of XLH in adult and pediatric patients six months of age and older. In the European Union, or the EU, and the United Kingdom, Crysvita is approved for the treatment of XLH with radiographic evidence of bone disease in children one year of age and older, adolescents, and adults. In Brazil, Colombia, and Mexico, Crysvita is approved for treatment of XLH in adult and pediatric patients one year of age and older. We have submitted regulatory filings in various other Latin American countries.

  Crysvita is also approved in the U.S. and Canada for the treatment of FGF23-related hypophosphatemia in tumor-induced osteomalacia, or TIO, associated with phosphaturic mesenchymal tumors that cannot be curatively resected or localized in adults and pediatric patients 2 years of age and older. TIO can lead to severe hypophosphatemia, osteomalacia, fractures, fatigue, bone and muscle pain, and muscle weakness.

  We are collaborating with Kyowa Kirin Co., Ltd., or KKC (formerly Kyowa Hakko Kirin Co., Ltd., or KHK), and Kyowa Kirin, a wholly owned subsidiary of KKC, on the development and commercialization of Crysvita globally.

  In July 2022, we sold 30% of our royalty interest in Crysvita in the U.S. and Canada to OMERS for $500.0 million, subject to a cap. Royalty payments to OMERS under the agreement will be based on net sales of the product beginning from April 2023, upon transfer of commercial responsibilities in the applicable North American territories to KKC.
- Mepsevii is an intravenous, or IV, enzyme replacement therapy, developed for the treatment of Mucopolysaccharidosis VII, also known as MPS VII or Sly syndrome, a rare lysosomal storage disease that often leads to multi-organ dysfunction, pervasive skeletal disease, and death. MPS VII is one of the rarest MPS disorders, affecting an estimated 200 patients in the developed world. Mepsevii is approved in the U.S. for the treatment of children and adults with MPS VII. In the EU and the United Kingdom, Mepsevii is approved under exceptional circumstances for the treatment of non-neurological manifestations of MPS VII for patients of all ages. In Italy, Mepsevii received reimbursement approval for the treatment of pediatric and adult patients with MPS VII. In Brazil and Mexico, Mepsevii is approved for the treatment of MPS VII for patients of all ages.
- Evkeeza is a fully human monoclonal antibody that binds to and blocks the function of angiopoietin-like 3 (ANGPTL3), a protein that plays a key role in lipid metabolism. Evkeeza is an approved therapy for the treatment of homozygous familial hypercholesterolemia, or HoFH, a rare inherited condition. HoFH occurs when two copies of the familial hypercholesterolemia (FH)-causing genes are inherited, one from each parent, resulting in dangerously high levels (>400 mg/dL) of LDL-C, or bad cholesterol. Patients with HoFH are at risk for premature atherosclerotic disease and cardiac events as early as their teenage years. Evkeeza is approved in the U.S., where it is marketed by our partner Regeneron

Pharmaceuticals (Regeneron), and the European Economic Area (EEA) as a first-in-class therapy for use together with diet and other low-density lipoprotein-cholesterol (LDL-C) lowering therapies to treat adults and adolescents aged 12 years and older with HoFH. There are approximately 3,000 to 5,000 patients with HoFH in the Ultragenyx key markets.

- UX143 (setrusumab), which is subject to our collaboration agreement with Mereo Biopharma 3 (Mereo), and the lead clinical asset in our bone endocrinology franchise, is a fully human monoclonal antibody that inhibits sclerostin, a protein that acts on a key bone-signaling pathway by inhibiting the activity of bone-forming cells and promoting bone resorption. Setrusumab is being studied for the treatment of osteogenesis imperfecta (OI) and has received orphan drug designation from the U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA), rare pediatric disease designation from the FDA, and was accepted into the EMA's Priority Medicines program (PRIME). There are an estimated 60,000 patients in the developed world affected by OI. A Phase 2/3 study of setrusumab in pediatric and young adult patients with OI was initiated in April 2022 and a separate study is currently being planned for younger children and an extension study for adults with OI.

Our small molecule products include the approved therapy Dojolvi® (triheptanoin):

- Dojolvi is a highly purified, synthetic, 7-carbon fatty acid triglyceride specifically designed to provide medium-chain, odd-carbon fatty acids as an energy source and metabolite replacement for people with long-chain fatty acid oxidation disorders, or LC-FAOD, which is a set of rare metabolic diseases that prevents the conversion of fat into energy and can cause low blood sugar, muscle rupture, and heart and liver disease. Dojolvi is approved and commercially available in the U.S. and Canada as a source of calories and fatty acids for the treatment of pediatric and adult patients with molecularly confirmed LC-FAOD. We have received marketing authorization from the Brazilian Health Regulatory Agency (ANVISA) and are in the process of seeking reimbursement approval. There are approximately 8,000 to 14,000 patients in the developed world with LC-FAOD.

Our clinical-stage gene therapy pipeline includes UX111, DTX401, DTX301, DTX201 and UX701:

- UX111 (formerly ABO-102) is an adeno-associated virus 9, or AAV9, gene therapy product candidate for the treatment of patients with Sanfilippo syndrome type A (MPS IIIA), a rare lysosomal storage disease with no approved treatment that primarily affects the central nervous system (CNS). There are approximately 3,000 to 5,000 patients in the developed world affected by Sanfilippo syndrome type A. The UX111 program has received Regenerative Medicine Advanced Therapy, Fast Track, Rare Pediatric Disease, and Orphan Drug designations in the U.S., and PRIME and Orphan Medicinal Product designations in the EU. Patients have been dosed with UX111 and are currently being followed in the ongoing, pivotal *Transpher A* Study.
- DTX401 is an adeno-associated virus 8, or AAV8, gene therapy clinical candidate for the treatment of patients with glycogen storage disease type Ia, or GSDIa, a disease that arises from a defect in G6Pase, an essential enzyme in glycogen and glucose metabolism. GSDIa is the most common genetically inherited glycogen storage disease, with an estimated 6,000 patients in the developed world affected by GSDIa. A Pediatric Investigation Plan, or PIP, was accepted by the EMA. DTX401 has been granted Orphan Drug Designation in both the U.S. and in the EU, and Regenerative Medicine Advanced Therapy (RMAT) designation and Fast Track designation in the U.S. Patients are currently being enrolled and dosed in the Phase 3 study of DTX401.
- DTX301 is an AAV8 gene therapy product candidate designed for the treatment of patients with ornithine transcarbamylase, or OTC, deficiency. OTC is part of the urea cycle, an enzymatic pathway in the liver that converts excess nitrogen, in the form of ammonia, to urea for excretion. OTC deficiency is the most common urea cycle disorder, and there are approximately 10,000 patients in the developed world with OTC deficiency, of which we estimate approximately 80% are classified as late-onset, our target population. DTX301 has received Orphan Drug Designation in both the U.S. and in the EU and Fast Track Designation in the U.S. Phase 3 study start-up activities are ongoing with the first patients in the U.S. expected to enter a 4-to 8-week baseline screening period in the second half of 2022, after which they would receive a single dose of DTX301 or placebo.
- DTX201 is a Factor VIII gene therapy program for the treatment of hemophilia A that is being developed in collaboration with Bayer Healthcare LLC, or Bayer. Hemophilia A is the most common form of hemophilia with approximately 144,000 patients in the developed world. A number of patients across multiple cohorts have been dosed with DTX201.
- UX701 is an AAV type 9 gene therapy product candidate designed to deliver stable expression of a truncated version of the ATP7B copper transporter following a single intravenous infusion to patients with Wilson disease. Wilson disease affects more than 50,000 individuals in the developed world. UX701 was granted Orphan Drug Designation in the U.S. and EU. Patients with Wilson disease are currently being enrolled and dosed in the first stage of the *Cyprus2+*study of UX701.

Our clinical-stage nucleic acid pipeline includes GTX-102 for the treatment of Angelman syndrome, and UX053 for the treatment of GSDIII:

- GTX-102 is an antisense oligonucleotide, or ASO, that is being developed for the treatment of Angelman syndrome, a debilitating and rare neurogenetic disorder caused by loss-of-function of the maternally inherited allele of the UBE3A gene. There are an estimated 60,000 patients in the developed world affected by Angelman syndrome. GTX-102 was granted Fast Track designation, Orphan Drug Designation and Rare Pediatric Disease Designation from the FDA. GTX-102 is being developed in an ongoing Phase 1/2 clinical study in the U.S., Canada, and the U.K.
- UX053 is an mRNA product candidate designed for the treatment of patients with GSDIII, a disease caused by a glycogen debranching enzyme (AGL) deficiency that results in glycogen accumulation in the liver and muscle. GSDIII affects more than 10,000 patients in the developed world. UX053 was granted Orphan Drug Designation in the U.S. and EU. The single-dose portion of a Phase 1/2 study of UX053 is currently ongoing.

The following table summarizes our approved products and clinical product candidate pipeline:

Products	Description	Indication	IND Stage[1]	Phase 1	Phase 2	Phase 3	Approved	Upcoming Milestones
Biologics								
Crysvita® (burosumab)[2]	Anti-FGF23 monoclonal antibody	XLH						
Crysvita® (burosumab)[2]	Anti-FGF23 monoclonal antibody	TIO						
Mepsevii® (vestronidase alfa)	Enzyme replacement	MPSVII						
Evkeeza® (evinacumab)[3]	Fully human monoclonal antibody	HoFH						
UX143 (setrusumab)[4]	Fully human monoclonal antibody	OI						Ph2/3 dosing update and Ph3 transition 2H22
Small Molecules								
Dojolvi® (triheptanoin)	Substrate replacement	LC-FAOD						
AAV Gene Therapy								
UX111	AAV9 Gene Therapy	MPS IIIA						Regulatory update around end of year
DTX401	AAV8 Gene Therapy	GSDIa						Ongoing enrollment of Ph3
DTX301	AAV8 Gene Therapy	OTC						Ongoing enrollment of Ph3
DTX201[5]	AAVhu37 Gene Therapy	HemA						
UX701	AAV9 Gene Therapy	Wilson						Ongoing enrollment of Stage 1
Nucleic Acid								
GTX-102	Antisense Oligonucleotide	Angelman Syndrome						Ongoing enrollment of Ph1/2
UX053	mRNA	GSDIII						Ph1/2 single ascending dose data 2H22

1: IND submitted or expected to be submitted within the near term
2: In collaboration with Kyowa Kirin Company
3: Ex-US collaboration with Regeneron Pharmaceuticals
4: In collaboration with Mereo BioPharma
5: Out licensed to Bayer

Clinical Product Candidates

GTX-102 for the treatment of Angelman Syndrome

In July 2022, we exercised our option to acquire GeneTx and closed on the acquisition for a purchase price of $75.0 million plus future milestone and royalty payments. Additional milestones include $30.0 million upon achievement of the earlier of a Phase 3 clinical study start or product approvals in Canada and the U.K. We also owe tiered royalty payments ranging from a mid-single to low double-digit percentage based on worldwide annual net sales.

In July 2022, we provided an interim data update on patients treated in Canada and the U.K. and the U.S. under each region's amended protocol for the phase 1/2 study of GTX-102. Under these amended protocols, 14 patients have begun treatment with GTX-102, ten under the U.K. and Canada protocol, and four under the U.S. protocol. Of these, seven patients have received cumulative doses over 20 mg, and 13 patients have over 147 days of exposure to treatment. There have been no treatment-related serious adverse events of any type nor adverse events related to lower extremity weakness observed in these patients. Cerebrospinal fluid (CSF) protein levels have remained stable throughout the course of the study consistent with absence of inflammation. As of the data cut-off in June 2022, a total of 11 patients had reached at least the Day 128 evaluation, with three patients reaching the Day 170 Pre-Maintenance Dose (PMD) evaluation. We evaluated patients across various clinical measurements, including AS Change Scale, AS Severity Scale, the Bayley Scales of Infant and Toddler Development (Bayley-4), the Vineland-3 adaptive behavior scale, and the Observed Reported Communication Ability (ORCA). Early and some statistically, significant quantitative measures suggest a dose dependent effect that will require continued follow-up of these patients in the maintenance phase.

In the U.K. and Canada, we submitted a protocol amendment that was approved in May 2022 to add additional dose-selection cohorts that will sequentially enroll new patients at incrementally higher starting doses based on age. A younger Cohort 6 will begin dosing at 7.5 mg and an older Cohort 7 at 10 mg. Once clinically sufficient efficacy is observed, two expansion cohorts will enroll 20 patients in each age range using the starting dose determined from the dose-selection cohorts. The first patient under this amendment has been dosed and enrollment for both Cohorts 6 and 7 is ongoing. Re-dosing of patients from the first three U.S. cohorts (Original 5) was approved in the Canadian protocol. As of the date of this filing, one of the Original 5 patients has been dosed under this protocol, with no reported safety issues.

We currently expect the next program update will be once we have determined an optimal dose and gathered substantial data from the expansion cohorts.

UX111 for the treatment of Sanfilippo syndrome type A or MPS IIIA

In May 2022, we announced an exclusive license agreement with Abeona Therapeutics for UX111 (formerly ABO-102). Under the terms of the agreement, we assumed responsibility for the UX111 program and in return Abeona is eligible to receive tiered royalties of up to 10% on net sales and certain commercial milestone payments following regulatory approval.

Abeona previously announced the completion of a successful Type B meeting with the U.S. FDA regarding the pivotal *Transpher A* trial to support filing and approval for UX111. Interim results from the *Transpher A* trial presented in an encore presentation at the 2022 American Society of Gene & Cell Therapy (ASGCT) conference demonstrated that neurocognitive development was preserved in children treated younger than 2 years or in children older than 2 years with a development quotient (DQ) > 60 (n=10) within normal range of a non-afflicted child after treatment with ABO-102 (3.0 x 10^13 vg/kg). The interim results also showed continued or stabilized cognitive function along with behavioral and developmental progress using standard assessments. Additionally, stabilization or increase in volumes of cortical gray matter, total cerebral, and amygdala was observed. Statistically significant reduction in liver volume was seen with UX111 treatment. Dose-dependent and statistically significant reductions in cerebrospinal fluid and plasma heparan sulfate, demonstrating replacement of enzyme activity consistent with levels required for disease correction in the central nervous system, have been sustained in treated patients for two years after treatment. As of the presentation, there have been no treatment-related serious adverse events and no clinically meaningful adverse events.

DTX401 for the treatment of glycogen storage disease type Ia, or GSDIa

In May 2022, additional longer-term safety and durability data from the ongoing Phase 1/2 study presented at the 2022 ASGCT conference showed sustained responses lasting more than 3.5 years following treatment with DTX401. All 12 patients in the study have demonstrated reductions in oral glucose replacement therapy, with a mean total daily reduction of 70% (p-value<0.0001) from baseline to the last available timepoint. Data also showed additional improvements of greater time spent in euglycemia and reduced average daily cornstarch intake, as measured by continuous glucose monitoring were also presented. As of May 2022, across the Phase 1/2 study, there have been no infusion-related adverse events and no treatment-related serious adverse events (SAEs) reported.

We are currently enrolling and dosing patients in the Phase 3 *GlucoGene* study of DTX401. The Phase 3 study has a 48-week primary efficacy analysis period and we plan to enroll approximately 50 patients eight years of age and older, randomized 1:1 to DTX401 (1.0 x 10^13 GC/kg dose) or placebo. The primary endpoint is the reduction in oral glucose replacement with cornstarch while maintaining glucose control.

DTX301 for the treatment of ornithine transcarbamylase, or OTC, deficiency

In May 2022, additional longer-term safety and durability data from the ongoing Phase 1/2 study presented at the 2022 ASGCT conference showed sustained responses lasting more than 4 years following treatment with DTX301. Seven of 11 patients, including four out of the five patients treated at the Phase 3 dose (1.7 x 10^13 GC/kg), have responded, and remain clinically and metabolically stable. Four complete responders have discontinued ammonia-scavenger medications and liberalized their diet within one year. As of May 2022, across all cohorts of the Phase 1/2 study, no treatment-related serious adverse events, infusion-associated reactions or dose-limiting toxicities have been reported.

We are currently in the process of initiating the Phase 3 *Enh3ance* study that will include a 64-week primary efficacy analysis period and plan to enroll approximately 50 patients 12 years of age and older, randomized 1:1 to DTX301 (1.7 x 10^13 GC/kg dose) or placebo. The co-primary endpoints are the percentage of patients who achieve a response as measured by discontinuation or reduction in baseline disease management and the 24-hour plasma ammonia levels. The first patients in the U.S. are expected to enter an approximate 4-to 8-week baseline screening period in the second half of 2022, after which they are expected to receive a single dose of DTX301 or placebo.

UX701 for the treatment of Wilson Disease

We are currently enrolling and dosing patients with Wilson disease in the first stage of the *Cyprus2+* study of UX701.

During the first stage of the study, the safety and efficacy of up to three dose levels of UX701 will be evaluated over the course of 52 weeks and a dose will be selected for further evaluation in stage 2. The sequential doses to be evaluated are 5.0 x 10^12 GC/kg, 1.0 x 10^13 GC/kg, and 2.0 x 10^13 GC/kg. In stage 2, a new cohort of patients will be randomized 2:1 to receive the selected dose of UX701 or placebo. The primary safety and efficacy analyses will be conducted at Week 52 of stage 2. The primary efficacy endpoints are change in 24-hour urinary copper concentration and percent reduction in standard of care medication by Week 52. After the initial 52-week study period, all patients will receive long term follow up in stage 3.

UX143 (setrusumab) for the treatment of Osteogenesis Imperfecta (OI), in collaboration with Mereo BioPharma 3 Limited, or Mereo

We are currently dosing patients in a pediatric and young adult Phase 2/3 study. The objective of the Phase 2/3 study will first focus on determining the optimal dose based on increases in collagen production using serum P1NP levels and an acceptable safety profile. Following determination of the dose, we currently intend to adapt the study into a pivotal Phase 3 stage, evaluating fracture reduction over an estimated 15 to 24 months as the primary endpoint, subject to regulatory review. We currently expect a separate Phase 2 study of patients under age five with OI to start in the second half of 2022. We will also continue to follow adult patients who were previously treated in the Phase 2b ASTEROID study that was conducted by Mereo.

UX053 for the treatment of glycogen storage disease type III, or GSDIII

We are dosing patients in a Phase 1/2 study of UX053 for the treatment of GSDIII. Part 1 of the study is open label with single-ascending doses. Part 2 is double-blind and will evaluate repeat doses at escalating levels. We currently expect preliminary data from Part 1 of the study and to initiate Part 2 of the study in the second half of 2022.

Other Developments

Partial sale of North America Crysvita royalty

In July 2022, we announced that we sold 30% of our royalty interest in Crysvita in the U.S. and Canada beginning from April 2023 to OMERS for $500.0 million, subject to a cap of $725.0 million.

Financial Operations Overview

We are a biopharmaceutical company with a limited operating history. To date, we have invested substantially all of our efforts and financial resources in identifying, acquiring, and developing our products and product candidates, including conducting clinical studies and providing selling, general and administrative support for these operations. To date, we have funded our operations primarily from the sale of our equity securities, revenues from our commercial products, the sale of certain future royalties, and strategic collaboration arrangements.

We have incurred net losses in each year since inception. Our net loss was $158.2 million and $310.5 million for the three and six months ended June 30, 2022, respectively, and $122.4 million and $258.6 million for the three and six months ended June 30, 2021, respectively. Net loss for the three and six months ended June 30, 2022 included losses of $10.2 million and $19.5 million, respectively, resulting from changes in fair value of our investments in Arcturus Therapeutics Holdings Inc. (Arcturus) and Solid Biosciences Inc. (Solid) equity securities. Net losses for the three and six months ended June 30, 2021, included losses of $31.0 million and $51.7 million, respectively, resulting from changes in the fair value of our investments in Arcturus and Solid. Other than changes in the fair value of our investments, substantially all of our net losses have resulted from costs incurred in connection with our research and development programs and from selling, general and administrative costs associated with our operations.

For the three and six months ended June 30, 2022 our total revenues were $89.3 million and $169.3 million, respectively, compared to $87.0 million and $186.4 million for the three and six months ended June 30, 2021, respectively. Revenue for the three and six months ended June 30, 2022 included $1.5 million and $4.7 million, respectively, from our collaboration and license agreement with Daiichi Sankyo Co., Ltd. (Daiichi Sankyo), as compared to $22.0 million and $64.7 million for the three and six months ended June 30, 2021, respectively. The decrease in collaboration revenue with Daiichi Sankyo was partially offset by higher revenue from Crysvita collaboration revenue in the profit-share territory, an increase in revenue for our approved products, and an increase in collaboration royalty revenue.

As of June 30, 2022, we had $706.1 million in available cash, cash equivalents, and marketable debt securities.

Critical Accounting Estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our Condensed Consolidated Financial Statements, which have been prepared in accordance with U.S. generally accepted accounting principles, or GAAP. The preparation of these Condensed Consolidated 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. There have been no material changes in our critical accounting policies during the six months ended June 30, 2022, as compared to those disclosed in "Management's Discussion and Analysis of Financial Condition and Results of Operations — Critical Accounting Policies and Significant Judgments and Estimates" in our Annual Report.

Results of Operations

Comparison of the three and six months ended June 30, 2022 to the three and six months ended June 30, 2021:

Revenue (dollars in thousands)

	Three Months Ended June 30,		Dollar	%
	2022	2021	Change	Change
Collaboration and license revenue:				
Crysvita collaboration revenue in profit-share territory	$ 51,609	$ 41,756	$ 9,853	24%
Crysvita royalty revenue in European territory	—	228	(228)	-100%
Daiichi Sankyo	1,479	21,956	(20,477)	-93%
Total collaboration and license revenue	53,088	63,940	(10,852)	-17%
Product sales:				
Crysvita	12,402	2,900	9,502	328%
Mepsevii	4,933	5,399	(466)	-9%
Dojolvi	13,497	10,047	3,450	34%
Total product sales	30,832	18,346	12,486	68%
Crysvita non-cash collaboration royalty revenue	5,423	4,689	734	16%
Total revenues	$ 89,343	$ 86,975	$ 2,368	3%

	Six Months Ended June 30,		Dollar	%
	2022	2021	Change	Change
Collaboration and license revenue:				
Crysvita collaboration revenue in profit-share territory	$ 96,773	$ 78,016	$ 18,757	24%
Crysvita royalty revenue in European territory	—	228	(228)	-100%
Daiichi Sankyo	4,728	64,706	(59,978)	-93%
Total collaboration and license revenue	101,501	142,950	(41,449)	-29%
Product sales:				
Crysvita	21,796	8,772	13,024	148%
Mepsevii	9,794	9,006	788	9%
Dojolvi	25,926	17,081	8,845	52%
Total product sales	57,516	34,859	22,657	65%
Crysvita non-cash collaboration royalty revenue	10,261	8,561	1,700	20%
Total revenues	$ 169,278	$ 186,370	$ (17,092)	-9%

For the three and six months ended June 30, 2022, our share of Crysvita collaboration revenue in the profit-share territory increased by $9.9 million and $18.8 million, respectively, as compared to the same periods in 2021. The increase primarily reflects the continuing increase in demand for Crysvita due to an increase in the number of patients on therapy.

In March 2020, we executed a license agreement with Daiichi Sankyo. For the three and six months ended June 30, 2022, the collaboration and license revenue from this arrangement decreased by $20.5 million and $60.0 million, respectively, as compared to the same periods in 2021. The decrease in the three and six months periods ended June 30, 2022 was related to the relative progress toward complete satisfaction of the individual performance obligation using an input measure of the technology transfer period, which was completed as of March 31, 2022.

The increase in product sales of $12.5 million and $22.7 million for the three and six months ended June 30, 2022, respectively, compared to the same periods in 2021 was primarily due to continued momentum from the commercial launch of Dojolvi in the U.S., continuing increase in demand for our other approved products, and an increase in sales of our products under our named patient program in certain countries.

The increase in Crysvita non-cash collaboration royalty revenue of $0.7 million and $1.7 million for the three and six months ended June 30, 2022, respectively, compared to the same periods in 2021 primarily reflects the launch progress by our collaboration partner in European countries and an increase in the number of patients on therapy.

Cost of Sales (dollars in thousands)

	Three Months Ended June 30,		Dollar	%
	2022	2021	Change	Change
Cost of sales	$ 8,270	$ 3,136	$ 5,134	164%

	Six Months Ended June 30,		Dollar	%
	2022	2021	Change	Change
Cost of sales	$ 14,370	$ 8,324	$ 6,046	73%

Cost of sales increased by $5.1 million and $6.0 million for the three and six months ended June 30, 2022, respectively, compared to the same periods in 2021. The increase was due to increased demand for our approved products, and amortization of the intangible asset related to our license from Regeneron for Evkeeza. This was also impacted by excess inventory write-downs of $1.2 million and $1.3 million recorded for the three and six months ended June 30, 2022, respectively, compared to nil and $1.7 million recorded for the three and six months ended June 30, 2021, respectively.

Research and Development Expenses (dollars in thousands)

Research and development expenses include internal and external costs incurred for research and development of our programs and program candidates and expenses related to certain technology that we acquire or license through business development transactions. These expenses consist primarily of clinical studies performed by contract research organizations, manufacturing of drug substance and drug product performed by contract manufacturing organizations, materials and supplies, fees from collaborative and other arrangements including milestones, licenses and other fees, personnel costs including salaries, benefits and stock-based compensation, and overhead allocations consisting of various support and infrastructure costs.

Commercial programs include costs for disease monitoring programs and certain regulatory and medical affairs support activities for programs after commercial approval. Clinical programs include study conduct and manufacturing costs related to clinical program candidates. Translational research includes costs for preclinical study work and costs related to preclinical programs prior to IND filing. Upfront license and milestone fees include any significant expenses related to strategic licensing agreements. Infrastructure costs include direct costs related to laboratory, IT, and equipment depreciation costs, and overhead allocations for human resources, IT and other allocable costs.

The following table provides a breakout of our research and development expenses by major program type and business activities:

	Three Months Ended June 30,		Dollar	%
	2022	2021	Change	Change
Commercial programs	$ 16,279	$ 12,641	$ 3,638	29 %
Clinical programs:				
Gene therapy programs	35,743	29,735	6,008	20 %
Nucleic acid and other biologic programs	21,852	10,571	11,281	107 %
Translational research	21,315	16,647	4,668	28 %
Infrastructure	17,683	14,374	3,309	23 %
Stock-based compensation	20,430	15,094	5,336	35 %
Other research and development	21,227	14,143	7,084	50 %
Total research and development expenses	$ 154,529	$ 113,205	$ 41,324	37 %

	Six Months Ended June 30,		Dollar	%
	2022	2021	Change	Change
Commercial programs	$ 29,343	$ 26,302	$ 3,041	12 %
Clinical programs:				
Gene therapy programs	76,023	51,097	24,926	49 %
Nucleic acid and other biologic programs	42,326	19,595	22,731	116 %
Translational research	41,642	29,260	12,382	42 %
Upfront license and milestone fees	—	50,000	(50,000)	-100 %
Infrastructure	34,067	29,236	4,831	17 %
Stock-based compensation	37,337	28,583	8,754	31 %
Other research and development	36,946	26,650	10,296	39 %
Total research and development expenses	$ 297,684	$ 260,723	$ 36,961	14 %

Total research and development expenses increased $41.3 million and $37.0 million for the three and six months ended June 30, 2022, respectively, compared to the same periods in 2021. The change in research and development expenses was primarily due to:

- for commercial programs, an increase of $3.6 million and $3.0 million for the three and six months ended June 30, 2022, respectively, primarily related to collaborative cost sharing with Regeneron for Evkeeza and increased R&D personnel allocations to commercial programs;
- for gene therapy programs, an increase of $6.0 million and $24.9 million for the three and six months ended June 30, 2022, respectively, primarily related to increases in clinical manufacturing expenses for DTX401 and DTX301 and the in-licensing of the UX111 program from Abeona Therapeutics;
- for nucleic acid and other biologic programs, an increase of $11.3 million and $22.7 million for the three and six months ended June 30, 2022, respectively, primarily related to the addition of clinical trial and manufacturing expenses related to UX053, following its IND approval in March 2021; increased clinical trial and manufacturing expenses related to the continued progress of the UX143 program in collaboration with Mereo; and expenses related to the continued progress of the GTX-102 program through GeneTx;
- for translational research, an increase of $4.7 million and $12.4 million for the three and six months ended June 30, 2022, respectively, primarily related to IND-enabling development costs for multiple research projects;
- for upfront license and milestone fees, a decrease of $50.0 million for the six months ended June 30, 2022 due to no fees incurred for the six months ended June 30, 2022, as compared to the $50.0 million upfront fee recognized for the Mereo license for the six months ended June 30, 2021;

- for infrastructure, an increase of $3.3 million and $4.8 million for the three and six months ended June 30, 2022, respectively, primarily related to increased expenses for support of our clinical and research program pipeline, expansion of laboratory space, implementation of COVID-related policies and safety protocols, depreciation of laboratory-related leasehold improvements and equipment, and IT-related expenses;
- for stock-based compensation, an increase of $5.3 million and $8.8 million for the three and six months ended June 30, 2022, respectively, primarily related to an increase in employee headcount; and
- for other research and development expenses, an increase of $7.1 million and $10.3 million for the three and six months ended June 30, 2022, respectively, primarily related to increased staffing to support internal manufacturing, increased travel, and increased administrative and general support.

We expect our annual research and development expenses to continue to increase in the future as we advance our product candidates through clinical development. The timing and amount of expenses incurred will depend largely upon the outcomes of current or future clinical studies for our product candidates as well as the related regulatory requirements, manufacturing costs, and any costs associated with the advancement of our preclinical programs.

Selling, General and Administrative Expenses (dollars in thousands)

	Three Months Ended June 30,		Dollar	%
	2022	2021	Change	Change
Selling, general and administrative	$ 68,137	$ 53,410	$ 14,727	28%

	Six Months Ended June 30,		Dollar	%
	2022	2021	Change	Change
Selling, general and administrative	$ 135,449	$ 106,668	$ 28,781	27%

Selling, general and administrative expenses increased by $14.7 million and $28.8 million for the three and six months ended June 30, 2022, respectively, compared to the same periods in 2021. The increases in selling, general and administrative expenses were primarily due to increases in personnel costs resulting from an increase in the number of employees in support of our commercial activities, commercialization costs, and professional services costs.

We expect selling, general and administrative expenses to continue to increase in the future to support our organizational growth related to our approved products and multiple clinical-stage product candidates.

Interest Income (dollars in thousands)

	Three Months Ended June 30,		Dollar	%
	2022	2021	Change	Change
Interest income	$ 899	$ 441	$ 458	104%

	Six Months Ended June 30,		Dollar	%
	2022	2021	Change	Change
Interest income	$ 1,393	$ 1,080	$ 313	29%

Interest income increased by $0.5 million and $0.3 million, for the three and six months ended June 30, 2022, respectively, compared to the same periods in 2021, primarily due to fluctuations in interest rates.

Change in Fair Value of Equity Investments (dollars in thousands)

	Three Months Ended June 30,		Dollar	%
	2022	2021	Change	Change
Change in fair value of equity investments	$ (10,184)	$ (31,046)	$ 20,862	-67%

	Six Months Ended June 30,		Dollar	%
	2022	2021	Change	Change
Change in fair value of equity investments	$ (19,513)	$ (51,665)	$ 32,152	-62%

For the three and six months ended June 30, 2022, we recorded a net decrease in the fair value of our equity investments of $10.2 million and $19.5 million, respectively, due to unrealized losses on our investments in Arcturus common stock of $5.6 million

and $10.6 million during the three and six months ended June 30, 2022, respectively, and unrealized losses on our investments in Solid common stock of $4.6 million and $8.9 million during the three and six months ended June 30, 2022, respectively.

For the three and six months ended June 30, 2021, we recorded a net decrease in the fair value of our equity investments of $31.0 million and $51.7 million, respectively, due to unrealized losses on our investments in Arcturus common stock of $16.4 million and $21.0 million during the three and six months ended June 30, 2021, respectively, and unrealized losses on our investments in Solid common stock of $14.6 million and $30.7 million during the three and six months ended June 30, 2021, respectively.

Given the historic volatility of the publicly traded stock price of Arcturus and Solid, the fair value adjustments of our equity investments may be subject to wide fluctuations which may have a significant impact on our earnings in future periods.

Non-cash Interest Expense on Liability Related to the Sale of Future Royalties (dollars in thousands)

	Three Months Ended June 30,		Dollar	%
	2022	2021	Change	Change
Non-cash interest expense on liability related to the sale of future royalties	$ (6,052)	$ (8,517)	$ 2,465	-29%

	Six Months Ended June 30,		Dollar	%
	2022	2021	Change	Change
Non-cash interest expense on liability related to the sale of future royalties	$ (12,636)	$ (16,935)	$ 4,299	-25%

The non-cash interest expense on liability related to the sale of future royalties decreased by $2.5 million and $4.3 million for the three and six months ended June 30, 2022, respectively, compared to the same periods in 2021 due to the capitalization of interest related to the construction-in-progress for the gene therapy manufacturing plant, slightly offset by the increase in the liability related to the sale of future royalties for net sales of Crysvita in the European territory. To the extent the royalty payments are greater or less than our initial estimates or the timing of such payments is materially different than our original estimates, we will prospectively adjust the effective interest rate.

Other Expense (dollars in thousands)

	Three Months Ended June 30,		Dollar	%
	2022	2021	Change	Change
Other expense	$ (930)	$ (67)	$ (863)	*

* not meaningful

	Six Months Ended June 30,		Dollar	%
	2022	2021	Change	Change
Other expense	$ (641)	$ (862)	$ 221	-26%

Other expense increased by $0.9 million and decreased by $0.2 million, respectively, for the three and six months ended June 30, 2022, compared to the same periods in 2021. These changes were primarily due to fluctuations in foreign exchange rates.

Provision for Income Taxes (dollars in thousands)

	Three Months Ended June 30,		Dollar	%
	2022	2021	Change	Change
Provision for income taxes	$ (302)	$ (463)	$ 161	-35%

	Six Months Ended June 30,		Dollar	%
	2022	2021	Change	Change
Provision for income taxes	$ (860)	$ (842)	$ (18)	2%

The provision for incomes taxes decreased and increased by a nominal amount for the three and six months ended June 30, 2022, respectively, compared to the same periods in 2021.

Liquidity and Capital Resources

To date, we have funded our operations primarily from the sale of our equity securities, revenues from our commercial products, the sale of certain future royalties, and strategic collaboration arrangements.

As of June 30, 2022, we had $706.1 million in available cash, cash equivalents, and marketable debt securities. We believe that our existing capital resources will be sufficient to fund our projected operating requirements for at least the next twelve months. Our cash, cash equivalents, and marketable debt securities are held in a variety of deposit accounts, interest-bearing accounts, corporate bond securities, commercial paper, U.S government securities, asset-backed securities, debt securities in government-sponsored entities, and money market funds. Cash in excess of immediate requirements is invested with a view toward liquidity and capital preservation, and we seek to minimize the potential effects of concentration and credit risk.

In July 2022, we sold 30% of our royalty interest in Crysvita in the U.S. and Canada to OMERS for $500.0 million, subject to a cap, beginning in April 2023 upon the transfer of commercial responsibilities in the applicable North American territories to KKC.

In May 2021, we entered into an Open Market Sale Agreement with Jefferies LLC, (Jefferies), pursuant to which we may offer and sell shares of our common stock having an aggregate offering proceeds up to $350.0 million, from time to time, in at-the-market (ATM) offerings through Jefferies. As of June 30, 2022, net proceeds from shares sold under the arrangement were approximately $78.9 million. No shares were sold under this arrangement for the three and six months ended June 30, 2022.

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

	Six Months Ended June 30,	
	2022	2021
Cash used in operating activities	$ (194,778)	$ (224,702)
Cash provided by (used in) investing activities	41,806	(223,670)
Cash provided by financing activities	7,449	25,277
Effect of exchange rate changes on cash	(1,346)	(323)
Net decrease in cash, cash equivalents and restricted cash	$ (146,869)	$ (423,418)

Cash Used in Operating Activities

Our primary use of cash is to fund operating expenses, which consist primarily of research and development and commercial 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 affected by the timing of when we pay these expenses, as reflected in the change in our outstanding accounts payable and accrued expenses.

Cash used in operating activities for the six months ended June 30, 2022 was $194.8 million and primarily reflected a net loss of $310.5 million and $10.3 million for non-cash collaboration royalty revenues related to the sale of future royalties to RPI Finance Trust (RPI), an affiliate of Royalty Pharma, offset by non-cash charges of $65.3 million for stock-based compensation, $3.4 million for the amortization of the premium paid on purchased marketable debt securities, $8.6 million for depreciation and amortization, $19.5 million for a change in fair value of equity investments in Arcturus and Solid, and $12.6 million for non-cash interest incurred on the liability related to the sale of future royalties to RPI. Cash used in operating activities also reflected a $8.9 million decrease due to an increase in accounts receivable primarily related to order timing, a $4.7 million decrease due to an increase in inventory primarily for Dojolvi and Mepsevii, a $6.8 million decrease due to an increase in prepaid expenses and other assets primarily due to an increase in prepaid manufacturing, partially offset by a decrease in receivables due from collaboration partners, and a decrease of $4.6 million in contract liabilities, net, related to the revenue recognized from the license agreements with Daiichi Sankyo, offset by a $41.3 million increase in accounts payable, accrued, and other liabilities primarily due to an increase in manufacturing and 2022 annual bonus accruals and an increase in accounts payable due to timing of payments, partially offset by the payout of the 2021 annual bonuses.

Cash used in operating activities for the six months ended June 30, 2021 was $224.7 million and reflected a net loss of $258.6 million and $8.6 million for non-cash collaboration royalty revenues related to the sale of future royalties to RPI, offset by non-cash charges of $51.3 million for stock-based compensation, $2.5 million for the amortization of the premium paid on purchased marketable debt securities, $6.5 million for depreciation and amortization, $51.7 million for a change in fair value of equity investments from Arcturus and Solid, and $16.9 million for non-cash interest incurred on the liability related to the sale of future royalties to RPI. Cash used in operating activities also reflected a $1.7 million decrease due to an increase in accounts receivable primarily related to higher revenues, a $2.2 million decrease due to an increase in inventory, a $9.0 million decrease due to an increase in prepaid expenses and other current assets primarily due to an increase in prepaid subscriptions, prepaid clinical studies, and prepaid fixed assets, a $11.5 million decrease in accounts payable, accrued liabilities, and other liabilities primarily due to the payout of 2020 annual bonuses, and a decrease of $62.7 million in contract liabilities, related to the revenue recognized from the license agreements with Daiichi Sankyo.

Cash Provided by (Used in) Investing Activities

Cash provided by investing activities for the six months ended June 30, 2022 was $41.8 million and was primarily related to proceeds from the sale of marketable debt securities of $43.0 million and maturities of marketable debt securities of $297.3 million,

offset by purchases of property, plant, and equipment of $63.1 million, primarily related to purchases for our gene therapy manufacturing plant, purchases of marketable debt securities of $205.3 million, and the payment to Regeneron for an intangible asset of $30.0 million.

Cash used in investing activities for the six months ended June 30, 2021 was $223.7 million and related to purchases of property, plant, and equipment of $36.5 million and purchases of marketable debt securities of $664.3 million, offset by proceeds from the sale of marketable debt securities of $70.5 million and maturities of marketable debt securities of $406.6 million.

Cash Provided by Financing Activities

Cash provided by financing activities for the six months ended June 30, 2022 was $7.4 million and was primarily comprised of $7.7 million in net proceeds from the issuance of common stock pursuant to equity plan awards.

Cash provided by financing activities for the six months ended June 30, 2021 was $25.3 million and was primarily comprised of $25.5 million in net proceeds from the issuance of common stock pursuant to equity plan awards.

Funding Requirements

We anticipate that, excluding non-recurring items, we will continue to generate annual losses for the foreseeable future as we continue the development of, and seek regulatory approvals for, our product candidates, and continue with commercialization of approved products. We will require additional capital to fund our operations, to complete our ongoing and planned clinical studies, to commercialize our products, to continue investing in early-stage research capabilities to promote our pipeline growth, to continue to acquire or invest in businesses or products that complement or expand our business, and to further develop our general infrastructure, including construction of our GMP gene therapy manufacturing facility, and such funding may not be available to us on acceptable terms or at all.

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 the scope of, or terminate one or more of our clinical studies, research and development programs, 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, products that we have begun to commercialize, and any products that we may develop in the future, including the construction of our own GMP gene therapy manufacturing plant;
- the number and characteristics of product candidates that we pursue;
- the cost, timing, and outcomes of regulatory approvals;
- the cost and timing of establishing our commercial infrastructure, and distribution capabilities;
- the magnitude and extent to which the COVID-19 pandemic impacts our business operations and operating results, as described in “Risk Factors – Risks Related to Our Business Operations;” and
- the terms and timing of any collaborative, licensing, marketing, distribution, acquisition, and other arrangements that we may establish, including any required upfront milestone, royalty, reimbursements or other payments thereunder.

We expect to satisfy future cash needs through existing capital balances, revenue from our commercial products, and through some combination of public or private equity offerings, debt financings, royalty sales, collaborations, strategic alliances, licensing arrangements, and other marketing and distribution arrangements. Please see "Risk Factors—Risks Related to Our Financial Condition and Capital Requirements."

Contractual Obligations and Commitments

Material contractual obligations arising in the normal course of business primarily consist of operating and finance leases and manufacturing and service contract obligations.

Manufacturing and service contract obligations primarily relate to manufacturing of inventory for our approved products. As of June 30, 2022, we had obligations of approximately $35.6 million, of which $34.9 million are due within one year.

The terms of certain of our licenses, royalties, development and collaboration agreements, as well as other research and development activities, require us to pay potential future milestone payments based on product development success. The amount and timing of such obligations are unknown or uncertain.

See Note 12 for information regarding our acquisition of GeneTx in July 2022.

Other than the update in manufacturing and service contract obligations and our acquisition of GeneTx noted above, there have been no material changes in our contractual obligations and commitments as compared to those disclosed in "Management's Discussion and Analysis of Financial Condition and Results of Operations — Contractual Obligations" in our Annual Report.

Off-Balance Sheet Arrangements

We have not engaged in any off-balance sheet arrangements, as contemplated by the rules and regulations of the SEC.

Item 3. ***Quantitative and Qualitative Disclosures about Market Risk***

Equity Risk

We have exposure to equity risk with respect to the equity investments that we hold in Arcturus and Solid. The carrying value of our equity investments held in Arcturus and Solid were $7.9 million and $4.8 million as of June 30, 2022, respectively. A hypothetical 10 percent decrease in the market price for our equity investments in Arcturus and Solid as of June 30, 2022 would decrease the fair value by $0.8 million and $0.5 million, respectively. Given the historic volatility of the publicly traded stock price of Arcturus and Solid, the fair value of our investments in Arcturus and Solid is subject to wide fluctuations which may have a significant impact on our net income (loss) in future periods.

Interest Rate Risk

Our exposure to market risk for changes in interest rates relates primarily to interest earned on our cash equivalents and marketable debt 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 June 30, 2022, we had cash, cash equivalents, and marketable debt securities totaling $706.1 million, compared to $999.1 million as of December 31, 2021, which include bank deposits, money market funds, U.S. government treasury and agency securities, and investment-grade corporate bond securities 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 continue to increase. A hypothetical 100 basis point change in interest rates during any of the periods presented would not have had a material impact on the fair market value of our cash equivalents and marketable debt securities as of June 30, 2022 or December 31, 2021. To date, we have not experienced a loss of principal on any of these investments and as of June 30, 2022, we did not record any allowance for credit loss from our investments.

Foreign Currency Risk

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. Volatile market conditions arising from the COVID-19 pandemic or global political instability may result in significant changes in exchange rates, and in particular a weakening of foreign currencies relative to the U.S. dollar may negatively affect our revenue and operating income as expressed in U.S. dollars. An adverse movement in foreign exchange rates could have a material effect on payments made to foreign suppliers and for license agreements. For the three and six months ended June 30, 2022, a majority of our revenue and expense activities and capital expenditures were denominated in U.S. dollars. A hypothetical 10% change in foreign exchange rates during any of the periods presented would not have had a material impact on our Condensed Consolidated Financial Statements.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Management carried out an evaluation, under the supervision and with the participation of our Chief Executive Officer and our Chief Financial Officer, of the effectiveness of our "disclosure controls and procedures" as of the end of the period covered by this Quarterly Report, pursuant to Rules 13a-15(b) and 15d-15(b) under the Securities Exchange Act of 1934, or the Exchange Act. In connection with that evaluation, our Chief Executive Officer and our Chief Financial Officer concluded that our disclosure controls and procedures were effective and designed to provide reasonable assurance that the information required to be disclosed is recorded, processed, summarized and reported within the time periods specified in the SEC rules and forms as of June 30, 2022. For the purpose of this review, disclosure controls and procedures means controls and procedures designed to ensure that information required to be disclosed by us in the reports that we file or submit is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. These disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports that we file or submit is accumulated and communicated to management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosure. In designing and evaluating the disclosure controls and procedures, our management recognized that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and our management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Changes in Internal Control over Financial Reporting

There have been no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the quarter ended June 30, 2022, that has materially affected, or is reasonably likely to materially affect our internal control over financial reporting.

PART II. OTHER INFORMATION

Item 1. *Legal Proceedings*

We are not currently a party to any material legal proceedings. We may, however, in the ordinary course of business face various claims brought by third parties or government regulators and we may, from time to time, make claims or take legal actions to assert our rights, including claims relating to our directors, officers, stockholders, intellectual property rights, employment matters and the safety or efficacy of our products. Any of these claims could subject us to costly litigation and, while we generally believe that we have adequate insurance to cover many different types of liabilities, our insurance carriers may deny coverage, may be inadequately capitalized to pay on valid claims, or our policy limits may be inadequate to fully satisfy any damage awards or settlements. If this were to happen, the payment of any such awards could have a material adverse effect on our consolidated operations, cash flows and financial position. Additionally, any such claims, whether or not successful, could damage our reputation and business.

Item 1A. *Risk Factors*

Investing in our common stock involves a high degree of risk. You should carefully consider the following material risks, together with all the other information in this Quarterly Report, including our financial statements and notes thereto, before deciding to invest in our common stock. The risks and uncertainties described below are not the only ones we face. Additional risk and uncertainties not presently known to us or that we presently deem less significant may also impair our business operations. If any of the following risks actually materialize, our operating results, financial condition, and liquidity could be materially adversely affected. As a result, the trading price of our common stock could decline and you could lose part or all of your investment. Our company's business, financial

condition and operating results can be affected by a number of factors, whether currently known or unknown, including but not limited to those described below, any one or more of which could, directly or indirectly, cause our actual financial condition and operating results to vary materially from past, or from anticipated future, financial condition and operating results. Any of these factors, in whole or in part, could materially and adversely affect our business, prospects, financial condition, operating results and stock price.

Because of the following factors, as well as other factors affecting our financial condition and operating results, past financial performance should not be considered to be a reliable indicator of future performance, and investors should not use historical trends to anticipate results or trends in future periods.

The following description of the risk factors associated with our business includes any material changes to and supersedes the description of the risk factors associated with our business previously disclosed in Part I, Item 1A of the Annual Report.

Risk Factor Summary

- We have a history of operating losses and anticipate that we will continue to incur losses for the foreseeable future.
- We have limited experience in generating revenue from product sales.
- We expect to need to raise additional capital to fund our activities.
- Clinical drug development is a lengthy, complex, and expensive process with uncertain outcomes.
- If we do not achieve our projected development goals in the time frames we announce and expect, we may experience delays in commercialization of our products and other adverse effects.
- We may experience difficulty in enrolling patients, which could delay or prevent clinical studies of our product candidates.
- The regulatory approval processes of the FDA and comparable foreign authorities are lengthy and inherently unpredictable.
- Our product candidates may cause undesirable or serious side effects that could delay or prevent their regulatory approval or result in other negative consequences.
- We face a multitude of manufacturing risks, particularly with respect to our gene therapy and mRNA product candidates.
- Our products will remain subject to regulatory scrutiny even if we obtain regulatory approval.
- Product liability lawsuits against us could cause us to incur substantial liabilities.
- We may not realize the full commercial potential of our product candidates if we are unable to source and develop effective biomarkers.
- We rely on third parties to conduct our nonclinical and clinical studies and perform other tasks for us.
- We are dependent on KKC for the clinical and commercial supply of Crysvita for all major markets and for the development and commercialization of Crysvita in certain major markets.
- We rely on third parties to manufacture our products and product candidates.
- The loss of, or failure to supply by, any of any of our single-source suppliers for our drug substance and drug product could adversely affect our business.
- The actions of distributors and specialty pharmacies could affect our ability to sell or market products profitably.
- A competitor could misappropriate or disclose our trade secrets.
- Our revenue may be adversely affected if the market opportunities for our products and product candidates are smaller than expected.
- Our competitors may develop therapies that are similar, more advanced, or more effective than ours.
- We may not successfully manage expansion of our company, including building an integrated commercial organization.
- Our exclusive rights to promote Crysvita in the U.S. and Canada will transition back to KKC.
- Commercial success of our products depends on the degree of market acceptance by physicians, patients, third-party payors, and others in the medical community.
- We face uncertainty related to insurance coverage and reimbursement status of our newly approved products.
- If we, or our third-party partners, are unable to maintain effective proprietary rights for our products or product candidates, we may not be able to compete effectively.
- Claims of intellectual property infringement may prevent or delay our development and commercialization efforts.
- We may not be successful in obtaining or maintaining necessary rights to our product candidates through acquisitions and in-licenses.
- We may face competition from biosimilars of our biologics product and product candidates or from generic versions of our small-molecule product and product candidates, which may result in a material decline in sales of affected products.
- We could lose license rights that are important to our business if we fail to comply with our obligations in the agreements under which we license intellectual property and other rights from third parties.

- We may become involved in lawsuits to protect or enforce our patents or the patents of our licensors, or be subject to claims that challenge the inventorship or ownership of our patents.
- Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.
- We may not be able to protect our intellectual property rights throughout the world.
- The ongoing COVID-19 pandemic has impacted our operations and could materially and adversely affect our business and operating results.
- We have no experience as a company developing or operating a manufacturing facility.
- Our success depends in part on our ability to retain our President and Chief Executive Officer and other qualified personnel.
- Our revenue may be impacted if we fail to obtain or maintain orphan drug exclusivity for our products.
- Our operating results may be adversely impacted if our intangible assets become impaired.
- We may not be successful in identifying, licensing, developing, or commercializing additional product candidates, or we may fail to capitalize on opportunities that may be more profitable.
- We may fail to comply with laws and regulations or changes in laws and regulations could adversely affect our business.
- We are exposed to risks related to international expansion of our business outside of the U.S.
- Our business may be adversely affected in the event of computer system failures or security breaches.
- We or our third-party partners may be adversely affected by earthquakes or other serious natural disasters that are not adequately protected by business continuity and disaster recovery plans.
- We may incur various costs and expenses and risks related to acquisition of companies or products or strategic transactions.
- The market price of our common stock is highly volatile.
- Future sales and issuances of our common stock could dilute the percentage ownership of our current stockholders and result in a decline in stock price.
- We do not intend to pay dividends on our common stock so any returns will be limited to the value of our 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 or could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.
- We face general risks related to our ability to maintain effective internal controls over financial reporting, additional tax liabilities related to our operations, our ability to use our net operating loss carryforwards and costs of litigation.

Risks Related to Our Financial Condition and Capital Requirements

We have a history of operating losses and anticipate that we will continue to incur losses for the foreseeable future.

We are a biopharmaceutical company with a history of operating losses, and anticipate continuing to incur operating losses for the foreseeable future. Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We have devoted substantially all of our financial resources to identifying, acquiring, and developing our products and product candidates, including conducting clinical studies, developing manufacturing processes, manufacturing product candidates for clinical studies, and providing selling, general and administrative support for these operations. The amount of our future net losses will depend, in part, on non-recurring events, the success of our commercialization efforts, and the rate of our future expenditures. 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;
- pursue preclinical and clinical development for additional indications for existing products and product candidates;
- change or add additional manufacturers or suppliers;
- expand upon or build our own manufacturing-related facilities and capabilities, including construction of our own GMP gene therapy manufacturing plant;
- seek regulatory and marketing approvals for our product candidates that successfully complete clinical studies;
- continue to establish Medical Affairs field teams to initiate relevant disease education;
- continue to establish a marketing and distribution infrastructure and field force to commercialize our products and any product candidates for which we may obtain marketing approval;
- continue to manage our international subsidiaries and establish new ones;
- continue to operate as a public company and comply with legal, accounting and other regulatory requirements;
- seek to identify, assess, license, acquire, and/or develop other product candidates, technologies, and/or businesses;
- make milestone or other payments under any license or other agreements;
- seek to maintain, protect, and expand our intellectual property portfolio;
- seek to attract and retain skilled personnel;
- create additional infrastructure, including facilities and systems, to support the growth of our operations, our product development, and our 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, inspection outcomes, 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.

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 limited experience in generating revenue from product sales.

Our ability to generate significant revenue from product sales depends on our ability, alone or with strategic collaboration partners, to successfully commercialize our products and to complete the development of, and obtain the regulatory and marketing approvals necessary to commercialize, our product candidates. Our ability to generate substantial future revenue from product sales, including named patient sales, depends heavily on our success in many areas, including, but not limited to:

- obtaining regulatory and marketing approvals with broad indications for product candidates for which we complete clinical studies;
- developing a sustainable and scalable manufacturing process for our products and any approved product candidates and establishing and maintaining supply and manufacturing relationships with third parties that can conduct the processes and provide adequate (in amount and quality) product supply to support market demand for our products and product candidates, if approved;

- launching and commercializing our products and product candidates for which we obtain regulatory and marketing approval, either directly or with a collaborator or distributor;
- obtaining market acceptance of our products and product candidates as viable treatment options;
- obtaining adequate market share, reimbursement and pricing for our products and product candidates;
- our ability to sell our products and product candidates on a named patient basis or through an equivalent mechanism and the amount of revenue generated from such sales;
- our ability to find patients so they can be diagnosed and begin receiving treatment;
- addressing any competing technological and market developments;
- negotiating favorable terms, including commercial rights, in any collaboration, licensing, or other arrangements into which we may enter, any amendments thereto or extensions thereof;
- maintaining, protecting, and expanding our portfolio of intellectual property rights, including patents, trade secrets, and know-how; and
- attracting, hiring, and retaining qualified personnel.

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, or any other reasons, we may not generate significant revenue from sales of our products, even if they receive regulatory approval.

We expect to need to raise additional capital to fund our activities. This additional financing may not be available on acceptable terms, if at all. Failure to obtain this necessary capital when needed may force us to delay, limit, or terminate our product development efforts or other activities.

As of June 30, 2022, our available cash, cash equivalents, and marketable debt securities were $706.1 million. We expect we will need additional capital to continue to commercialize our products, and to develop and obtain regulatory approval for, and to commercialize, all of 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 and commercial supplies of our products and product candidates;
- the cost of creating additional infrastructure, including facilities and systems, such as our GMP gene therapy manufacturing plant;
- the number and characteristics of the product candidates that we pursue;
- the cost, timing, and outcomes of regulatory approvals;
- the cost and timing of establishing and operating our international subsidiaries;
- the cost and timing of establishing and operating field forces, marketing, and distribution capabilities;
- the cost and timing of other activities needed to commercialize our products; and
- the terms and timing of any collaborative, licensing, acquisition, and other arrangements that we may establish, including any required milestone, royalty, and reimbursements or other payments thereunder.

Any additional fundraising efforts may divert our management's attention from their day-to-day activities, which can adversely affect our ability to develop our product candidates and commercialize our products. In addition, we cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. The terms of any financing may adversely affect the holdings or the rights of our stockholders and the issuance of additional securities by us, whether equity or debt, 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. If we incur debt, it 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 have in the past sought and may in the future seek funds through a sale of future royalty payments similar to our transactions with Royalty Pharma and OMERS or through collaborative partnerships, strategic alliances, and licensing or other arrangements, such as our transaction with Daiichi Sankyo, and we may be required to relinquish rights to some of our technologies or product candidates, future revenue streams, research programs, and other 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, or at all, we may be required to significantly curtail, delay, or discontinue one or more of our research or development programs or the commercialization of our products and any approved 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

Clinical drug development involves a lengthy, complex, and expensive process with uncertain outcomes and the potential for substantial delays, and the results of earlier studies may not be predictive of future study results.

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, complex, 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. Product candidates that have shown promising results in early-stage clinical studies may still suffer significant setbacks or fail in subsequent clinical studies. The safety or efficacy results generated to date in clinical studies do not ensure that later clinical studies will demonstrate similar results. Further, we have, and may in the future, expect to continue to report preliminary or interim data from our clinical trials. Preliminary or interim data from our clinical trials are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and/or more patient data become available. Such data may show initial evidence of clinical benefit, but as patients continue to be assessed and more patient data become available, there is a risk that any therapeutic effects are no longer durable in patients and/or decrease over time or cease entirely. As a result, preliminary or interim data should be considered carefully and with caution until the final data are available. Results from investigator-sponsored studies or compassionate-use studies may not be confirmed in company-sponsored studies or may negatively impact the prospects for our programs. Additionally, given the nature of the rare diseases we are seeking to treat, we often have to devise newly-defined endpoints to be tested in our studies, which can lead to some subjectivity in interpreting study results and could result in regulatory agencies not agreeing with the validity of our endpoints, or our interpretation of the clinical data, and therefore delaying or denying approval. Given the illness of the patients in our studies and the nature of their rare diseases, we may also be required or choose to conduct certain studies on an open-label basis. We have in the past, and may in the future elect to review interim clinical data at multiple time points during the studies, which could introduce bias into the study results and potentially result in denial of approval.

In the biopharmaceutical industry, 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 despite having progressed through nonclinical 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.

Scenarios that can prevent successful or timely completion of clinical development include but are not limited to:

- delays or failures in generating sufficient preclinical, toxicology, or other *in vivo* or *in vitro* data to support the initiation or continuation of human clinical studies or filings for regulatory approval;
- failure to demonstrate a starting dose for our product candidates in the clinic that might be reasonably expected to result in a clinical benefit;

- delays or failures in developing gene therapy, messenger RNA (mRNA), DNA, small interfering RNA (siRNA) or other novel and complex product candidates, which are expensive and difficult to develop and manufacture;
- delays resulting from a shutdown, or uncertainty surrounding the potential for future shutdowns of the U.S. government, including the FDA;
- delays or failures in reaching a consensus with regulatory agencies on study design;
- delays in reaching agreement on acceptable terms with contract research organizations, or CROs, clinical study sites, and other clinical trial-related vendors;
- failure or delays in obtaining required regulatory agency approval and/or IRB or EC approval at each clinical study site or in certain countries;
- failure to correctly design clinical studies which may result in those studies failing to meet their endpoints or the expectations of regulatory agencies;
- changes in clinical study design or development strategy resulting in delays related to obtaining approvals from IRBs or ECs and/or regulatory agencies to proceed with clinical studies;
- imposition of a clinical hold by regulatory agencies after review of an IND application or amendment, another 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 and/or ICH's good clinical practices requirements or applicable regulatory guidelines in other countries;
- delays in patients' completion of studies or their returns for post-treatment follow-up;
- patients dropping out of a study;
- adverse events associated with the product candidate occurring that are viewed to outweigh its potential benefits;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;
- greater than anticipated costs associated with clinical studies of our drug candidates, including as a result of hyperinflation;
- 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 or nonclinical studies or to abandon drug development programs;
- competing clinical studies of potential alternative product candidates or investigator-sponsored studies of our product candidates; 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 negatively impact our ability to generate revenue. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional toxicology, comparability or other studies to bridge our modified product candidates to earlier versions. Clinical study delays could also shorten any periods during which our products have commercial exclusivity and may allow our competitors to bring products to market before we do, which could negatively impact our ability to obtain orphan exclusivity and to successfully commercialize our product candidates and may harm our business and results of operations.

If we do not achieve our projected development goals in the time frames we announce and expect, the commercialization of our products may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

For planning purposes, we estimate the timing of the accomplishment of various scientific, clinical, regulatory, and other product development goals, which we sometimes refer to as milestones. These milestones may include the commencement or completion of scientific studies and clinical trials, the timing of patient dosing, the timing, type or clarity of data from clinical trials, the submission or acceptance of regulatory filings, and the potential approval of such regulatory filings. We periodically make public announcements about the expected timing of some of these milestones. All of these milestones are based on a variety of assumptions, but the actual timing of these milestones can vary dramatically from our estimates. If we do not meet these publicly

announced milestones, the commercialization of our products may be delayed and the credibility of our management may be adversely affected and, as a result, our stock price may decline.

We may find it difficult to identify and 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 example, we estimate that approximately 6,000 patients worldwide suffer from GSDIa, for which DTX401 is being studied, and these all may not be treatable if they are immune to the AAV viral vector.

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 patients to 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. The process of finding and diagnosing patients is costly and time-consuming, especially since the rare diseases we are studying are commonly underdiagnosed. We also may not be able to identify, recruit, and enroll a sufficient number of appropriate patients to complete our clinical studies because of demographic criteria for prospective patients, the perceived risks and benefits of the product candidate under study, the proximity and availability of clinical study sites for prospective patients, and the patient referral practices of physicians. The availability and efficacy of competing therapies and clinical studies can also adversely impact enrollment. If patients are unwilling to participate in our studies for any reason (such as drug-related side effects), the timeline for and our success in recruiting patients, conducting studies, and obtaining regulatory approval of potential products may be delayed or impaired, 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. 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.

The regulatory approval processes of the FDA and comparable foreign authorities are lengthy, time consuming, and inherently unpredictable. Even if we achieve positive results in our pre-clinical and clinical studies, if we are ultimately unable to obtain timely regulatory approval for our product candidates, our business will be substantially harmed.

Our future success is dependent on our ability to successfully commercialize our products and develop, obtain regulatory approval for, and then successfully commercialize one or more product candidates. 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. We have only obtained regulatory approval for three products that we have developed, 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. Further, as the clinical trial requirements of regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the product candidates, the regulatory approval process for novel product candidates, such as our gene therapy product candidates, can be more expensive and take longer than for other product candidates, leading to fewer product approvals. To date, very few gene therapy products have received regulatory approval in the U.S. or Europe. The regulatory framework and oversight over development of gene therapy products has evolved and may continue to evolve in the future. Within the FDA, the Center for Biologics Evaluation and Research (CBER) regulates gene therapy products. Within the CBER, the review of gene therapy and related products is consolidated in the Office of Cellular, Tissue and Gene Therapies, and the FDA has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its reviews. The CBER works closely with the National Institutes of Health (NIH). The FDA and the NIH have published guidance with respect to the development and submission of gene therapy protocols. For example, in January 2020, the FDA issued final guidance to set forth the framework for the development, review and approval of gene therapies. The final guidance pertains to the development of gene therapies for the treatment of specific disease categories, including rare diseases, and to manufacturing and long-term follow up issues relevant to gene therapy, among other topics. At the same time the FDA issued new draft guidance describing the FDA's approach for determining whether two gene therapy products were the same or different for the purpose of assessing orphan drug exclusivity; the draft guidance was finalized by the FDA in September 2021.

To obtain regulatory approval in the U.S. and other jurisdictions, we must comply with numerous and varying requirements regarding safety, efficacy, chemistry, manufacturing and controls, clinical studies (including good clinical practices), commercial sales, pricing, and distribution of our product candidates, as described in "Item 1. Business – Government Regulation" of our Annual Report. Even if we are successful in obtaining approval in one jurisdiction, we cannot ensure that we will obtain approval in any other jurisdictions. In addition, approval policies, regulations, positions of the regulatory agencies on study design and/or endpoints, or the type and amount of clinical data necessary to gain approval may change during the course of a product candidate's clinical development, which may cause delays in the approval or the decision not to approve an application. Communications with the regulatory agencies during the approval process are also unpredictable; favorable communications early in the process do not ensure that approval will be obtained and unfavorable communications early on do not guarantee that approval will be denied. Applications for our product candidates could fail to receive regulatory approval, or could be delayed in receiving regulatory approval, for many reasons, including but not limited to the following:

- regulatory authorities may disagree with the design, implementation, or conduct of our clinical studies;
- regulatory authorities may change their guidance or requirements for a development program for a product candidate;
- the population studied in the clinical program may not be sufficiently broad or representative to assure efficacy and safety in the full population for which we seek approval;
- 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 an NDA, or biologics license application, or BLA, or other submission or to obtain regulatory approval;
- we may be unable to demonstrate to regulatory authorities that a product candidate's risk-benefit ratio for its proposed indication is acceptable;
- regulatory authorities may fail to approve the manufacturing processes, test procedures and specifications, or facilities used to manufacture our clinical and commercial supplies;
- the U.S. government may be shut down, which could delay the FDA;
- the FDA may be delayed in responding to our applications or submissions due to competing priorities or limited resources, including as a result of the COVID-19 pandemic;
- failure of our nonclinical or clinical development to comply with an agreed upon Pediatric Investigational Plan (PIP), which details the designs and completion timelines for nonclinical and clinical studies and is a condition of marketing authorization in the EU; and
- the approval policies or regulations of regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

Furthermore, the disease states we are evaluating often do not have clear regulatory paths for approval and/or do not have validated outcome measures. In these circumstances, we work closely with the regulatory authorities to define the approval path and may have to qualify outcome measures as part of our development programs. Additionally, many of the disease states we are targeting are highly heterogeneous in nature, which may impact our ability to determine the treatment benefit of our potential therapies.

This lengthy and uncertain approval process, as well as the unpredictability of the clinical and nonclinical studies, may result in our failure to obtain regulatory approval to market any of our product candidates, or delayed regulatory approval.

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 or further development, and could result in a more restrictive label, the delay or denial of regulatory approval by the FDA or other comparable foreign authorities, or 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, restricted distribution, a communication plan for healthcare providers, and/or other elements to assure safe use. Our product candidates are in development and the safety profile has not been established. Further, as one of the goals of Phase 1 and/or 2 clinical trials is to identify the highest dose of treatment that can be safely provided to study participants, adverse side effects, including serious adverse effects, have occurred in certain studies as a result of changes to the dosing regimen during such studies and may occur in future studies. Results of our studies or investigator-sponsored trials 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.

Additionally, notwithstanding our prior or future regulatory approvals for our product candidates, if 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 product's label or restrict the product's approved use;
- we may be required to create a REMS plan;
- patients and physicians may elect not to use our products, or reimbursement authorities may elect not to reimburse for them; 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.

Serious adverse events in clinical trials involving gene therapy product candidates may damage public perception of the safety of our product candidates, increase government regulation, and adversely affect our ability to obtain regulatory approvals for our product candidates or conduct our business.

Gene therapy remains a novel technology. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. For example, certain gene therapy trials using AAV8 vectors (although at significantly higher doses than those used in our gene therapy product candidates) and other vectors led to several well-publicized adverse events, including cases of leukemia and death. The risk of cancer or death remains a concern for gene therapy and we cannot assure you that it will not occur in any of our planned or future clinical studies. In addition, there is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material. Serious adverse events in our clinical trials, or other clinical trials involving gene therapy products, particularly AAV gene therapy products such as candidates based on the same capsid serotypes as our product candidates, or occurring during use of our competitors' products, even if not ultimately attributable to the relevant product candidates, and the resulting publicity, could result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our gene therapy product candidates, stricter labeling requirements for those gene therapy product candidates that are approved and a decrease in demand for any such gene therapy product candidates.

Gene therapy and mRNA, DNA and siRNA product candidates are novel, complex, expensive and difficult to manufacture. We could experience manufacturing problems that result in delays in developing and commercializing these programs or otherwise harm our business.

The manufacturing process used to produce our gene therapy, mRNA, DNA and siRNA product candidates is novel, complex, and has not been validated for commercial use. Several factors could cause production interruptions, including equipment malfunctions, regulatory inspections, facility contamination, raw material shortages or contamination, natural disasters, disruption in utility services, human error or disruptions in the operations of our suppliers. Further, given that cGMP gene therapy, mRNA, DNA and siRNA manufacturing is a nascent industry, there are a small number of CMOs with the experience necessary to manufacture our gene therapy product candidates and we may have difficulty finding or maintaining relationships with such CMOs or hiring experts for internal manufacturing and accordingly, our production capacity may be limited.

Our gene therapy, mRNA, DNA and siRNA product candidates require processing steps that are more complex than those required for most small molecule drugs. Moreover, unlike small molecules, the physical and chemical properties of a biologic such as gene therapy, mRNA, DNA and siRNA product candidates generally cannot be fully characterized. As a result, assays of the finished product candidate may not be sufficient to ensure that the product candidate is consistent from lot to lot or will perform in the intended manner. Accordingly, we employ multiple steps to control the manufacturing process to assure that the process works reproducibly, and the product candidate is made strictly and consistently in compliance with the process. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, noncompliance with regulatory requirements, product recalls, product liability claims or insufficient inventory. We may encounter problems achieving adequate quantities and quality of clinical-grade materials that meet FDA, the EMA or other applicable standards or specifications with consistent and acceptable production yields and costs.

In addition, FDA, the EMA and other foreign regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, FDA, the EMA or other foreign regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay product launches or clinical trials, which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects.

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

Our products and any product candidates that are approved in the future remain subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, distribution, 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 U.S. and requirements of comparable foreign regulatory authorities, as described in “Item 1. Business – Government Regulation” of our Annual Report.

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 Good Manufacturing Practices (GMP) regulations. As such, we and our contract manufacturers are subject to continual review and inspection to assess compliance with GMP and adherence to commitments made in any NDA, BLA, MAA, or other comparable application for approval in another jurisdiction. Regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our products, product candidates or the associated quality systems for compliance with the regulations applicable to the activities being conducted. If we, our collaborators, such as KKC, 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, the temporary or permanent suspension of a clinical study or commercial sales, recalls or seizures of product or the temporary or permanent closure of a facility or withdrawal of product approval. If supply from one approved manufacturer is interrupted due to failure to maintain regulatory compliance, 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 delays in product supply. The regulatory agencies may also require additional studies if a new manufacturer, material, testing method or standard is relied upon for commercial production. Switching manufacturers, materials, test methods or standards may involve substantial costs and may result in a delay in our desired clinical and commercial timelines. Accordingly, we and others with whom we work are required 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 other conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical studies, and surveillance to monitor the safety and efficacy of the product candidate. 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 was obtained via the accelerated approval or conditional marketing authorization pathways, we would 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. We will be required to report certain adverse events and manufacturing problems, if any, to the FDA and comparable foreign regulatory authorities. The holder of an approved NDA, BLA, MAA, or other comparable application must submit new or supplemental applications and obtain approval for certain changes to the approved product, product labeling, or manufacturing process.

If we fail to comply with applicable regulatory requirements, or there are safety or efficacy problems with a product, a regulatory agency or enforcement authority may, among other things:

- issue warning or notice of violation 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;
- seize or detain products, or require a product recall; or
- require entry into a consent decree.

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.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit commercialization of our approved products or product candidates.

We face an inherent risk of product liability exposure related to the testing of our approved products and product candidates in human clinical trials, as well as in connection with commercialization of our current and future products. If we cannot successfully defend ourselves against claims that any of our approved products or product candidates caused injuries, we could incur substantial liabilities. There can be no assurance that our product liability insurance, which provides coverage in the amount of $15.0 million per incident and $15.0 million in the aggregate, will be sufficient in light of our current or planned clinical programs. 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, or losses may exceed the amount of insurance that we carry. A 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.

If we are unable to identify, source, and develop effective biomarkers, or our collaborators are unable to successfully develop and commercialize companion diagnostics for our product candidates, or experience significant delays in doing so, we may not realize the full commercial potential of our product candidates.

We are developing companion diagnostic tests to identify the right patients for certain of our product candidates and to monitor response to treatment. In certain cases, diagnostic tests may need to be developed as companion diagnostics and regulatory approval obtained in order to commercialize some product candidates. We currently use and expect to continue to use biomarkers to identify the right patients for certain of our product candidates. We may also need to develop predictive biomarkers in the future. We can offer no assurances that any current or future potential biomarker will in fact prove predictive, be reliably measured, or be accepted as a measure of efficacy by the FDA or other regulatory authorities. In addition, our success may depend, in part, on the development and commercialization of companion diagnostics. We also expect the FDA will require the development

and regulatory approval of a companion diagnostic assay as a condition to approval of our gene therapy product candidates. There has been limited success to date industrywide in developing and commercializing these types of companion diagnostics. Development and manufacturing of companion diagnostics is complex and there are limited manufacturers with the necessary expertise and capability. Even if we are able to successfully develop companion diagnostics, we may not be able to manufacture the companion diagnostics at a cost or in quantities or on timelines necessary for use with our product candidates. To be successful, we need to address a number of scientific, technical and logistical challenges. We are currently working with a third party to develop companion diagnostics, however, we have little experience in the development and commercialization of diagnostics and may not ultimately be successful in developing and commercializing appropriate diagnostics to pair with any of our product candidates that receive marketing approval. We rely on third parties for the automation, characterization and validation, of our bioanalytical assays, companion diagnostics and the manufacture of its critical reagents.

Companion diagnostics are subject to regulation by FDA and similar regulatory authorities outside the U.S. as medical devices and require regulatory clearance or approval prior to commercialization. In the U.S., companion diagnostics are cleared or approved through FDA's 510(k) premarket notification or premarket approval, or PMA, process. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted 510(k) premarket notification, PMA or equivalent application types in jurisdictions outside the U.S., may cause delays in the approval, clearance or rejection of an application. Given our limited experience in developing and commercializing diagnostics, we expect to rely in part or in whole on third parties for companion diagnostic design and commercialization. We and our collaborators may encounter difficulties in developing and obtaining approval or clearance for the companion diagnostics, including issues relating to selectivity/specificity, analytical validation, reproducibility, or clinical validation. Any delay or failure by us or our collaborators to develop or obtain regulatory approval of the companion diagnostics could delay or prevent approval of our product candidates.

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 be exposed to sub-optimal quality and reputational harm, 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 parties, including CROs, collaborative partners, and independent investigators to analyze, collect, monitor, and manage data for our ongoing nonclinical and clinical programs. We rely on third parties for execution of our nonclinical and clinical studies, and for estimates regarding costs and efforts completed, and we control only certain aspects of their activities. We and our CROs and other vendors and partners are required to comply with GMP, GCP, and GLP, which are regulations and guidelines enforced by the FDA, the Competent Authorities of the Member States of the European Economic Area, and comparable foreign regulatory authorities for all of our product candidates in 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 other vendors and partners, including the sites at which clinical studies are conducted, 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 deny approval and/or require us to perform additional nonclinical and clinical studies before approving our marketing applications, which would delay the approval process. We cannot make assurances that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical studies comply with GCP regulations or that nonclinical studies comply with GLP regulations. In addition, our clinical studies must be conducted with products produced under GMP regulations. If the regulatory authorities determine that we have failed to comply with GLP, GMP, or GCP regulations, they may deny approval of our product candidates and/or we may be required to repeat clinical or nonclinical studies, which would delay the regulatory approval process.

Our CROs and other vendors and partners are not our employees and we cannot control whether or not they devote sufficient time and resources to our on-going nonclinical and clinical programs, except for the limited remedies available to us under our agreements with such third parties. If our vendors and partners 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 and other vendors and partners may also generate higher costs than anticipated as a result of changes in scope of work or otherwise. As a result, the commercial prospects for our product candidates would be harmed, our costs could increase, and our ability to generate revenue could be delayed.

If any of our relationships with these third parties terminate, we may not be able to enter into arrangements with alternative vendors or do so on commercially reasonable terms. Switching or adding additional vendors involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new vendor commences work. As a result, delays may occur, which can materially impact our ability to meet our desired clinical development timelines. Our efforts to manage our relationships with our vendors and partners can provide 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 business prospects.

We also rely on third parties in other ways, including efforts to support patient diagnosis and identify patients, to assist our finance and legal departments, and to provide other resources for our business. Use of these third parties could expose us to sub-optimal quality, missed deadlines, and non-compliance with applicable laws, all of which could result in reputational harm to us and negatively affect our business.

We are dependent on KKC for the clinical and commercial supply of Crysvita for all major markets and for the development and commercialization of Crysvita in certain major markets, and KKC's failure to provide an adequate supply of Crysvita or to commercialize Crysvita in those markets could result in a material adverse effect on our business and operating results.

Under our agreement with KKC, KKC has the sole right to commercialize Crysvita in Europe and, at certain specified times, in the U.S., Canada, and Turkey, subject to a limited promotion right we retained. Our partnership with KKC 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:

- KKC has no obligation under our agreement to use diligent efforts to commercialize Crysvita in Europe. The timing and amount of any royalty payments that are made by KKC based on sales of Crysvita in Europe will depend on, among other things, the efforts, allocation of resources, and successful commercialization of Crysvita by KKC in Europe;
- the timing and amount of any payments we may receive under our agreement with KKC will depend on, among other things, the efforts, allocation of resources, and successful commercialization of Crysvita by KKC in the U.S. and Canada under our agreement;
- KKC may change the focus of its commercialization efforts or pursue higher-priority programs;
- KKC may make decisions regarding the indications for our product candidates in countries where it has the sole right to commercialize the product candidates that limit commercialization efforts in those countries or in countries where we have the right to commercialize our product candidates;
- KKC may make decisions regarding market access and pricing in countries where it has the sole right to commercialize our product candidates which can negatively impact our commercialization efforts in countries where we have the right to commercialize our product candidates;
- KKC may fail to manufacture or supply sufficient drug product of Crysvita in compliance with applicable laws and regulations or otherwise for our development and clinical use or commercial use (including as a result of the COVID-19 pandemic), which could result in program delays or lost revenue;
- KKC may elect to develop and commercialize Crysvita indications with a larger market than XLH and at a lower price, thereby reducing the profit margin on sales of Crysvita for any orphan indications, including XLH;
- if KKC were to breach or terminate the agreement with us, we would no longer have any rights to develop or commercialize Crysvita or such rights would be limited to non-terminated countries;
- KKC may terminate its agreement with us, adversely affecting 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 KKC may be greater than anticipated.

We rely on third parties to manufacture our products and our product candidates and we are subject to a multitude of manufacturing risks, any of which could substantially increase our costs and limit the supply of our product and product candidates.

As we currently lack the resources and the capability to manufacture our products and most of our product candidates on a clinical or commercial scale, we rely on third parties to manufacture our products and product candidates. Although we oversee the contract manufacturers, we cannot control the manufacturing process of, and are substantially dependent on, our contract manufacturing partners for compliance with the regulatory requirements. See "*-Even if we obtain regulatory approval for our product candidates, our products will remain subject to regulatory scrutiny"* risk factor above. Further, we depend on our

manufacturers to purchase from third-party suppliers the materials necessary to produce our products and product candidates. There are a limited number of suppliers for raw materials that we use to manufacture our drugs, placebos, or active controls, and there may be a need to identify alternate suppliers to prevent or mitigate a possible disruption of the manufacture of the materials necessary to produce our products and product candidates for our clinical studies, and, if approved, ultimately for commercial sale. We also do not have any control over the process or timing of the acquisition of these raw materials by our manufacturers. We may also experience interruptions in supply of product if the product or raw material components fail to meet our quality control standards or the quality control standards of our suppliers.

Further, manufacturers that produce our products and product candidates may not have experience producing our products and product candidates at commercial levels and may not produce our products and product candidates at the cost, quality, quantities, locations, and timing needed to support profitable commercialization. We have not yet secured manufacturing capabilities for commercial quantities of all of our product candidates and may be unable to negotiate binding agreements with manufacturers to support our commercialization activities on commercially reasonable terms. Even if our third-party product manufacturers develop acceptable manufacturing processes that provide the necessary quantities of our products and product candidates in a compliant and timely manner, the cost to us for the supply of our products and product candidates manufactured by such third parties may be high and could limit our profitability. For instance, KKC is our sole supplier of commercial quantities of Crysvita. The supply price to us for commercial sales of Crysvita in Latin America and the transfer price for commercial sales of the product in the U.S. and Canada is 35% of net sales through December 31, 2022 and 30% thereafter, which is higher than the typical cost of sales for companies focused on rare diseases.

The process of manufacturing our products and product candidates is complex, highly regulated, and subject to several risks, including but not limited to those listed below.

- The process of manufacturing our products and 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 our products and 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 products and product candidates or in the manufacturing facilities in which our products and product candidates are made, such manufacturing facilities may need to be closed for an extended period of time to investigate and remedy the contamination.
- The manufacturing facilities in which our products and product candidates are made could be adversely affected by equipment failures, labor shortages, raw material shortages, natural disasters, power failures, actual or threatened public health emergencies, and numerous other factors.

Any adverse developments affecting manufacturing operations for our products and product candidates may result in shipment delays, inventory shortages, lot failures, withdrawals or recalls, or other interruptions in the supply of our products and product candidates. Due to their stage of development, small volume requirements, and infrequency of batch production runs, we carry limited amounts of safety stock for our products and product candidates. We have, and may in the future, be required to take inventory write-offs and incur other charges and expenses for products and 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 products and most of our product candidates are currently acquired from single-source suppliers. The loss of these suppliers, or their failure to supply us with the necessary drug substance or drug product, could materially and adversely affect our business.

We acquire most of the drug substances and drug products for our products and product candidates from single sources. If any single source supplier breaches an agreement with us, or terminates the agreement in response to an alleged breach by us or otherwise becomes unable or unwilling to fulfill its supply obligations, we would not be able to manufacture and distribute the product or product candidate until a qualified alternative supplier is identified, which could significantly impair our ability to commercialize such product or delay the development of such product candidate. For example, the drug substance and drug product for Crysvita are made by KKC pursuant to our license and collaboration agreement with KKC. The drug substance and drug product for Mepsevii are currently manufactured by Rentschler under a commercial supply and services agreement, accompanying purchase orders, and other agreements. Pharmaceutical-grade drug substance for Dojolvi is manufactured by IOI Oleo pursuant to a supply agreement, and the drug product for Dojolvi is prepared by Haupt Pharma AG, pursuant to a master services agreement. Single source suppliers are also used for our gene therapy programs. We cannot provide assurances that identifying alternate sources, if available at all, and establishing relationships with such sources would not result in significant expense or delay in the commercialization of our products or the development of our product candidates. Additionally, we may not be able to enter into supply arrangements with an alternative supplier on commercially reasonable terms or at all. The terms of any new agreement may also be less favorable or more costly than the terms we have with our current supplier. A delay in the commercialization of our products or 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.

The actions of distributors and specialty pharmacies could affect our ability to sell or market products profitably. Fluctuations in buying or distribution patterns by such distributors and specialty pharmacies could adversely affect our revenues, financial condition, or results of operations.

We rely on commercial distributors and specialty pharmacies for a considerable portion of our product sales and such sales are concentrated within a small number of distributors and specialty pharmacies. The financial failure of any of these parties could adversely affect our revenues, financial condition or results of operations. Our revenues, financial condition or results of operations may also be affected by fluctuations in buying or distribution patterns of such distributors and specialty pharmacies. These fluctuations may result from seasonality, pricing, wholesaler inventory objectives, or other factors.

Risks Related to Commercialization of Our Products and Product Candidates

If the market opportunities for our products and 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 products and product candidates are small, and the addressable patient population potentially even smaller, 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. Some of our current products or clinical programs may be most appropriate for patients with more severe forms of their disease. For instance, while adults make up the majority of the XLH patients, they often have less severe disease that may reduce the penetration of Crysvita in the adult population relative to the pediatric population. Given the overall rarity of the diseases we target, it is difficult to project the prevalence of the more severe forms, or the other subsets of patients that may be most suitable to address with our products and product candidates, which may further limit the addressable patient population to a small subset. 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 products and 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 products and product candidates may be limited or may not be amenable to treatment with our products and product candidates, and new patients may become increasingly difficult to identify or access. Further, even if we obtain significant market share for our products and product candidates, because the potential target populations are very small, we may never become or remain profitable nor generate sufficient revenue growth to sustain our business.

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 treatments that may compete with our products and product candidates. See "Item 1. Business – Competition" in our Annual Report.

We have competitors both in the U.S. and internationally, including major multinational pharmaceutical companies, specialty pharmaceutical companies, biotechnology companies, startups, academic research institutions, government agencies, and public and private research institutions. 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 products and product candidates uneconomical or obsolete, and we may not be successful in marketing our products and product candidates against competitors.

We may not be able to effectively manage the expansion of our organization, including building an integrated commercial organization. If we are unable to expand our existing commercial infrastructure or enter into agreements with third parties to market and sell our products and product candidates, as needed, we may be unable to increase our revenue.

We expect to need additional managerial, operational, marketing, financial, legal, and other resources to support our development and commercialization plans and strategies. In order to successfully commercialize our products as well as any additional products that may result from our development programs or that we acquire or license from third parties, we are building and expanding our commercial infrastructure in North America, Europe, Latin America and the Asia-Pacific region. This infrastructure consists of both office-based as well as field teams with technical expertise, and will be expanded as we approach the potential approval dates of additional products that result from our development programs. 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, as a company, have limited, recent experience selling and marketing our product and only some of our employees have prior experience promoting other similar products in the past while employed at other companies. As we increase the number and range of our commercialized products, we may experience additional complexities to our sales process and strategy and have difficulties in allocating sufficient resources to sales and marketing of certain products. Further, as we launch additional products or as demand for our products change, our initial estimate of the size of the required field force may be materially more or less than the size of the field force actually required to effectively commercialize our product candidates. As such, we may be required to hire large teams to adequately support the commercialization of our products and product candidates or we may incur excess costs in an effort to optimize the hiring of commercial personnel. 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 a large internal team or the support of a third party to perform key commercial functions, we may be unable to compete successfully against these more established companies.

Our exclusive rights to promote Crysvita in the U.S. and Canada will transition back to KKC.

Pursuant to the terms of our collaboration and license agreement with KKC, or the collaboration agreement, we have the sole right to promote Crysvita in the U.S. and Canada, or the profit-share territory for a specified period of time, with KKC increasingly participating in the promotion of the product until the transition date of April 2023, which is the fifth anniversary of the commercial launch of the product in the U.S. After the transition date, KKC will have the right to promote the product, subject to a limited promotion right retained by us. The transition of responsibilities to KKC will require significant effort and may result in the diversion of management's attention to transition activities. We may also encounter unexpected difficulties or incur unexpected costs in connection with such transition activities. Further, we cannot assure that we will have adequate commercial activity to support our North America field force and other aspects of our commercial infrastructure in the territory after the transition date and we may fail to retain members of our field teams due to such uncertainties. After the transition date, we will also solely bear the expenses related to the promotion of Crysvita in the profit-share territory pursuant to our limited promotion right, rather than share such expenses with KKC. Collaboration with KKC may not result in a seamless transition of responsibilities for KKC to promote the product in the profit-share territory after the transition date and the commercial success of Crysvita in the profit-share territory after the transition date will depend on, among other things, the efforts and allocation of resources of KKC.

The commercial success of any current or future product 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 current and future products will depend in part on the medical community, patients, and payors accepting our current and future products as medically useful, cost-effective, and safe. Any product that we bring to the market may not gain market acceptance by physicians, patients, payors, and others in the medical community. The degree of market acceptance of any of our current and future products 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 effectiveness of our field forces and marketing efforts;
- 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 payors on the benefits of the product candidates require significant resources and may never be successful. If our current and future products fail to achieve an adequate level of acceptance by physicians, patients, 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 products and 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. We expect the cost of a single administration of gene therapy products, such as those we are developing, to be substantial, when and if they achieve regulatory approval. Accordingly, the availability and adequacy of coverage and reimbursement by governmental and private payors are essential for most patients to afford expensive treatments such as ours, assuming approval. Sales of our products and product candidates, if approved, will depend substantially, both domestically and abroad, on the extent to which their costs 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 payors. If coverage and reimbursement are not available, are available only to limited levels, or are not available on a timely basis, we may not be able to successfully commercialize our products and product candidates, if approved. For example, deteriorating economic conditions and political instability in certain Latin American countries and in Turkey continue to cause us to experience significant delays in receiving approval for reimbursement for our products and consequently impact our product commercialization timelines in such regions. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to sustain our overall enterprise.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the U.S., the Centers for Medicare & Medicaid Services, or CMS, an agency within the U.S. Department of Health and Human Services, 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 or private payors will decide with respect to reimbursement for products such as ours, especially our gene therapy product candidates as there is a limited body of established practices and precedents for gene therapy products.

Outside the U.S., 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 will put pressure on the pricing and usage of our products and 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 U.S., the reimbursement for our products may be reduced compared with the U.S. and may be insufficient to generate commercially reasonable revenue and profits.

Moreover, increasing efforts by governmental and third-party payors in the U.S. and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for new products and, as a result, they may not cover or provide adequate payment for our products and product candidates. We expect to experience pricing pressures in connection with the sale of any of our products and product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations, additional legislative changes, and statements by elected officials. For example, proposals have been discussed to tie U.S. drug prices to the cost in other countries, several states in the U.S. have introduced legislation to require pharmaceutical companies to disclose their costs to justify the prices of their products, and an “Affordable Drug Pricing Task-Force” has been formed in the U.S. House of Representatives with the goal of combating the increased costs of prescription drugs. Drug pricing is also expected to remain a focus for the current Presidential Administration and Congress. The downward pressure on healthcare costs in general, and with respect to prescription drugs, surgical procedures, and other treatments in particular, 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 products, 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, our products, and our 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 U.S. and in other countries with respect to our proprietary technologies, our products, and our product candidates.

We have sought to protect our proprietary position by filing patent applications in the U.S. and abroad related to our novel technologies, products and product candidates 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 products or product candidates in the U.S. 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. Third parties may challenge the validity, enforceability, or scope of any issued patents which may result in such patents being narrowed, found unenforceable, or invalidated. Furthermore, even if the patents and patent applications we own or in-license are unchallenged, they may not adequately protect our intellectual property, provide exclusivity for our products or product candidates, or prevent others from designing around our claims. Any of these outcomes could impair our ability to prevent competition from third parties.

We, independently or together with our licensors, have filed several patent applications covering various aspects of our products or 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 could impair the exclusivity position of our products or deprive us of rights necessary for the successful commercialization of any product candidates that are approved. 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.

Our current patents or applications covering methods of use and certain compositions of matter do not provide complete patent protection for our products and product candidates in all territories. For example, there are no issued patents covering the Crysvita composition of matter in Latin America, where we have rights to commercialize the compound. Therefore, a competitor could develop the same antibody or a similar antibody as well as other approaches that target FGF23 for potential commercialization in Latin America, subject to any intellectual property rights or regulatory exclusivities awarded to us. If we cannot obtain and maintain effective patent rights for our products or 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 U.S., the natural expiration of a patent is generally 20 years after its effective filing date. 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 or biosimilar medications.

Patent term extensions under the Hatch-Waxman Act in the U.S. and under supplementary protection certificates in Europe may not be available to extend the patent exclusivity term for our products and product candidates, and we cannot provide any assurances that any such patent term extension will be obtained and, if so, for how long. Furthermore, we may not receive an extension if we fail to apply within applicable deadlines, fail to apply prior to expiration of relevant patents, or otherwise fail to satisfy applicable requirements. Moreover, the length of the extension could be less than we request. If we do not have sufficient patent terms or regulatory exclusivity to protect our products, our business and results of operations may 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 U.S. 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 U.S. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the U.S. 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 in-licensed patents or pending applications, or that we or our licensor were the first to file for patent protection of such inventions.

In 2011, the Leahy-Smith America Invents Act (the Leahy-Smith Act) was signed into law and introduced significant changes to the prosecution of U.S. patent applications and to the procedures for challenging U.S. patents. The effects of these changes still remain unclear owing to the evolving nature of the law and the lengthy timelines associated with court system review and interpretation. However, 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 products, 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 products or 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. The confidentiality agreements entered into with our employees, consultants, scientific advisors, contractors and other third parties that we rely on in connection with the development, manufacture and commercialization of our products may not be sufficient to protect our proprietary technology and processes, which increase the risk that such trade secrets may become known by our competitors or may be inadvertently incorporated into the technology of others.

The physical security of our premises and physical and electronic security of our information technology systems may not preserve the integrity and confidentiality of our data and trade secrets. 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.

The assignment agreements we enter into with our employees and consultants to assign their inventions to us, and the confidentiality agreements we enter into with our employees, consultants, advisors, and any third parties who have access to our proprietary know-how, information, or technology may not have been duly executed and we cannot assure 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.

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 others. 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, inter partes reviews, post grant reviews, 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 other 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 products or product candidates may be subject to claims of infringement of the patent rights of these other parties.

Other parties may assert that we are employing their proprietary technology without authorization. There may be patents or patent applications with claims to materials, formulations, methods of manufacture, or methods for treatment relevant to the use or manufacture of our products or product candidates. We have conducted freedom to operate analyses with respect only to our products and certain of our product candidates, and therefore we do not know whether there are any patents of other parties that would impair our ability to commercialize all of our 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 U.S. and abroad that is relevant or necessary to the commercialization of our products or 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 are relevant to our products or product candidates.

We are aware of certain U.S. and foreign patents owned by third parties that a court might construe to be valid and relevant to one or more of our gene therapy product candidates, certain methods that may be used in their manufacture, or certain formulations comprising one or more of our gene therapy candidates. We are also aware of certain U.S. and foreign patents owned by third parties that relate to anti-sclerostin antibodies and their use, and which a court might construe to be valid and relevant to setrusumab. We are additionally aware of certain U.S. and foreign patents owned by third parties that relate to nucleic acid-containing lipid particles or to certain mRNA modifications, and which a court might construe to be valid and relevant to UX053. There is a risk that one or more of these third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that one or more of these patents is valid, enforceable, and infringed, in which case the owners of any such patents may be able to block our ability to commercialize a product candidate unless we obtained a license under the applicable patents, or until such patents expire. However, 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 continue commercialization of our products, or block our ability to 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.

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 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 the corresponding program.

We may face competition from biosimilars, which may have a material adverse impact on the future commercial prospects of our biological products and product candidates.

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 our biological products (Crysvita, Mepsevii and Evkeeza) and our biological product candidates. In the U.S., the Biologics Price Competition and Innovation Act of 2009, or BPCI Act, was included in the Affordable Care Act and 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. The BPCI Act prohibits the FDA from approving a biosimilar or interchangeable product that references a brand biological product until 12 years after the licensure of the reference product, but permits submission of an application for a biosimilar or interchangeable product to the FDA four years after the reference product was first licensed. The BPCI Act does not prevent another company from developing a product that is highly similar to the innovative product, generating its own data, and seeking approval. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation and meaning are subject to uncertainty. Modification of the BPCI Act, or changes to the FDA's interpretation or implementation of the BPCI Act, could have a material adverse effect on the future commercial prospects for our biological products and product candidates.

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.

Competitors could enter the market with generic versions of Dojolvi or our small-molecule product candidates, which may result in a material decline in sales of affected products.

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, and 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 "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 enforce its patents, approval of the ANDA is stayed for 30 months, or as lengthened or shortened by the court.

Accordingly, competitors could file ANDAs for generic versions of our small-molecule product, Dojolvi, or 505(b)(2) NDAs that reference Dojolvi. For the patents listed for Dojolvi 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 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 products and product candidates is dependent on third parties.

While we normally seek and gain the right to fully prosecute the patents relating to our products or product candidates, there may be times when patents relating to our products or product candidates are controlled by our licensors. This is the case with our license agreements with KKC and Regeneron, who are primarily responsible for the prosecution of certain patents and patent applications covering Crysvita and Evkeeza, respectively.

In addition, we have in-licensed patents and patent applications owned by the University of Pennsylvania, relating to the AAV8 vector used in DTX301 and DTX401, and the AAV9 vector used in UX701. These patents and patent applications are licensed or sublicensed by REGENX and sublicensed to us. We do not have the right to control the prosecution of these patent applications, or the maintenance of any of these patents. In addition, under our agreement with REGENX, we do not have the first right to enforce the licensed patents, and our enforcement rights are subject to certain limitations that may adversely impact our ability to use the licensed patents to exclude others from commercializing competitive products. Moreover, REGENX and the University of Pennsylvania may have interests which differ from ours in determining whether to enforce and the manner in which to enforce such patents.

We also have in-licensed patents and patent applications owned by Arcturus relating to the cationic lipid used in UX053. We do not have the right to control the prosecution of these patent applications, or the maintenance of any of these patents. In addition, under our agreement with Arcturus, we do not have the first right to enforce these patents, and our enforcement rights are subject to certain limitations that may adversely impact our ability to use these licensed patents to exclude others from commercializing competitive products. Moreover, Arcturus may have interests which differ from ours in determining whether to enforce and the manner in which to enforce such patents.

If KKC, Regeneron, the University of Pennsylvania, REGENX, Arcturus or any of our future licensing partners fail to appropriately prosecute, maintain, and enforce patent protection for the patents covering any of our products or product candidates, our ability to develop and commercialize those products or 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 product candidates.

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.

We may become involved in lawsuits to protect or enforce our patents or the patents of our licensors, or be subject to claims that challenge the inventorship or ownership of our patents or other intellectual property, which could be expensive, time consuming, and result in unfavorable outcomes.

Competitors may infringe our patents or the patents of our licensors. If we or one of our licensing partners were to initiate legal proceedings against a third party to enforce a patent covering our products or one of our product candidates, the defendant could counterclaim that the patent covering our product or product candidate is invalid and/or unenforceable. In patent litigation in the U.S., 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 or derivation proceedings now available under the Leahy-Smith Act provoked by third parties or brought by us or declared or instituted 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. In addition, the validity of our patents could be challenged in the USPTO by one of the new post grant proceedings (*i.e.*, *inter partes* review or post grant review) now available under the Leahy-Smith Act. Our defense of litigation, interference proceedings, or post grant proceedings under the Leahy-Smith Act may fail and, even if successful, may result in substantial costs and distract our management and other employees.

We may in the future also be subject to claims that former employees, collaborators, or other third parties have an interest in our patents as an inventor or co-inventor. In addition, we may have ownership 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 or ownership. If we fail to successfully defend against such litigation or 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.

Even if we are successful in defending against such litigation and claims, such proceedings could result in substantial costs and distract our management and other employees. 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 litigation. There could also be public announcements of the results of hearings, motions, or other interim proceedings or developments related to such litigation or claims. 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 certain individuals who were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Our efforts to vet our employees, consultants, and independent contractors and prevent their use of the proprietary information or know-how of others in their work for us may not be successful, and we may in the future be subject to claims that our employees, consultants, or independent contractors have wrongfully used or disclosed confidential information of third parties. 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 distract 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 biotechnology and pharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology and pharmaceutical industries involves both technological and legal complexity. Therefore, obtaining and enforcing such patents is costly, time consuming, and inherently uncertain. In addition, the U.S. 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. For example, in *Association for Molecular Pathology v. Myriad Genetics, Inc.*, the Supreme Court ruled that a "naturally occurring DNA segment is a product of nature and not patent eligible merely because it has been isolated," invalidating Myriad Genetics' patents on the BRCA1 and BRCA2 genes. Certain claims of our licensed U.S. patents covering DTX301 and DTX401 relate to isolated AAV8 vectors, capsid proteins, or nucleic acids. To the extent that such claims are deemed to be directed to natural products, or to lack an inventive concept above and beyond an isolated natural product, a court may decide the claims are invalid under *Myriad*. Additionally, there have been recent proposals for additional changes to the patent laws of the U.S. and other countries that, if adopted, could impact our ability to obtain patent protection for our proprietary technology or our ability to enforce our proprietary technology. Depending on future actions by the U.S. Congress, the U.S. courts, the USPTO and the relevant law-making bodies in other countries, 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 our products or product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the U.S. can be less extensive than those in the U.S. 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 U.S. Further, licensing partners such as KKC and Regeneron 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 U.S., or from selling or importing products made using our inventions in and into the U.S. 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 U.S. 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, could put 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

Actual or threatened public health epidemics or outbreaks, including the ongoing COVID-19 pandemic, have and could again materially and adversely impact our business and operating results.

A public health epidemic or outbreak, and the public and governmental efforts to mitigate the spread of such disease, could materially and adversely impact the commercialization of our products, development and regulatory approval of our product candidates and our clinical trial operations and significantly disrupt our business operations as well as those of our third-party suppliers, CRO and collaboration partners that we rely on. In March 2020, the World Health Organization declared the novel coronavirus strain COVID-19 a pandemic.

Our clinical trial activities, including the initiation and completion of such activities and the timing thereof, have been and are expected to continue to be significantly delayed or disrupted by COVID-19. For instance, the pandemic has impacted enrollment of patients in certain of our clinical trials for our product candidates as patients have been more reluctant to conduct in-person visits at the sites due to concerns over COVID-19. Changes in local regulations in response to COVID-19 have also required us to change the way our clinical trials are conducted and certain data from our clinical trials were delayed as a result. Further, healthcare resources have been and may continue to be diverted away from the conduct of clinical trials, such as the diversion of hospitals serving as our clinical trial sites, in response to the COVID-19 pandemic. We have also had difficulties in recruiting clinical site investigators and clinical staff for our studies, and may continue to experience such difficulties. Any of these events, including if we are required to initiate new or additional sites in response to such events, could require us to incur substantial increased expenses, delay the development and commercialization of our product candidates, delay the timing of anticipated data releases, and impact our operating results.

The COVID-19 pandemic has also impacted the timing of review of our submissions and may continue to do so in the future. The pandemic has also significantly impacted our commercialization efforts for our products. Illness from the more contagious omicron variant impacted the availability of certain of our field and sales medical teams and also resulted in staffing shortages at offices, clinics and hospitals, making it difficult to maintain consistent contact with our current patients or identify new patients for our commercialized products and product candidates. Further, certain of our patients may experience interruptions in insurance coverage due to job loss or change in employment status due to the economic impact from the pandemic, which would limit patient access to our products. Effects from government budgetary constraints, either in the U.S. or internationally, due to the economic impact of the pandemic, such as changes to state coverage rules under Medicaid programs in the U.S., could also impact continued insurance coverage and reimbursement for our products. Any of these events could impact our ability to commercialize our products and adversely affect our operating results and revenue.

We have experienced delays in delivery of ancillary clinical trial materials due to government-imposed mandates and other restrictions from COVID-19 and may in the future experience delays or interruptions in supply of drug product or raw materials, or incur increased costs or expenses. For instance, the Presidential Executive Order invoking the Defense Production Act of 1950 has caused certain of our third-party manufacturers or suppliers to prioritize and allocate more resources and capacity to supply materials to other companies engaged in the study or manufacture of treatments or vaccinations for COVID-19, which has resulted in delays or shortages in supply of such materials to us. Any of these events could adversely impact our clinical trial activities and our ability to meet commercial demand for our product and product candidates and result in loss of revenue. In response to these events, we continue to seek and secure alternative sources of supply of drug product or raw materials in an attempt to avoid future potential delays in supply of product, which may result in additional expenses. We have also experienced interruptions or delays in sourcing certain equipment, materials and resources, and increased costs for certain raw materials, related to construction of our gene therapy manufacturing facility as a result of COVID-19, which could delay the anticipated timing for completion of the plant or result in significant additional expenses.

We have opened our offices in the U.S. and there is the risk that employees may transmit or contract COVID-19 when they return to work onsite despite the safety protocols we have implemented. If members of our management and other key personnel in critical functions across our organization are unable to perform their duties or have limited availability due to illness from COVID-19, we may not be able to execute on our business strategy and/or our operations may be negatively impacted. Further, as our offices reopen, we have offered a significant percentage of our employees flexibility in the amount of time they work in the office. Our new office model, our vaccination policy or other workforce actions taken in response to the COVID-19 pandemic, may not meet the expectations of our workforce, which could adversely impact our ability to attract and retain certain employees.

The COVID-19 pandemic has already impacted our operations and those of our third-party partners. The magnitude and extent to which the outbreak may impact or continue to impact our business operations, clinical trial activities, product candidate approvals, supply chain and commercialization of our products and product candidates will continue to remain highly dependent on future developments, which are very uncertain and cannot be predicted with confidence, such as the duration of the outbreak, the scope and magnitude of any resurgence in the outbreak due to virus mutations, such as the omicron or other new variants or other factors, the timing and efficacy of treatments and vaccines against virus mutations, the public acceptance of vaccines, the duration of, or implementation of additional, restrictions to contain the outbreak and the effectiveness of other actions taken in the U.S. and other countries to contain and address the pandemic. This pandemic also amplifies many of the other risks described throughout the “Risk Factors” section of this Quarterly Report on Form 10-Q.

We have no experience as a company developing or operating a manufacturing facility and may experience unexpected costs or delays or ultimately be unsuccessful in developing a facility.

During the fourth quarter 2020, we completed our purchase of land located in the Town of Bedford, Massachusetts for construction of our gene therapy manufacturing facility and began construction of the base building for the facility, which is currently expected to be completed in 2023. We do not have experience as a company, however, in developing a manufacturing facility and we may experience unexpected costs or delays or ultimately be unsuccessful in developing the facility or capability. We are dependent on key partners for delivery of power, electricity and other utilities to our manufacturing facility and we cannot assure that such services will be provided at the facility without interruptions, delays or unexpected costs. Further, as described in the risk factor above entitled, “Actual or threatened public health epidemics or outbreaks, including the ongoing COVID-19 pandemic, have and could again materially and adversely impact our business and operating results,” the COVID-19 pandemic has adversely impacted delivery of raw materials, and increased costs for certain materials, for construction of our facility. As we expand our commercial footprint to multiple geographies, we may establish multiple manufacturing facilities, which may lead to regulatory delays or prove costly. Even if we are successful, we cannot assure that such additional capacity will be required or that our investment will be recouped. Further, our manufacturing capabilities could be affected by cost-overruns, unexpected delays, equipment failures, lack of capacity, labor shortages, natural disasters, power failures, program failures, actual or threatened public health emergencies, and numerous other factors that could prevent us from realizing the intended benefits of our manufacturing strategy.

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 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. Our investments and efforts in human capital management may not 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. In the U.S., 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 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 EU, 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 Dojolvi for the treatment of fatty acid oxidation disorders in the U.S. and for various subtypes of LC-FAOD in Europe, as well as for Crysvita, Mepsevii, DTX301, DTX401 and UX701 in the U.S. 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 or the same drug can be approved for a different indication unless there are other exclusivities such as new chemical entity exclusivity preventing such approval. 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.

Our operating results would be adversely impacted if our intangible assets become impaired.

As a result of the accounting for our acquisition of Dimension Therapeutics, Inc. (Dimension) in November 2017, we have recorded on our Consolidated Balance Sheet intangible assets for in-process research and development (IPR&D) related to DTX301 and DTX401. We have also recorded contract-based intangible assets related to our license from third parties for certain assets related to Dojolvi following FDA approval of the product, in addition to the intangible asset related to our license from Regeneron for Evkeeza. We test the intangible assets for impairment annually during the fourth quarter and more frequently if events or changes in circumstances indicate that it is more likely than not that the asset is impaired. If the associated research and development effort is abandoned, the related assets will be written-off and we will record a noncash impairment loss on our Condensed Consolidated Statement of Operations. We have not recorded any impairments related to our intangible assets through the end of June 30, 2022.

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

The success of our business depends upon our ability to identify, license, discover, develop, or commercialize additional product candidates in addition to the continued clinical testing, potential approval, and commercialization of our existing product candidates. Research programs to identify and develop new product candidates, such as those under our collaboration with Arcturus, 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 technical, financial or human resources to acquire or discover additional product candidates;
- we may face competition in obtaining and/or developing additional product candidates;
- our product candidates may not succeed in research, discovery, 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 regulatory authorities, patients, the medical community, or 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 may expend our limited resources to pursue a particular product, product candidate or indication and fail to capitalize on products, product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus our sales, marketing and research programs on certain products, product candidates or for specific indications. As a result, we may forego or delay pursuit of opportunities with other products or product candidates or other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product or product candidate, we may relinquish valuable rights through collaboration, licensing, or other royalty arrangements in cases in which it would have been advantageous for us to retain sole development and commercialization rights or we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

Changes to healthcare and FDA laws, regulations, and policies may have a material adverse effect on our business and results of operations.

As described under "Item 1. Business - Government Regulation" in our Annual Report and in the Risk Factor above entitled " – *The insurance coverage and reimbursement status of newly approved products is uncertain*" there have been and continue to be a number of legislative initiatives to contain healthcare costs and to modify the regulation of drug and biologic products. We expect that additional state and federal healthcare reform measures and regulations will be adopted in the future, including proposals to reduce the exclusivity protections provided to already approved biological products and to provide biosimilar and interchangeable biologic products an easier path to approval. Any of these measures and regulations could limit the amounts that federal and state governments will pay for healthcare products and services, result in reduced demand for our product candidates or additional pricing pressures and affect our product development, testing, marketing approvals and post-market activities.

Failure to comply with laws and regulations could harm our business and our reputation.

Our business is subject to regulation by various federal, state, local and foreign governmental agencies, including agencies responsible for monitoring and enforcing employment and labor laws, workplace safety, and tax laws and regulations. In certain jurisdictions, these regulatory requirements may be more stringent than those in the U.S., and in other circumstances these requirements may be more stringent in the U.S.

In particular, our operations are directly, and 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 and patient privacy regulations, including the EU General Data Protection Regulation and the California Consumer Privacy Act (CCPA), as described in "Item 1. Business – Government Regulation" of our Annual Report. 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. For instance, one of our programs has been the subject of review by applicable governmental authorities of compliance with various fraud and abuse laws; we cannot assure that such program, or our other operations or programs, will not be challenged from time to time by such authorities for violation of such laws. Further, as we and our employees increasingly use social media tools as a means of communication with the public, there is a risk that the use of social media by us or our employees to communicate about our products or business may cause to be found in violation of applicable laws, despite our attempts to monitor such social media communications through company policies and guidelines. In addition, our employees may knowingly or inadvertently make use of social media in ways that may not comply with our company policies or other legal or contractual requirements, which may give rise to liability, lead to the loss of trade secrets or other intellectual property, or result in public exposure of personal information of our employees, clinical trial patients, customers, and others. 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, disgorgement of profits, and the curtailment or restructuring of our operations. If any governmental sanctions, fines or penalties are imposed, or if we do not prevail in any possible civil or criminal litigation, our business, operating results, financial condition and our reputation could be harmed. In addition, responding to any action will likely result in a significant diversion of management's attention and resources and an increase in professional fees.

Our research and development activities, including our process and analytical development activities in our quality control laboratory, and our 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, such as viruses, and other hazardous compounds, which subjects us to laws and regulations governing such activities. In some cases, these hazardous materials and various wastes resulting from their use are stored at our or 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 or environmental damage that could result 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. We cannot guarantee that the safety procedures utilized by us and our third-party manufacturers for handling and disposing of these materials comply with the standards prescribed by these laws and regulations, 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.

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

Our business strategy includes international expansion. We currently conduct clinical studies and regulatory activities and we also commercialize products outside of the U.S. Doing business internationally involves a number of risks, including but not limited to:

- multiple, conflicting, and changing laws and regulations such as privacy and data regulations, transparency regulations, tax laws, export and import restrictions, employment laws, regulatory requirements, and other governmental approvals, permits, and licenses;
- introduction of new health authority requirements and/or changes in health authority expectations;
- supply chain disruptions, changes to regulatory processes and other adverse effects resulting from the United Kingdom's withdrawal from the EU, commonly referred to as Brexit;
- 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 for, 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 on 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 and political and economic instability, including wars, terrorism, political unrest, results of certain elections and votes, actual or threatened public health emergencies and outbreak of disease (including for example, the COVID-19 pandemic), boycotts, adoption or expansion of government trade restrictions, and other business restrictions;
- certain expenses including, among others, expenses for travel, translation, and insurance;
- regulatory and compliance risks that relate to maintaining accurate information and control over commercial operations 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, including those under the U.K. Bribery Act and similar foreign laws and regulations; and
- regulatory and compliance risks relating to doing business with any entity that is subject to sanctions administered by the Office of Foreign Assets Control of the U.S. Department of the Treasury.

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

Risks generally associated with the expansion of our enterprise resource planning (ERP) system may adversely affect our business and results of operations or the effectiveness of our internal controls over financial reporting.

We are in the process of expanding our company-wide ERP system to upgrade certain existing business, operational, and financial processes related to our gene therapy manufacturing facility, which we currently expect to be completed in 2023. The ERP expansion is a complex and time-consuming project. Our results of operations could be adversely affected if we experience time delays or cost overruns during the ERP expansion process, or if the ERP system or associated process changes do not give rise to the benefits that we expect. This project has required and may continue to require investment of capital and human resources, the re-engineering of processes of our business, and the attention of many employees who would otherwise be focused on other aspects of our business. Any deficiencies in the design and implementation of the expanded ERP system could result in potentially much higher costs than we had incurred and could adversely affect our ability to develop and launch solutions, provide services, fulfill contractual obligations, file reports with the SEC in a timely manner, operate our business or otherwise affect our controls environment. Any of these consequences could have an adverse effect on our results of operations and financial condition.

Our business and operations may be materially adversely affected in the event of computer system failures or security breaches.

Cybersecurity incidents, including phishing attacks and attempts to misappropriate or compromise confidential or proprietary information or sabotage enterprise IT systems are becoming increasingly frequent and more sophisticated. The information and data processed and stored in our technology systems, and those of our strategic partners, CROs, contract manufacturers, suppliers, distributors or other third parties for which we depend to operate our business, may be vulnerable to loss, damage, denial-of-service, unauthorized access or misappropriation. Data security breaches can occur as a result of malware, hacking, business email compromise, ransomware attacks, phishing or other cyberattacks directed by third parties. We, and certain of the third parties for which we depend on to operate our business, have experienced cybersecurity incidents, including third-party unauthorized access to and misappropriation of financial information. Further, risks of unauthorized access and cyber-attacks have increased as most of our personnel, and the personnel of many third-parties with which we do business, have adopted remote working arrangements as a result of the COVID-19 pandemic. Improper or inadvertent employee behavior, including data privacy breaches by employees, contractors and others with permitted access to our systems, pose a risk that sensitive data may be exposed to unauthorized persons or to the public. A system failure or security breach that interrupts our operations or the operations at one of our third-party vendors or partners could result in intellectual property and other proprietary or confidential information being lost or stolen or a material disruption of our drug development programs and commercial operations. For example, the loss of clinical trial data from ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications, loss of trade secrets or inappropriate disclosure of confidential or proprietary information, including protected health information or personal identifiable information of employees or former employees, access to our clinical data, or disruption of the manufacturing process, we could incur liability and the further development of our drug candidates could be delayed. Further, we could incur significant costs to investigate and mitigate such cybersecurity incidents. A security breach that results in the unauthorized access, use or disclosure of personal identifiable information also requires us to notify individuals, governmental authorities, credit reporting agencies, or other parties, as applicable, pursuant to privacy and security laws and regulations or other obligations. Such a security breach could harm our reputation, erode confidence in our information security measures, and lead to regulatory scrutiny and result in penalties, fines, indemnification claims, litigation and potential civil or criminal liability.

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 one of our laboratories are located in the San Francisco Bay Area, and our collaboration partner for Crysvita, KKC, 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. We have also experienced power outages as a result of wildfires in the San Francisco Bay Area which are likely to continue to occur in the future. 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 may be inadequate 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.

We may acquire companies or products or engage in strategic transactions, which could divert our management's attention and cause us to incur various costs and expenses, or result in fluctuations with respect to the value of such investment, which could impact our operating results.

We may acquire or invest in businesses or products that we believe could complement or expand our business or otherwise offer growth opportunities. For example, we acquired Dimension in November 2017 and GeneTx in July 2022. The pursuit of potential acquisitions or investments may divert the attention of management and may cause us to incur various costs and expenses in identifying, investigating, and pursuing them, whether or not they are consummated. We may not be able to identify desirable acquisitions or investments or be successful in completing or realizing anticipated benefits from such transactions. We may experience difficulties in assimilating the personnel, operations and products of the acquired companies, management's attention may be diverted from other business concerns and we may potentially lose key employees of the acquired company. If we are unable to successfully or timely integrate the operations of acquired companies with our business, we may incur unanticipated liabilities and be unable to realize the revenue growth, synergies and other anticipated benefits resulting from the acquisition, and our business, results of operations and financial condition could be materially and adversely affected.

The value of our investments in other companies or businesses may also fluctuate significantly and impact our operating results quarter to quarter or year to year. For instance, in June 2019, we purchased 2,400,000 shares of common stock of Arcturus and in May 2020, we exercised our option to purchase an additional 600,000 shares of Arcturus' common stock pursuant to the terms of our equity purchase agreement with Arcturus; we have subsequently sold an aggregate of 2,500,000 shares. We also purchased 7,825,797 shares of common stock of Solid in October 2020. We have elected to apply the fair value option to account for our equity investments in Arcturus and Solid. As a result, increases or decreases in the stock price of Arcturus and Solid common stock will result in accompanying changes in the fair value of our investments, and cause substantial volatility in, our operating results for the reporting period. As the fair value of our investments in Arcturus and Solid is dependent on the stock price of Arcturus and Solid, which has recently seen wide fluctuations, the value of our investments and the impact on our operating results may similarly fluctuate significantly from quarter to quarter and year to year such that period-to-period comparisons may not be a good indication of the future value of the investments and our future operating results.

Risks Related to Ownership of Our Common Stock

The market price of our common stock may be highly volatile.

The market price of our common stock has been, and is likely to continue to be, volatile, including for reasons unrelated to changes in our business. 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, MAA, 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, MAA, or other regulatory submission;
- the perception of limited market sizes or pricing for our products and product candidates;
- decisions by our collaboration partners with respect to the indications for our products and product candidates in countries where they have the right to commercialize the products and product candidates;
- decisions by our collaboration partners regarding market access and pricing in countries where they have the right to commercialize our products and product candidates;
- failure to successfully develop and commercialize our products and product candidates;
- the level of revenue we receive from our commercialized products or from named patient sales;
- 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 products and product candidates or the inability to do so at acceptable prices;
- adverse regulatory decisions;
- introduction of new products, services, or technologies by our competitors;
- changes in or failure to meet or exceed financial projections or other guidance we may provide to the public;
- changes in or failure to meet or exceed the financial projections or other expectations of the investment community;
- the perception of the pharmaceutical industry or our company by the public, legislatures, regulators, and the investment community;
- the perception of the pharmaceutical industry's approach to drug pricing;
- announcements of significant acquisitions, strategic partnerships, joint ventures, or capital commitments by us, our strategic collaboration partners, or our competitors;
- the integration and performance of any businesses we have acquired or may acquire;

- 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 investigations, regulatory proceedings or lawsuits, including patent or stockholder litigation;
- securities or industry analysts' reports regarding our stock, or their failure to issue such reports;
- changes in the market valuations of similar companies;
- general market or macroeconomic conditions, including the impact from the COVID-19 pandemic;
- 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.

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 2014 Incentive Plan, or the 2014 Plan, our management is authorized to grant stock options and other equity-based awards to our employees, directors, and consultants. At June 30, 2022, 1,972,064 shares were available for future grants under the 2014 Plan. Through January 1, 2024, the number of shares available for future grant under the 2014 Plan will automatically increase on January 1 of each year by the lesser of 2,500,000 shares or 4% 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.

Pursuant to our 2014 Employee Stock Purchase Plan, or 2014 ESPP, eligible employees can acquire shares of our common stock at a discount to the prevailing market price. At June 30, 2022, 4,640,287 shares were available for issuance under the 2014 ESPP. Through January 1, 2024, the number of shares available for issuance under the 2014 ESPP will automatically increase on January 1 of each year by the lesser of 1,200,000 shares or 1% 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.

In February 2021, our board of directors adopted the Employment Inducement Plan (the Inducement Plan) with a maximum of 500,000 shares available for grant under the plan. At June 30, 2022, 403,963 shares were available for issuance under the Inducement Plan. If our board of directors elects to increase the number of shares available for future grant under the 2014 Plan, the 2014 ESPP, or the Inducement Plan, our stockholders may experience additional dilution, which could cause our stock price to fall.

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. We currently intend to retain all available funds and any future earnings, if any, 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 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 resolution adopted by the board 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. Further, no stockholder is permitted to cumulate votes at any election of directors because this right is not included in our amended and restated certificate of incorporation.

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.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware is the sole and exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or employees.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware is the sole and exclusive forum for (1) any derivative action or proceeding brought on our behalf, (2) any action asserting a claim of breach of fiduciary duty owed by any of our directors, officers, or other employees to us or to our stockholders, (3) any action asserting a claim against us arising under the Delaware General Corporation Law or under our amended and restated certificate of incorporation or bylaws, or (4) any action against us asserting a claim governed by the internal affairs doctrine. The choice of forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers, or other employees, which may discourage such lawsuits against us and our directors, officers, and other employees. Alternatively, if a court were to find the choice of forum provision contained in our amended and restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could harm our business, operating results and financial condition.

General Risk Factors

If we are unable to maintain effective internal control over financial reporting, investors may lose confidence in the accuracy and completeness of our financial reports and the market price of our stock may decrease.

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 are required to perform system and process evaluation and testing of our internal controls over financial reporting to allow management to report on the effectiveness of our internal controls over financial reporting, as required by Section 404(a) of the Sarbanes-Oxley Act. Section 404(b) of the Sarbanes-Oxley Act also requires our independent auditors to attest to, and report on, this management assessment. Ensuring that we have adequate internal controls in place so that we can produce accurate financial statements on a timely basis is a costly and time-consuming effort that will need to be evaluated frequently. If we are not able to comply with the requirements of Section 404 or if we or our independent registered public accounting firm are unable to attest to the effectiveness of our internal control over financial reporting, investors may lose confidence in the accuracy and completeness of our financial reports, 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.

We may incur additional tax liabilities related to our operations.

We have a multinational tax structure and are subject to income tax in the U.S. and various foreign jurisdictions. Our effective tax rate is influenced by many factors including changes in our operating structure, changes in the mix of our earnings among countries, our allocation of profits and losses among our subsidiaries, our intercompany transfer pricing agreements and rules relating to transfer pricing, the availability of U.S. research and development tax credits, and future changes in tax laws and regulations in the U.S. and foreign countries. Significant judgment is required in determining our tax liabilities including management's judgment for uncertain tax positions. The Internal Revenue Service, other domestic taxing authorities, or foreign taxing authorities may disagree with our interpretation of tax laws as applied to our operations. Our reported effective tax rate and after-tax cash flows may be materially and adversely affected by tax assessments in excess of amounts accrued for our financial statements. This could materially increase our future effective tax rate thereby reducing net income and adversely impacting our results of operations for future periods.

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. To the extent that we continue to generate taxable losses, unused taxable losses will, subject to certain limitations, 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, or the IRC, 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 NOL carryforwards, and other pre-change tax attributes (such as research tax credits) to offset its post-change income may be limited. An analysis to determine limitations upon our NOL carryforwards and other pre-change tax attributes for ownership changes that have occurred previously has been performed, resulting in a permanent decrease of federal and state NOL carryforwards in the amount of $7.2 million and a permanent decrease in federal research tax credit carryforwards in the amount of $0.2 million. As a result of these decreases and others that may occur as a result of future ownership changes, our ability to use our pre-change NOL carryforwards and other tax attribute carryforwards to offset U.S. federal taxable income and tax liabilities is limited and may become subject to even greater limitations, which could potentially accelerate or permanently increase future federal tax liabilities for us. In addition, there may be periods during which the use of state income tax NOL carryforwards and other state tax attribute carryforwards (such as state research tax credits) are suspended or otherwise limited, which could potentially accelerate or permanently increase future state tax liabilities for us.

Litigation may substantially increase our costs and harm our business.

We have been, and may in the future become, party to lawsuits including, without limitation, actions and proceedings in the ordinary course of business relating to our directors, officers, stockholders, intellectual property, and employment matters and policies, which will cause us to incur legal fees and other costs related thereto, including potential expenses for the reimbursement of legal fees of officers and directors under indemnification obligations. The expense of defending against such litigation may be significant and there can be no assurance that we will be successful in any defense. Further, the amount of time that may be required to resolve such lawsuits is unpredictable, and these actions may divert management's attention from the day-to-day operations of our business, which could adversely affect our business, results of operations, and cash flows. Litigation is subject to inherent uncertainties, and an adverse result in such matters that may arise from time to time could have a material adverse effect on our business, results of operations, and financial condition.

Item 2. ***Unregistered Sales of Equity Securities and Use of Proceeds***

None.

Item 3. ***Defaults Upon Senior Securities***

None.

Item 4. ***Mine Safety Disclosures***

Not applicable.

Item 5. ***Other Information***

None.

Item 6. ***Exhibits***

		Incorporated by Reference			
Exhibit Number	**Exhibit Description**	**Form**	**Date**	**Number**	**Furnished or Filed Herewith**
3.1	Amended and Restated Certificate of Incorporation	8-K	2/5/2014	3.1	
3.2	Amended and Restated Bylaws	8-K	2/5/2014	3.2	
4.1	Form of Common Stock Certificate	S-1	11/8/2013	4.2	
4.2	Form of Indenture	S-3ASR	2/12/2021	4.2	
10.1*	Royalty Purchase Agreement by and among Rare Delaware Inc., Ultragenyx Pharmaceutical Inc. and OCM LS23 Holdings LP dated as of July 14, 2022.				X
10.2*	Unit Purchase Agreement by and among Ultragenyx Pharmaceutical Inc., GeneTx Biotherapeutics LLC, the Unitholders and Deborah A. Guagliardo, dated as of July 15, 2022				X
31.1	Certification of Principal Executive Officer Required Under Rule 13a-14(a) or Rule 15d-14(a) of the Exchange Act				X
31.2	Certification of Principal Financial Officer Required Under Rule 13a-14(a) or Rule 15d-14(a) of the Exchange Act				X
32.1**	Certification of Principal Executive Officer and Principal Financial Officer Required Under Rule 13a-14(b) or Rule 15d-14(b) of the Exchange Act and 18 U.S.C. 1350				X
101.INS	XBRL Instance Document, formatted in Inline XBRL				X
101.SCH	Inline XBRL Taxonomy Extension Schema Document				X
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document				X
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document				X
101.LAB	Inline XBRL Taxonomy Extension Labels Linkbase Document				X
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document				X
104	Cover Page Interactive Data File, formatted in Inline XBRL (included in Exhibit 101).				

* Certain confidential portions of this exhibit were omitted by means of marking such portions with asterisks because the identified confidential portions (i) are not material and (ii) would be competitively harmful if publicly disclosed.

** The certification attached as Exhibit 32.1 that accompanies this Quarterly Report is furnished to, and not deemed filed with, the SEC and is not to be incorporated by reference into any filing of the Registrant under the Securities Act or the Exchange Act, whether made before or after the date of this Form 10-Q, irrespective of any general incorporation language contained in such filing.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

ULTRAGENYX PHARMACEUTICAL INC.

Date: July 28, 2022

By: /s/ Emil D. Kakkis
Emil D. Kakkis, M.D., Ph.D.
President and Chief Executive Officer
(Principal Executive Officer)

Date: July 28, 2022

By: /s/ Mardi C. Dier
Mardi C. Dier
Executive Vice President and Chief Financial Officer
(Principal Financial Officer)

Date: July 28, 2022

By: /s/ Theodore A. Huizenga
Theodore A. Huizenga
Senior Vice President and Chief Accounting Officer
(Principal Accounting Officer)

Exhibit 10.1

[***] = CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY BRACKETS, HAS BEEN OMITTED BECAUSE THE INFORMATION (I) IS NOT MATERIAL AND (II) WOULD BE COMPETITIVELY HARMFUL IF PUBLICLY DISCLOSED

Royalty Purchase Agreement

By and Between

Rare Delaware Inc.

Ultragenyx Pharmaceutical Inc.

and

OCM LS23 Holdings LP

Dated as of July 14, 2022

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Page

Index of Exhibits

ROYALTY PURCHASE AGREEMENT

This Royalty Purchase Agreement (this "Agreement"), dated as of July 14, 2022 (the "Effective Date"), is made and entered into by and among Rare Delaware Inc., a Delaware corporation ("Seller"), Ultragenyx Pharmaceutical Inc., a Delaware corporation and the direct parent of Seller ("Ultragenyx") and OCM LS23 Holdings LP, an Ontario limited partnership ("Buyer").

RECITALS:

WHEREAS, pursuant to the License Agreement, Ultragenyx and Licensee granted to each other certain licenses and other rights and, commencing on the Profit Share Territory Transition Date, Licensee will have the exclusive right to (among other activities) sell the Licensed Product in the Profit Share Territory, and Licensee, in partial consideration thereof, agreed to pay the Royalty and other payments to Ultragenyx from and after the Profit Share Territory Transition Date;

WHEREAS, Ultragenyx has sold the Royalty to Seller, and Buyer now desires to purchase the Purchased Interest from Seller, while Seller desires to sell the Purchased Interest to Buyer.

NOW THEREFORE, in consideration of the representations, warranties, covenants and agreements set forth herein and for good and valuable consideration, the receipt and adequacy of which are hereby acknowledged, Seller, Ultragenyx and Buyer hereby agree as follows:

ARTICLE 1

DEFINED TERMS AND RULES OF CONSTRUCTION

Section 1.1 Definitions. As used in this Agreement, the following terms shall have the following meanings:

"Affiliate" shall have the meaning ascribed to the term Affiliate in Section 1.1.1 of the License Agreement. In the case of Buyer, the term Affiliate also includes any Person in respect of which OMERS Administration Corporation, as administrator of the OMERS primary pension plan and trustee of the pension funds thereunder, holds, directly or indirectly, more than 50% of the equity interests of such Person.

"Agreement" is defined in the preamble.

"Amendment No. 3" means that certain Amendment No. 3 to the Collaboration and License Agreement by and between Ultragenyx and Licensee, dated as of September 29, 2017.

"Applicable Law" means, with respect to any Person, all laws, rules, regulations and order of Governmental Entities applicable to such Person or any of its properties or assets;

"Applicable Patents" is defined in Section 6.12(c).

"Bankruptcy Laws" means, collectively, bankruptcy, insolvency, reorganization, moratorium, fraudulent conveyance, fraudulent transfer or other similar laws affecting the enforcement of creditors' rights generally.

"Bill of Sale" is defined in Section 3.3.

"Business Day" means any day other than (I) a Saturday or Sunday or (ii) a day on which banking institutions located in New York, New York or Toronto, Ontario are permitted or required by Applicable Law to remain closed.

"Buyer" is defined in the preamble.

"Buyer Bank Account" is defined in Section 6.2(c).

"Buyer Closing Certificate" is defined in Section 3.2(c).

"Buyer GP" means OCM LS23 Holdings G.P. Inc., in its capacity as general partner of Buyer.

"Buyer Indemnified Parties" is defined in Section 8.1(a).

"Closing" is defined in Section 3.1.

"Closing Date" means the date on which the Closing occurs.

"Determination" is defined in Section 6.17(b).

"Disclosure Schedule" means the Disclosure Schedule, dated as of the Effective Date, delivered to Buyer by Ultragenyx and Seller concurrently with the execution of this Agreement.

"Disputes" is defined in Section 4.1(l)(ii).

"Drug Substance" shall have the meaning ascribed to the term Drug Substance in Section 1.1.15 of the License Agreement.

"Excluded Liabilities and Obligations" is defined in Section 2.2.

"Field" shall have the meaning ascribed to the term Field in Section 1.1.22 of the License Agreement.

"Governmental Entity" means any: (i) nation, principality, republic, state, commonwealth, province, territory, county, municipality, district or other jurisdiction of any nature; (ii) federal, state, provincial, local, municipal, foreign or other government; (iii) governmental or quasi-governmental authority of any nature (including any governmental division, subdivision, department, agency, bureau, branch, office, commission, council, board, instrumentality, officer, official, representative, organization, unit, body or other entity and any court, arbitrator or other tribunal); (iv) multi-national organization or body; or (v) individual,

body or other entity exercising, or entitled to exercise, any executive, legislative, judicial, administrative, regulatory, police, military or taxing authority or power of any nature.

"In-Licenses" shall have the meaning ascribed to the term In-Licenses in Section 1.1.36 of the License Agreement.

"Indemnified Party" is defined in Section 8.2.

"Indemnifying Party" is defined in Section 8.2.

"Intended Tax Treatment" is defined in Section 6.17(a).

"Intercompany Sale Documents" means that certain Purchase and Sale Agreement by and between Ultragenyx and Seller, dated as of July 14, 2022 and that certain Promissory Note issued by Seller to Ultragenyx, dated as of July 14, 2022.

"Joint Invention" shall have the meaning ascribed to the term Joint Invention in Section 1.1.37 of the License Agreement.

"Joint Invention Patents" means all Patent Rights claiming or covering any Joint Inventions.

"Judgment" means any judgment, order, writ, injunction, citation, award or decree of any nature.

"KHK Invention Patents" means all Patent Rights claiming or covering any KHK Inventions.

"KHK Inventions" shall have the meaning ascribed to the term KHK Inventions in Section 1.1.39 of the License Agreement.

"KHK Know-How" shall have the meaning ascribed to the term Licensed Know-How in Section 1.1.46 of the License Agreement.

"KHK Patent Rights" shall have the meaning ascribed to the term Licensed Patent Rights in Section 1.1.47 of the License Agreement.

"Knowledge of Seller" means the actual knowledge of the individuals listed on Schedule 1.1-S of the Disclosure Schedule, after due inquiry.

"Knowledge of Ultragenyx" means the actual knowledge of the individuals listed on Schedule 1.1-U of the Disclosure Schedule, after due inquiry.

"License Agreement" means that certain Collaboration and License Agreement, dated as of August 29, 2013, by and between Ultragenyx and Licensee, as amended by that certain Amendment No. 1, dated as of August 24, 2015, that certain Amendment No. 2, dated as of

November 28, 2016, Amendment No. 3, that certain Amendment No. 4, dated as of January 29, 2018, that certain Amendment No. 5, dated as of April 30, 2018, that certain Amendment No. 6, dated as of February 1, 2019, that certain Amendment No. 7, dated as of December 5, 2018, that certain Amendment No. 8, dated as of July 4, 2019, that certain Amendment No. 9, dated as of December 23, 2019, that certain Amendment No. 10, dated as of April 1, 2020 and that certain Amendment No. 11, dated as of December 17, 2021.

"Licensed IP" means, collectively, the Licensed KHK IP and the Licensed UGNX IP.

"Licensed KHK IP" means the KHK Patent Rights, the KHK Know-How, the KHK Inventions and Licensee's interest in the Joint Inventions.

"Licensed UGNX IP" means the UGNX Inventions and Ultragenyx's interest in the Joint Inventions.

"Licensed Patents" is defined in Section 4.1(l)(i).

"Licensed Product" shall have the meaning ascribed to the term Licensed Product in Section 1.1.48 of the License Agreement.

"Licensee" means Kyowa Kirin Co. Ltd. (formerly, Kyowa Hakko Kirin Co., Ltd.) and any successor thereof, as permitted pursuant to the terms of this Agreement and the License Agreement.

"Licensee Consent" is the consent letter attached hereto as Exhibit C.

"Licensee Instruction Letter" is defined in Section 6.2(b).

"Lien" means any mortgage, lien, pledge, charge, adverse claim, security interest, encumbrance or restriction of any kind, including any restriction on use, transfer or exercise of any other attribute of ownership of any kind.

"Loss" means any and all Judgments, damages, Taxes, losses, claims, costs, liabilities and expenses, including reasonable fees and out-of-pocket expenses of counsel.

"Material Adverse Effect" shall mean (i) a material adverse effect on the legality, validity or enforceability of any provision of the Intercompany Sale Documents or this Agreement, (ii) a material adverse effect on the ability of Seller or Ultragenyx to perform any of its obligations hereunder or under any of the Intercompany Sale Documents, (iii) a material adverse effect on the rights or remedies of Buyer hereunder, (iv) a material adverse effect on the rights of Seller or Ultragenyx under the License Agreement related to the Royalty or the Profit Share Territory or (v) an adverse effect on the timing, amount or duration of the payments to be made to Buyer in respect of any portion of the Purchased Interest or the right of Buyer to receive such payments in any material respect (but excluding in each case any event, circumstance or change based on market conditions generally applicable to the industry in which Seller or

Ultragenyx operates or in any specific jurisdiction or geographical area, such as drug reimbursement rates or the commercial launch of a potentially competitive product).

"Mutually Agreed" means:

(a) for matters: (x) solely related to the Royalty or the Profit Share Territory, or (y) that would reasonably be expected (with or without the giving of notice or passage of time, or both) to result in a Material Adverse Effect, Seller or Ultragenyx shall take (or refrain from taking) such reasonable actions in respect of each such matter as are reasonably requested by Buyer;

(b) except with respect to matters related to routine intellectual property maintenance and prosecution, for matters under the License Agreement that: (x) do not relate, directly or indirectly, to the Royalty or the Profit Share Territory and (y) would not reasonably be expected (with or without the giving of notice or passage of time, or both) to result in a Material Adverse Effect, Seller or Ultragenyx shall have the right (subject to providing prior, reasonably detailed written notice to Buyer) to take (or refrain from taking) such actions in respect of each such matter as Seller or Ultragenyx, acting reasonably, deems appropriate; or

(c) except with respect to matters related to routine intellectual property maintenance and prosecution, for matters (x) involving a UGNX Invention Patent in the Profit Share Territory or a Joint Invention Patent (but subject to the extent of Seller's or Ultragenyx's rights under Section 10.2.1 of the License Agreement) in the Profit Share Territory, including patent term restoration, extension or adjustment, supplementary protection certificates and the like or any other similar foreign equivalent, or (y) all other matters under the License Agreement that (i) do not meet the criteria set forth in clauses (a) or (b) above, and (ii) would not reasonably be expected (with or without the giving of notice or the passage of time, or both) to result in a Material Adverse Effect, Seller or Ultragenyx shall take (or refrain from taking) actions in respect of each such matter as Seller or Ultragenyx and Buyer, each acting reasonably, mutually agree.

"Net Sales" shall have the meaning ascribed to the term Net Sales in Section 1.1.54 of the License Agreement.

"Opinion" is defined in Section 3.5.

"Patent Rights" shall have the meaning ascribed to the term Patent Rights in Section 1.1.58 of the License Agreement.

"Permitted Liens" means any (i) mechanic's, materialmen's, and similar liens for amounts not yet due and payable, (ii) statutory liens for taxes not yet due and payable or for taxes that the taxpayer is contesting in good faith, (iii) any liens in favor of, or granted to, Licensee pursuant to the License Agreement, (iv) any liens created, permitted or required by the Transaction Documents in favor of Buyer or its Affiliates, (v) Liens related to "march in" rights of the United States government under 35 U.S.C. §§ 200 – 212, and implementing

regulations, and (vi) other liens and encumbrances not incurred in connection with the borrowing of money by Ultragenyx, Seller or any other Person that do not materially and adversely affect the use or value of the affected assets provided that, in each case, such liens are automatically released upon the sale or other transfer of the affected assets (it being understood that any obligations secured by such "Permitted Liens" shall remain the obligations of Ultragenyx).

"Person" means any individual, firm, corporation, company, partnership, limited liability company, trust, joint venture, association, estate, trust, Governmental Entity or other entity, enterprise, association or organization.

"Pharmacovigilance Agreement" means that certain Pharmacovigilance Agreement by and between [***] and Ultragenyx, dated as of May 13, 2021, as amended June 8, 2022.

"Prime Rate" means the prime rate published by the Wall Street Journal, from time to time, as the prime rate.

"Proceeds" means any amounts actually recovered by Ultragenyx or Seller as a result of any settlement or resolution of any actions, suits, proceedings, claims or disputes related to the License Agreement related to or involving the Royalty.

"Profit Share Territory" shall have the meaning ascribed to the term Profit Share Territory in Section 1.1.67 of the License Agreement.

"Profit Share Territory Transition Date" shall mean April 26, 2023.

"Purchase Price" means $500,000,000.

"Purchased Interest" means the right to receive payments in amounts equal to the Royalty.

"Quality Technical Agreement" means that certain Quality and Technical Agreement by and between [***] and Ultragenyx, dated as of March 29, 2018.

"Representative" means, with respect to any Person, (i) any direct or indirect stockholder, member or partner of such Person and (ii) any manager, director, officer, employee, agent, advisor or other representative (including attorneys, accountants, consultants, bankers, financial advisors and actual and potential lenders and investors) of such Person.

"Royalty" means, on any date prior to the occurrence of the earlier of (a) the date on which aggregate payments on account of the Purchased Interest owed to Buyer hereunder equal the Royalty Cap or (b) the date of the last Royalty payment under the License Agreement, 30% of the following (in each case, as determined without giving effect to the transactions under the Intercompany Sale Documents): (i) all payments owed to Ultragenyx under Section

7.2.2 of the License Agreement with respect to Net Sales of a Licensed Product in the Profit Share Territory from and after the Profit Share Territory Transition Date, (ii) any payments to Ultragenyx under the License Agreement in lieu of such payments of the foregoing clause (i), (iii) any payments to Ultragenyx under Section 10.5.3(i) of the License Agreement relating to a claim for Competing Product Infringement (as defined in the License Agreement) in the Profit Share Territory that occurred from and after the Profit Share Territory Transition Date, (iv) any payments to Ultragenyx under Section 15.5(d), (e) or (h) of the License Agreement with respect to the Profit Share Territory from and after the Profit Share Territory Transition Date, and (v) any interest payments to Ultragenyx under Section 7.4 of the License Agreement assessed on any payments described in the foregoing clauses (i), (ii), (iii) or (iv).

For greater certainty: (i) such 30% portion of the foregoing amounts represents 100% of Seller's entitlement thereto after giving effect to the transactions under the Intercompany Sale Documents and before giving effect to the sale of the Purchased Interest hereunder, and (ii) as between Ultragenyx, Seller and Buyer, Ultragenyx will remain entitled to 100% of all amounts payable under the License Agreement on account of Net Sales before the Profit Share Territory Transition Date, including subsequent adjustments to the reserves for the respective period.

Notwithstanding the foregoing and for the avoidance of doubt, the term "Royalty" shall exclude: (i) any payments under Article 7 of the License Agreement, except those payments arising pursuant to Sections 7.2.2 and 7.4 described above, (ii) any damages or royalties payable to a Third Party under Section 10.6.1 of the License Agreement, (iii) reimbursements to Ultragenyx or Seller for costs and expenses incurred in connection with the preparation, filing, prosecution and maintenance of patent applications and patents in the KHK Patent Rights under Section 10.1.1 or the Joint Inventions under Section 10.2.1 of the License Agreement or litigation costs under Sections 10.5.3, 10.6.2, or 10.6.3 of the License Agreement; (iv) any profit sharing payments and related cost reimbursements to Ultragenyx or Seller under Sections 7.1 and 7.2.1 of the License Agreement, or any milestone payments or upfront payments payable to Seller, if any, under Article 7 of the License Agreement, and (v) any indemnity payments to Ultragenyx or Seller and its Affiliates under Section 14.2 of the License Agreement that are not in respect of the Royalty.

"Royalty Cap" means $725,000,000.

"Royalty Reduction" is defined in Section 4.1(j)(xii).

"Royalty Reports" means the quarterly reports deliverable by Licensee pursuant to Section 7.2.4 of the License Agreement redacted to remove all information with respect to Licensed Products outside the Profit Share Territory.

"Royalty Termination Date" means the earlier of (a) the date on which aggregate payments on account of the Purchased Interest actually received by Buyer equal the Royalty Cap or (b) the date of payment of the Purchased Interest in respect of the last Royalty payment under the License Agreement in respect of the Profit Share Territory.

"Seller" is defined in the preamble.

"Seller Bank Account" is defined in Section 6.2(a).

"Seller Closing Certificate" is defined in Section 3.2(a).

"Seller Indemnified Parties" is defined in Section 8.1(b).

"Tax" or "Taxes" means any federal, state, local or non-U.S. income, gross receipts, license, payroll, employment, excise, severance, occupation, premium, windfall profits, environmental, customs duties, capital stock, franchise, profits, withholding, social security, unemployment, disability, real property, personal property, abandoned property, value added, alternative or add-on minimum, estimated or other tax of any kind whatsoever, including any interest, penalty or addition thereto, whether disputed or not.

"Third Party" shall have the meaning ascribed to the term Third Party in Section 1.1.77 of the License Agreement.

"Transaction Documents" means this Agreement, the Bill of Sale, the Disclosure Schedule, the Licensee Instruction Letter, and the Licensee Consent.

"UCC" means Article 9 of the New York Uniform Commercial Code, as in effect from time to time.

"UGNX Inventions" shall have the meaning ascribed to the term UGNX Inventions in Section 1.1.80 of the License Agreement.

"UGNX Invention Patents" means all Patent Rights claiming or covering any UGNX Inventions.

"Ultragenyx" is defined in the preamble.

"Ultragenyx Closing Certificate" is defined in Section 3.2(b).

Section 1.2 Certain Interpretations. Except where expressly stated otherwise in this Agreement, the following rules of interpretation apply to this Agreement:

(a) "either" and "or" are not exclusive and "include," "includes" and "including" are not limiting and shall be deemed to be followed by the words "without limitation;"

(b) "extent" in the phrase "to the extent" means the degree to which a subject or other thing extends, and such phrase does not mean simply "if;"

(c) "hereof," "hereto," "herein" and "hereunder" and words of similar import when used in this Agreement refer to this Agreement as a whole and not to any particular provision of this Agreement;

(d) references to a Person are also to its permitted successors and assigns;

(e) definitions are applicable to the singular as well as the plural forms of such terms;

(f) unless otherwise indicated, references to an "Article", "Section" or "Exhibit" refer to an Article or Section of, or an Exhibit to, this Agreement, and references to a "Schedule" refer to the corresponding part of the Disclosure Schedule;

(g) references to "$" or otherwise to dollar amounts refer to the lawful currency of the United States;

(h) references to a law include any amendment or modification to such law and any rules and regulations issued thereunder, whether such amendment or modification is made, or issuance of such rules and regulations occurs, before or after the date of this Agreement; and

(i) for purposes of the defined terms in Section 1.1, any term that is defined by reference to the License Agreement refers to the License Agreement as of the Effective Date, as amended in accordance with this Agreement.

Section 1.3 Headings. The table of contents and the descriptive headings of the several Articles and Sections of this Agreement and the Exhibits and Schedules are for convenience only, do not constitute a part of this Agreement and shall not control or affect, in any way, the meaning or interpretation of this Agreement.

ARTICLE 2

PURCHASE, SALE AND ASSIGNMENT OF THE PURCHASED INTEREST

Section 2.1 Closing; Purchase Price. Upon the terms and subject to the conditions of this Agreement, at the Closing, Seller shall sell, transfer, assign and convey to Buyer, and Buyer shall purchase, acquire and accept from Seller, free and clear of all Liens, the Purchased Interest. The purchase price to be paid to Seller for the sale, transfer, assignment and conveyance of the Purchased Interest to Buyer is the Purchase Price.

Section 2.2 No Assumed Obligations, Etc. Notwithstanding any provision in this Agreement to the contrary, Buyer is purchasing, acquiring and accepting only the Purchased Interest, and is not assuming any liability or obligation of Ultragenyx or Seller of whatever nature, whether presently in existence or arising or asserted hereafter, under the License Agreement or otherwise, all of which liabilities and obligations shall remain liabilities or

obligations of Ultragenyx or Seller, as the case may be, and as between Ultragenyx or Seller, on the one hand, and Buyer, on the other hand, Ultragenyx and Seller shall exclusively remain jointly and severally responsible for the satisfaction and performance thereof (the "Excluded Liabilities and Obligations"). For the avoidance of doubt, the Excluded Liabilities and Obligations shall include any deductions or withholdings made by the Licensee (or any of its Affiliates) from any payment of Royalties on account of Taxes that arise as a result of amounts paid to Ultragenyx (including any assignee thereof) pursuant to the License Agreement prior to or following the Closing Date, other than Tax deductions or withholdings (including any interest and penalties thereon or related thereto) that are required under Applicable Law as a result of the payment of Royalties to or for the benefit of Seller. Except as specifically set forth herein in respect of the Purchased Interest purchased, acquired and accepted hereunder, Buyer does not, by such purchase, acquisition and acceptance, acquire any other contract rights of Ultragenyx or Seller under the License Agreement or any other assets of Ultragenyx or Seller.

Section 2.3 True Sale. It is the intention of the parties hereto that the sale, transfer, assignment and conveyance contemplated by this Agreement constitute a sale of the Purchased Interest from Seller to Buyer (and not a financing transaction, borrowing or loan) for all applicable purposes. Accordingly, Seller shall treat the sale, transfer, assignment and conveyance of the Purchased Interest as a sale of an "account" or a "payment intangible" (as appropriate) in accordance with the UCC, and Seller hereby authorizes Buyer to file financing statements (and continuation statements with respect to such financing statements when applicable) naming Seller as the debtor and Buyer as the secured party in respect of the Purchased Interest. Not in derogation of the foregoing statement of the intent of the parties hereto in this regard, and for the purposes of providing additional assurance to Buyer in the event that, despite the intent of the parties hereto, the sale, transfer, assignment and conveyance contemplated hereby is hereafter held not to be a sale, Seller does hereby grant to Buyer, as security for the obligations of Seller hereunder, a first priority security interest in and to all right, title and interest of Seller, in, to and under the Purchased Interest and any "proceeds" (as such term is defined in the UCC) thereof, and Seller does hereby authorize Buyer, from and after the Closing, to file such financing statements (and continuation statements with respect to such financing statements when applicable) as are necessary to perfect such security interest.

ARTICLE 3

CLOSING

Section 3.1 Closings; Payment of Purchase Price.

(a) Closing. The purchase and sale of the Purchased Interest shall take place on the Effective Date, subject to the conditions set forth in Article 5 being satisfied, or at such other place, time and date as the parties hereto may mutually agree (the "Closing"). At the Closing, Buyer shall deliver (or cause to be delivered) payment of the Purchase Price to Seller or

its designee by wire transfer of immediately available funds to one or more accounts specified by Seller on Exhibit A, without any deduction or withholding on account of any Taxes.

Section 3.2 Closing Certificates.

(a) Seller's Closing Certificate. At the Closing, Seller shall deliver to Buyer a certificate of the Secretary of Seller, dated as of the Closing Date, certifying (i) as to the incumbency of the officer of Seller executing this Agreement, (ii) as to the attached copies of Seller's certificate of incorporation, bylaws and resolutions adopted by Seller's board of directors authorizing (A) the execution and delivery by Seller of the Intercompany Sale Documents and the consummation by Seller of the transactions contemplated thereby, and (B) the execution and delivery by Seller of this Agreement and the consummation by Seller of the transactions contemplated hereby and (iii) that the conditions set forth in Section 5.1(a), Section 5.1(b) and Section 5.1(c) have been satisfied (the "Seller Closing Certificate").

(b) Ultragenyx's Closing Certificate. At the Closing, Ultragenyx shall deliver to Buyer a certificate of the Secretary of Ultragenyx, dated as of the Closing Date, certifying (i) as to the incumbency of the officer of Ultragenyx executing this Agreement, (ii) as to the attached copies of Ultragenyx's certificate of incorporation, bylaws and resolutions adopted by Ultragenyx's board of directors authorizing (A) the execution and delivery by Ultragenyx of the Intercompany Sale Documents and the consummation by Ultragenyx of the transactions contemplated thereby, and (B) the execution and delivery by Ultragenyx of this Agreement and the consummation by Ultragenyx of the transactions contemplated hereby and (iii) that the conditions set forth in Section 5.1(a), Section 5.1(b) and Section 5.1(c) have been satisfied (the "Ultragenyx Closing Certificate").

(c) Buyer's Closing Certificate. At the Closing, Buyer shall deliver to Seller and to Ultragenyx a certificate of an authorized officer thereof, certifying (i) as to the incumbency of the officer of Buyer executing this Agreement and (ii) that the conditions set forth in Section 5.2(a) and Section 5.2(b) have been satisfied (the "Buyer Closing Certificate").

Section 3.3 Bill of Sale. At the Closing, upon confirmation of the receipt of the Purchase Price, Seller shall deliver to Buyer a duly executed bill of sale evidencing the sale, transfer, assignment and conveyance of the Purchased Interest, substantially in the form attached hereto as Exhibit B (the "Bill of Sale").

Section 3.4 [Reserved].

Section 3.5 Legal Opinion. At the Closing, Gibson, Dunn & Crutcher LLP, as counsel to Ultragenyx and Seller, shall deliver to Buyer a duly executed legal opinion in the form previously agreed by the parties hereto (the "Opinion").

Section 3.6 Form W-9. At the Closing, each of Ultragenyx and Seller shall deliver to Buyer a valid, properly executed IRS Form W-9 certifying that it is exempt from U.S. federal withholding tax and "backup" withholding tax.

Section 3.7 Form W-8BEN-E. At the Closing, Buyer shall deliver to Seller and Ultragenyx a valid, properly executed IRS Form W-8BEN-E certifying that Buyer is exempt from U.S. federal withholding tax with respect to any and all payments in respect of the Purchased Interest.

Section 3.8 Data Room. Ultragenyx shall instruct Cowen and Company to deliver to Buyer, within [***] Business Days following the Closing, an electronic copy of all of the information and documents posted to the virtual data room established by Cowen and Company as of the Effective Date in connection with the transactions contemplated hereby and made available to Buyer.

ARTICLE 4

REPRESENTATIONS AND WARRANTIES

Section 4.1 Seller's and Ultragenyx's Representations and Warranties. Except as set forth in the Disclosure Schedule, Seller and Ultragenyx jointly and severally represent and warrant to Buyer that as of the Effective Date:

(a) Existence; Good Standing. Each of Seller and Ultragenyx is a corporation duly incorporated, validly existing and in good standing under the laws of Delaware. Seller is a wholly owned subsidiary of Ultragenyx. Each of Seller and Ultragenyx is duly licensed or qualified to do business and is in corporate good standing in each jurisdiction in which the nature of the business conducted by it or the character or location of the properties and assets owned, leased or operated by it makes such licensing or qualification necessary, except where the failure to be so licensed or qualified and in corporate good standing has not and would not reasonably be expected to have, either individually or in the aggregate, a Material Adverse Effect.

(c) Authorization. Each of Seller and Ultragenyx has all requisite corporate power and authority to execute, deliver and perform its obligations under the Intercompany Sale Documents and this Agreement. The execution, delivery and performance of the Intercompany Sale Documents and this Agreement, and the consummation of the transactions contemplated thereby and hereby, have been duly authorized by all necessary corporate action on the part of Seller and Ultragenyx.

(d) Enforceability. The Intercompany Sale Documents and this Agreement have been duly executed and delivered and constitute a valid and binding obligation of each of Seller and Ultragenyx, enforceable against each of Seller and Ultragenyx in accordance with their respective terms, except as such enforceability may be limited by applicable bankruptcy, securities, insolvency, or similar laws relating to, or affecting generally the enforcement of, creditors' rights and remedies, or indemnification or by other equitable principles of general application.

(e) No Conflicts. The execution, delivery and performance by each of Seller and Ultragenyx of the Intercompany Sale Documents and this Agreement and the consummation of the transactions contemplated thereby and hereby do not and shall not (i) contravene or conflict with the organizational documents of Seller or Ultragenyx, (ii) contravene or conflict with or constitute a material default under any Applicable Law or Judgment binding upon or applicable to Seller or Ultragenyx, (iii) contravene or conflict with or constitute a default under the License Agreement or (iv) contravene or conflict with or constitute a material default under any other material contract or material agreement binding upon or applicable to Seller or Ultragenyx including the Royalty Purchase Agreement dated as of December 17, 2019 between Ultragenyx and RPI Finance Trust.

(f) Consents. Except for the Licensee Consent, a true, correct and complete copy of which is attached hereto as Exhibit C, and the consents that have been obtained on or prior to the Closing or filings required by the federal securities laws or stock exchange rules, no consent, approval, license, order, authorization, registration, declaration or filing with or of any Governmental Entity or other Person is required to be done or obtained by Seller or Ultragenyx in connection with (i) the execution and delivery by each of Seller and Ultragenyx of the Intercompany Sale Documents or this Agreement, (ii) the performance by Seller and Ultragenyx of its obligations under the Intercompany Sale Documents or this Agreement or (iii) the consummation by Seller and Ultragenyx of any of the transactions contemplated by the Intercompany Sale Documents or this Agreement.

(g) No Litigation.

(i) There is no action, suit, investigation or proceeding pending before any Governmental Entity or, to the Knowledge of Seller and to the Knowledge of Ultragenyx, threatened to which Seller is a party that, individually or in the aggregate would, if determined adversely, reasonably be expected to have a Material Adverse Effect.

(ii) There is no action, suit, investigation or proceeding pending before any Governmental Entity or, to the Knowledge of Ultragenyx, threatened to which Ultragenyx is a party that, individually or in the aggregate would, if determined adversely, reasonably be expected to have a Material Adverse Effect.

(h) Compliance with Applicable Laws.

(i) Seller is not in violation of, and to the Knowledge of Seller and to the Knowledge of Ultragenyx, Seller is not under investigation with respect to nor has Seller been threatened to be charged with or given notice of any violation of, any Applicable Law or Judgment applicable to Seller, which violation would reasonably be expected to have a Material Adverse Effect.

(ii) Ultragenyx is not in violation of, and to the Knowledge of Ultragenyx, Ultragenyx is not under investigation with respect to nor has Ultragenyx been threatened to be charged with or given notice of any violation of, any Applicable Law or Judgment applicable to Ultragenyx, which violation would reasonably be expected to have a Material Adverse Effect.

(i) No Undisclosed Events or Circumstances.

(i) Except for the transactions contemplated hereby, no event or circumstance has occurred or exists with respect to Seller, its Affiliates, or their respective businesses, properties, operations or financial condition, which, under Applicable Law, requires public disclosure or announcement by Seller but which has not been so publicly announced or disclosed and which, individually or in the aggregate, would constitute a Material Adverse Effect. There is no action, suit, claim, investigation or proceeding pending or, to the Knowledge of Seller and to the Knowledge of Ultragenyx, threatened against Seller or any of its Affiliates which questions the validity of the Intercompany Sale Documents or this Agreement or the transactions contemplated thereby or hereby or any action taken or to be taken pursuant thereto or hereto. There is no action, suit, claim, investigation or proceeding pending or, to the Knowledge of Seller and to the Knowledge of Ultragenyx, threatened, against or involving Seller or any of its Affiliates, or any of their respective properties or assets that would be reasonably be expected to result in a Material Adverse Effect.

(ii) Except for the transactions contemplated hereby, no event or circumstance has occurred or exists with respect to Ultragenyx, its Affiliates, or their respective businesses, properties, operations or financial condition, which, under Applicable Law, requires public disclosure or announcement by Ultragenyx but which has not been so publicly announced or disclosed and which, individually or in the aggregate, would constitute a Material Adverse Effect. There is no action, suit, claim, investigation or proceeding pending or, to the Knowledge of Ultragenyx, threatened against Ultragenyx or any of its Affiliates which questions the validity of the Intercompany Sale Documents or this Agreement or the transactions contemplated thereby or hereby or any action taken or to be taken pursuant thereto or hereto. There is no action, suit, claim, investigation or proceeding pending or, to the Knowledge of Ultragenyx, threatened, against or involving Ultragenyx or any of its Affiliates, or any of their respective properties or assets that would be reasonably be expected to result in a Material Adverse Effect.

(j) License Agreement; Intercompany Sale Documents. Attached hereto as Exhibits D-1, D-2 and D-3 are true, correct and complete copies of, respectively, the License Agreement, the Pharmacovigilance Agreement and the Intercompany Sale Documents. Seller has delivered to Buyer true, correct and complete copies of (A) all material communications

between Ultragenyx and Licensee since April 2018 relating, directly or indirectly, to the Royalty or to the Licensed Products in the Profit Share Territory, and (B) all minutes from and meeting materials of the JSC (as such term is defined in the License Agreement) since April 2018, related, directly or indirectly, to the Royalty or the Licensed Products in the Profit Share Territory, and redacted to reflect solely such information.

(i) No Other Agreements. Except as set forth on Schedule 4.1(j)(i)(A) of the Disclosure Schedule, the License Agreement, the Pharmacovigilance Agreement and the Quality Technical Agreement are the only agreements, instruments, arrangements, waivers or understandings (collectively, "Contracts") between Ultragenyx (or any predecessor or Affiliate thereof), on the one hand, and Licensee (or any predecessor or Affiliate thereof), on the other hand, relating to the subject matter thereof, and there are no other Contracts between Ultragenyx (or any predecessor or any Affiliate thereof), on the one hand, and Licensee (or any predecessor or Affiliate thereof), on the other hand, that relate, directly or indirectly, to the License Agreement, the Licensed IP, the Licensed Products (including the development or commercialization thereof), or the Royalty in the Profit Share Territory. Except as set forth on Schedule 4.1(j)(i)(B) of the Disclosure Schedule, Ultragenyx has not proposed or received any proposal, to amend or waive any provision of (1) the License Agreement since December 17, 2021, or (2) the Pharmacovigilance Agreement in each case of clause (1) and (2) in any manner that (x) would result in a breach of this Agreement or (y) would otherwise reasonably be expected (with or without the giving of notice or the passage of time, or both) to have a Material Adverse Effect. Ultragenyx and Seller have not agreed to amend or waive any provision of the Intercompany Sale Documents.

(ii) Licenses/Sublicenses. Except as set forth on Schedule 4.1(j)(ii) of the Disclosure Schedule, to the Knowledge of Ultragenyx, there are no licenses or sublicenses entered into by Licensee or any other Person (or any predecessor or Affiliate thereof) in respect of Licensee's rights and obligations under the License Agreement (including any Licensed IP) related, directly or indirectly, to the Profit Share Territory. Ultragenyx has not received any notice from Licensee pursuant to Section 13.1.1 of the License Agreement.

(iii) Validity and Enforceability of License Agreement and Intercompany Sale Documents. The License Agreement and the Intercompany Sale Documents are legal, valid, binding, enforceable, and in full force and effect. The License Agreement and the Intercompany Sale Documents will continue to be legal, valid, binding, enforceable, and in full force and effect on identical terms except, with respect to the License Agreement, for such terms modified as expressly set forth in the Licensee Consent and the Licensee Instruction Letter, immediately following the consummation of the transactions contemplated by this Agreement. No party to the License Agreement or the Intercompany Sale

Documents is in material, substantial and ongoing breach thereof, there is no ongoing breach under the License Agreement that would reasonably be expected (with or without the giving of notice or the passage of time, or both) to have a Material Adverse Effect, and no event has occurred that with notice or lapse of time would constitute a material breach of the License Agreement or a breach that would reasonably be expected (with or without the giving of notice or the passage of time, or both) to have a Material Adverse Effect, or permit termination, modification, or acceleration, under the License Agreement or the Intercompany Sale Documents. No party to the License Agreement or the Intercompany Sale Documents has repudiated any provision of the License Agreement or the Intercompany Sale Documents and Ultragenyx has not received any notice in connection with the License Agreement challenging the validity, enforceability or interpretation of any provision of such agreement, including the obligation to pay any portion of the Royalty without set-off of any kind. Neither Ultragenyx nor Seller has received any notice challenging the validity, enforceability or interpretation of any provision of the Intercompany Sale Documents.

(iv) Licensed Product. Burosumab is a Drug Substance and is the active ingredient in the Licensed Products. Licensee and its Affiliates are required to pay royalties under Section 7.2.2 of the License Agreement on all Net Sales by or on behalf of them and any of their (sub)licensees of any Licensed Products in the Field in the Profit Share Territory. As a result of the transactions under the Intercompany Sale Documents, as between Seller and Ultragenyx, Seller has the exclusive right to receive the Royalty on Net Sales of the Licensed Products in the Field in the Profit Share Territory for so long as Licensee, one of its Affiliates or any of its or their (sub)licensees is selling the Licensed Products in any country in the Profit Share Territory.

(v) No Liens or Assignments by Seller or Ultragenyx. Each of Seller and Ultragenyx has not, except for Permitted Liens and as contemplated hereby, conveyed, assigned or in any other way transferred or granted any Liens upon or security interests with respect to all or any portion of its right, title and interest in and to the Royalty, Ultragenyx's interest in the Joint Invention Patents, the License Agreement or the Intercompany Sale Documents to any other Person.

(vi) No Waivers or Releases. Ultragenyx has not granted any material waiver under the License Agreement with respect to the Royalty or the Profit Share Territory and has not released Licensee, in whole or in part, from any of its material obligations with respect to the Royalty or the Profit Share Territory under the License Agreement, and no waiver granted by Ultragenyx under the License Agreement with respect to the Royalty or the Profit Share Territory and no release of Licensee thereunder would reasonably be expected

(with or without the giving of notice or the passage of time, or both) to have a Material Adverse Effect.

(vii) No Termination. Ultragenyx has not (A) given Licensee any notice of termination of the License Agreement (whether in whole or in part) or any notice expressing any intention to terminate the License Agreement or (B) received any notice of termination of the License Agreement (whether in whole or in part) or any notice expressing any intention to terminate either the License Agreement. To the Knowledge of Ultragenyx, no event has occurred that would give rise to the expiration or termination of the License Agreement. Neither Ultragenyx nor Seller has terminated or has any intention of terminating the Intercompany Sale Documents in whole or in part.

(viii) No Breaches or Defaults. There is and has been no material breach or default under any provision of the License Agreement either by Ultragenyx (or any predecessor thereof) or, to the Knowledge of Ultragenyx, by Licensee (or any predecessor thereof), and there is no event that upon notice or the passage of time, or both, would reasonably be expected to give rise to any material breach or default either by Ultragenyx or, to the Knowledge of Ultragenyx, by Licensee.

(ix) Payments Made. Ultragenyx has received from Licensee (including from one or more Affiliates of Licensee in accordance with Section 13.6 of the License Agreement) the full amount of the payments due and payable under the License Agreement.

(x) No Assignments by Licensee. Ultragenyx has not consented to any assignment, delegation or other transfer by Licensee or any of its predecessors of any of their rights or obligations under the License Agreement with respect to the Profit Share Territory, and, to the Knowledge of Ultragenyx, Licensee has not assigned or otherwise transferred or granted any Liens upon or, with respect to any of its rights or obligations under the License Agreement with respect to the Profit Share Territory or, to the Knowledge of Ultragenyx, any portion of its right, title and interest in and to the Licensed KHK IP with respect to the Profit Share Territory, in each case, to any Person.

(xi) No Indemnification Claims. Ultragenyx has not notified Licensee or any other Person of any claims for indemnification under the License Agreement nor has Ultragenyx received any claims for indemnification under the License Agreement pursuant to Article 14 thereof.

(xii) No Royalty Reductions. To the Knowledge of Ultragenyx, the amount of the Royalty that will be due and payable under Section 7.2.2 of the License Agreement is not subject to any claim by Licensee alleging a right of set-off, counterclaim, credit, reduction or deduction by contract or otherwise

against such Royalty, including in respect of any royalties payable by Ultragenyx to Licensee pursuant to Section 7.2.3 of the License Agreement (each, a "Royalty Reduction"). To the Knowledge of Ultragenyx, no event or condition exists that, upon notice or passage of time or both, would reasonably be expected to permit Licensee to claim, or have the right to claim, a Royalty Reduction.

(xiii) No Notice of Infringement. Ultragenyx has not received any written notice from, or given any written notice to, Licensee pursuant to Sections 10.5.1 or 10.6.1 of the License Agreement.

(xiv) Audits. Ultragenyx has not initiated, pursuant to Section 9.2 of the License Agreement any inspection or audit of books of accounts or other records pertaining to Net Sales, the calculation of royalties or other amounts payable to Ultragenyx under the License Agreement.

(xv) In-Licenses. To the Knowledge of Ultragenyx, and with respect to each agreement constituting an In-License, (A) such agreement is valid and in full force and effect, and binding and enforceable on Licensee and its counterparty, (B) Licensee has not given the counterparty to any such agreement any notice of termination or any notice expressing an intention to terminate such agreement, and Licensee has not received the same from such counterparty, (C) there is no and has been no material breach under any provision of such agreement by Licensee or its counterparty, and (D) Licensee has not proposed, or received any proposal, to amend or waive any provision of such agreement in a manner that would reasonably be expected to result in a Material Adverse Effect.

(k) Title to Royalty. After giving effect to the Intercompany Sale Documents, Seller is the exclusive owner of and has good and marketable title to the Royalty free and clear of all Liens (other than Permitted Liens). Upon payment of the Purchase Price by Buyer, Buyer will acquire, subject to the terms and conditions set forth in this Agreement and the License Agreement, good and marketable title to the Purchased Interest, free and clear of all Liens (other than Permitted Liens and Liens created by Buyer). No Person other than Buyer pursuant to this Agreement has any oral or written contract, or any option, right or privilege of any nature capable of becoming a contract, for the purchase or acquisition from Seller of any of the Royalty.

(l) Intellectual Property.

(i) Schedule 4.1(l)(i) of the Disclosure Schedule lists all non-expired Joint Invention Patents and, to the Knowledge of Ultragenyx, all non-expired KHK Patent Rights (collectively, the "Licensed Patents"). To the Knowledge of Ultragenyx, Licensee is the sole owner of, or is in possession of a valid license to, all of the KHK Patent Rights. To the Knowledge of Ultragenyx, Ultragenyx and Licensee collectively are the sole owners of, and collectively have the sole

interest in, the Joint Invention Patents. Ultragenyx is the sole owner of, and has sole interest in, its undivided half interest in each of the Joint Invention Patents. Schedule 4.1(l)(i) of the Disclosure Schedule specifies as to each of the Licensed Patents, as applicable, the jurisdictions by or in which each such patent has issued as a patent or such patent application has been filed, including the respective patent numbers and application numbers and issue and filing dates, and, to the Knowledge of Ultragenyx, the owner of such Licensed Patent. Neither Ultragenyx nor any of its Affiliates owns any Patent Rights that are UGNX Invention Patents, and, to the Knowledge of Ultragenyx, neither KHK nor any of its Affiliates owns any Patent Rights that are KHK Invention Patents.

(ii) Except as set forth in Schedule 4.1(l)(ii) of the Disclosure Schedule: (A) (1) there is no pending or, to the Knowledge of Ultragenyx, threatened opposition, interference, reexamination, *inter partes* review, post-grant review, injunction, claim, suit, action, citation, summons, subpoena, complaint, arbitration, mediation, demand, decree or other dispute, disagreement, proceeding or claim to which Ultragenyx is a party or, to the Knowledge of Ultragenyx, to which Licensee is a party, and (2) to the Knowledge of Ultragenyx, there is no pending or threatened hearing, inquiry or investigation (by the International Trade Commission or any other Governmental Entity), (collectively (1) and (2), "Disputes") involving any Joint Invention Patent; (B) to the Knowledge of Ultragenyx, the Joint Invention Patents are not subject to any outstanding injunction, judgment, order, decree, ruling, change, settlement or other disposition of a Dispute; and (C) to the Knowledge of Ultragenyx, no court, tribunal, or government agency in any jurisdiction has found any Joint Invention Patent invalid or unenforceable. Except as set forth in Schedule 4.1(l)(ii) of the Disclosure Schedule, to the Knowledge of Ultragenyx: (1) there are no pending or threatened Disputes involving any KHK Patent Right; (2) the KHK Patent Rights are not subject to any outstanding injunction, judgment, order, decree, ruling, change, settlement or other disposition of a Dispute; (3) no court, tribunal, or government agency in any jurisdiction has found any KHK Patent Right invalid or unenforceable.

(iii) Except as set forth in Schedule 4.1(l)(iii), subsequent to the issuance of the Licensed Patents, Ultragenyx has not, and to the Knowledge of Ultragenyx, Licensee has not, filed any disclaimer or made or permitted any other voluntary reduction in the term or subject matter claimed in any of the Licensed Patents. All of the issued Joint Invention Patents are in full force and effect and have not lapsed, expired or otherwise terminated, and, to the Knowledge of Ultragenyx, are valid and enforceable. Ultragenyx has not, and, to the Knowledge of Ultragenyx, Licensee has not, (A) received any written notice relating to the lapse, expiration or other termination of any of the Joint Invention Patents or (B) any written legal opinion that alleges that any of the issued Joint Invention Patents is invalid or unenforceable. To the Knowledge of

Ultragenyx, all of the issued KHK Patent Rights identified in Schedule 4.1(l)(i) are in full force and effect and have not lapsed, expired or otherwise terminated and are valid and enforceable. To the Knowledge of Ultragenyx, Licensee has not received any written notice relating to the lapse, expiration or other termination of any of the issued KHK Patent Rights identified in Schedule 4.1(l)(i) or any written legal opinion that alleges that any of the issued KHK Patent Rights is invalid or unenforceable.

(iv) To the Knowledge of Ultragenyx, there is no Person who is or claims to be an inventor under any of the Joint Invention Patents who is not a named inventor thereof.

(v) Ultragenyx has not, and, to the Knowledge of Ultragenyx, Licensee has not, received any written notice of any claim by any Person challenging the inventorship or ownership of, the rights of Ultragenyx or Licensee, as applicable, in and to, or the patentability, validity or enforceability of, any Licensed Patent, or asserting that the development, manufacture, importation, sale, offer for sale or use of any Licensed Product infringes any patent or other intellectual property rights of such Person.

(vi) To the Knowledge of Ultragenyx, the discovery and development of the Licensed Products did not and does not infringe, misappropriate or otherwise violate any patent rights or other intellectual property rights owned by any Third Party. Neither Ultragenyx nor, to the Knowledge of Ultragenyx, Licensee, has, except pursuant to the In-Licenses, and except for two patent families added to the KHK Patent Rights in Amendment No. 3, in-licensed any patents or other intellectual property rights covering the manufacture, use, sale, offer for sale or import of the Licensed Products.

(vii) To the Knowledge of Ultragenyx, the manufacture, use, marketing, sale, offer for sale, importation or distribution of the Licensed Products has not and will not, infringe, misappropriate or otherwise violate any patent rights or other intellectual property rights owned by any other Person.

(viii) To the Knowledge of Ultragenyx, no Third Party has infringed, misappropriated or otherwise violated, or is infringing, misappropriating or otherwise violating, any of the Licensed Patents.

(ix) All required maintenance fees, annuities and similar payments with respect to the Licensed Patents for which Ultragenyx controls the prosecution and maintenance in accordance with Section 10.1.1 or 10.2.1 of the License Agreement, and to the Knowledge of Ultragenyx, with respect to all other Licensed Patents, have been paid timely.

(m) UCC Representation and Warranties. Ultragenyx's exact legal name is, and for the immediately preceding ten years Ultragenyx's exact legal name has been, "Ultragenyx Pharmaceutical Inc.". Ultragenyx is, and for the prior ten years Ultragenyx has been, incorporated in Delaware. Seller's exact legal name is, and since its date of incorporation, Seller's exact legal name has been, "Rare Delaware Inc.". Seller is, and since its incorporation has been, incorporated in Delaware.

(n) Solvency. Following consummation of the transactions contemplated by the Intercompany Sale Documents and assuming consummation of the transactions contemplated by this Agreement, (i) the fair saleable value of the assets of each of Ultragenyx and Seller will be greater than the sum of its debts, liabilities and other obligations, including contingent liabilities, (ii) the present fair saleable value of the assets of each of Ultragenyx and Seller will be greater than the amount that would be required to pay its probable liabilities on its existing debts, liabilities and other obligations, including contingent liabilities, as they become absolute and matured, (iii) each of Seller and Ultragenyx will be able to pay its debts, liabilities and other obligations, including contingent obligations, as they come due in the ordinary course, (iv) neither Ultragenyx nor Seller has incurred, will incur or has any present plans or intentions to incur debts or other obligations or liabilities beyond its ability to pay such debts or other obligations or liabilities as they become due and payable, and (v) neither Ultragenyx nor Seller will become bankrupt or will be rendered insolvent under applicable Bankruptcy Laws.

(o) Brokers' Fees. Other than Cowen and Company, there is no investment banker, broker, finder, financial advisor or other intermediary who has been retained by or is authorized to act on behalf of Seller or Ultragenyx who might be entitled to any fee or commission in connection with the transactions contemplated by this Agreement.

Section 4.2 Buyer's Representations and Warranties. Buyer represents and warrants to Seller and Ultragenyx that as of the Effective Date:

(a) Existence; Good Standing. Buyer is a limited partnership formed and existing under the laws of the Province of Ontario, Canada. Buyer GP is a corporation duly incorporated, validly existing and in good standing under the laws of the Province of Ontario, Canada, and is the sole general partner of Buyer.

(b) Authorization. Buyer has the requisite partnership right, power and authority to execute, deliver and perform its obligations under this Agreement. The execution, delivery and performance of this Agreement, and the consummation of the transactions contemplated hereby, have been duly authorized by all necessary action on the part of Buyer GP in its capacity as general partner of Buyer.

(c) Enforceability. This Agreement has been duly executed and delivered by Buyer GP in its capacity as general partner of Buyer and constitutes the valid and binding obligation of Buyer, enforceable against Buyer in accordance with its terms, except as may be

limited by applicable Bankruptcy Laws or by general principles of equity (whether considered in a proceeding in equity or at law).

(d) No Conflicts. The execution, delivery and performance by Buyer GP in its capacity as general partner of Buyer of this Agreement do not and shall not (i) contravene or conflict with the organizational documents of Buyer or Buyer GP, (ii) contravene or conflict with or constitute a default under any material provision of any Applicable Law binding upon or applicable to Buyer or Buyer GP or (iii) contravene or conflict with or constitute a default under any material contract or other material agreement or Judgment binding upon or applicable to Buyer.

(e) Consents. No consent, approval, license, order, authorization, registration, declaration or filing with or of any Governmental Entity or other Person is required to be done or obtained by Buyer in connection with (i) the execution and delivery by Buyer GP in its capacity as general partner of Buyer of this Agreement, (ii) the performance by Buyer of its obligations under this Agreement, other than the filing of financing statement(s) in accordance with Section 2.3, or (iii) the consummation by Buyer of any of the transactions contemplated by this Agreement.

(f) No Litigation. There is no action, suit, investigation or proceeding pending or, to the knowledge of Buyer, threatened before any Governmental Entity to which Buyer is a party that would, if determined adversely, reasonably be expected to prevent or materially and adversely affect the ability of Buyer to perform its obligations under this Agreement.

(g) Financing. Buyer will have sufficient cash on hand to pay the entire Purchase Price at the Closing. Buyer acknowledges that its obligations under this Agreement are not contingent on obtaining financing.

(h) Brokers' Fees. There is no investment banker, broker, finder, financial advisor or other intermediary who has been retained by or is authorized to act on behalf of Buyer who might be entitled to any fee or commission in connection with the transactions contemplated by this Agreement.

Section 4.3 No Implied Representations and Warranties. EXCEPT AS EXPRESSLY SET FORTH IN SECTION 4.1, NEITHER SELLER NOR ULTRAGENYX MAKES ANY REPRESENTATION OR WARRANTY, EXPRESSED OR IMPLIED, AT LAW OR IN EQUITY, INCLUDING WITH RESPECT TO MERCHANTABILITY OR FITNESS FOR ANY PARTICULAR PURPOSE, AND ANY SUCH REPRESENTATIONS OR WARRANTIES ARE HEREBY EXPRESSLY DISCLAIMED. BUYER ACKNOWLEDGES THAT, EXCEPT AS SPECIFICALLY PROVIDED IN THIS ARTICLE 4 AND THE DISCLOSURE SCHEDULES, NEITHER SELLER NOR ULTRAGENYX HAS ASSUMED ANY RESPONSIBILITIES OF ANY KIND WITH RESPECT TO ANY ACT OR OMISSION OF LICENSEE WITH RESPECT TO THE DESIGN, DEVELOPMENT, MANUFACTURE, USE, SALE, DISTRIBUTION, MARKETING OR OTHER ACTIVITIES OF LICENSEE WITH RESPECT TO ANY OF THE LICENSED PRODUCTS.

ARTICLE 5

CONDITIONS TO CLOSING

Section 5.1 Conditions to Buyer's Obligations. The obligations of Buyer to consummate the transactions contemplated hereunder on the Closing Date are subject to the satisfaction or waiver, at or prior to the Closing Date, of each of the following conditions precedent.

(a) Each of Seller and Ultragenyx shall have performed and complied in all material respects with all, and shall not be in material breach of any, agreements, covenants, obligations and conditions required to be performed and complied by it under this Agreement at or prior to the Closing Date.

(b) The representations and warranties of each of Seller and Ultragenyx contained in Article 4 shall be true and correct in all material respects as of the Closing Date as though made at and as of the Closing Date, except to the extent any such representation or warranty expressly speaks as of a particular date, in which case it shall be true and correct in all material respects as of such date; provided, that to the extent that any such representation or warranty is qualified by the term "material" or "Material Adverse Effect," such representation or warranty (as so written, including the term "material" or "Material Adverse Effect") shall be true and correct in all respects as of the Closing Date or such other date, as applicable.

(c) After the date of this Agreement, there shall not have occurred any fact, circumstance, effect, change, event or development that, individually or in the aggregate, has resulted, or would reasonably be likely to result, in a Material Adverse Effect.

(d) There shall not have been issued and be in effect any Judgment of any Governmental Entity enjoining, preventing or restricting the consummation of the transactions contemplated by this Agreement.

(e) There shall not have been instituted or be pending any action or proceeding by any Governmental Entity or any other Person (i) challenging or seeking to make illegal, to delay materially or otherwise directly or indirectly to restrain or prohibit the consummation of the transactions contemplated hereby, (ii) seeking to obtain material damages in connection with the transactions contemplated hereby or (iii) seeking to restrain or prohibit Buyer's receipt of the Purchased Interest.

(f) Seller shall have delivered to Buyer the duly executed Bill of Sale.

(g) [Reserved]

(h) Seller shall have delivered to Buyer the duly executed Opinion.

(i) Seller shall have delivered to Buyer the duly executed Seller Closing Certificate.

(j) Ultragenyx shall have delivered to Buyer the duly executed Ultragenyx Closing Certificate.

Section 5.2 Conditions to Seller's Obligations. The obligations of Seller and Ultragenyx to consummate the transactions contemplated hereunder on the Closing Date are subject to the satisfaction or waiver, at or prior to the Closing Date, of each of the following conditions precedent:

(a) Buyer shall have performed and complied in all material respects with all, and shall not be in material breach of any, agreements, covenants, obligations and conditions required to be performed and complied by it under this Agreement at or prior to the Closing Date.

(b) The representations and warranties of Buyer contained in Section 4.2 shall be true and correct in all material respects as of the Closing Date as though made at and as of the Closing Date, except to the extent any such representation or warranty expressly speaks as of a particular date, in which case it shall be true and correct in all material respects as of such date; provided, that to the extent that any such representation or warranty is qualified by the term "material," such representation or warranty (as so written, including the term "material") shall be true and correct in all respects as of the Closing Date or such other date, as applicable.

(c) There shall not have been issued and be in effect any Judgment of any Governmental Entity enjoining, preventing or restricting the consummation of the transactions contemplated by this Agreement.

(d) There shall not have been instituted or be pending any action or proceeding by any Governmental Entity or any other Person (i) challenging or seeking to make illegal, to delay materially or otherwise directly or indirectly to restrain or prohibit the consummation of the transactions contemplated hereby, (ii) seeking to obtain material damages in connection with the transactions contemplated hereby or (iii) seeking to restrain or prohibit Buyer's receipt of the Purchased Interest.

(e) Buyer shall have delivered to Seller and Ultragenyx the duly executed Buyer Closing Certificate.

ARTICLE 6

COVENANTS

Ultragenyx and Seller covenant in favor of Buyer, on a joint and several basis, as follows:

Section 6.1 Disclosures. Except for a press release previously approved in form and substance by Seller, Ultragenyx and Buyer or any other public announcement using substantially the same text as such press release, neither Buyer, Seller nor Ultragenyx shall, and each party hereto shall cause its respective Representatives, Affiliates and Affiliates' Representatives not to, issue a press release or other public announcement or otherwise make any public disclosure with respect to this Agreement or the subject matter hereof without the prior written consent of the other party hereto (which consent shall not be unreasonably withheld, conditioned or delayed), except as may be required by Applicable Law or stock exchange rule (in which case the party hereto required to make the press release or other public announcement or disclosure shall allow the other party hereto reasonable time to comment on such press release or other public announcement or disclosure in advance of such issuance and shall consider such comments in good faith).

Section 6.2 Payments of Purchased Interest.

(a) Within [***] days following the Closing Date, Seller shall establish a bank account with Ultragenyx's primary bank, the sole owner and beneficiary of which account shall be Seller (the "Seller Bank Account"). Seller agrees that the sole purpose of the Seller Bank Account will be to receive deposits of the Royalty from the Licensee. Until the Royalty Termination Date, (i) Seller shall maintain and retain exclusive control of the Seller Bank Account and shall not grant to any Person any Lien thereon or in respect of funds held on deposit therein, and (ii) Seller shall use commercially reasonable efforts to promptly (x) notify Buyer of each receipt of any payment on account of Royalties from the Licensee following such receipt, and (y) provide to Buyer copies of account statements issued by Seller's bank in respect of the Seller Bank Account on a periodic basis and, in any event, not less than quarterly.

(b) Within [***] Business Days following the establishment of the Seller Bank Account, Ultragenyx shall deliver the Licensee Instruction Letter, substantially in the form attached hereto as Exhibit E, with the Seller Bank Account information reflected therein at time of such delivery, to the Licensee to provide notice of the sale, assignment, transfer and conveyance of the Royalty to Seller and directing the Licensee to pay the Royalty to the Seller Bank Account. Promptly following the delivery of the Licensee Instruction Letter to the Licensee, Ultragenyx shall provide evidence thereof to Buyer. Ultragenyx and Seller shall not revoke, amend or otherwise change the Licensee Instruction Letter in any respect prior to the Royalty Termination Date without the prior written consent of Buyer.

(c) Until the Royalty Termination Date, upon receipt of payment on account of the Royalties due to Buyer hereunder, Seller shall promptly (and in any event within [***] Business Days) pay the Purchased Interest to Buyer, without set-off or deduction of any kind by Seller except as expressly contemplated in Section 6.17, by wire transfer of immediately available funds to the account specified in Exhibit F or to such other account as Buyer may specify by written notice to Seller and Ultragenyx from time to time (the "Buyer Bank Account"). The parties agree that upon receipt of any payment on account of Royalties, the Purchased Interest shall immediately become payable and from and after such time, Seller shall

(i) hold the funds comprising such payment in trust for the exclusive benefit of Buyer until the Purchased Interest is paid to Buyer, and (ii) have no right, title or interest in such payment and it shall not grant any Lien in respect thereof. For the avoidance of doubt, any payments by the Licensee to Seller in error or that constitute adjustments to reserves shall be excluded from this Section 6.2(c).

(d) Commencing on the Closing Date and until the Royalty Termination Date, if, notwithstanding the terms of the Licensee Instruction Letter, any payment of any portion of the Royalty is made to Ultragenyx, Ultragenyx shall pay the corresponding Purchased Interest, without set-off or deduction of any kind by Ultragenyx except as expressly contemplated in Section 6.17, to Buyer on behalf of Seller, promptly (and in any event within [***] Business Days) after the receipt thereof, by wire transfer of immediately available funds to the Buyer Bank Account. Ultragenyx shall notify Buyer of such wire transfer and provide reasonable details regarding the Royalty payment so received by Ultragenyx. Ultragenyx agrees that, in the event any payment of the Royalty is paid to Ultragenyx, Ultragenyx shall (i) until the Purchased Interest is paid to Buyer on behalf of Seller, hold such payment received in trust for the exclusive benefit of Seller to enable Seller to pay the Purchased Interest to Buyer in accordance with this Agreement and (ii) have no right, title or interest in such payment and that it shall not grant any Lien in respect thereof.

(e) Commencing on the Closing Date and at all times thereafter if, notwithstanding the terms of the Licensee Instruction Letter, any payment due under the License Agreement that does not constitute Royalties is made to Seller or Buyer, Seller or Buyer, as applicable, shall pay such amount, without set-off or deduction of any kind by Seller or Buyer, as applicable, to Ultragenyx, promptly (and in any event within [***] Business Days) after the receipt thereof, by wire transfer of immediately available funds to an account designated in writing by Ultragenyx. Seller or Buyer, as applicable, shall notify Ultragenyx of such wire transfer and provide reasonable details regarding the erroneous payment so received by Seller or Buyer. Each of Seller and Buyer (in each case, in respect of itself only) agrees that, in the event any payment due under the License Agreement that does not constitute Royalties is paid to Seller or Buyer, Seller or Buyer, as applicable, shall (i) until paid to Ultragenyx, hold such payment received in trust for the exclusive benefit of Ultragenyx and (ii) have no right, title or interest in such payment and that it shall not grant any Lien in respect thereof.

(f) A late fee of [***] shall accrue on all unpaid amounts on an annualized basis with respect to any sum payable under this Section 6.2 beginning [***] Business Days after receipt of such payment.

Section 6.3 Royalty Reduction. If Licensee exercises any Royalty Reduction against any payment of the Royalty, such Royalty Reduction shall not reduce any payment of the Purchased Interest otherwise payable to Buyer, and if such Royalty Reduction reduces any payment of the Purchased Interest to less than the full amount of the Royalty, then Seller shall promptly (and in any event within [***] Business Days following the payment of the Royalty affected by such Royalty Reduction) make a true-up payment to Buyer such that Buyer receives

the full amount of such Purchased Interest payments that would have been payable to Buyer had such Royalty Reduction not occurred.

Section 6.4 Royalty Reports; Notices and Other Information from the Licensee. Promptly (and in any event within [***] Business Days) following the receipt by Ultragenyx of any Royalty Report or other material notices or correspondence relating, directly or indirectly, to the Royalty or the Licensed Product in the Profit Share Territory that has been provided to Ultragenyx under, or in respect of, the License Agreement, Ultragenyx shall furnish a true, correct and complete copy of the same to Buyer.

Section 6.5 Notices and Other Information to the Licensee. Ultragenyx shall not send (or refrain from sending), without the prior written consent of Buyer, any material written notice or correspondence to Licensee that (a) relates, directly or indirectly, to the Royalty or the Profit Share Territory or (b) would, or relates directly or indirectly to a matter that would, reasonably be expected (with or without the giving of notice or passage of time, or both) to result in a Material Adverse Effect.

Section 6.6 Inspections and Audits of Licensee. Ultragenyx shall consult and coordinate with Buyer regarding the time, manner and conduct of any inspection or audit of Licensee under Section 9.2.1 of the License Agreement. At the written request of Buyer upon not less than [***] days' prior written notice, Ultragenyx shall, to the extent permitted under Section 9.2.1 of the License Agreement, provide written notice to Licensee to cause an inspection or audit during normal business hours not more than [***] each calendar year, and under a customary non-disclosure agreement, by an independent, mutually agreed public accounting firm to be made for the purpose of determining the correctness of Royalty payments made under the License Agreement. With respect to any inspection or audit requested by Buyer with respect to the Royalty, Ultragenyx shall, for purposes of Section 9.2.1 of the License Agreement, select such independent, mutually agreed public accounting firm as Buyer shall recommend for such purpose (as long as such independent certified public accountant is reasonably acceptable to Licensee as required by Section 9.2.1 of the License Agreement). Buyer shall pay Ultragenyx the expenses of any inspection or audit requested by Buyer (including the fees and expenses of such independent public accounting firm designated for such purpose) that would otherwise be borne by Ultragenyx pursuant to the License Agreement (if and as such expenses are actually incurred by Ultragenyx); provided, however, that as between Buyer and Ultragenyx, Ultragenyx shall be solely responsible for any of such fees and expenses that do not relate exclusively to the inspection or audit of Licensee in respect of sales of Licensed Product in the Profit Share Territory and the Royalty. Ultragenyx shall deliver to Buyer a copy of the results of any audit conducted pursuant to Section 9.2.1 of the License Agreement within [***] Business Days following Ultragenyx's receipt thereof, with information redacted that Ultragenyx reasonably determines is not relevant for determining the correctness of Royalty payments made under the License Agreement.

Section 6.7 Amendment or Assignment of License Agreement. Ultragenyx shall not, except as Mutually Agreed, assign, amend, modify, supplement or restate (or consent

to any assignment, amendment, modification, supplement or restatement of) any provision of the License Agreement; provided that, Ultragenyx may amend, modify, supplement or restate the License Agreement without the prior written consent of Buyer so long as such amendment, modification, supplement or restatement relates to the transition for the Profit Share Territory and does not adversely affect, directly or indirectly, the Royalty in any manner, including the timing, amount or duration thereof. Subject to the foregoing, promptly, and in any event within [***] Business Days, following receipt by Ultragenyx of any final assignment, amendment, modification, supplement or restatement of the License Agreement, Ultragenyx shall furnish a copy of the same to Buyer; provided, however, that Ultragenyx shall furnish to Buyer a copy of the final form of any assignment, amendment, modification, supplement or restatement of the License Agreement that relates to the transition for the Profit Share Territory at least [***] Business Days prior to the execution thereof, and Ultragenyx will cause its representatives to meet with representatives of Buyer to discuss the content thereof if requested by Buyer within such [***] Business Day period.

Section 6.8 Maintenance of License Agreement. Ultragenyx shall comply in all material respects with its obligations under the License Agreement and shall not take any action or forego any action that would reasonably be expected to constitute a material breach or default thereof. Promptly, and in any event within [***] Business Days, after receipt of any (written or oral) notice from Licensee of an alleged breach or default under the License Agreement relating, directly or indirectly, to the Royalty or to the Licensed Products in the Profit Share Territory or of other material breach by Ultragenyx under the License Agreement, Ultragenyx shall give written notice thereof to Buyer, including delivering Buyer a copy of any such written notice. After consultation with Buyer and as Mutually Agreed, Ultragenyx shall use its reasonable best efforts to cure any such breach or default by it under the License Agreement and shall give written notice to Buyer upon curing any such breach or default. In connection with any dispute regarding an alleged breach that is related, directly or indirectly, to the Royalty or could reasonably be expected (with or without the giving of notice or passage of time, or both) to have a Material Adverse Effect, Ultragenyx shall employ such counsel, reasonably acceptable to Ultragenyx, as Buyer may select. Buyer and Ultragenyx shall pay [***]% and [***]%, respectively, of the costs and expenses of such counsel in connection with any dispute regarding any such breach by the Licensee, and Ultragenyx shall pay [***]% of the costs and expenses of such counsel in connection with any dispute regarding any such breach by Ultragenyx; provided that, for greater certainty, as between Buyer and Ultragenyx, Ultragenyx shall be solely responsible for all costs and expenses of counsel in connection with any dispute regarding a breach by Licensee to the extent relating to any jurisdiction other than the Profit Share Territory. Ultragenyx shall not, except as Mutually Agreed, (a) forgive, release or compromise any amount owed to or becoming owed to Ultragenyx under the License Agreement in respect of the Royalty or (b) waive any obligation of, or grant any consent to, the Licensee under, in respect of or related, directly or indirectly, to the Royalty. Ultragenyx shall not exercise or enforce its applicable rights under the License Agreement, or omit to do so, in any manner that would reasonably be expected (with or without the giving of notice or the passage of time, or both) to have a Material Adverse Effect.

Section 6.9 Enforcement of License Agreement.

(a) Notice of Breaches by Licensee. Promptly (and in any event within [***] Business Days) after Ultragenyx becomes aware of, or comes to believe in good faith that there has been, a material breach of the License Agreement by Licensee, Ultragenyx shall provide written notice of such breach to Buyer. In addition, Ultragenyx shall provide to Buyer a copy of any written notice of such breach or alleged breach of the License Agreement delivered by Ultragenyx to Licensee as soon as practicable and in any event not less than [***] Business Days following such delivery.

(b) Enforcement of License Agreement. In the case of any material breach by Licensee referred to in Section 6.9(a), Ultragenyx shall consult with Buyer regarding the timing, manner and conduct of any enforcement of Licensee's obligations under the License Agreement. Following such consultation, Ultragenyx shall, (i) as Mutually Agreed, exercise such rights and remedies relating to any such breach as shall be available to Ultragenyx, whether under the License Agreement or by operation of law and, (ii) if such breach is solely related to the Royalty or could reasonably be expected (with or without the giving of notice or passage of time, or both) to have a Material Adverse Effect, employ such counsel reasonably acceptable to Ultragenyx as Buyer shall recommend for such purpose.

(c) Allocation of Proceeds and Costs of Enforcement. Each of Buyer and Ultragenyx shall bear its own fees and expenses incurred in enforcing Licensee's obligations under the License Agreement pursuant to this Section 6.9, provided that Buyer shall pay [***]% of the costs and expenses of any counsel employed by Ultragenyx pursuant to Section 6.9(b)(ii). The Proceeds resulting from any enforcement of Licensee's obligations under the License Agreement undertaken at Buyer's request pursuant to this Section 6.9 shall be applied first to reimburse Ultragenyx and Buyer for any expenses incurred by them in connection with such enforcement (including notwithstanding the occurrence of the Royalty Termination Date), with the remainder of the Proceeds distributed to (i) Buyer if the breach by Licensee is solely related to the Royalty or [***] if the breach by Licensee is otherwise related to the Profit Share Territory or royalties payable in respect of sales of Licensed Product in the Profit Share Territory or (ii) Ultragenyx for all other breaches by Licensee that do not relate, directly or indirectly, to the Profit Share Territory or royalties payable in respect of sales of Licensed Product in the Profit Share Territory. Ultragenyx hereby assigns and, if not presently assignable, agrees to assign to Buyer the amount of Proceeds due to Buyer in accordance with this Section 6.9.

Section 6.10 Termination of License Agreement. Ultragenyx shall not, without the prior written consent of Buyer, (i) exercise any right to terminate the License Agreement, in whole or in part (but only if termination of such part could reasonably be expected (with or without the giving of notice or passage of time, or both) to have a Material Adverse Effect), (ii) agree with Licensee to terminate the License Agreement, in whole or in part (but only if termination of such part could reasonably be expected (with or without the giving of notice or passage of time, or both) to have a Material Adverse Effect), or (iii) take, or permit any Affiliate or sublicensee to take, any action that would reasonably be expected to give Licensee the right

to terminate the License Agreement, in whole or in part (but only if termination of such part could reasonably be expected (with or without the giving of notice or passage of time, or both) to have a Material Adverse Effect). Ultragenyx shall not take any action, fail to take an action or permit an action to be taken, that would give Licensee the right to terminate the License Agreement under Section 15.2.1 or 15.2.2 thereof or Section 4 of Amendment No. 3.

Section 6.11 Preservation of Rights. Ultragenyx shall not, except as Mutually Agreed, hereafter sell, transfer, hypothecate, assign or in any manner convey or mortgage, pledge or grant a security interest or other encumbrance of any kind in any of its interest in any portion of the License Agreement or any of its interest in the Joint Invention Patents that could reasonably be expected (with or without the giving of notice or passage of time, or both) to have a Material Adverse Effect. Neither Ultragenyx nor Seller shall hereafter subject to a Lien (other than a Permitted Lien), sell, transfer, assign, convey title (in whole or in part), grant any right to, or otherwise dispose of any portion of the Royalty.

Section 6.12 Enforcement; Defense; Prosecution and Maintenance.

(a) Buyer and Ultragenyx shall promptly inform each other of any suspected infringement by a Third Party they become aware of in the Profit Share Territory with respect to any of the Licensed Patents or any other patent right claiming the composition of matter of, or the method of making or using, any Licensed Product in the Profit Share Territory. Ultragenyx shall (i) provide to Buyer a copy of any written notice of any suspected infringement in the Profit Share Territory of any of the Licensed Patents and all pleadings filed in such action and (ii) notify Buyer of any material developments in any claim, suit or proceeding resulting from such infringement that are delivered by Licensee to Ultragenyx under Section 10.5.1 of the License Agreement or otherwise as soon as practicable and in any event not less than [***] Business Days following such delivery.

(b) If Ultragenyx has the right to join an enforcement action in the Profit Share Territory as set forth in Section 10.5.1 of the License Agreement, Ultragenyx shall, if requested in writing by Buyer, promptly, and in any event within [***] Business Days after receipt of such request, exercise such right as instructed by Buyer and, if requested by Buyer, Ultragenyx shall employ such counsel reasonably acceptable to Ultragenyx as Buyer shall recommend for such purpose. Ultragenyx shall not join any infringement action in the Profit Share Territory under Section 10.5.1 of the License Agreement without Buyer's prior consent.

(c) Promptly (and in any event within [***] Business Days) following Ultragenyx receiving written notice from the Licensee pursuant to Section 10.1.1 of the License Agreement or, if applicable where Licensee was appointed the lead-party, Section 10.2.1 of the License Agreement, of the Licensee's intention to allow any of the KHK Patent Rights in the Profit Share Territory or the Joint Invention Patents in the Profit Share Territory to lapse or become abandoned or to not file patent applications for any of the KHK Patent Rights in the Profit Share Territory or Joint Invention Patents in the Profit Share Territory (such Patent Rights, the "Applicable Patents"), Ultragenyx shall inform Buyer of such notice and, as Mutually

Agreed, Ultragenyx shall exercise its rights under Section 10.1.1 of the License Agreement or, if applicable where Licensee was appointed the lead-party, Section 10.2.1 of the License Agreement, to assume the prosecution and maintenance of any such Applicable Patents.

(d) Ultragenyx shall act as Mutually Agreed to: (i) take any and all actions, and prepare, execute, deliver and file any and all agreements, documents and instruments, that are reasonably necessary or desirable to diligently prosecute, preserve and maintain any Licensed Patents in the Profit Share Territory for which it controls the prosecution and maintenance, including in accordance with Section 10.1.1 of the License Agreement and, if applicable Section 10.2.1 (where Ultragenyx was appointed the lead-party or Ultragenyx assumed such role from Licensee as permitted pursuant to Section 6.12(c) above) of the License Agreement, including payment of maintenance fees or annuities on any such Licensed Patents, (ii) prosecute any corrections, substitutions, reissues, reviews and reexaminations of any Licensed Patents in the Profit Share Territory, for which it controls the prosecution and maintenance, including in accordance with Section 10.1.1 of the License Agreement and, if applicable Section 10.2.1 (where Ultragenyx was appointed the lead-party or Ultragenyx assumed such role from Licensee as permitted pursuant to Section 6.12(c) above) of the License Agreement, and any other forms of patent term restoration in any applicable jurisdiction in the Profit Share Territory, (iii) diligently enforce and defend any Licensed Patents for which it controls the defense and enforcement in the Profit Share Territory, including by bringing any legal action for infringement or defending any counterclaim of invalidity or unenforceability or action of a Third Party for declaratory judgment of non-infringement or non-interference, and (iv) not disclaim or abandon, or fail to take any action necessary or desirable to prevent the disclaimer or abandonment (including through lack of enforcement against Third Party infringers), of any Licensed Patents in the Profit Share Territory for which it controls the prosecution and maintenance, including in accordance with Section 10.1.1 of the License Agreement and, if applicable Section 10.2.1 (where Ultragenyx was appointed the lead-party or Ultragenyx assumed such role from Licensee as permitted pursuant to Section 6.12(c) above) of the License Agreement. For purposes of compliance with this Section 6.12(d), Ultragenyx shall employ such counsel, reasonable acceptable to Ultragenyx, as Buyer shall recommend for such purpose.

(e) Ultragenyx shall be responsible for all out-of-pocket costs and expenses (including the fees and expenses of counsel) incurred by Ultragenyx in connection with Ultragenyx's actions pursuant to Section 6.12 (b), (c) or (d), as applicable; provided, however, that, if Ultragenyx's actions under any such Section 6.12 (b), (c) or (d) (or any clauses thereof): (i) were taken pursuant to the mutual agreement of Ultragenyx and Buyer, Buyer shall promptly on demand reimburse Ultragenyx for 30% of all such out-of-pocket costs and expenses (including the fees and expenses of counsel) reasonably incurred by Ultragenyx in connection with Ultragenyx's actions pursuant to any such Sections (or any clauses thereof), provided that such demand shall be in writing and shall be accompanied by reasonably detailed invoices or similar supporting evidence of the costs and expenses for which Ultragenyx seeks reimbursement, or (ii) were taken solely at the direction or request of Buyer (and not pursuant to the mutual agreement of Ultragenyx and Buyer), Buyer shall promptly on demand reimburse Ultragenyx for 100% all such out-of-pocket costs and expenses (including the fees and expenses

of counsel) reasonably incurred by Ultragenyx in connection with Ultragenyx's actions pursuant to any such Sections (or any clauses thereof), provided that such demand shall be in writing and shall be accompanied by reasonably detailed invoices or similar supporting evidence of the costs and expenses for which Ultragenyx seeks reimbursement, and provided further that Ultragenyx shall apply the remainder of any proceeds and other amounts recovered through such actions following application of the recoveries sharing allocation between Ultragenyx and the Licensee pursuant to Section 10.5.3 of the License Agreement (A) first, to reimburse Buyer for 70% of all of the amounts reimbursed by Buyer to Seller pursuant to this clause (ii), and (B) second, 70% to Ultragenyx and 30% to Buyer.

Section 6.13 Additional Covenants Regarding Seller.

(a) Commencing on the Closing Date and until the Royalty Termination Date (except as otherwise contemplated by this Agreement or as required by Applicable Law or except as Mutually Agreed), Seller shall not:

(i) amend Article VII, Section 9 of Seller's bylaws without the prior written consent of Buyer;

(ii) incur, create, assume or otherwise become liable for any indebtedness, or amend, modify or refinance any indebtedness or make any loans, advances or capital contributions to, or investments in, any other Person.

(b) Seller shall provide to Buyer at least [***] Business Days' prior written notice prior to any change to its legal name, jurisdiction of formation or entity type.

(c) Ultragenyx covenants and agrees that it or its Affiliates shall continue to own all of the voting and equity interests of Seller until the Royalty Termination Date. Ultragenyx and Seller agree not to amend, modify or terminate, in whole or in part, or to waive any right under any of the Intercompany Sale Documents prior to the Royalty Termination Date.

Section 6.14 No Disposition of Royalty. Except as permitted by Section 10.3, Seller shall remain the exclusive owner of, and shall continue to have good and marketable title to, the Royalty free and clear of all Liens (other than Permitted Liens). Except as permitted by Section 10.3, Seller shall not sell, assign, transfer or convey, or grant any Lien (other than Permitted Liens) on, the Royalty or agree to do any of the foregoing.

Section 6.15 Efforts to Consummate Transactions. Subject to the terms and conditions of this Agreement, each of Seller, Ultragenyx and Buyer shall use its commercially reasonable efforts to take, or cause to be taken, all actions and to do, or cause to be done, all things reasonably necessary under Applicable Law to consummate the transactions contemplated by this Agreement. Each of Buyer, Seller and Ultragenyx agrees to execute and deliver such other documents, certificates, agreements and other writings and to take such other actions as may be reasonably necessary in order to consummate or implement

expeditiously the transactions contemplated by this Agreement and the Intercompany Sale Documents.

Section 6.16 Further Assurances. After the Closing, Seller, Ultragenyx and Buyer agree to execute and deliver such other documents, certificates, agreements and other writings and to take such other actions as may be reasonably necessary in order to give effect to the transactions contemplated by this Agreement and the Intercompany Sale Documents.

Section 6.17 Tax Matters.

(a) Notwithstanding anything to the contrary in the Transaction Documents, the parties to this Agreement shall treat (i) the transactions contemplated by this Agreement as a sale of the Purchased Interest for U.S. federal Tax purposes, and to the extent applicable, U.S. state, local and non-U.S. Tax purposes, and (ii) any and all payments on account of the Purchased Interest made pursuant to this Agreement after the Closing Date as made directly from Seller to Buyer for such Tax purposes (the "Intended Tax Treatment"). The parties hereto shall cooperate to effect the foregoing Intended Tax Treatment for U.S. federal Tax purposes, and to the extent applicable, U.S. state, local and non-U.S. Tax purposes.

(b) The parties to this Agreement acknowledge and agree that all payments to Buyer under the Transaction Documents are expected to be made without any withholding for or on account of any Tax. In the event that Seller subsequently reasonably determines in consultation with Buyer that any such withholding has become required, whether as a result of a change in Applicable Law after the date hereof or a determination within the meaning of Section 1313(a) of the Code (a "Determination") that is inconsistent with the Intended Tax Treatment (or otherwise), Seller shall use commercially reasonable efforts to give Buyer notice of such withholding at least [***] Business Days before any Tax is withheld, and the parties hereto shall use commercially reasonable efforts to cooperate with Buyer to implement such measures (such as by providing certifications or other tax forms, in each case, subject to Buyer's reimbursement of reasonable out-of-pocket expenses incurred by the other parties) that are reasonably necessary or advisable to reduce or eliminate such withholding. In the event that any amount of Tax is withheld from any payment to a party pursuant to this Section 6.17(b), such amount of Tax shall be deemed to have been received by such party for all purposes of this Agreement. Notwithstanding anything to the contrary in the Transaction Documents (including Section 6.3), neither Ultragenyx nor Seller shall have any obligation to gross-up or otherwise pay any other party any amounts with respect to any Tax withholding except to the extent that such Tax withholding constitutes Excluded Liabilities and Obligations.

(c) The parties hereto agree not to take any position that is inconsistent with the provisions of Section 6.17(a) on any Tax return or in any audit or other administrative or judicial proceeding or otherwise unless (i) the other party hereto has provided its prior written consent to such action(s), or (ii) there is a Determination that is inconsistent with the Intended Tax Treatment. If there is an inquiry by any Governmental Entity of Ultragenyx, Seller or Buyer related to this Section 6.17, the parties hereto shall cooperate with each other in responding to such inquiry in a reasonable manner consistent with this Section 6.17. In the event that a

Governmental Entity makes a Determination that a payment previously made to Buyer pursuant to this Agreement was subject to withholding Taxes and, in connection with such Determination, Seller or Ultragenyx remits (or is required pursuant to such Determination to remit) such Taxes to the Governmental Entity, Seller and Ultragenyx will have the right to (x) offset such amount (including any interest and penalties) against future payments under this Agreement or (y) invoice Buyer for such amount (which shall be payable by Buyer within [***] days following its receipt of such invoice).

ARTICLE 7

CONFIDENTIALITY

Section 7.1 Confidentiality. Except as provided in this Article 7 or otherwise agreed in writing by the parties, the parties hereto agree that each party and its Affiliates (the "Receiving Party") shall, and shall cause its Affiliates to, keep confidential, and shall not, and shall cause its Affiliates not to, publish, otherwise disclose or use for any purpose other than as provided for in this Agreement (which includes the exercise of any rights or the performance of any obligations hereunder) any information furnished to it or its Affiliates by or on behalf of the other party (the "Disclosing Party") pursuant to this Agreement (such information, "Confidential Information" of the Disclosing Party), except for that portion of such information that:

(a) was already known to the Receiving Party, other than under an obligation of confidentiality, at the time of disclosure by the Disclosing Party, as documented by contemporaneous business records;

(b) was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party;

(c) became generally available to the public or otherwise part of the public domain after its disclosure and other than through any act or omission of the Receiving Party in breach of this Agreement;

(d) is subsequently disclosed to the Receiving Party on a non-confidential basis by a Third Party without obligations of confidentiality with respect thereto, as documented by written business records in existence prior to the receipt of such information from the disclosing Party; or

(e) is independently developed by the Receiving Party or any of its Affiliates, as evidenced by written records, without the use of or reference of the Confidential Information.

Section 7.2 Authorized Disclosure.

(a) Either party may disclose Confidential Information with the prior written consent of the Disclosing Party or to the extent such disclosure is reasonably necessary in the following situations:

(i) exercising a party's rights or performing a party's obligations hereunder;

(ii) complying with Applicable Laws, including regulations promulgated by securities exchanges;

(iii) complying with a valid order of a court of competent jurisdiction or other Governmental Entity;

(iv) for regulatory, tax or customs purposes;

(v) for audit purposes, provided that each recipient of Confidential Information must be bound by customary obligations of confidentiality and non-use prior to any such disclosure;

(vi) disclosure to its Affiliates and Representatives on a need-to-know basis, provided that each recipient of Confidential Information must be bound by customary obligations of confidentiality and non-use prior to any such disclosure; or

(vii) regarding the terms and conditions of the License Agreement, the Intercompany Sale Documents or this Agreement, to the Receiving 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 Receiving Party will be jointly responsible for any disclosure of Confidential Information by any such Person inconsistent with the confidentiality obligations owed by the Receiving Party hereunder.

(b) Notwithstanding the foregoing, in the event the Receiving Party is required to make a disclosure of the Disclosing Party's Confidential Information pursuant to Sections 7.2(a)(i), (ii), (iii) or (iv), it will, except where impracticable, give reasonable advance notice to the Disclosing Party of such disclosure, consider in good faith any comments of the Disclosing Party regarding such disclosure, and use reasonable efforts to secure confidential treatment of such information. In any event, Buyer shall not file any patent application based upon or using the Confidential Information of Seller or Ultragenyx provided hereunder.

Section 7.3 Termination of Confidential Disclosure Agreement. Effective upon the Closing Date, the Confidential Disclosure Agreement dated as of May 23, 2022 between Ultragenyx and OMERS Capital Markets, a division of OMERS Administration Corporation

("**OMERS**") shall terminate and be of no further force and effect and shall be superseded by the provisions of this Article 7. OMERS shall have the benefit of, and be entitled to rely on, this Section 7.3.

ARTICLE 8

INDEMNIFICATION

Section 8.1 General Indemnity. Subject to Section 8.3, from and after the Closing:

(a) Seller and Ultragenyx hereby agree to jointly and severally indemnify, defend and hold harmless Buyer and its Affiliates and its and their respective directors, officers, partners, agents and employees (the "Buyer Indemnified Parties") from, against and in respect of all Losses suffered or incurred by Buyer Indemnified Parties to the extent arising out of or resulting from (i) any breach of any of the representations or warranties (in each case, when made) of Seller or Ultragenyx in this Agreement, Seller Closing Certificate and the Ultragenyx Closing Certificate, (ii) any breach of any of the covenants or agreements of Seller or Ultragenyx in this Agreement; provided, however, that the foregoing shall exclude any indemnification to any Buyer Indemnified Party (x) to the extent resulting from the gross negligence, willful misconduct, or fraud of any Buyer Indemnified Party or (y) to the extent resulting from acts or omissions of Seller, Ultragenyx or any of their Affiliates based upon written instructions from any Buyer Indemnified Party (unless Seller or Ultragenyx is otherwise liable for such Losses pursuant to the terms of this Agreement), and (iii) the Excluded Liabilities and Obligations; and

(b) Buyer hereby agrees to indemnify, defend and hold harmless each of Seller and Ultragenyx, their Affiliates and their and their Affiliates' directors, officers, agents and employees ("Seller Indemnified Parties") from, against and in respect of all Losses suffered or incurred by Seller Indemnified Parties to the extent arising out of or resulting from (i) any breach of any of the representations or warranties (in each case, when made) of Buyer in this Agreement and Buyer Closing Certificate or (ii) any breach of any of the covenants or agreements of Buyer in this Agreement; provided, however, that the foregoing shall exclude any indemnification to any Seller Indemnified Party (x) to the extent resulting from the gross negligence, willful misconduct, or fraud of any Seller Indemnified Party or (y) to the extent resulting from acts or omissions of Buyer or any of its Affiliates based upon written instructions from any Seller Indemnified Party (unless Buyer is otherwise liable for such Losses pursuant to the terms of this Agreement).

Section 8.2 Notice of Claims. If either a Buyer Indemnified Party, on the one hand, or a Seller Indemnified Party, on the other hand (such Buyer Indemnified Party on the one hand and such Seller Indemnified Party on the other hand being hereinafter referred to as an "Indemnified Party"), has suffered or incurred any Losses for which indemnification may be sought under this Article 8, the Indemnified Party shall so notify the other party from whom indemnification is sought under this Article 8 (the "Indemnifying Party") promptly in writing

describing such Loss, the amount or estimated amount thereof, if known or reasonably capable of estimation, and the method of computation of such Loss, all with reasonable particularity and containing a reference to the provisions of this Agreement in respect of which such Loss shall have occurred. If any claim, action, suit or proceeding is asserted or instituted by or against a third party with respect to which an Indemnified Party intends to claim any Loss under this Article 8, such Indemnified Party shall promptly notify the Indemnifying Party of such claim, action, suit or proceeding and tender to the Indemnifying Party the defense of such claim, action, suit or proceeding. A failure by an Indemnified Party to give notice and to tender the defense of such claim, action, suit or proceeding in a timely manner pursuant to this Section 8.2 shall not limit the obligation of the Indemnifying Party under this Article 8, except to the extent such Indemnifying Party is actually prejudiced thereby.

Section 8.3 Limitations on Liability. No party hereto shall be liable for any consequential (including lost profits), punitive, special, indirect or incidental damages under this Article 8 (and no claim for indemnification hereunder shall be asserted) as a result of any breach or violation of any covenant or agreement of such party (including under this Article 8) in or pursuant to this Agreement. Notwithstanding the foregoing, Buyer shall be entitled to make indemnification claims, in accordance with the procedures set forth in this Article 8, for Losses that include any portion of the Purchased Interest that Buyer was or would have been entitled to receive but did not receive timely or at all due to any indemnifiable events under this Agreement, and such portion of the Purchased Interest shall not be deemed consequential, punitive, special, indirect, incidental damages or lost profits for any purpose of this Agreement. Other than with respect to any fraud, willful misconduct, or intentional misrepresentation, (a) in no event shall an Indemnifying Party's aggregate liability for Losses under Section 8.1(a)(i) or Section 8.1(b)(i) exceed the Purchase Price less the Purchased Interest payments actually received by Buyer, and (b) no Indemnifying Party shall have any liability for Losses under Section 8.1(a)(i), Section 8.1(a)(ii) or Section 8.1(b) unless and until the aggregate amount of all Losses incurred by the Indemnified Party equals or exceeds $[***], in which event such Indemnifying Party shall be liable for all Losses including such amount.

Section 8.4 Third Party Claims. Upon providing notice to an Indemnifying Party by an Indemnified Party pursuant to Section 8.2 of the commencement of any action, suit or proceeding against such Indemnified Party by a third party with respect to which such Indemnified Party intends to claim any Loss under this Article 8, such Indemnifying Party shall have the right to defend such claim, at such Indemnifying Party's expense and with counsel of its choice reasonably satisfactory to the Indemnified Party. If the Indemnifying Party assumes the defense of such claim, the Indemnified Party shall, at the request of the Indemnifying Party, use commercially reasonable efforts to cooperate in such defense; provided, that the Indemnifying Party shall bear the Indemnified Party's out-of-pocket costs and expenses reasonably incurred in connection with such cooperation. So long as the Indemnifying Party is conducting the defense of such claim as provided in this Section 8.4, the Indemnified Party may retain separate co-counsel at its expense and may participate in the defense of such claim, and neither the Indemnified Party nor the Indemnifying Party shall consent to the entry of any Judgment or enter into any settlement with respect to such claim without the prior written

consent of the other unless such Judgment or settlement (A) provides for the payment by the Indemnifying Party of money as sole relief (if any) for the claimant (other than customary and reasonable confidentiality obligations relating to such claim, Judgment or settlement), (B) results in the full and general release of the Indemnified Party from all liabilities arising out of, relating to or in connection with such claim and (C) does not involve a finding or admission of any violation of any law, rule, regulation or Judgment, or the rights of any Person. In the event the Indemnifying Party does not or ceases to conduct the defense of such claim as so provided, (i) the Indemnified Party may defend against, and consent to the entry of any reasonable Judgment or enter into any reasonable settlement with respect to, such claim in any manner it may reasonably deem to be appropriate, (ii) the Indemnifying Party shall reimburse the Indemnified Party promptly and periodically for the reasonable out-of-pocket costs of defending against such claim, including reasonable attorneys' fees and expenses against reasonably detailed invoices, and (iii) the Indemnifying Party shall remain responsible for any Losses the Indemnified Party may suffer as a result of such claim to the full extent provided in this Article 8.

Section 8.5 Exclusive Remedy. Except as set forth in Section 10.10, from and after Closing, the rights of the parties hereto pursuant to (and subject to the conditions of) this Article 8 shall be the sole and exclusive remedy of the parties hereto and their respective Affiliates with respect to any claims (whether based in contract, tort or otherwise) resulting from or relating to any breach of the representations, warranties covenants and agreements made under this Agreement or any certificate, document or instrument delivered hereunder, and each party hereto hereby waives, to the fullest extent permitted under Applicable Law, and agrees not to assert after Closing, any other claim or action in respect of any such breach. Notwithstanding the foregoing, claims for fraud shall not be waived or limited in any way by this Article 8.

ARTICLE 9

TERMINATION

Section 9.1 Grounds for Termination. This Agreement may be terminated at any time prior to the Closing:

(a) by mutual written agreement of Buyer, Seller and Ultragenyx; or

(b) by Buyer upon notice in writing to Seller and Ultragenyx at any time after [***] from the Effective Date, if by such date the Closing shall not have been consummated for any reason other than a material breach by Buyer of any of its representations, warranties, covenants, agreements or obligations under this Agreement.

Section 9.2 Automatic Termination. Unless earlier terminated as provided in Section 9.1, this Agreement shall continue in full force and effect until the Royalty Termination Date, at which point this Agreement shall automatically terminate, except with respect to any rights that shall have accrued prior to such termination.

Section 9.3 Survival. Notwithstanding anything to the contrary in this Article 9, the following provisions shall survive termination of this Agreement: Section 6.1 (Disclosures), Section 6.2(b) (Payments Received in Error; Interest), Article 7 (Confidentiality), Article 8 (Indemnification), Section 9.3 (Survival) and Article 10 (Miscellaneous). Termination of the Agreement shall not relieve any party of liability in respect of breaches under this Agreement by any party on or prior to termination.

ARTICLE 10

MISCELLANEOUS

Section 10.1 Notices. All notices and other communications under this Agreement shall be in writing and shall be by email with PDF attachment, facsimile, courier service or personal delivery to the following addresses, or to such other addresses as shall be designated from time to time by a party hereto in accordance with this Section 10.1:

If to Seller, to it at:

Rare Delaware Inc.
c/o Ultragenyx Pharmaceutical
60 Leveroni Court
Novato, CA 94949
Attention: Karah Parschauer
Email: [***]

If to Ultragenyx, to it at:

Ultragenyx Pharmaceutical
60 Leveroni Court
Novato, CA 94949
Attention: Karah Parschauer
Email: [***]

With copies to:

Gibson, Dunn & Crutcher LLP
555 Mission Street
San Francisco, CA 94105
Attention: Ryan Murr and Karen Spindler
Email: [***]; [***]

If to Buyer, to it at:

OCM LS23 Holdings LP
c/o OMERS Capital Markets
900 – 100 Adelaide St W

Toronto, ON M5H 0E2, Canada
Attention: Robert Missere, President
Email: [***]

With a copy to:

OMERS Capital Markets
900 – 100 Adelaide St W
Toronto, ON M5H 0E2, Canada
Attention: Danial Lam, Managing Director
Email: [***] and [***]

All notices and communications under this Agreement shall be deemed to have been duly given (i) when delivered by hand, if personally delivered, (ii) when received by a recipient, if sent by email, or (iii) upon receipt when sent by overnight delivery via commercial overnight courier service.

Section 10.2 Expenses. Except as otherwise provided herein, all fees, costs and expenses (including any legal, accounting and banking fees) incurred in connection with the preparation, negotiation, execution and delivery of this Agreement and to consummate the transactions contemplated hereby shall be paid by the party hereto incurring such fees, costs and expenses.

Section 10.3 Assignment.

(a) Neither Ultragenyx nor Seller shall sell, assign or otherwise transfer, as applicable, (i) all or any portion of its interest in the Joint Invention Patents, (ii) all or any portion of its interest in the Royalty, the Intercompany Sale Documents and the License Agreement that relates to the Royalty or to the Profit Share Territory, (iii) all or any portion of its interest in this Agreement or (iv) all or any portion of its interest in the License Agreement that does not relate to the Royalty or to the Profit Share Territory (but only, in the case of clause (iv), if the sale, assignment or transfer of such portion would reasonably be expected (with or without the giving of notice or the passage of time, or both) to have a Material Adverse Effect) to any third party or to the Licensee by operation of law, merger, change of control, or otherwise, unless in connection therewith (A) such Person acquires all of Ultragenyx's and Seller's respective interests in all of the Joint Invention Patents, the License Agreement, the Intercompany Sale Documents and this Agreement and (B) prior to closing any such transaction, Ultragenyx or Seller, as applicable, causes such Person to deliver a writing to Buyer in which (1) if such Person is not the Licensee, such Person assumes all of the obligations of Ultragenyx or Seller, as applicable, to Buyer under this Agreement, and (2) if such Person is the Licensee, the Licensee assumes all of the obligations of Ultragenyx and Seller to Buyer hereunder and agrees to pay the

Purchased Interest directly to Buyer notwithstanding any subsequent termination of the License Agreement by the Licensee.

(b) Buyer may assign this Agreement, provided that Buyer promptly thereafter notifies each of Seller and Ultragenyx and any such assignee promptly thereafter agrees in writing to be bound by the obligations of Buyer contained in this Agreement, and in any event such assignment shall be of the Agreement in its entirety.

(c) Any purported assignment in violation of this Section 10.3 shall be null and void.

(d) This Agreement shall be binding upon, inure to the benefit of and be enforceable by, the parties hereto and their respective permitted successors and assigns.

Section 10.4 Amendment and Waiver.

(a) This Agreement may be amended, modified or supplemented only in a writing signed by each of the parties hereto. Any provision of this Agreement may be waived only in a writing signed by the parties hereto granting such waiver.

(b) No failure or delay on the part of any party hereto in exercising any right, power or remedy hereunder shall operate as a waiver thereof, nor shall any single or partial exercise of any such right, power or remedy preclude any other or further exercise thereof or the exercise of any other right, power or remedy. No course of dealing between the parties hereto shall be effective to amend, modify, supplement or waive any provision of this Agreement.

Section 10.5 Entire Agreement. This Agreement, the Exhibits annexed hereto and the Disclosure Schedule constitute the entire understanding between the parties hereto with respect to the subject matter hereof and supersede all other understandings and negotiations with respect thereto.

Section 10.6 No Third Party Beneficiaries. Except as set forth in Section 7.3, this Agreement is for the sole benefit of Seller, Ultragenyx and Buyer and their permitted successors and assigns and nothing herein expressed or implied shall give or be construed to give to any Person, other than the parties hereto and such successors and assigns, any legal or equitable rights hereunder.

Section 10.7 Governing Law. This Agreement shall be governed by, and construed in accordance with, the laws of the State of New York without giving effect to any choice or conflict of law provision or rule that would cause the application of the laws of any other jurisdiction.

Section 10.8 JURISDICTION; VENUE.

(a) EACH OF THE PARTIES HERETO HEREBY IRREVOCABLY AND UNCONDITIONALLY SUBMITS, FOR ITSELF AND ITS RESPECTIVE PROPERTY AND ASSETS, TO THE EXCLUSIVE JURISDICTION OF ANY NEW YORK STATE COURT OR FEDERAL COURT OF THE UNITED STATES OF AMERICA SITTING IN NEW YORK COUNTY, NEW YORK, AND ANY APPELLATE COURT THEREOF, IN ANY ACTION OR PROCEEDING ARISING OUT OF OR RELATING TO THIS AGREEMENT, OR FOR RECOGNITION OR ENFORCEMENT OF ANY JUDGMENT IN RESPECT THEREOF, AND BUYER, SELLER AND ULTRAGENYX HEREBY IRREVOCABLY AND UNCONDITIONALLY AGREE THAT ALL CLAIMS IN RESPECT OF ANY SUCH ACTION OR PROCEEDING MAY BE HEARD AND DETERMINED IN ANY SUCH NEW YORK STATE COURT OR, TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, IN SUCH FEDERAL COURT. BUYER AND SELLER HEREBY AGREE THAT A FINAL JUDGMENT IN ANY SUCH ACTION OR PROCEEDING SHALL BE CONCLUSIVE AND MAY BE ENFORCED IN OTHER JURISDICTIONS BY SUIT ON THE JUDGMENT OR IN ANY OTHER MANNER PROVIDED BY APPLICABLE LAW. EACH OF BUYER, SELLER AND ULTRAGENYX HEREBY SUBMITS TO THE EXCLUSIVE PERSONAL JURISDICTION AND VENUE OF SUCH NEW YORK STATE AND FEDERAL COURTS. EACH OF BUYER, SELLER AND ULTRAGENYX AGREES, TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, THAT PROCESS MAY BE SERVED ON BUYER, SELLER OR ULTRAGENYX IN THE SAME MANNER THAT NOTICES MAY BE GIVEN PURSUANT TO SECTION 10.1 HEREOF.

(b) EACH OF THE PARTIES HERETO HEREBY IRREVOCABLY AND UNCONDITIONALLY WAIVES, TO THE FULLEST EXTENT IT MAY LEGALLY AND EFFECTIVELY DO SO, ANY OBJECTION THAT IT MAY NOW OR HEREAFTER HAVE TO THE LAYING OF VENUE OF ANY ACTION OR PROCEEDING ARISING OUT OF OR RELATING TO THIS AGREEMENT IN ANY NEW YORK STATE OR FEDERAL COURT. EACH OF BUYER, SELLER AND ULTRAGENYX HEREBY IRREVOCABLY WAIVES, TO THE FULLEST EXTENT PERMITTED BY APPLICABLE LAW, THE DEFENSE OF AN INCONVENIENT FORUM TO THE MAINTENANCE OF SUCH ACTION OR PROCEEDING IN ANY SUCH COURT.

Section 10.9 Severability. If any term or provision of this Agreement shall for any reason be held to be invalid, illegal or unenforceable in any situation in any jurisdiction, then, to the extent that the economic and legal substance of the transactions contemplated hereby is not affected in a manner that is materially adverse to any party hereto, all other terms and provisions of this Agreement shall nevertheless remain in full force and effect and the enforceability and validity of the offending term or provision shall not be affected in any other situation or jurisdiction.

Section 10.10 Specific Performance. Each of the parties acknowledges and agrees that the other parties would be damaged irreparably in the event any of the provisions of this Agreement are not performed in accordance with their specific terms or otherwise are breached or violated. Accordingly, notwithstanding Section 8.5, each of the parties agrees that, without posting bond or other undertaking, the other parties shall be entitled to an injunction or injunctions to prevent breaches or violations of the provisions of this Agreement and to

enforce specifically this Agreement and the terms and provisions hereof in any action, suit or other proceeding instituted in any court of the United States or any state thereof having jurisdiction over the parties and the matter in addition to any other remedy to which it may be entitled, at law or in equity. Each party further agrees that, in the event of any action for specific performance in respect of such breach or violation, it shall not assert that the defense that a remedy at law would be adequate.

Section 10.11 Relationship of the Parties. The relationship between Buyer one the one hand and Seller and Ultragenyx on the other hand is solely that of purchaser and seller, and neither party has any fiduciary or other special relationship with the other party or any of its Affiliates. The Transaction Documents do not constitute a partnership or similar agreement, and nothing contained herein shall be deemed to constitute Ultragenyx, Seller, Buyer and/or any other person as a partnership, an association, a joint venture or any other kind of entity or legal form for any purposes, including any tax purposes. The parties agree that they shall not take any inconsistent position with respect to such treatment in a filing with any Governmental Entity.

Section 10.12 Counterparts. This Agreement may be executed in any number of counterparts and by the parties hereto in separate counterparts, each of which when so executed shall be deemed to be an original and all of which taken together shall constitute one and the same agreement. Copies of executed counterparts transmitted by telecopy, facsimile or other similar means of electronic transmission, including "PDF," shall be considered original executed counterparts, provided receipt of such counterparts is confirmed.

[*Signature Page Follows*]

IN WITNESS WHEREOF, the parties hereto have caused this Royalty Purchase Agreement to be executed and delivered by their respective representatives thereunto duly authorized as of the Effective Date.

RARE DELAWARE INC.

By: /s/ Mardi C. Dier
Name: Mardi C. Dier
Title: Vice President and Treasurer

ULTRAGENYX PHARMACEUTICAL INC.

By: /s/ Emil D. Kakkis, M.D., Ph.D.
Name: Emil D. Kakkis, M.D., Ph.D.
Title: President and Chief Executive Officer

IN WITNESS WHEREOF, the parties hereto have caused this Royalty Purchase Agreement to be executed and delivered by their respective representatives thereunto duly authorized as of the Effective Date.

OCM LS23 HOLDINGS LP, by its general partner, OCM LS23 HOLDINGS G.P. INC.

By: /s/ Robert Missere
Name: Robert Missere
Title: President

By: /s/ Danial K. Lam
Name: Danial K. Lam
Title: Vice President

Exhibit 10.2

[***] = CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY BRACKETS, HAS BEEN OMITTED BECAUSE THE INFORMATION (I) IS NOT MATERIAL AND (II) WOULD BE COMPETITIVELY HARMFUL IF PUBLICLY DISCLOSED

UNIT PURCHASE AGREEMENT

by and among

ULTRAGENYX PHARMACEUTICAL INC.,

GENETX BIOTHERAPEUTICS LLC,

THE UNITHOLDERS LISTED ON SCHEDULE A

and

Deborah A. Guagliardo, as the Representative

Dated as of July 15, 2022

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UNIT PURCHASE AGREEMENT

THIS UNIT PURCHASE AGREEMENT (this "Agreement") is made and entered into as of this 15th day of July, 2022, by and among Ultragenyx Pharmaceutical Inc., a Delaware corporation ("Buyer"), GeneTx Biotherapeutics LLC, a Delaware limited liability company (the "Company"), the holders of Units listed on Schedule A (the "Unitholders"), and Deborah A. Guagliardo, an individual, solely in her capacity as representative of the Unitholders (the "Representative").

RECITALS

WHEREAS, the Unitholders of the Company each granted an exclusive option (collectively, the "Option") to Buyer to acquire such holder's Series A Preferred Units of the Company (the "Series A Preferred Units"), if any, such holder's Class A Common Units of the Company (the "Class A Common Units"), if any, such Class B Common Units of the Company (the "Class B Common Units"), such holder's profits interests of the Company (the "Profits Interests" and, collectively with the Series A Preferred Units, Class A Common Units and Class B Common Units, the "Units"), if any, pursuant to the terms and conditions set forth in the Unitholder Option Agreement, dated as of August 13, 2019, as amended, by and among Buyer, the Company and the Unitholders (the "Unitholder Option Agreement"); and

WHEREAS, on July 2, 2022, Buyer exercised the Option, and pursuant to the terms and conditions of the Unitholder Option Agreement, Buyer, the Company and the Unitholders are entering into this Agreement to effectuate the sale of all outstanding Units to Buyer.

NOW, THEREFORE, in consideration of the premises and of the mutual representations, warranties, covenants and agreements herein contained, and for other good and valuable consideration (the receipt and sufficiency of which are hereby acknowledged), the parties hereto, intending to be legally bound, hereby agree as follows:

ARTICLE I
UNITS; CLOSING

Section I.1 Unit Purchase. Upon the terms and conditions of this Agreement, at the Closing, the Unitholders shall sell to Buyer, and Buyer shall purchase from the Unitholders all of the outstanding Units, free and clear of all Liens. Effective of the Closing, each Unitholder hereby assigns and transfers to Buyer all of such Unitholder's Units, and consents to the admission of Buyer as a member of the Company with respect to such Units.

Section I.2 Closing. Upon the terms and subject to the conditions hereof, the closing of the purchase and sale of the Units pursuant to Section 1.1 (the "Closing") will take place at 10:00 a.m. Boston time on the date hereof, or at such other time as may be mutually agreed by Buyer and Seller, by electronic exchange of documents and signatures. The date on which the Closing occurs is referred to herein as the "Closing Date."

Section I.3 Calculation of the Purchase Consideration

(a) Attached hereto as Exhibit A is a statement (the "Estimated Initial Purchase Consideration Calculation") setting forth the Company's good faith calculation and estimate of the

Estimated Initial Purchase Consideration (as defined below) and the components thereof accompanied by reasonable supporting detail and documentation, and (ii) the percentage and aggregate amount of the Estimated Initial Purchase Consideration that each Unitholder shall be entitled to receive in accordance with the Allocation Schedule. For purposes of this Agreement, the following terms shall have the following meanings:

(i) "Estimated Initial Purchase Consideration" means an amount equal to the Company's good faith calculation and estimate of the following as of the Closing Date: (a) the Base Consideration, plus (b) the Estimated Net Working Capital Adjustment (which may be a negative number), minus (c) the amount of Closing Date Company Indebtedness, minus (d) the amount of Transaction Costs, plus (e) the amount of Closing Cash, minus (f) if Buyer provided a Notice of Interest on or before the Early Option Exercise Deadline, but there was not a Closing under this Agreement as of the Early Option Exercise Deadline, the Option Extension Premium, if any.

(ii) "Base Consideration" means an amount equal to: (i) if this Agreement is entered into as a result of delivery of a Notice of Interest on or before the Early Option Deadline, fifty million Dollars ($50,000,000), (ii) if this Agreement is entered into as a result of delivery of a Notice of Interest after the Early Option Deadline but on or before the Interim Option Deadline, seventy-five million Dollars ($75,000,000), or (iii) if this Agreement is entered into as a result of delivery of a Notice of Interest after the Interim Option Deadline, one hundred twenty-five million Dollars ($125,000,000).

(iii) "Estimated Net Working Capital Adjustment" means (a) if the Estimated Net Working Capital exceeds the NWC Target Amount, then the amount by which Estimated Net Working Capital exceeds the NWC Target Amount or (b) if the NWC Target Amount exceeds the Estimated Net Working Capital, then the amount by which the NWC Target Amount exceeds the Estimated Net Working Capital; provided, that any amount which is calculated pursuant to clause (b) above shall be deemed to be a negative number.

(iv) "Estimated Net Working Capital" means the Company's good faith calculation and estimate of the following as of the Closing Date: (a) the current assets of the Company, on a consolidated basis, as of the close of business on the day immediately prior to the Closing Date (consisting solely of the line item current asset accounts specified on Exhibit B), less (b) the current liabilities of the Company, on a consolidated basis, as of the close of business on the day immediately prior to the Closing Date (consisting solely of the line item current liabilities specified on Exhibit B), in each case, determined in accordance with GAAP and using the same accounting methods, practices, principles, policies and procedures as were used in the preparation of the audited Financial Statements. Estimated Net Working Capital shall not include any amounts of Closing Cash, Closing Date Company Indebtedness, Transaction Costs, any accruals for employee or consultant bonuses, deferred or current Tax assets or deferred Tax liabilities. For the avoidance of doubt, the Company Pass-Through Expenses shall not be included in the calculation of Estimated Net Working Capital.

(b) As soon as practicable, but in any event within [***] days after the Closing Date, Buyer shall, or shall cause the Company to, prepare and deliver to the Representative, a statement setting forth the Buyer's good faith calculation and estimate of the proposed Definitive Initial Purchase Consideration (as defined below) and the components thereof accompanied by reasonable supporting detail and documentation (the "Closing Date Calculations"). For purposes of this Agreement, the following terms shall have the following meanings:

(i) "Definitive Initial Purchase Consideration" means an amount equal to the following as of the Closing Date: (a) the Base Consideration, plus (b) the Definitive Net Working Capital Adjustment (which may be a negative number), minus (c) the amount of Closing Date Company Indebtedness, minus (d) the amount of Transaction Costs, plus (e) the amount of Closing Cash, minus (f) if Buyer provided a Notice of Interest on or before the Early Option Exercise Deadline, but there was not a Closing under this Agreement as of the Early Option Exercise Deadline, the Option Extension Premium, if any.

(ii) "Definitive Net Working Capital Adjustment" means (a) if the Definitive Net Working Capital exceeds the NWC Target Amount, then the amount by which Definitive Net Working Capital exceeds the NWC Target Amount or (b) if the NWC Target Amount exceeds the Definitive Net Working Capital, then the amount by which the NWC Target Amount exceeds the Definitive Net Working Capital; provided, that any amount which is calculated pursuant to clause (b) above shall be deemed to be a negative number.

(iii) "Definitive Net Working Capital" means the following as of the Closing Date: (a) the current assets of the Company, on a consolidated basis, as of the close of business on the day immediately prior to the Closing Date (consisting solely of the line item current asset accounts specified on Exhibit B), less (b) the current liabilities of the Company, on a consolidated basis, as of the close of business on the day immediately prior to the Closing Date (consisting solely of the line item current liabilities specified on Exhibit B), in each case, determined in accordance with GAAP and using the same accounting methods, practices, principles, policies and procedures as were used in the preparation of the audited Financial Statements. Definitive Net Working Capital shall not include any amounts of Closing Cash, Closing Date Company Indebtedness, Transaction Costs, any accruals for employee or consultant bonuses, deferred or current Tax assets or deferred Tax liabilities. For the avoidance of doubt, the Company Pass-Through Expenses shall not be included in the calculation of Definitive Net Working Capital.

(c) From and after the Closing Date until the final determination of the Definitive Initial Purchase Consideration in accordance with this Section 1.3, Buyer shall (i) make available to the Representative and its accountants and advisors, the Company's books, records and other documents relevant to the preparation of the Closing Date Calculations and (ii) give the Representative reasonable access, during normal business hours and upon reasonable notice, to the personnel and accountants of the Company involved in the preparation of the Closing Date Calculations.

(d) The Representative shall have [***] days following receipt of the Closing Date Calculations to review such calculations (the "Review Period"). The Representative may, on or prior to the last day of the Review Period, give to Buyer a written notice of dispute which sets forth, in reasonable detail, its objections to the Closing Date Calculations, including the Company's revised calculation of the Definitive Net Working Capital Adjustment (a "Purchase Consideration Dispute Notice"). Unless the Representative provides a Purchase Consideration Dispute Notice to Buyer on or prior to the last day of the Review Period, (i) the Definitive Net Working Capital and Definitive Net Working Capital Adjustment set forth in the Closing Date Calculations shall be deemed to be the final Definitive Net Working Capital and Definitive Net Working Capital Adjustment, (ii) the Closing Cash set forth in the Closing Date Calculations shall be deemed to be the final Closing Cash, (iii) the Closing Date Company Indebtedness set forth in the Closing Date Calculations shall be deemed to be the final Closing Date Company Indebtedness, (iv) the Transaction Costs set forth in the Closing Date Calculations shall be deemed to be the final Transaction Costs and (v) the Definitive Initial Purchase Consideration set forth in the Closing Date

Calculations shall be deemed to be the final Definitive Initial Purchase Consideration. If the Representative provides a Purchase Consideration Dispute Notice to Buyer on or prior to the last day of the Review Period, the Representative and Buyer will use commercially reasonable efforts to resolve the dispute set forth in such Purchase Consideration Dispute Notice during the [***]-day period commencing on the date Buyer receives the applicable Purchase Consideration Dispute Notice from the Representative and any mutually agreed extension thereof. If the Representative and Buyer do not obtain a final resolution within such [***] day period or extension thereof, then the items remaining in dispute shall be submitted promptly (but in any event within [***] days) by the Representative and Buyer to a nationally or regionally recognized accounting firm that shall be independent and free of conflicts from, and mutually acceptable to, each of Buyer and the Representative (such firm, the "Accounting Firm"). The terms of appointment and engagement of the Accounting Firm shall be as agreed upon between the Representative and Buyer (it being understood that the Accounting Firm shall consider only those items or amounts in the Closing Date Calculations as to which there is disagreement as set forth in the Purchase Consideration Dispute Notice and that the Accounting Firm shall be functioning as an expert and not as an arbitrator). The Accounting Firm shall be required to render a determination regarding the applicable dispute within thirty [***] after engagement of the Accounting Firm, which determination must be in writing and must set forth, in reasonable detail, the basis therefor. In making its determination regarding such applicable dispute, the Accounting Firm shall select, with respect to each item in dispute, an amount between Buyer's position as set forth in the Closing Date Calculations and the Representative's position as set forth in the Purchase Consideration Dispute Notice or equal to either such amount. In connection with the resolution of any dispute, the Accounting Firm shall have access to all documents, records, work papers, facilities and personnel necessary to make its determination. Absent fraud by the Representative or Buyer or manifest error by the Accounting Firm, the determination of the Accounting Firm shall be conclusive and binding upon the Representative and Buyer for purposes of this Agreement. The Accounting Firm will revise the Closing Date Calculations, the Definitive Net Working Capital Adjustment and the Definitive Initial Purchase Consideration as appropriate to reflect the resolution of any objections thereto pursuant to this Section 1.3(d). The "Final Statement of Purchase Consideration" shall mean the Closing Date Calculations together with any revisions thereto pursuant to this Section 1.3(d).

(e) If the Representative and Buyer submit any unresolved disputes to the Accounting Firm for resolution as provided in Section 1.3(d), the fees and expenses of the Accounting Firm pursuant to this Section 1.3 shall initially be borne fifty percent (50%) each by Buyer and the Representative (on behalf of the Unitholders in accordance with the Allocation Schedule); provided, that upon the final determination by the Accounting Firm, the fees and expenses of the Accounting Firm shall be paid by each of the Buyer and Representative (on behalf of the Unitholders) in proportion to the amounts by which Buyer's estimate of the Definitive Net Working Capital Adjustment and the Company's estimate of the Definitive Net Working Capital Adjustment each differ from the final Definitive Net Working Capital Adjustment determined by the Accounting Firm and updated in the Final Statement of Purchase Consideration.

(f) Buyer, the Company and the Representative agree that the procedure set forth in this Section 1.3 for resolving disputes with respect to the Definitive Initial Purchase Consideration shall be the sole and exclusive method for resolving any such disputes (except for disputes relating to Company Indebtedness or Transaction Costs, in each case, to the extent not deducted from the Definitive Initial Purchase Consideration, absent fraud by the Representative or Buyer or manifest error by the Accounting Firm).

Section I.4 Closing Payments of Estimated Initial Purchase Consideration.

(a) At the Closing, Buyer shall make the following payments to, or on behalf of, the Unitholders, as applicable:

(i) At the Closing, Buyer shall pay on behalf of the Company to the holders of Closing Date Company Indebtedness as set forth in the pay-off letters delivered to Buyer pursuant to Section 5.1(c), the sums necessary to pay the Closing Date Company Indebtedness in accordance with the wire instructions set forth in such pay-off letters.

(ii) At the Closing, Buyer shall pay on behalf of the Company to the Persons to which Transaction Costs are owed, the sums necessary to pay such Transaction Costs as set forth in the list delivered to Buyer pursuant to Section 5.1(d), in accordance with wire transfer instructions set forth therein.

(iii) At the Closing, Buyer shall deliver to the Representative for further distribution to the Unitholders, the Closing Date Cash Payment, by wire transfer of immediately available funds to the account provided by the Representative to Buyer prior to the Closing. "Closing Date Cash Payment" means an amount equal to the Estimated Initial Purchase Consideration minus the sum of (a) the Escrow Amount and (b) the Representative's Fund. Promptly following the Representative's receipt of such funds, the Representative shall pay to each Unitholder the applicable amounts to which such Unitholder is entitled pursuant to the Estimated Initial Purchase Consideration Calculation and the Allocation Schedule.

(iv) At the Closing, Buyer shall deposit the Escrow Amount with the Escrow Agent in accordance with the Escrow Agreement, with such Escrow Amount to be invested, at the Representative's election, in an interest bearing account for the benefit of the Unitholders.

(v) At the Closing, Buyer shall deposit the Representative's Fund with the Representative at an account designated by the Representative prior to the Closing.

(vi) At or immediately prior to the Closing, Buyer shall also pay to the Company any amount outstanding for Company Pass-Through Expenses.

Subject to this Section 1.4(a), from and after the Closing, the Unitholders shall cease to have any rights with respect to any Units, other than the right to receive the applicable portion of the Definitive Initial Purchase Consideration, the Escrow Amount, the Milestone Payments, the Priority Review Voucher Payments, and the Royalty Payments with respect to such Units in accordance with the terms and conditions set forth in this Agreement.

(b) Each of the Unitholders hereby acknowledges and agrees that the Definitive Initial Purchase Consideration, the Escrow Amount, the Milestone Payments, the Priority Review Voucher Payments, and the Royalty Payments are being allocated among the Unitholders (i) as if the transactions contemplated hereby were a "Liquidation Event" as defined in the Amended and Restated Limited Liability Company Operating Agreement of the Company dated October 19, 2018 (the "Operating Agreement"), and (ii) in accordance with the Operating Agreement, Applicable Law and the terms and conditions of the Units. Prior to the Closing, the Company delivered to Buyer a schedule prepared by the Company (the

"Allocation Schedule"), certified by the Company's principal executive officer to be true, correct and accurate, which sets forth the allocation of Definitive Initial Purchase Consideration, the Escrow Amount, the Milestone Payments, the Priority Review Voucher Payments, and the Royalty Payments among the Unitholders in accordance with the prior sentence, which Allocation Schedule contains (x) the mailing addresses, telephone numbers and email addresses for each Unitholder and (y) the type and number of Units owned by each Unitholder. Upon making payments in accordance with the Allocation Schedule and the terms of this Agreement, whether such payments are made directly to the Unitholders or to the Representative for further distribution to the Unitholders in accordance with the Allocation Schedule, Buyer shall be deemed to have satisfied its obligations to make such payments with respect to the transactions contemplated by this Agreement and shall have no further obligations to the Unitholders with respect to such payments.

Section I.5 Actual Adjustment Payments.

(a) If the Actual Adjustment is a positive amount, Buyer shall pay such positive amount to the Representative (for further distribution to the Unitholders in accordance with the Allocation Schedule), by wire transfer or delivery of immediately available funds, in each case, within [***] Business Days after the date on which the Definitive Initial Purchase Consideration is finally determined pursuant to Section 1.3.

(b) If the Actual Adjustment is a negative amount, Buyer shall be entitled to payment from the Escrow Amount in the amount equal to the absolute value of such Actual Adjustment in accordance with the terms of the Escrow Agreement.

(c) Payments made pursuant to this Section 1.5 shall be treated for all purposes, including Tax purposes, as adjustments to the Definitive Initial Purchase Consideration to the extent permitted by Applicable Law.

Section I.6 Milestone Payments, Priority Review Voucher Payments and Royalty Payments.

(a) The Unitholders shall be entitled to receive from Buyer after the Closing, as additional payments for the Units (such additional payments paid pursuant to Section 1.6(b)(i), Section 1.6(b)(ii), Section 1.6(b)(iv), and Section 1.6(b)(v); or Section 1.6(c)(i) through Section 1.6(c)(iv) (inclusive), Section 1.6(c)(vi), and Section 1.6(c)(vii); or Section 1.6(d)(i), Section 1.6(d)(iii), and Section 1.6(d)(iv), the "Milestone Payments,"; such additional payments paid pursuant to Section 1.6(b)(iii), Section 1.6(c)(v), or Section 1.6(d)(ii), the "Priority Review Voucher Payments"); and such additional payments paid pursuant to (i) Section 1.6(b)(vi), (ii) Section 1.6(c)(viii), and Section 1.6(c)(ix) (as applicable) or (iii) Section 1.6(d)(v), the "Royalty Payments"), subject to the terms and conditions of this Section 1.6. Any Milestone Payments, Priority Review Voucher Payments, or Royalty Payments payable by Buyer shall be paid, by wire transfer or delivery of immediately available funds, to the Representative for further distribution to the Unitholders in accordance with the Allocation Schedule.

(b) In the event (and only in the event) this Agreement was entered into as a result of delivery of a Notice of Exercise on or before the Early Option Deadline, then, upon the achievement of any of the following set forth in this Section 1.6(b) by or on behalf of Buyer or any of its Affiliates or Licensees with respect to a Royalty-Bearing Product, Buyer shall pay the Milestone Payment set forth below corresponding to each such milestone, Company's portion of the net Priority Review Voucher

proceeds, and the applicable Royalty Payments. If more than one of the commercialization milestones set forth below is achieved in the same fiscal year, then all Milestone Payments for all such milestones shall be paid at the end of such Calendar Year:

(i) within [***] days after Buyer's receipt of [***] of a Royalty-Bearing Product conducted in accordance with the [***], [***] Dollars ($[***]);

(ii) within [***] after [***] a Royalty-Bearing Product, [***] Dollars ($[***]);

(iii) an amount equal to (i) [***][(***]%) of the cash value of consideration received (net of any reasonable banker's fee, commission or other similar fee payable upon such sale) upon sale by the Buyer of a Priority Review Voucher to a Third Party in an arms' length transaction, which amount shall be paid within [***] Business Days after the closing of such sale, or if such consideration is received in installments, within [***] Business Days after the receipt of each such installment; or (ii) [***] Dollars ($[***]) if a Priority Review Voucher is retained to be used by Buyer or its Affiliate (including, after the Closing, the Company), which decision to retain such Priority Review Voucher must be made within [***]([***])[***]] of the issuance of such Priority Review Voucher, and which amount shall be paid on the earlier to occur of (i) the [***]-[***] anniversary of the award of such Priority Review Voucher, and (ii) within [***] Business Days after [***];

(iv) within [***] days after [***], [***] Dollars ($[***]);

(v) commercialization milestones as follows, in each case within [***] days after the close of the Calendar Year in which such milestone occurs:

(1) [***] Dollars ($[***]), after the first occurrence of aggregate worldwide Net Sales of at least [***] Dollars ($[***]) of Royalty-Bearing Products during any Calendar Year;

(2) [***] Dollars ($[***]), after the first occurrence of aggregate worldwide Net Sales of at least [***] Dollars ($[***]) of Royalty-Bearing Products during any Calendar Year; and

(3) [***] Dollars ($[***]), after the first occurrence of aggregate worldwide Net Sales of at least [***] Dollars ($[***]) of Royalty-Bearing Products during any Calendar Year;

(vi) subject to Section 1.6(f), royalty payments as follows, based on worldwide aggregate annual Net Sales of all Royalty-Bearing Products, such payment to be made in each case within [***] days after the close of the applicable fiscal [***]:

Annual Net Sales of Royalty-Bearing Products	Royalty Rate Paid on the Portion of Annual Net Sales
Portion up to and including $[***]	[***]%
Portion greater than $[***]	[***]%

On or about the date Buyer makes each royalty payment in accordance with this Section 1.6(b)(vi), Buyer shall submit a royalty report to the Representative showing the calculation of Net Sales for the applicable fiscal [***] on a country-by-country basis, including gross sales of Royalty-Bearing Products by Selling Parties, a listing of applicable deductions, and the total royalty payment due for such fiscal [***].

(c) In the event (and only in the event) this Agreement was entered into as a result of delivery of a Notice of Interest after the Early Option Deadline and on or before the Interim Option Deadline, then, upon the achievement of any of the following set forth in this Section 1.6(c) with respect to a Royalty-Bearing Product by or on behalf of Buyer or any of its Affiliates or Licensees, Buyer shall pay the Milestone Payment set forth below corresponding to each such milestone, Company's portion of the net Priority Review Voucher proceeds, and the applicable Royalty Payments. If more than one of the commercialization milestones set forth below is achieved in the same fiscal year, then all Milestone Payments for all such milestones shall be paid at the end of such Calendar Year:

(i) Within [***] days after the first CA Approval, [***] Dollars ($[***]); where "CA Approval" means, with respect to a Royalty-Bearing Product, approval from the Department of Health Canada to market or sell any such Royalty-Bearing Product in Canada; provided that no such payment will be due if Buyer has paid thirty million Dollars ($30,000,000) pursuant to the following (ii) or (iii) this Section 1.6(c), individually or in combination;

(ii) Within [***] days after the first UK Approval, [***] Dollars ($[***]); where "UK Approval" means, with respect to a Royalty-Bearing Product, approval from the Medicines and Healthcare Products Regulatory Agency to market or sell any such Royalty-Bearing Product in the United Kingdom; provided that no such payment will be due if Buyer has paid thirty million Dollars ($30,000,000) pursuant to the foregoing (i) or following (iii) in this Section 1.6(c), individually or in combination;

(iii) Within [***] days after the dosing of the first patient in the first Phase 3 Clinical Study of a Royalty-Bearing Product, thirty million Dollars ($30,000,000), less any amounts paid pursuant to the foregoing (i) or (ii) in this Section 1.6(c); provided that in the event a clinical study that was not initially viewed to be a Phase 3 Clinical Study of a Royalty-Bearing Product but the results of such Clinical Study serve as the basis for the submission of an NDA or its foreign equivalent, then this milestone shall be deemed to have been achieved upon the submission of such NDA or its foreign equivalent and the corresponding milestone shall be paid within [***] days thereafter.

For the avoidance of doubt, the combined total amount payable under Sections 1.6(c)(i), 1.6(c)(ii) and 1.6(c)(iii) shall not exceed thirty million Dollars ($30,000,000).

(iv) within [***] days after [***] Royalty-Bearing Product, [***] Dollars ($[***]);

(i) an amount equal to (i) thirty percent (30%) of the cash value of consideration received (net of any reasonable banker's fee, commission or other similar fee payable upon such sale) upon sale by the Buyer of a Priority Review Voucher to a Third Party in an arms' length transaction, which amount shall be paid within [***] Business Days after the closing of such sale, or if such consideration is received in installments, within [***] Business Days after the receipt of each such installment; or (ii) twenty-five million Dollars ($25,000,000) if a Priority Review Voucher is retained to be used by Buyer or its Affiliate (including, after the Closing, the Company), which decision to retain such Priority Review Voucher must be made within [***] ([***][***] of the issuance of such Priority Review Voucher, and which amount shall be paid within [***] days after such decision;

(ii) within [***)] days after the [***], [***] Dollars ($[***]);

(iii) commercialization milestones as follows, in each case within [***] days after the close of the Calendar Year in which such milestone occurs:

(1) [***] Dollars ($[***]), after the first occurrence of aggregate worldwide Net Sales of at least [***] Dollars ($[***]) of Royalty-Bearing Products during any Calendar Year;

(2) [***] Dollars ($[***]), after the first occurrence of aggregate worldwide Net Sales of at least [***] Dollars ($[***]) of Royalty-Bearing Products during any Calendar Year; and

(3) [***] Dollars ($[***]), after the first occurrence of aggregate worldwide Net Sales of at least [***] Dollars ($[***]) of Royalty-Bearing Products during any Calendar Year;

(iv) subject to Section 1.6(f), royalty payments on Net Sales of Full Royalty Products as follows, based on worldwide aggregate annual Net Sales of all Royalty-Bearing Products, such payment to be made in each case within [***] days after the close of the applicable fiscal [***], with royalties to be paid at each of the following tiers and pursuant to this Section 1.6(c)(viii) and Section 1.6(c)(ix), to be calculated according to the principles set forth in Exhibit C:

Annual Net Sales of all Royalty-Bearing Products	**Royalty Rate Paid on the Portion of Annual Net Sales of Full Royalty Products**
Portion up to and including $[***]	[***]%
Portion greater than $[***] and less than or equal to $[***]	[***]%
Portion greater than $[***]	[***]%

On or about the date Buyer makes each royalty payment in accordance with this Section 1.6(c)(viii), Buyer shall submit a royalty report to the Representative showing the calculation of Net Sales for the applicable fiscal [***] on a country-by-country basis, including gross sales of Full Royalty Products by Selling Parties, a listing of applicable deductions, and the total royalty payment due for such fiscal [***].

(v) subject to Section 1.6(f), royalty payments on Net Sales of Reduced Royalty Products as follows, based on worldwide aggregate annual Net Sales of all Royalty-Bearing Products, such payment to be made in each case within [***] days after the close of the applicable fiscal [***], with royalties to be paid at each of the following tiers and pursuant to Section 1.6(c)(viii) and this Section 1.6(c)(ix), to be calculated according to the principles set forth in Exhibit D:

Annual Net Sales of all Royalty-Bearing Products	**Royalty Rate Paid on the Portion of Annual Net Sales of all Reduced Royalty Products**
Portion up to and including $[***]	[***]%
Portion greater than $[***] and less than or equal to $[***]	[***]%
Portion greater than $[***]	[***]%

On or about the date Buyer makes each royalty payment in accordance with this Section 1.6(c)(ix), Buyer shall submit a royalty report to the Representative showing the calculation of Net Sales for the applicable fiscal [***] on a country-by-country basis, including gross sales of all Reduced Royalty Products by Selling Parties, a listing of applicable deductions, and the total royalty payment due for such fiscal [***].

(d) In the event (and only in the event) this Agreement was entered into as a result of delivery of a Notice of Interest after the Interim Option Deadline, then, upon the achievement of any of the following set forth in this Section 1.6(d) by or on behalf of Buyer or any of its Affiliates or Licensees with respect to a Royalty-Bearing Product, Buyer shall pay the Milestone Payment set forth below corresponding to such milestone, Company's portion of the net Priority Review Voucher proceeds, and the applicable Royalty Payments. If more than one of the commercialization milestones set forth below is achieved in the same fiscal year, then all Milestone Payments for all such milestones shall be paid at the end of such Calendar Year:

(i) within [***] days after the [***] Royalty-Bearing Product, [***] Dollars ($[***]);

(ii) an amount equal to (i) [***] of the cash value of consideration received (net of any reasonable banker's fee, commission or other similar fee payable upon such sale) upon sale by the

Buyer of a Priority Review Voucher to a Third Party in an arms' length transaction, which amount shall be paid within [***] Business Days after the closing of such sale, or if such consideration is received in installments, within [***] Business Days after the receipt of each such installment; or (ii) [***] Dollars ($[***]) if a Priority Review Voucher is retained to be used by Buyer or its Affiliate (including, after the Closing, the Company), which decision to retain such Priority Review Voucher must be made within [***] ([***]) [***] of the issuance of such Priority Review Voucher, and which amount shall be paid within [***] days after such decision;

(iii) within [***] days after [***], [***] Dollars ($[***]);

(iv) commercialization milestones as follows, in each case within [***] days after the close of the Calendar Year in which such milestone occurs:

(1) [***] Dollars ($[***]), after the first occurrence of aggregate worldwide Net Sales of at least [***] Dollars ($[***]) of Royalty-Bearing Products during any Calendar Year;

(2) [***] Dollars ($[***]), after the first occurrence of aggregate worldwide Net Sales of at least [***] Dollars ($[***]) of Royalty-Bearing Products during any Calendar Year; and

(3) [***] Dollars ($[***]), after the first occurrence of aggregate worldwide Net Sales of at least [***] Dollars ($[***]) of Royalty-Bearing Products during any Calendar Year;

(v) subject to Section 1.6(f), royalty payments as follows, based on worldwide aggregate annual Net Sales of all Royalty-Bearing Products, such payment to be made in each case within [***] days after the close of the applicable fiscal [***]:

Annual Net Sales of Royalty-Bearing Products	**Royalty Rate Paid on the Portion of Annual Net Sales of Royalty-Bearing Products**
Portion up to and including $[***]	[***]%
Portion greater than $[***] and less than or equal to $[***]	[***]%
Portion greater than $[***]	[***]%

On or about the date Buyer makes each royalty payment in accordance with this Section 1.6(d)(v), Buyer shall submit a royalty report to the Representative showing the calculation of Net Sales for the applicable fiscal [***] on a country-by-country basis, including gross sales of Royalty-Bearing Products by Selling Parties, a listing of applicable deductions, and the total royalty payment due for such fiscal [***].

(e) The Milestone Payments, Priority Review Voucher Payments, and Royalty Payments (if any) are intended to be treated as [***] and any corresponding provisions of state, local, or non-U.S. law. If any Milestone Payment, Priority Review Voucher Payments, or Royalty Payment is actually paid by Buyer, interest may be imputed on such amount, as required by Section 483 or 1274 of the Code.

(f) Additional Royalty Provisions.

(i) In no event shall Milestone Payments, Priority Review Voucher Payments, or Royalty Payments be due and payable under more than one of Section 1.6(b), Section 1.6(c) and Section 1.6(d), nor shall any Milestone Payment be due more than once under any subsection of any such Section.

(ii) By way of example for royalties payable under Section 1.6(d)(v), if in a Calendar Year the worldwide aggregate annual Net Sales of Royalty-Bearing Products for which Royalty Payments are due under such Section were $2,000,000,000, and no reductions pursuant to Section 1.6(f)(iv) apply, then the Royalty Payment for the Calendar Year would be: (([***]% x $[***) + ([***]% x $[***]) + ([***]% x $[***])) = $[***] + $[***] + $[***] = $[***]. An analogous calculation shall apply to Royalty Payments due under Section 1.6(b)(vi) or Section 1.6(c)(viii) and Section 1.6(c)(ix) (as applicable).

(iii) Subject to this Section 1.6(f)(iii), on a Royalty-Bearing Product-by-Royalty-Bearing Product and country-by-country basis, the Royalty Payments due under Section 1.6(b)(vi), Section 1.6(c)(viii) and Section 1.6(c)(ix) (as applicable), or Section 1.6(d)(v), will be payable on aggregate annual Net Sales of a Royalty-Bearing Product in a country during the period commencing on the First Commercial Sale of such Royalty-Bearing Product in a country until the latest of (A) expiration of the last Valid Claim within the Company Intellectual Property described in clause (a) of the definition of Royalty-Bearing Product, (B) [***] after First Commercial Sale of such Royalty-Bearing Product in such country, or (C) expiration of all Regulatory Exclusivities for such Royalty-Bearing Product in such country (the "Royalty Term").

(iv) Reductions.

(1) If, at any time during the Royalty Term for a Royalty-Bearing Product, Generic Competition exists in a given country with respect to a Reference Product, then Buyer may reduce the royalties due hereunder for such Reference Product [***] by [***] for so long as such Generic Competition exists.

(2) If during the Royalty Term for a given Royalty-Bearing Product in a particular country, there is no Valid Claim within the Company Intellectual Property within such country, then, as from the date this Section 1.6(f)(iv)(2) applies, the applicable royalty rate for Net Sales of such Royalty-Bearing Product otherwise due pursuant to Section 1.6(b)(vi), Section 1.6(c)(viii) and Section 1.6(c)(ix) (as applicable), or Section 1.6(d)(v) shall be reduced by [***].

(3) If after the Effective Date, Company, Buyer or a Selling Party determines that it is in the best interest of one or more Selling Parties to obtain one or more licenses to Third Party patent rights or other intellectual property so as to prevent a claim by a Third Party that the Development, Commercialization or Manufacturing of a Royalty-Bearing Product infringes the intellectual property of a Third Party and a Selling Party obtains any such license, Buyer and its Affiliates may deduct from any royalty payment made hereunder up to an amount equal to [***] of the royalties paid to such

Third Party licensors for the use, manufacture, import, export or sale of such Royalty-Bearing Product in such country up to an amount equal to [***] of the royalties owed for any fiscal [***] hereunder. No Third Party payments that exceeded the amount eligible for a deduction in any fiscal [***] may be carried over into any succeeding period.

(4) Notwithstanding anything in this Agreement to the contrary, under no circumstances shall the reductions set forth in this Section 1.6(f)(iv) cause the total royalties due in any financial [***] to be reduced by more than [***] of the amount that would otherwise be due without giving effect to this Section 1.6(f)(iv).

(v) Only one royalty will be due with respect to the sale of the same unit of Royalty-Bearing Product. Only one royalty will be due hereunder on the sale of a Royalty-Bearing Product even if the manufacture, use, sale, offer for sale, or importation of such Royalty-Bearing Product would otherwise infringe more than one claim of the Patents within the Company Intellectual Property.

(vi) With respect to Net Sales of Royalty-Bearing Products invoiced in Dollars, the Royalty Payments will be expressed in Dollars. When conversion of payments from any foreign currency is required to be undertaken by Buyer, the Dollar equivalent will be calculated using the average exchange rate for such currency to Dollars over the applicable fiscal [***] as reported by OANDA (www.OANDA.com), or another resource agreed upon by Buyer and the Representative.

(vii) Buyer shall keep and maintain, and shall require all of its Licensees, to keep and maintain complete, accurate, and continuous records for a period of [***] from the date of creation, which show the manufacture, transfer, internal use, and other disposition of Royalty-Bearing Products. Such records shall include general ledger records showing cash receipts and expenses, and records which include production records, customers, and related information, in each case to the extent necessary and in sufficient detail to determine the amounts payable hereunder. Buyer shall permit a nationally or regionally recognized firm reasonable access annually during and within [***] after the end of the Royalty Term, to audit during ordinary business hours and on reasonable notice, such records for the prior [***] as may be necessary to verify or determine royalties or other payments paid or payable under this Agreement; provided that each year shall be subject to no more than one audit. Buyer shall pay the Company unpaid amounts due hereunder, plus interest as set forth in Section 1.6(g), within [***] days after receiving a written audit report. Notwithstanding the foregoing, Buyer shall have the right to reasonably dispute all or a portion of such written audit report by providing written notice and reasonable grounds for such dispute within [***] days after receiving such written audit report. In such event, the Parties shall use good faith efforts to promptly resolve such dispute. The Representative shall pay the cost and expense of the audit unless the agreed-upon results of the audit reveal an under-reporting or an underpayment due to the Company of [***] or more, in which case Buyer shall reimburse the Company for the reasonable out-of-pocket costs and expenses of the audit within [***] days after receipt of an invoice.

(g) All amounts due to the Representative shall be made in Dollars. The Milestone Payments, Priority Review Voucher Payments, Royalty Payments, and other payments set forth in this Agreement shall, if overdue, bear interest until payment at the monthly rate of [***] per month or the maximum amount permitted under law, whichever is less. The acceptance of the payment of such interest shall not foreclose the Company from exercising any other rights or remedies it may have.

(h) Diligence Obligations.

(i) Buyer will use Commercially Reasonable Efforts to Develop and Commercialize a Royalty-Bearing Product. Such Development and Commercialization may be performed directly by Buyer or by subcontractors and Licensees. Not later than [***] days after each [***] anniversary of the Effective Date and lasting until the [***], Buyer shall provide the Representative with reasonably detailed written reports of [***], including without limitation an update of (i) [***], (ii) [***], (iii) [***], and (iv) [***] and Buyer shall make relevant personnel reasonably available to the Representative during normal business hours by phone to answer questions regarding the most recent report. Not later than [***] days after receipt of such reports from Buyer, Representative shall forward such reports to the Unitholders, which reports will be Confidential Information for purposes of Section 6.2. The Company and the Unitholders acknowledge that any potential obligation to pay Milestone Payments, Priority Review Voucher Payments, or Royalty Payments will not create any express or implied obligation for Buyer to operate the Business in any particular manner in order to maximize such Milestone Payments, Priority Review Voucher Payments, or Royalty Payments. Without limiting the generality of the foregoing, Buyer shall have no liability to the Company or the Unitholders, and the Company, the Representative and the Unitholders shall not be entitled to make any claim for lost Milestone Payments, Priority Review Voucher Payments, or Royalty Payments if Buyer used Commercially Reasonable Efforts to Develop and Commercialize a Royalty-Bearing Product. Notwithstanding the foregoing, Buyer may cease to Develop or Commercialize any or all Royalty-Bearing Products, and thereafter shall comply with the provisions of Section 1.6(i) below.

(ii) From time to time after the Closing, Buyer may publish information regarding the utility of Biomarkers in the identification, testing, response, safety monitoring or treatment of Angelman Syndrome.

(iii) EACH PARTY HEREBY DISCLAIMS ANY REPRESENTATION OR WARRANTY THAT THE RESEARCH, DEVELOPMENT, MANUFACTURE, OR COMMERCIALIZATION OF ANY PRODUCT OR COMPONENT THEREOF WILL BE SUCCESSFUL.

(i) Buyer shall give Representative written notice within [***] days) after the date of Cessation. Buyer hereby grants to Representative (i) an exclusive first option during the Negotiation Period to acquire an exclusive, worldwide, field limited, royalty-bearing license on commercially reasonable terms to practice and commercially exploit the Intellectual Property Controlled by Buyer that is necessary to Develop, Commercialize or Manufacture Royalty-Bearing Products and is exclusively used for such purpose(s) and (ii) a nonexclusive, worldwide, field limited, royalty-bearing license on commercially reasonable terms to practice and commercially exploit the Intellectual Property Controlled by Buyer that is necessary to Develop, Commercialize or Manufacture Royalty-Bearing Products and is nonexclusively used for such purpose(s). Buyer and Representative shall negotiate in good faith to determine the terms of such license. If Buyer and Representative are unable to agree on a license agreement during the Negotiation Period, Buyer shall have no obligation to Representative under this Section 1.6(i).

Section I.7 Representative.

(a) By virtue of the execution of this Agreement or acceptance of any consideration pursuant to this Agreement, each Unitholder irrevocably nominates, constitutes and appoints the Representative as the true and lawful agent and attorney-in-fact of such Unitholder, with full power in his, her or its name and on his, her or its behalf to act according to the terms of the Transaction Documents

in the discretion of the Representative, and to do all things and to perform all acts, including (1) amending the Transaction Documents, (2) waiving rights, (3) discharging liabilities and obligations, (4) making all decisions relating to the determination of the Definitive Initial Purchase Consideration pursuant to Section 1.3, (5) determining, disputing and facilitating the disbursement of the Milestone Payments, the Priority Review Voucher Payments, or Royalty Payments, (6) defending and settling of any claims under Section 7.6, (7) facilitating the disbursement of the Escrow Amount (or any portion thereof) in accordance with this Agreement and the Escrow Agreement, (8) receiving from Buyer and distributing to the respective Unitholders any written reports delivered by Buyer pursuant to Section 1.6(h), and (9) executing and delivering all agreements, certificates, receipts, instructions and other instruments contemplated by, or deemed advisable in connection with, the Transaction Documents. Any such actions taken by the Representative on behalf of the Unitholders as provided hereunder shall be binding on all Unitholders, and Deborah A. Guagliardo hereby accepts such appointment. This power of attorney and all authority hereby conferred is coupled with an interest, is granted in consideration of the mutual covenants and agreements made herein, shall be irrevocable and shall not be terminated by any act of any one or more Unitholders, or by operation of Law, whether by death or other event. As to TAMUS, the Representative must obtain written approval to defend or settle any claims of TAMUS. Furthermore, the power of attorney provided to the Representative by TAMUS is revocable and subject to termination.

(b) All decisions and actions by the Representative, including any agreement between the Representative and Buyer relating to the determination of the Definitive Initial Purchase Consideration pursuant to Section 1.3, the determination, dispute and facilitating the disbursement of the Milestone Payments, the Priority Review Voucher Payments, or Royalty Payments, or the defense or settlement of any claims for which the Unitholders may be required to indemnify the Buyer Indemnified Parties pursuant to Section 7.6 hereof as well as facilitating the disbursement of all or any portion of the Escrow Amount pursuant to this Agreement and the Escrow Agreement in respect thereof, shall be binding upon the Unitholders, and no Unitholder shall have the right to object, dissent, protest or otherwise contest the same. As to TAMUS, the Representative must obtain written approval to defend or settle any claims of TAMUS.

(c) Each Unitholder agrees that: (i) Buyer and its Affiliates shall be able to rely conclusively on the instructions and decisions of the Representative as to the determination of the Definitive Initial Purchase Consideration pursuant to Section 1.3, the determination, dispute and facilitating the disbursement of the Milestone Payments, the Priority Review Voucher Payments, or Royalty Payments, and the settlement of any claims for indemnification by a Buyer Indemnified Party pursuant to Section 7.6 hereof, facilitating the disbursement of the Escrow Amount (or any portion thereof) via the Representative pursuant to the Escrow Agreement or any other actions required to be taken by the Representative under the Transaction Documents, and no Unitholder shall have any cause of action against any Buyer Indemnified Party for any action taken by such Person in reliance upon the instructions or decisions of the Representative; (ii) Buyer shall be required to file and negotiate any claims or disputes related to or in connection with the Transaction Documents, including the determination of the Estimated Initial Purchase Consideration and Definitive Initial Purchase Consideration pursuant to Section 1.3, the determination, dispute and disbursement of the Milestone Payments, the Priority Review Voucher Payments, or Royalty Payments, or indemnification by the Unitholders pursuant to Section 7.6 hereof, only with the Representative (on behalf of the Unitholders) and not with any Unitholder; (iii) all actions, decisions and instructions of the Representative shall be conclusive and binding upon all Unitholders and no Unitholder shall have any cause of action against the Representative, and the Representative shall have no liability to any Unitholder for any action taken or omitted, decision made or

instruction given by the Representative under or in connection with this Agreement, the Escrow Agreement or any other agreements entered into in connection with the transactions contemplated by this Agreement, except for fraud, bad faith, gross negligence or willful misconduct by the Representative; (iv) the provisions of this Section 1.8 are independent and severable, are irrevocable and coupled with an interest and shall be enforceable notwithstanding any rights or remedies that any Unitholder may have in connection with the transactions contemplated hereby; and (v) the provisions of this Section 1.7 shall be binding upon the executors, heirs, legal representatives and successors of each Unitholder, and any references in this Agreement to a Unitholder shall mean and include the successors to such Unitholder's rights hereunder, whether pursuant to testamentary disposition, the laws of descent and distribution or otherwise. As to TAMUS, the Representative must obtain written approval to defend or settle any claims of TAMUS.

(d) The Unitholders will indemnify, defend and hold harmless the Representative from and against any losses, liabilities, damages, claims, penalties, fines, forfeitures, actions, fees, costs and expenses (including the fees and expenses of counsel and experts and their staffs and all expense of document location, duplication and shipment) (collectively, "Representative Losses") arising out of or in connection with the Representative's enforcement of its rights under this Agreement, the Escrow Agreement or any other agreement entered into in connection with the transactions contemplated by this Agreement, in each case as such Representative Loss is suffered or incurred and (i) is actually caused by the fraud, gross negligence or willful misconduct of the Unitholders or the Company or (ii) relates directly to a dispute (including a claim for indemnification) between Buyer and the Unitholders. If not paid directly to the Representative by the Unitholders, any such Representative Losses may be recovered by the Representative from (i) the funds in the Representative's Fund; (ii) any Escrow Amounts at such time as any such amounts would otherwise be distributable to the Unitholders; and (iii) any Milestone Payments, Priority Review Voucher Payments, or Royalty Payments at such time as any such amounts would otherwise be distributable to the Unitholders; provided, that while this Section 1.7 allows the Representative to be paid from each of the aforementioned sources of funds, this does not prevent the Representative from seeking any remedies available to it at law or otherwise. The Unitholders acknowledge and agree that the foregoing indemnities will survive the resignation or removal of the Representative or the termination of this Agreement.

(e) At the Closing, Buyer shall deliver [***] Dollars ($[***]) to the Representative (the "Representative's Fund"), which Representative's Fund shall be maintained by the Representative in a segregated client account. The Representative's Fund shall be used for the purposes of paying directly, or reimbursing the Representative for, any Third Party expenses in connection with the transactions contemplated by this Agreement. The Unitholders will not receive any interest or earnings on the Representative's Fund and irrevocably transfer and assign to the Representative any ownership right that they may otherwise have had in any such interest or earnings. The Representative will hold these funds separate from its corporate funds, will not use these funds for its operating expenses or any other corporate purposes and will not voluntarily make these funds available to its creditors in the event of bankruptcy. As soon as practicable following the completion of the Representative's responsibilities, the Representative will distribute the balance of the Representative's Fund to the Unitholders in accordance with the Allocation Schedule. The Representative may make earlier distributions of such portions of the Representative's Fund to the Unitholders to the extent the Representative determines, in its sole discretion, such portions are no longer required to be retained. In the event the Representative determines, in its sole discretion, that the Representative's Fund is insufficient to satisfy expenses that are reasonably likely to be incurred, each Unitholder hereby authorizes the Representative to withhold

amounts reasonably anticipated to be required from the disbursement of Milestone Payments, Priority Review Voucher Payments, and Royalty Payments. The Unitholders acknowledge that the Representative is not providing any investment supervision, recommendations or advice. The Representative shall have no responsibility or liability for any loss of principal of the Representative's Fund other than as a result of its bad faith or fraud. For Tax purposes, the Representative's Fund shall be treated as having been received and voluntarily set aside by the Unitholders at the time of the Closing. Notwithstanding anything to the contrary herein, Buyer shall have no liability or obligation to any Person in connection with, or relating to, the Representative's Fund.

(f) If Deborah A. Guagliardo resigns or subsequently becomes unable to serve as the Representative, the successor determined by operation of the next sentence shall, upon written notice to Buyer, become the Representative for all purposes hereunder. If, at any time, there is no Representative hereunder, the written agreement of Unitholders representing at least fifty-one percent (51%) of the outstanding Voting Units (as defined in the Operating Agreement) as of immediately prior to the Closing shall be sufficient to appoint a new Representative, and upon the approval by Buyer of such Representative, such appointment will be binding on all Unitholders, provided that Buyer may, after a reasonable time period during which there is no Representative hereunder, appoint a Person as Representative upon written notice to the Unitholders. If the Buyer appoints a Person as Representative in such fashion, the Unitholders may replace such Representative at any time (i) by the written agreement of Unitholders representing at least fifty-one percent (51%) of the outstanding Voting Units (as defined in the Operating Agreement) as of immediately prior to the Closing and (ii) upon the approval by the Buyer of such Representative (not to be unreasonably withheld). Notwithstanding anything to the contrary in this Agreement, no change in the identity of the Representative shall be effective unless and until Buyer receives notice of such change in accordance with Section 9.1.

Section I.8 Withholding. Buyer, the Company, the Representative and their agents shall be entitled to deduct and withhold from any amounts payable hereunder any amounts it may be required to deduct and withhold under any applicable Tax Law. Amounts withheld under this Section 1.8 shall be treated for all purposes of this Agreement as having been paid to the Person in respect of which such deduction and withholding was made.

Section I.9 Tax Treatment.

(a) For Tax purposes the parties agree to treat the exercise of the Option as the purchase of the Units in respect of which the Option was exercised by Buyer in exchange for such Unitholders' rights to a share of the Definitive Initial Purchase Consideration, the Escrow Amount, the Milestone Payments, the Priority Review Voucher Payments, the Royalty Payments and the Representative's Fund, each subject to any adjustments as provided for pursuant to the terms of this Agreement. For the avoidance of doubt, the Milestone Payments, the Priority Review Voucher Payments, and Royalty Payments shall be treated as contingent deferred consideration for the purchase of the Units. Furthermore, solely for U.S. income Tax purposes, the parties agree to treat Buyer as the owner of the Escrow Amount and agree that all interest and earnings earned from the investment and reinvestment thereof, or any portion thereof, shall be allocated to Buyer pursuant to Section 468B(g) of the Code and Proposed Treasury Regulation Section 1.468B-8 (and any comparable provision of state or local law). Buyer shall be entitled to receive distributions from the Escrow Amount on a quarterly basis in an amount equal to [***]%) of any such interest and earnings allocated to Buyer. Unless otherwise required by Applicable Law, the parties agree, that they and their Affiliates shall (A) prepare and file all Tax Returns

required to be filed by the parties or their Affiliates in a manner consistent with the foregoing, and (B) take no position on any Tax Return, or in any audit or other proceeding with respect to Taxes in a manner that is inconsistent with the foregoing, except as to TAMUS, which is an agency of the State of Texas and is required to follow the accounting policies and procedures for an agency of the State of Texas.

(b) Notwithstanding anything in Section 1.9(a) to the contrary, the parties will not be precluded from complying with accounting practices consistently applied (including to capitalize, amortize or expense any payments hereunder for accounting purposes), as reasonably determined in good faith by each respective party or their respective advisors, even if inconsistent with Section 1.9(a).

Section I.10 Purchase Price Allocation. Buyer shall prepare a draft IRS Form 8594, allocating the Option Premium, Purchase Consideration and all other relevant items, as determined for U.S. federal income Tax purposes, to the assets of the Company in accordance with the allocation methodology attached hereto as Schedule 1.10, which methodology has been mutually agreed to by the parties hereto, and which the parties agree is consistent with Code Section 1060 and the Treasury Regulations promulgated thereunder (and any similar provisions of state, local, or non-U.S. Law). Buyer shall deliver such draft IRS Form 8594 to Representative no later than [***] days after the final determination of the Purchase Consideration in accordance with this Section 1.10. Buyer shall timely file IRS Form 8594 in accordance with such draft IRS Form 8594 and the Company, and the Unitholders and Representative, shall file all other Tax Returns in a manner consistent with such draft IRS Form 8594. Neither Buyer nor any of the Unitholders shall take any position for Tax purposes (whether in audits, Tax Returns, or otherwise) that is inconsistent with such final IRS Form 8594 unless otherwise required by applicable Law, provided that the parties may use a different methodology for financial reporting purposes.

ARTICLE II

REPRESENTATIONS AND WARRANTIES OF THE COMPANY

The Company represents and warrants to Buyer as of the date hereof as follows, except as disclosed by the Company in the written Disclosure Schedule provided to Buyer in accordance with the Unitholder Option Agreement and attached hereto as Schedule B (the "Disclosure Schedule"). The Disclosure Schedule shall be arranged in sections and subsections corresponding to the numbered and lettered sections and subsections contained in this Article II. The disclosures in any section or subsection of the Disclosure Schedule corresponding to any section or subsection of this Article II shall qualify other sections and subsections in this Article II if indicated by cross-references to such other sections and subsections or if the applicability of such disclosure to any other applicable representation or warranty is readily apparent from the text of such disclosure.

Section II.1 Due Organization. The Company is a limited liability company duly organized, validly existing and in good standing under the laws of the State of Delaware. The Company is duly qualified to transact business as a foreign limited liability company and is in good standing in each of the jurisdictions listed in Section 2.1(a) of the Disclosure Schedule, which jurisdictions are the only ones in which the ownership or leasing of the Company's assets or properties or the conduct of the Business requires such qualification, except where the failure to be so qualified or in good standing would not have a Material Adverse Effect. The Company has full limited liability company power and authority to own or lease and to operate and use its assets and properties and to carry on its business as now conducted. Except as set forth in Section 2.1(b) of the Disclosure Schedule, true and complete copies of (a) the

Operating Agreement and other organizational documents and all amendments thereto and (b) the minute books of the Company since the formation of the Company, have been delivered or made available to Buyer. The Company is not in Default under, or in violation of, any provision of the Operating Agreement or other organizational documents, each as amended and in effect as of the date hereof.

Section II.2 Authorization; No Conflict.

(a) The Company has full limited liability company power and authority to execute, deliver and perform its obligations under this Agreement and the other Transaction Documents to which the Company is a party. The execution, delivery and performance by the Company of this Agreement and any other Transaction Documents to which the Company is a party have been duly authorized and approved by all requisite limited liability company action and do not require any further authorization or consent of the Company or its members or board of managers. This Agreement has been duly authorized, executed and delivered by the Company and is the legal, valid and binding obligation of the Company enforceable in accordance with its terms, except as enforceability may be limited by applicable bankruptcy, insolvency, reorganization, moratorium or similar Applicable Laws affecting the enforcement of creditors' rights generally and by the effect of general principles of equity (regardless of whether enforcement is considered in a proceeding in equity or at law).

(b) Except as set forth in Section 2.2(b) of the Disclosure Schedule, neither the execution and delivery by the Company of this Agreement or any other Transaction Documents to which the Company is a party nor the consummation of any of the transactions contemplated hereby or thereby, nor compliance with or fulfillment of the terms, conditions and provisions hereof or thereof, in each case by the Company, will:

(i) conflict with, result in a Default, or an event creating rights of acceleration, termination, modification or cancellation or a loss of rights under, any Contract to which the Company is a party or by which the Company is bound or to which any of its properties and assets are subject (including any Material Contract) or any Permit affecting the properties, assets or the Business;

(ii) result in the creation or imposition of any Lien upon any of the properties or assets of the Company;

(iii) conflict with or result in a Default under, any provision of the Operating Agreement or other organizational documents of the Company;

(iv) conflict with or result in a violation or breach of any Applicable Law or any Court Order to which the Company is a party; or

(v) require the approval, consent, authorization or act of, or the making by the Company of any declaration, filing, notice or registration with, any Person.

(c) The board of managers of the Company at a meeting duly called and held, or by written consent in lieu thereof, has unanimously approved this Agreement and the other Transaction Documents to which the Company is a party and the transactions contemplated hereby and thereby.

Section II.3 Capitalization.

(a) Section 2.3(a) of the Disclosure Schedule accurately sets forth the capitalization of the Company as of immediately prior to the Closing, including (i) the type and number of membership interests held by each member of the Company and (ii) the name of any holder or beneficial owner of, or of any Person having the right to acquire beneficial ownership of, any membership interests of, or any other voting or Equity Participation in the Company, in each case, as of immediately prior to the Closing. The Units represent all of the issued and outstanding equity interests of the Company and are duly authorized and validly issued. Except as set forth on Section 2.3(a) of the Disclosure Schedule, none of the issued and outstanding Units have been issued in violation of, or are subject to, any preemptive rights, rights of first offer, rights of first refusal or subscription rights. There are no declared and unpaid dividends or other distributions with respect to any Units. The rights, privileges and preferences of the Units are as stated in the Operating Agreement. No claim has been made or, to the Knowledge of the Company, threatened against the Company asserting that any Person other than a Person listed on Section 2.3(a) of the Disclosure Schedule is the holder or beneficial owner of, or has the right to acquire beneficial ownership of, any equity interests of, or any other voting right or Equity Participation in the Company.

(b) Other than as set forth in Section 2.3(b) of the Disclosure Schedule, there are no agreements, arrangements, options, warrants, calls, rights or commitments of any character relating to the issuance, sale, purchase or redemption of any membership interests or other equity interests of the Company, whether on conversion of other securities or otherwise. Except for the Operating Agreement and the Transaction Documents and as set forth in Section 2.3(b) of the Disclosure Schedule, the Company is not a party to any, and to the Knowledge of the Company there exists no member agreement, voting trust agreement or any other similar contract, agreement, arrangement, commitment, plan or understanding restricting or otherwise relating to the voting, dividend, ownership or transfer rights of any membership interests of the Company.

(c) Section 2.3(c) of the Disclosure Schedule lists each Contract of the Company relating to the Company's Profits Interests. The Company has provided to Buyer a true and accurate copy of each Contract identified on Section 2.3(c) of the Disclosure Schedule.

(d) The Company does not currently own or control, directly or indirectly, any interest in any other corporation, partnership, trust, joint venture, limited liability company, association, or other business entity. The Company is not a participant in any joint venture, partnership or similar arrangement.

Section II.4 Financial Statements.

(a) Section 2.4(a) of the Disclosure Schedule attaches the following financial statements (the "Financial Statements"): true, complete and correct copies of the Company's (a) audited balance sheet and income statement as of December 31, 2020 and December 31, 2021and for the year then ended and (b) unaudited balance sheets and income statements as of June 30, 2022and for the six-month period then ended. Each Financial Statement (including the notes thereto) has been prepared from the books and records of the Company and in accordance with GAAP (except that unaudited interim financial statements are subject to normal and recurring year-end adjustments which will not be material in amount or effect and do not include footnotes) and fairly presents in all material respects the financial condition and results of operations of the Company as of the dates, and for the periods, indicated thereon. Section 2.4(a) of the Disclosure Schedule contains a true, correct and complete list of all of the Company

Indebtedness as of the date of this Agreement and identifies for each item of Company Indebtedness the outstanding principal and accrued but unpaid interest as of the date of this Agreement.

(b) The Company has provided Buyer the audit reports of the Company's auditors in respect of the audited Financial Statements. The Company has not withheld any material information from the auditors. No auditor of the Company has ever declined or indicated its inability to issue an opinion with respect to any financial statements of the Company.

(c) The Company maintains adequate internal accounting controls that are designed to ensure that: (i) material transactions are executed with management's general or specific authorization; (ii) transactions are recorded as necessary to permit preparation of the financial statements of the Company in accordance with GAAP and to maintain accountability for the assets of the Company; and (iii) accounts, books and ledgers related to the Business are properly kept, and are accurate and complete in all material respects. There have been no instances of fraud by the Company or its officers, whether or not material, that occurred during any period covered by the Financial Statements.

Section II.5 No Undisclosed Liabilities. There are no liabilities whatsoever of the Company or facts or circumstances that would reasonably be expected to give rise to liabilities of the Company, whether accrued, contingent, absolute, determined, determinable, or otherwise, other than (a) liabilities fully recorded or reserved for in the balance sheet as of December 31, 2021, and (b) current liabilities incurred since December 31, 2021, in the ordinary course of business consistent with past practice and which are not material, except as set forth in Section 2.5 of the Disclosure Schedule. Except for Indebtedness reflected in Section 2.5 of the Disclosure Schedule which is to be paid off prior to Closing, Company does not have any Indebtedness outstanding at the date hereof. The Company is not in default with respect to any such Indebtedness or any instrument relating thereto.

Section II.6 Absence of Changes. Except as set forth in the Financial Statements or Section 2.6 of the Disclosure Schedule, since the Effective Date of the Unitholder Option Agreement, (a) the Company has conducted its business in the ordinary course of business, (b) there has not been any Material Adverse Effect and (c) there has not been any action which, if taken after the Effective Date of the Unitholder Option Agreement without Buyer's consent, would have violated the provisions of Section 5.1 or 5.2 of the Unitholder Option Agreement.

Section II.7 Real Property.

(a) The Company does not own, and has never owned, any Real Property.

(b) Section 2.7(b) of the Disclosure Schedule sets forth a true and complete list of all of the Company's Leased Real Property, if any, including the street address of each parcel of Leased Real Property, the landlord under the lease and the current use of such Leased Real Property. The Company has a valid leasehold interest in the Leased Real Property, free and clear of all Liens (other than Permitted Liens). Except as set forth on Section 2.7(b) of the Disclosure Schedule, no Person other than the Company has any right to use, occupy or lease all or any portion of the Leased Real Property. To the Knowledge of the Company, no Default has occurred in the due observance of any Permit applicable to the Leased Real Property.

Section II.8 Environmental Matters. The Company's conduct of the Business and the current use of the Leased Real Property are in compliance in all material respects, and has at all times been in compliance in all material respects, with all Environmental Laws. There are no facts which would give rise to non-compliance of the Company with any Environmental Laws, either in the conduct by the Company of its Business or in the current use of the Leased Real Property. There have been no disposal, releases or threatened releases of Hazardous Materials by the Company on, from or under the Leased Real Property except in compliance with Environmental Laws. The Company holds and is and has at all times been in compliance in all material respects with all Permits required pursuant to any Environmental Law necessary for the conduct of any Hazardous Materials Activities and other Business as such activities and business are currently being conducted.

Section II.9 Assets. The Company owns and has good and valid title to, or a valid leasehold interest in, or a valid and enforceable license to use, all of its material Assets, free and clear of all Liens (other than Permitted Liens). The Assets constitute all of the assets, properties and rights used by the Company for the conduct of the Business. All of the material tangible Assets have been maintained in a reasonably prudent manner and are in good operating condition and repair, ordinary wear and tear excepted.

Section II.10 Taxes.

(a) All Tax Returns required to be filed by the Company (i) have been duly and timely filed (taking into account any extension of time to file granted to or obtained by the Company) with the appropriate Governmental Authorities and (ii) were complete and correct in all material respects.

(b) All Taxes due and payable by the Company (whether or not shown as due on its Tax Returns) have been fully paid.

(c) The Company has deducted, withheld and timely paid to the appropriate Governmental Authority all Taxes required to be deducted, withheld or paid on behalf of another Person, and the Company has complied in all material respects with its reporting and recordkeeping obligations relating thereto.

(d) There are no audits, inquiries in writing, examinations, investigations, reassessments or litigations by any Governmental Authority relating to Taxes with respect to the Company pending or currently in progress, and no such audits, inquiries, examinations, investigations, reassessments or litigations or other Actions are, to the Knowledge of the Company, threatened or contemplated.

(e) All deficiencies asserted or assessments made against the Company as a result of any examinations by any taxing authority have been fully resolved and any Taxes due have been fully paid, and no assessment of Tax has been proposed in writing against the Company or any of its assets or property.

(f) No written claim has ever been made by an authority in a jurisdiction where the Company does not file Tax Returns that the Company is or may be subject to taxation by that jurisdiction that remains unresolved.

(g) The Company is not party to or bound by any closing agreement pursuant to Section 7121 of the Code (or any similar provision of any state, local or non-U.S. Law), offer in compromise or other agreement with any taxing authority.

(h) The Company has not waived any statute of limitations in respect of Taxes or agreed to any extension of time (other than any automatic extension) with respect to a Tax assessment or deficiency. There is no power of attorney given by or binding upon the Company with respect to Taxes for any period for which the statute of limitations (including any waivers or extensions) has not yet expired that is currently in effect.

(i) The Company has not participated in a "listed transaction" within the meaning of Treasury Regulation Section 1.6011-4(b) or any similar provision of state, local or foreign Tax Law, and the Company has not participated in a transaction that is described as a "reportable transaction" within the meaning of Treasury Regulations Section 1.6011-4(b)(1).

(j) There are no liens for Taxes (other than Taxes not yet due and payable) upon any of the assets of the Company.

(k) The Company is not a party to or bound by, and has no obligation under, any tax allocation or sharing or similar contract or arrangement or any agreement pursuant to which it will have any obligation to make any payments computed by reference to the Taxes, taxable income or taxable losses of any other Person after the Closing Date. The Company does not have any liability for the Taxes of any other Person, as a transferee or successor, or otherwise.

(l) The Company does not own any asset described in Section 197(f)(9) of the Code.

(m) The Company has never undergone prior ownership changes as defined in Section 382 of the Code.

(n) The Company has since its inception been properly treated as a partnership for U.S. federal income tax purposes. The Company uses the Calendar Year and the accrual method of accounting for income Tax purposes.

(o) The Company does not own any stock or other ownership interests in (i) any corporation which is a passive foreign investment company within the meaning of Section 1297 of the Code or a controlled foreign corporation within the meaning of Section 957 of the Code or (ii) any partnership, joint venture, limited liability company, or other entity taxed as a partnership or other pass-through entity for U.S. federal income tax purposes (or other arrangement or contract which could be treated as a partnership for U.S. federal income tax purposes).

(p) The Company will not be required to include any material amount in taxable income or exclude any item of deduction or loss from taxable income for any taxable period (or portion thereof) ending after the Closing Date as a result of (a) any change in method of accounting for a taxable period ending on or prior to the Closing Date; (b) use of an improper method of accounting for a taxable period ending on or prior to the Closing Date; (c) any "closing agreement" as described in Section 7121 of the Code (or any corresponding or similar provision of state, local or non-U.S. income Tax Law) executed on or prior to the Closing Date, (d) any installment sale or open transaction disposition made on or prior to

the Closing Date; (e) any prepaid amount received on or prior to the Closing Date; (f) any election made under Section 108(i) of the Code; (g) Tax incurred pursuant to Section 965 of the Code (or any corresponding or similar provision of state, local, or foreign Tax law); or (h) any similar election, action or agreement that would have the effect of deferring any liability for Taxes of the Company from any period ending on or before the Closing Date to any period ending after such date. The Company is not required to make any adjustment under Section 481(a) of the Code.

Section II.11 Employees and Labor Matters.

(a) Section 2.11(a) of the Disclosure Schedule sets forth a true and complete list of each employee of and any other individual service provider (including independent contractors) to the Company (including any Person on a leave of absence), with job title or position, primary work location, date of commencement of employment or service, current annual base salary rate and current target bonus opportunity. To the Knowledge of the Company, no such employee or service provider has any present intention to terminate his or her employment or service relationship with the Company. There are no collective bargaining agreements or other Contracts with any labor organization binding on the Company or any of its subsidiaries. The Company is not subject to any (i) unfair labor practice complaint pending before a Governmental Authority, (ii) pending or, to the Knowledge of the Company, threatened or anticipated labor strike, slowdown, work stoppage, picketing, lockout, or other organized labor disturbance, or (iii) labor unions, works councils or other organizations representing, or to the Knowledge of the Company, purporting or attempting to represent any employees of or other service provider to the Company. There are no labor disputes currently subject to any grievance procedure, arbitration or litigation and there is no representation petition pending or, to the Knowledge of the Company, threatened or anticipated with respect to any employee of the Company. The Company has not engaged in any unfair labor practices within the meaning of the National Labor Relations Act.

(b) Section 2.11(b) of the Disclosure Schedule also contains a complete and accurate list of all of the independent contractors, consultants, temporary employees, leased employees or other agents employed or used by the Company and classified by the Company as other than employees, or compensated other than through wages paid by the Company through the Company's payroll department ("Contingent Workers"), showing for each Contingent Worker such individual's role in the business, fee or compensation arrangements and other contractual terms with the Company.

(c) To the extent that any Contingent Workers are or were engaged by the Company, the Company currently classifies and has properly classified and treated them as Contingent Workers (as distinguished from Form W-2 employees) in accordance with applicable law and for the purpose of all employee benefit plans and perquisites.

(d) The Company (i) has not violated any Law or Contract relating to labor or employment in any jurisdiction, and no such violation is anticipated or (ii) is not involved in any Action in relation to any present, former or prospective employee, manager or consultant or any trade union or other employee representative body and, to the Knowledge of the Company, there is no such Action pending or threatened.

(e) The Company is in compliance in all material respects with Laws regarding employment and employment practices, workers' compensation, exempt and non-exempt status, worker classification, mass layoffs, terms and conditions of employment, worker safety, wages and hours, civil

rights, discrimination, immigration and collective bargaining. Without limiting the generality of the foregoing, there have been no claims of harassment, discrimination, retaliatory act or similar actions against any employee, officer, manager or other service provider of the Company since the Effective Date of the Unitholder Option Agreement, and to the Knowledge of the Company, no facts exist that could reasonably be expected to give rise to such claims or actions. To the Knowledge of the Company, no employees, independent contractors or other service providers of the Company are in any material respect in violation of any term of any employment contract, nondisclosure agreement, non-competition agreement, invention assignment agreement or any restrictive covenant to a former employer or service recipient relating to the right of any such employee, independent contractor or other service provider to be employed or retained by the Company because of the nature of the business or to the use of trade secrets or proprietary information.

(f) Each employee and Contingent Worker of the Company is subject to non-competition, non-solicitation, confidentiality and invention assignment obligations in favor of the Company, and Section 2.11(f) of the Disclosure Schedule lists each such Contract containing such non-competition, non-solicitation, confidentiality and invention assignment obligations, all of which Contracts have been provided to Buyer.

Section II.12 Employee Benefit Plans.

(a) Section 2.12(a) of the Disclosure Schedule sets forth a true, complete and correct list of all Company Benefit Plans. Each Company Benefit Plan is and has been established, maintained, operated, funded and administered in compliance in all material respects with its terms, ERISA, the Code, any other Applicable Law and the terms of any related documents or Contracts. The Company has made available to Buyer true, correct and complete copies of the plan documents or agreements underlying each Company Benefit Plan (or, to the extent no such copies exist, a description of the material terms) and the following, as applicable: (i) any amendments to the plan document or agreement; (ii) the three most recent (A) Form 5500 and attached schedules, (B) audited financial statements, and (C) actuarial valuation reports; (iii) summary plan description and summaries of material modifications thereto; (iv) determination or opinion letter, if any, received from the Internal Revenue Service; and (v) any material written communications to or from any Governmental Authority or summaries thereof. All contributions, distributions, reserves, reimbursements and premium payments required to be made with respect to each Company Benefit Plan have been timely made or have been properly accrued in accordance with the provisions of each of the Company Benefit Plans, Applicable Law and GAAP. There are no Actions or other proceedings pending or, to the Knowledge of the Company, threatened against or involving any Company Benefit Plan. No Company Benefit Plan is mandated by a government or is subject to the laws of a jurisdiction outside of the United States.

(b) Each Company Benefit Plan that is intended to be "qualified" under Section 401(a) of the Code is so qualified and has received a favorable determination or opinion letter from the Internal Revenue Service as to its qualified status, and the exempt status of its accompanying trust under Section 501(a) of the Code, and, to the Knowledge of the Company, no fact or event has occurred since the date of such letter that could adversely affect the qualified status of any such Company Benefit Plan or the exempt status of any such trust, or require corrective action under the IRS Employee Plans Compliance Resolution System in order to maintain such qualification.

(c) Since the Effective Date of the Unitholder Option Agreement, no Action (excluding claims for immaterial benefits incurred in the ordinary course) has been brought or is pending or, to the Knowledge of the Company, threatened or anticipated against or with respect to any Company Benefit Plan. There are no audits, inquiries, investigations or proceedings pending or, to the Knowledge of the Company, threatened, by the Internal Revenue Service, Department of Labor or other Governmental Authority with respect to any Company Benefit Plan. To the Knowledge of the Company, the Company has no liability by reason of an individual who performs or performed services for the Company in any capacity being improperly excluded from participating in a Company Benefit Plan. With respect to each Company Benefit Plan that is funded mostly or partially through an insurance policy, the Company has no material liability in the nature of retroactive rate adjustment, losing sharing arrangement or other actual or contingent liability arising wholly or partially out of events occurring on or before the Closing Date.

(d) No Company Benefit Plan is, and neither the Company nor any ERISA Affiliate has ever maintained, established, sponsored, participated in or contributed to (or had any obligation to contribute to) or otherwise has any current or contingent liability or obligation under or with respect to, (i) a "pension plan" within the meaning of Section 3(2) of ERISA or any other plan that is (or was within the prior six years) subject to Section 302 or Title IV of ERISA or Section 412 or 430 of the Code, (ii) a "multiemployer plan" within the meaning of Section 3(37) of ERISA, (iii) a "multiple employer plan" within the meaning of Section 413(c) of the Code, (iv) a "multiple employer welfare arrangement" within the meaning of Section 3(40) of ERISA, or (v) a "funded welfare benefit plan" (within the meaning of Section 419 of the Code. The Company does not have any current or contingent liability or obligation with respect to any "employee benefit plan" (as defined in Section 3(3) of ERISA) by reason of, at any time, being considered a single employer with any other Person under Section 414 of the Code, and neither the Company nor any ERISA Affiliate has ever incurred any liability under Title IV of ERISA that has not been paid in full.

(e) The Company does not have any current or contingent obligations for retiree health or life insurance or other welfare benefits (and no Company Benefit Plan has ever provided for such benefits), other than continuation coverage required under Section 4980B of the Code or Part 6 of Subtitle B of Title I of ERISA (or similar state law) for which the covered Person pays the full cost of coverage, and the Company has never promised to provide such post-termination benefits.

(f) Neither the Company nor, to the Knowledge of the Company, any Third Party, has engaged in any "prohibited transactions" (within the meaning of Section 406 of ERISA or Section 4975 of the Code) with respect to any Company Benefit Plan, other than any such transactions that are covered by a statutory or administrative exemption, and no such non-exempt "prohibited transaction" with respect to any Company Benefit Plan is reasonably expected to occur as a result of any action or inaction by the Company or, to the Knowledge of the Company, any Third Party.

(g) No Company Benefit Plan is or ever was a "non-qualified deferred compensation plan" as defined under Section 409A of the Code and the regulations thereunder. No Person is entitled to a gross-up, make-whole or other similar reimbursement payment from the Company in respect of any Tax (including federal, state, local and foreign income, excise and other Taxes (including Taxes imposed under Code Section 409A or 4999)) or interest or penalty related thereto. No payment to be made under any Company Benefit Plan is or, to Knowledge of the Company, will be, subject to the penalties of Section 409A(a)(1) of the Code.

(h) Neither the Company nor any of its officers or management-level employees has made any promises or commitments to create any employee benefit plan or agreement or modify or change any Company Benefit Plan. To the Knowledge of the Company, no event, condition or circumstance exists that could reasonably be expected to result in an increase in the benefits or compensation provided under any Company Benefit Plan or the expense of maintaining any Company Benefit Plan from the level of benefits or expense incurred for the most recent fiscal year ending before the Closing Date. To the Knowledge of the Company, no event, condition or circumstance exists that could prevent the amendment or termination of any Company Benefit Plan other than in accordance with its terms.

(i) Except as set forth on Section 2.12(i) of the Disclosure Schedule, no Company Benefit Plan contains any provision or is subject to any Law that, as a result of the execution of this Agreement or the consummation of the transactions contemplated hereby (either alone or in conjunction with any other event) or upon any related, concurrent, or subsequent termination of employment of any current or former employee, officer, manager or consultant of the Company, would (i) entitle any Person to any payment, forgiveness of indebtedness, funding or distribution under or with respect to any Company Benefit Plan, (ii) increase, accelerate or vest any compensation or benefit, (iii) trigger any obligation to fund any Company Benefit Plan, (iv) require severance, termination, change-in-control, retention or similar payments, (v) provide any term of employment or compensation guaranty, (vi) forgive any Company Employee Indebtedness, or (vii) result in any "parachute payment" (within the meaning of Section 280G of the Code, whether or not such payment is considered to be reasonable compensation for services rendered), or (viii) result in any requirement to pay any tax "gross-up" or similar "make-whole" payments to any current or former employee, officer, director or consultant of the Company.

(j) No Company Benefit Plan is subject to the laws of any jurisdiction outside the United States.

Section II.13 Compliance with Law; Permits.

(a) The Company has complied at all times, and is now complying, with all Applicable Law in all material respects, required for the conduct of its Business or by which any of its properties or assets are bound. Since its formation, the Company has not received any written communication (or, to the Knowledge of the Company, any other communication) from any Governmental Authority or Person alleging noncompliance with any Applicable Law.

(b) The Company owns or holds, and has owned or held at all times, all Permits necessary or required for the conduct of the Business and to own, lease and operate its properties and assets, and such Permits are valid and in full force and effect and are owned by the Company free and clear of all Liens except Permitted Liens. Section 2.13(b) of the Disclosure Schedule sets forth a true, correct and complete list of all Permits issued to the Company, including the names of the Permits and their respective dates of issuance and expiration, copies of which have been provided to Buyer. The Company is conducting, and has always conducted, its business in compliance in all material respects with the requirements, standards, criteria and conditions set forth in such Permits and the Company has not received notice regarding any actual or possible violation of any Permit, or any failure to comply in any respect with any term or requirement of any Permit or any actual or possible revocation, withdrawal, suspension, cancellation, termination, or modification of any Permit. To the Knowledge of the Company is not in Default with respect to any such Permits. The transactions contemplated hereby will not result

in a Default under or a breach or violation of, or adversely affect the rights and benefits afforded to the Company by, any Permit issued to the Company. There is no Action pending or, to the Knowledge of the Company, threatened that could result in the termination, revocation, suspension, modification, nonrenewal or restriction of any Permit issued to the Company or the imposition of any fine, penalty or other sanctions for violation of any legal or regulatory requirements relating to any Permit issued to the Company.

Section II.14 Legal Proceedings. Except as described in Section 2.14 of the Disclosure Schedule, there are no, and since the Effective Date of the Unitholder Option Agreement, there have been no, Actions pending or, to the Knowledge of the Company, threatened, with respect to, against or affecting the Company, the Business, or any Assets, or seeking to prevent or delay the transactions contemplated hereby, and no written notice of any Action involving or relating to the Company, whether pending or to the Knowledge of Company threatened, has been received by the Company or, to the Knowledge of the Company, any Related Party. There are no judgments, Court Orders, injunctions, decrees, stipulations or awards (whether rendered by a court, administrative agency or other Governmental Authority, by arbitration or otherwise) against or involving the Company. There is no material Action by the Company currently pending or which the Company intends to initiate.

Section II.15 Contracts and Commitments.

(a) Section 2.15(a) of the Disclosure Schedule sets forth a true, complete and correct list of (Contracts listed or required to be listed in Section 2.15(a) of the Disclosure Schedule, each, a "Material Contract"):

(i) each Contract or group of related Contracts to which the Company is a party or by which the Business, the Company or any of the Assets are bound that are not terminable by the Company upon notice of thirty (30) days or less without penalty and that involve aggregate obligations, Liabilities, revenues, benefits or other consideration exceeding fifty thousand Dollars ($50,000);

(ii) each Contract between, on the one hand, the Company, and on the other hand, any Affiliate or Related Party (other than (A) employment and consulting agreements, (B) customary confidentiality, assignment of inventions, (C) employee benefits generally made available to employees of the Company, and (D) any Transaction Document);

(iii) each Contract evidencing or relating to any Company Indebtedness or Company Employee Indebtedness, including any loan or credit agreement, security agreement, guaranty, indenture, note, mortgage, pledge, conditional sale or title retention agreement, equipment obligation or lease purchase agreement to which the Company is a party or by which the Company or any of the Assets are bound (including all guaranties issued by any Related Party relating to the Company);

(iv) any guarantee by the Company, or any similar commitment by the Company with respect to, the obligations, liabilities (whether accrued, absolute, contingent or otherwise) or indebtedness of any other Person, other than those entered into in the ordinary course of business consistent with past practice;

(v) any contract or agreement that contains any (A) exclusive dealing obligation, (B) "clawback" or similar undertaking requiring the reimbursement or refund of any fees, (C) "most

favored nation" or similar provision granted by the Company or (D) provision that grants a Third Party any right of first refusal or right of first offer or similar right or that limits or purports to limit the ability of the Company to own, operate, sell, transfer, pledge or otherwise dispose of any assets or business;

(vi) each Contract concerning the establishment or operation of a partnership, joint venture or similar enterprise or any strategic alliance, collaboration, joint development or similar arrangement, relating to the Company, or to which the Company is a party or by which the Business or any of the Assets are bound;

(vii) each Contract that grants any option, right of first refusal or right of first offer or similar right or that limits or purports to limit the ability of the Company to own, operate, sell, transfer, pledge or otherwise dispose of any material amount of its Assets, equity or business;

(viii) each Contract to which the Company is a party or by which the Business, the Company or any of the Assets are bound with any Governmental Authority, or any Contract with any academic institution, research center or Governmental Authority that relates to any Asset, the Business or the Company, including the development or other creation of any Intellectual Property;

(ix) each Contract to which the Company is party, or by which the Business or any of the Assets are bound (A) that would entitle any Third Party to receive a license or any other right with respect to any Intellectual Property of Buyer or any of Buyer's Affiliates following the Closing, (B) pursuant to which the Company is granted by any other Person any license, sublicense or other right to use, or a covenant not to sue with respect to, or any option to license any Intellectual Property, in each case, other than non-exclusive licenses for a commercially available, off-the-shelf software application and other non-exclusive licenses granted by a Third Party service provider under its pre-existing Intellectual Property rights in the ordinary course of business (such Contracts referred to in this Subsection B, collectively, the "Company In-Licenses"), (C) pursuant to which the Company granted to any other Person any license, sublicense or other right to use, or a covenant not to sue with respect to, or any option to license any Intellectual Property, other than non-exclusive licenses granted in the ordinary course of business to a Third Party service provider under such Intellectual Property for the limited purpose of performing services to the Company (such Contracts, collectively, the "Company Out-Licenses"), (D) pursuant to which the Company assigned or agreed to assign to any Person, or any Person assigned or agreed to assign to the Company, any Intellectual Property, (E) pursuant to which any research or development activities are conducted with respect to any Product or any Intellectual Property, or (F) that involve the payment of royalties or other amounts calculated upon the revenues or income of the Company or income or revenues related to any Product or Company Intellectual Property;

(x) each Contract that (A) limits or purports to limit the ability of the Company to compete in any line of business or geographical area (including any covenant restricting the relating to any product or product line or any other Assets), (B) requires the Company to indemnify or hold harmless any Person with respect to infringements of proprietary rights (other than indemnification obligations arising from clinical trial, contract research organizations, contract manufacturing organizations, material transfer, purchase, sale or license agreements entered into in the ordinary course of business consistent with past practice) or for breach of fiduciary duties, or (C) that limits or purports to limit that Company from soliciting any customer of any Person or from soliciting or hiring any employee, consultant or independent contractor of any Person;

(xi) each Contract that requires the Company to provide in kind consideration;

(xii) each Contract that involves real property;

(xiii) any settlement agreement or settlement-related agreement (including any agreement in connection with which any employment-related claim is settled) that provides for ongoing obligations that will last after the Closing;

(xiv) any agreement involving any current or former member of the Company or any Affiliate thereof;

(xv) each Contract that relates to the purchase or sale of any material assets (other than clinical trial and material transfer agreements entered into in the ordinary course of business consistent with past practice);

(xvi) each Contract that results in any Person holding a power of attorney from the Company;

(xvii) each Contract that prohibits, or is terminable other than by the Company upon, or would reasonably be expected to result in or accelerate obligations to the Company in excess of fifty thousand Dollars ($50,000) upon, the announcement or consummation of any of the transactions contemplated by the Unitholder Option Agreement or this Agreement;

(xviii) each Contract to which the Company is a party relating to any contract research, supply or manufacturing arrangements and any Contract with any Third Party to develop, promote or market any Product or that otherwise grant any rights (including any license) or impose any obligations to develop, manufacture, supply distribute, market, sell, offer, import or otherwise commercialize any compound, product or technology; and

(xix) each Contract to which the Company is a party or by which any of the Assets are bound for the employment of any individual or for the consulting services of any Person, or any retention bonus, indemnification or severance Contract.

(b) Each Material Contract is in full force and effect. As to the Company, and to the Company's Knowledge as to all other parties to such contracts, all of the Material Contracts are valid, binding and enforceable against the Company in accordance with their terms except as enforcement may be limited by applicable bankruptcy, insolvency, reorganization, moratorium and other laws affecting enforcement of creditors' rights generally and except insofar as the availability of equitable remedies may be limited by Applicable Law. Neither the Company nor, to the Knowledge of the Company, any other party to any Material Contract, is in Default under, or has improperly terminated, revoked or accelerated, any Material Contract, and there exists no condition or event which, after notice, lapse of time or both, would constitute any such Default, termination, revocation or acceleration. Neither the Company nor any of its Affiliates has received any written notice of Default under any Material Contract. The Company has not waived in writing any of its rights under any Material Contract. The Company is not currently paying liquidated damages in lieu of performance under any Material Contract. Except as set forth on Section 2.15(b) of the Disclosure Schedule, no Person has any option or any other right to participate in the

development, commercialization or marketing of any Products under any Contract to which the Company is party or by which it is bound.

Section II.16 Intellectual Property.

(a) Company Registrations.

(i) Section 2.16(a)(i) of the Disclosure Schedule sets forth a true, correct and complete list of all existing Intellectual Property Registrations that are included in the Company Intellectual Property (the "Company Registrations"); and in each case with respect to Company Registrations, enumerating specifically the owner(s), the application, grant or registration number, as applicable, title, jurisdiction in which filing was made or from which registration issued, date of application, and date of grant or registration. All assignments of all granted Patents and all Patent applications that were filed prior to the date hereof within the Company Registrations have been executed and recorded (including, as applicable, any assignments from any inventor of any such Intellectual Property) as of the Closing Date, in accordance with all Applicable Law. To the Company's Knowledge, all filing, prosecuting, recording, registering, issuance, renewal, maintenance and other fees, annuities and payments that have been due and payable with respect to, or actions required to be taken to maintain, the Company Registrations as of the Closing Date have been, as applicable, timely paid or taken by or on behalf of the Company or its applicable Affiliate(s) or, as applicable with respect to Company Licensed IP, the licensor.

(ii) To the Knowledge of the Company, all of the Company Registrations are subsisting and in full force and effect, and are valid and enforceable. The Company Intellectual Property includes all Intellectual Property owned, used or held for use by the Company in the operation of the Business as conducted as of the date of this Agreement. To the Knowledge of the Company, the Company Intellectual Property includes all Intellectual Property necessary for the operation of the Business as the Company currently contemplates such business to be conducted.

(iii) Section 2.16(a)(iii) of the Disclosure Schedule sets forth a true, correct and complete list of all Company In-Licenses, and a true, correct and complete list of all Company Out-Licenses. The consummation of the transactions upon the Closing under this Agreement shall not: (A) require the consent, approval or authorization of any counterparty to any Company In-License or Company Out-License; (B) give rise to any right of termination for any Company In-License or Company Out-License; (C) result in any loss of the Company's rights to material Company Licensed IP under any Company In-License; or (D) result in the amendment of any terms and conditions of any Company In-License or Company Out-License such that the Company's rights to any of the Company Licensed IP under any such agreement are not materially similar to those in effect immediately prior to the Closing.

(b) Prosecution Matters. As of the Closing Date, there are no inventorship challenges, or opposition, reexamination, nullity or interference proceedings or other challenges to ownership, registrability, patentability, enforceability or validity declared, commenced or, to the Knowledge of the Company, threatened with respect to any Company Registrations. The Company and all Third Parties acting on the Company's behalf have complied in all material respects with all of their obligations and duties to the respective patent, trademark and copyright offices, including the duty of candor and disclosure to the U.S. Patent and Trademark Office, with respect to all Company Registrations filed by or on behalf of the Company. To the Knowledge of the Company, there is no information that could

reasonably be expected to result in a challenge to, or otherwise adversely affect the ownership, registrability, patentability, enforceability or validity of any Company Registrations.

(c) Ownership. Each item of Company Registrations is solely and exclusively owned, or available for use pursuant to a valid and enforceable written license agreement, by the Company. The Company is the sole and exclusive owner of all right, title and interest in, to and under all Company Owned Intellectual Property, free and clear of any Liens (other than Permitted Liens). No Company Owned Intellectual Property and no Company Licensed IP is subject to any (i) consent, settlement, decree, order, injunction, judgment or ruling, cancellation or reexamination proceeding or any other proceeding challenging their scope or validity other than proceedings for pending applications or registrations, or (ii) Contract (other than a Company In-License or pursuant to the terms of any agreement set forth on 2.16(c)(ii) of the Disclosure Schedule), in each case restricting or otherwise limiting the use, ownership, validity, enforceability, disposition or exploitation thereof. As of the date of this Agreement, with respect to the Company Registrations, (i) no post grant review (PGR), inter partes review (IPR), opposition, extension of time to oppose, interference, rejection, or refusal to register has been filed in connection with any such application other than a grant refusal communication or a registration refusal communication issued by a Governmental Authority, and (ii) the ownership of the Company's right, title and interest therein is recorded with the applicable Governmental Authority. Other than Third Party licensors under any of the Company In-Licenses, Third Party licensees under any of the Company Out-Licenses and joint owners indicated on Section 2.16(a)(i) of the Disclosure Schedule, no other Person has, any right, title or interest in or to any Company Intellectual Property. Except for any fees payable to a Governmental Authority to issue, register or maintain any of the Company Registrations and for any license or royalty payments required pursuant to a Company In-License, to the Company's Knowledge no payment by the Company of any kind is required to be made to any Person (including directors, officers, employees, consultants, contractors and agents of the Company) based on the use of any Intellectual Property. Other than the Company Out-Licenses, the Company has not granted to any Person any license (including any sublicense), option, assignment or other similar agreement (including any covenants not to sue) or any other right, title or interest with respect to any Company Registrations.

(d) Protection Measures. To the Knowledge of the Company, none of the material Know-How Controlled by the Company that is Confidential Information has been disclosed by or on behalf of the Company nor by any Person to any Third Party unless, in each case, such disclosure was made pursuant to an appropriate written confidentiality agreement and to the Knowledge of the Company there has not been any material breach by any such Person of any such agreement. As of the Closing Date, the Company has taken reasonable steps to maintain and protect each item of Company Intellectual Property that is Confidential Information, including commercially reasonable measures at least commensurate with industry standards to maintain the confidentiality of confidential information treated as trade secrets and other confidential information using not less than a reasonable degree of care under the circumstances. The Company has required each of its current and former employees, consultants and independent contractors who is or was involved in the creation or development of any Company Owned Intellectual Property to enter into agreements with the Company pursuant to which each such Person agreed and is bound to maintain and protect the confidential information of the Company and has agreed to assign to the Company, or confirm prior assignment, whether directly to the Company or to the Company via assignment to such Person's employer in the course of such Person's employment, sole ownership of all Intellectual Property authored, developed or otherwise created by such Person in the course of such Person's employment or other engagement with the Company all in accordance with all Applicable Law.

(e) Intellectual Property Infringement. To the Knowledge of the Company, the operations of the Company do not and have not infringe(d), misappropriate(d) or otherwise violate(d) the Intellectual Property rights of any other Person. The Company has not received any charge, complaint, claim, demand, communication or other notice, and the Company is not or has not been a party to any lawsuit, claim, action or proceeding, where any Person has (i) alleged that the Company has infringed, misappropriated or otherwise violated any Intellectual Property rights of any other Person (including pursuant to any written notice or demand that the Company must license or refrain from using any Intellectual Property of any other Person), or (ii) challenged the validity, enforceability, ownership, patentability or registrability of, or has claimed any right, title or interest in or to, any Company Intellectual Property, and, with respect to each of clauses (i) and (ii), no such claim has been asserted or threatened in writing. To the Knowledge of the Company, no Person has infringed, misappropriated or otherwise violated any Company Intellectual Property and the Company has not filed or threatened in writing any claims alleging that a Third Party has infringed, misappropriated or otherwise violated any Company Owned Intellectual Property.

(f) Activities with Governmental Authorities or Universities. Except as set forth in Section 2.16(f) of the Disclosure Schedule, no academic institution, academic or governmental research center or Governmental Authority, nor any Person working for or on behalf of any of the foregoing, has, or will be entitled to have, any right, title or interest (including any "march in" or co-ownership rights) in or to any Company Intellectual Property (including any claim or option to any of the foregoing).

(g) Inventors. With respect to Patents in the Company Owned Intellectual Property, (i) there are no other inventors of such Patents other than the listed inventors in the Patents, including as the term "inventor" is defined and interpreted under United States patent law or patent laws of other relevant jurisdictions, and (ii) all inventors of any inventions claimed or covered by any such Patent included in the Company Registrations have each assigned, or have agreed to assign, or confirm prior assignment, whether, (a) in the case of inventors who are employees of the Company, directly to the Company or, (b) in the case of inventors who are employed by a service provider, to the relevant service provider in the course of such Person's employment and the service provider has assigned, or has agreed to assign, or confirm prior assignment of such service provider's entire right, title and interest, in each case ((a) and (b)) in and to any Intellectual Property rights in and to such inventions to the Company, or, (c) if the Company acquired any such Patents or other Intellectual Property, the Person who employed or otherwise engaged such inventor, and such Person has assigned the same to the Company (or any previous owner or acquirer of such Patents or other Intellectual Property). No claims have been asserted in writing challenging the inventorship of any of the Patents included in the Company Registrations.

Section II.17 Absence of Claims; Business Relationships with Affiliates. Except as set forth on Section 2.17 of the Disclosure Schedule, and excluding this Agreement, the Transaction Documents and the transactions contemplated hereby and thereby, neither any Related Party of the Company nor any Affiliate thereof, either directly or indirectly, Controls any asset, property or right, tangible or intangible, used or held for use by the Company, or to the Knowledge of the Company, has any claim or cause of action against the Company, or is owed any payment or other obligation by the Company, or is a party to any Contract with the Company or engaged in any transaction, arrangement or course of dealing with the Company (excluding this Agreement, the Transaction Documents and the transactions contemplated hereby and thereby). No event has occurred, and no circumstance or condition exists, that has resulted in, or would reasonably be expected to result in, any claim by a service provider or manager of the Company for indemnification or advancement of expenses related thereto pursuant to (x) the terms of

the Operating Agreement, (y) any indemnification agreement or other Contract between the Company and any such service provider or manager, or (z) any Applicable Law.

Section II.18 Allocation Schedule. As of immediately prior to the Closing, the allocations set forth in the Allocation Schedule are true, complete and accurate, have been calculated in accordance with Section 2.18 of the Disclosure Schedule and comply with the requirements of Applicable Law, the Operating Agreement and other organizational documents, the Unitholder Option Agreement, this Agreement, and any applicable Contracts. No holder of any Equity Participation of the Company will be entitled to receive any consideration in connection with the Definitive Initial Purchase Consideration (or any Milestone Payment, Priority Review Voucher Payment, or Royalty Payment, or amount required to be released to the Unitholders from the Escrow Amount or the Representative's Fund) except as set forth in the Allocation Schedule.

Section II.19 Brokers and Agents. Except as set forth in Section 2.19 of the Disclosure Schedule, neither the Company nor any Person acting on its behalf has employed, paid or entered into any Contract which has or will result in an obligation to pay any fee or commission to any broker, finder or similar intermediary for or on account of the transactions contemplated by the Unitholder Option Agreement or this Agreement.

Section II.20 Regulatory Matters.

(a) The operations of the Company (inclusive of the Products, services, and advertising and promotional materials) are, and have been at all times, in compliance with all applicable Healthcare Laws. The Products are, and have, been manufactured, researched and developed by, or on behalf of, the Company in compliance with all applicable Healthcare Laws. The Company has obtained all applicable approvals, authorizations, licenses, registrations, and Permits required by the FDA and any other Governmental Authority responsible for the oversight and enforcement with respect to Healthcare Laws, including to permit any manufacturing, labeling, storing, testing, research and development of each Product as previously conducted or currently being conducted by or on behalf of the Company. Section 2.20(a) of the Disclosure Schedule sets forth a complete and accurate list of (i) all licenses, certifications, orders, clearances, exemptions, authorizations, and approvals granted or pending with the FDA or any other Governmental Authority to research, develop, or market any Product used in the conduct of the Business; (ii) each clinical trial protocol submitted by the Company to the FDA or any other Governmental Authority; (iii) all other filings or submissions made by the Company pursuant to the Federal Food, Drug, and Cosmetic Act, as amended ("FD&C Act") and its respective implementing orders and regulations, or any similar Applicable Law; and (iv) any material correspondence between the Company and the FDA or any other Governmental Authority concerning the Company or a Product. The Company has delivered to Buyer each of the items described in (i)-(iv) above.

(b) The Company has not received any notice that the FDA or any other Governmental Authority responsible for oversight or enforcement of any Healthcare Law, or any institutional review board (or similar body responsible for oversight of human subjects research) or institutional animal care and use committees (or similar body responsible for oversight of animal research), has initiated, or threatened to initiate, any action, suit, proceeding or order to restrict or suspend, nonclinical research on or clinical study of any Product, or to recall, suspend or otherwise restrict the manufacture of any Product, or in which the Governmental Authority alleges or asserts a failure to comply, with applicable Healthcare Laws.

(c) There are no lawsuits, actions, arbitrations, proceedings, charges, complaints or, to the Knowledge of the Company, investigations pending, or to the Knowledge of the Company, threatened, with respect to any alleged violation by the Company, any of its Affiliates, or to the Knowledge of the Company, any of its subcontractors, partners or collaborators of the FD&C Act or any other Healthcare Law, and the Company, its Affiliates, and to the Knowledge of the Company, its subcontractors, partners and collaborators are not party to or subject to, nor is any Product subject to, any corporate integrity agreements, monitoring agreements, consent decrees, deferred prosecution agreements, settlement orders or similar Contracts with or imposed by any Governmental Authority related to any Healthcare Law, and no such Contract is currently pending or, to the Knowledge of the Company, threatened. The Company is not a defendant or named party in any unsealed qui tam/False Claims Act litigation. The Company is not resubmitting or planning to resubmit to the government any data reported under the Medicaid Rebate Statute, Medicare Part B Drug Pricing requirements, 340B Program requirements, Veterans Health Care Act of 1992, or the Medicare Part D Coverage Gap Discount Program, or refunding any monies owed due to a resubmission.

(d) Neither the Company, nor to the Knowledge of the Company, any Person engaged by the Company for contract research, contract manufacturing, consulting, other collaboration services, partners or collaborators with respect to any Product has made an untrue statement of a material fact or a fraudulent statement to the FDA or any Governmental Authority responsible for enforcement or oversight with respect to Healthcare Laws, or failed to disclose a material fact required to be disclosed to the FDA or other such Governmental Authority that, at the time such disclosure was made, would reasonably be expected to provide a basis for the FDA to invoke its policy respecting "Fraud, Untrue Statements of Material Facts, Bribery, and Illegal Gratuities" set forth in 56 Fed. Reg. 46191 (September 10, 1991), or for any other Governmental Authority to invoke a similar policy.

(e) Neither the Company nor any of its current or former members, officers, partners, or employees, nor to the Knowledge of the Company, contractors or agents have been or are currently suspended, excluded or debarred from any federal contracting or healthcare programs, or threatened with or currently subject to an investigation or proceeding that could result in suspension, exclusion or debarment under state or federal statutes or regulations, or assessed or threatened with assessment of civil monetary penalties. Neither the Company nor any of its current or former members, officers, partners, employees, nor to the Knowledge of the Company, contractors or agents have been convicted of any crime or engaged in any conduct that has previously caused or would reasonably be expected to result in (i) disqualification or debarment under 21 U.S.C. Sections 335 or any similar law, rule or regulation of any other Governmental Authorities, or (ii) exclusion under 42 U.S.C. Section 1320a-7 or any similar law, rule or regulation of any Governmental Authorities.

(f) All pre-clinical studies and clinical trials conducted or being conducted with respect to any Product or the Business by or, to the Knowledge of the Company, at the direction of the Company have been and are being conducted in material compliance with all Applicable Law, including (i) standard medical and scientific research procedures and all Healthcare Laws, as applicable, including 21 C.F.R. Parts 11, 50, 54, 56, 58 and 312 and comparable foreign Laws; (ii) the applicable requirements of Good Laboratory Practices and Good Clinical Practices and applicable regulations and guidances that relate to the proper conduct of clinical studies and requirements relating to the protection of human subjects (including "Informed Consent" as such term is defined under Applicable Law in the jurisdictions where clinical trials were or are being conducted); and (iii) Applicable Law governing the privacy of patient medical records and other personal information and data. The Company has not received any notifications

or other communications from any institutional review board (IRB), ethics committee, safety monitoring committee or Governmental Authority raising any issues in any jurisdiction requiring the termination or suspension or investigation of any clinical studies conducted by, or on behalf of, the Company, or in which the Company has participated and, to the Knowledge of the Company, no such action has been threatened and there is nothing that would warrant any such action. Complete and correct copies of all material Regulatory Materials, including scientific and clinical data of the Company with respect to all Products or the Business, have been made available to Buyer. Such Regulatory Materials are complete and correct in all material respects and have been maintained in compliance with all formal filing and maintenance requirements. The Company has made available to Buyer complete and correct copies of all serious adverse event reports, periodic adverse event reports, non-clinical expedited safety reports, and all other Governmental Authority communications, documents and other information submitted by the Company to or received by the Company from any Governmental Authority, including inspection reports, warning letters and similar documents, relating to the Company, the conduct of the Business, or any Product.

(g) There have been no (i) FDA Form 483 inspection observations, (ii) establishment inspection reports, (iii) warning, untitled or action letters, (iv) orders, (v) enforcement actions or (vi) other documents or actions that assert lack of compliance in any material respect with any applicable Law, in all such cases, relating to, arising out of or in connection with the Business or any Product. There has not been, nor, to the Knowledge of the Company, is there currently under consideration by the Company, its partners, collaborators or any Governmental Authority, any recall, market withdrawal, safety alert, "Dear Doctor" letter, public health notification or other safety communication in respect of any Product.

(h) If applicable, the manufacture of the Products are being conducted in compliance with current good manufacturing practices, as defined by the FDA, including, as applicable, the FDA's Current Good Manufacturing Practices set forth in 21 C.F.R. Parts 210 and 211 and any successor legislation or regulations, and comparable foreign Laws.

(i) The Company owns or has the right to use and access (including the right to make copies of) all material information and data generated in all development activities and all nonclinical, toxicology and other studies, and clinical studies and trials (together with data sets associated with such studies) with respect to the Products, in each case, undertaken by or on behalf of Company.

Section II.21 Insurance. Section 2.21 of the Disclosure Schedule sets forth a true, correct and complete list of all insurance policies carried by, or maintained on behalf of, the Company (the "Insurance Policies"), the amounts and types of insurance coverage available thereunder and all insurance loss runs and workers' compensation claims received for the past three (3) policy years. There is no material claim pending under any such Insurance Policies. Such insurance policies are sufficient for compliance with Applicable Law and for compliance with any obligations under any Contract of the Company; provided that, if the Company obtains and maintains insurance in accordance with Section 8.4 of the Program Agreement, Buyer may not assert a claim for a breach of this Section 2.21 with respect to any Contract that only requires a commercially reasonable amount of insurance (or similar standard) and does not explicitly require specific insurance amounts and/or types. The Company does not have any self-insurance or co-insurance programs, except as identified in the Disclosure Schedule. The Company has delivered to Buyer true, complete and correct copies of all such Insurance Policies. With respect to each Insurance Policy, the Company is not in Default (including any breach or Default with respect to the giving of notice) under any material provision of any such Insurance Policy, and no event has occurred which, after notice or the lapse of time or both, would constitute a Default of a material provision of such Insurance Policy or

permit termination or modification under such Insurance Policy. The Company has not received notice of cancellation or nonrenewal of any such Insurance Policy.

Section II.22 Certain Business Practices. None of the managers, officers or employees of the Company or any of their Affiliates or, to the Knowledge of the Company, agents of the Company or any of their Affiliates, has, in each case in connection with the Business, (a) used any funds for unlawful contributions, gifts, entertainment or other unlawful expenses, including expenses related to political activity, (b) made any unlawful payment to foreign or domestic government officials or employees or to foreign or domestic political parties or campaigns, made any bribes or kickback payments or violated any provision of the Foreign Corrupt Practices Act of 1977, as amended, or similar legislation, or (c) made any payment to any customer or supplier of the Company, or given any other consideration to any such customer or supplier in respect of the Business that violates Applicable Law or (d) made any other unlawful payment.

Section II.23 Books and Records. The Company has made and kept (and given Buyer access to copies of) its true, correct and complete Books and Records and accounts, which, in reasonable detail, accurately and fairly reflect, in all material respects, the activities of the Company. The minute books of the Company made available to Buyer accurately and adequately reflect in all material respects all action previously taken by the members of the Company, the board of managers and committees of the board of managers of the Company. The copies of the membership interest records of the Company previously made available to Buyer are true, correct and complete, and accurately reflect all transactions effected in the membership interest of the Company through and including the date hereof.

Section II.24 Bank Accounts. Section 2.24 of the Disclosure Schedule contains a true, correct and complete list of all bank accounts maintained by the Company, including each account number and the name and address of each bank and the name of each Person who has signature power with respect to each such account or power of attorney to act on behalf of the Company.

Section II.25 Privacy.

(a) In connection with its collection, storage, transfer (including any transfer across national borders) or use of any personally identifiable health information from any clinical trial participants (collectively "Personal Information"), the Company is and has been in compliance with all applicable Laws (including HIPAA, and other applicable Laws governing the privacy and security of Personal Information) in all relevant jurisdictions, the Company's privacy policies and the requirements of any contract or codes of conduct to which the Company is a party.

(b) The Company has commercially reasonable physical, technical, organizational and administrative security measures and policies in place to protect all Personal Information to the extent collected by the Company or on its behalf from and against unauthorized access, use, modification, disclosure, or other misuse. The Company is and has been in compliance with all Laws relating to data loss, theft and breach of security notification obligations. There has been no unauthorized access to or other misuse of such information constituting a "Security Incident" or "Breach of Unsecured Protected Health Information" as those terms are defined under HIPAA.

(c) The Company has in place policies necessary to protect the privacy and security of Personal Information, including policies and procedures reasonably intended to respond to complaints

received alleging violation of applicable privacy or security standards and to identify and report all Breaches of Personal Information in accordance with the Company's legal and contractual obligations and have performed a thorough assessment of the potential risks and vulnerabilities to the confidentiality, integrity, and availability of electronic Personal Information held by the Company.

(d) No action has been asserted or threatened in a formal notice or commenced against the Company alleging non-compliance with HIPAA, a data security violation, or a violation of any Person's privacy, personal information, or data rights.

(e) The Company is not subject to the European Union General Data Protection Regulation (EU) 2016/679 ("GDPR"), any Laws implementing or supplementing GDPR in each applicable jurisdiction, nor any other Law relating to data privacy and security in any jurisdiction outside the United States. The Company has not transferred any Personal Information across any national borders.

(f) The Company is not subject to any contractual requirements, privacy policies or other legal obligations that, following Closing, would prohibit Buyer from receiving and using any Personal Information. The Company is not the owner or host of any database that is subject to a registration or notification requirement with any Governmental Authority.

Section II.26 Suppliers. The relationships of the Company with its suppliers and service providers are good commercial working relationships. None of the Company's suppliers or service providers has canceled, terminated, or otherwise materially altered or notified Company, of any intention or otherwise threatened to cancel, terminate, or materially alter its relationship with the Company effective prior to, as of, or within one year after, the Closing. There has not been, and the Company has no reasonable basis to expect that there will be, any change in relations with suppliers or service providers, as a result of the transactions contemplated by this Agreement or the other Transaction Documents. There is not any present condition or state of facts or circumstances related to the Company's service providers and suppliers that would reasonably be expected to prevent the Business from being carried on after the Closing Date in the same manner as it is presently being carried on.

ARTICLE III
REPRESENTATIONS AND WARRANTIES OF UNITHOLDERS

To induce Buyer to enter into the Transaction Documents and consummate the transactions contemplated thereby, each Unitholder represents and warrants, except for TAMUS, which represents, severally and not jointly, to Buyer as follows:

Section III.1 Due Organization; Authorization; Binding Agreement. If such Unitholder is an entity, such Unitholder is duly organized, validly existing and in good standing under the Laws of the jurisdiction in which it is incorporated or constituted (or to the extent such concepts are not recognized in such jurisdiction, such entity is up to date in filing its corporate or annual return), and the consummation of the transactions contemplated hereby are within such Unitholder's corporate or organizational powers and have been duly authorized by all necessary corporate or organizational actions on the part of such Unitholder. If such Unitholder is an individual, such Unitholder has the legal capacity to execute and deliver this Agreement and the other Transaction Documents to which such Unitholder is a party, to own, hold, sell and transfer pursuant to this Agreement and the other Transaction Documents to which such Unitholder is a party the Units owned by such Unitholder and to consummate the transactions

contemplated hereby and thereby. Such Unitholder has full power and authority to execute, deliver and perform this Agreement and any other Transaction Documents to which such Unitholder is a party. This Agreement and any other Transaction Documents to which such Unitholder is a party has been duly and validly executed and delivered by such Unitholder, and constitutes a legal, valid and binding obligation of such Unitholder enforceable against such Unitholder in accordance with its terms, subject to the effects of bankruptcy, insolvency, fraudulent conveyance, reorganization, moratorium and other similar Laws relating to or affecting creditors' rights generally and general equitable principles (whether considered in an Action in equity or at law).

Section III.2 Non-Contravention. The execution and delivery by such Unitholder of this Agreement and the other Transaction Documents to which such Unitholder is a party does not, and the performance by such Unitholder of such Unitholder's obligations hereunder and thereunder and the consummation by such Unitholder of the transactions contemplated hereby and thereby will not (a) violate any Law applicable to such Unitholder or such Unitholder's Units, (b) except as may be required by U.S. federal securities Law, require any consent, approval, order, authorization or other action by, or filing with or notice to, any Person (including any Governmental Authority) under, violate, conflict with, constitute a Default (with or without the giving of notice or the lapse of time or both) under, or give rise to any right of termination, cancellation or acceleration under, or result in the creation of any Liens on any of such Unitholder's Units pursuant to, any contract, agreement, trust, commitment, Court Order, judgment, writ, stipulation, settlement, award, decree or other instrument binding on such Unitholder or any Applicable Law, or (c) if such Unitholder is an entity, conflict with, or result in a breach of or violate any provision of such Unitholder's certificate or articles of incorporation, bylaws, limited liability or operating agreement, partnership agreement, trust agreement or similar organizational documents.

Section III.3 Ownership of Units; Total Units. Such Unitholder is the record and beneficial owner of such Unitholder's Units and has good and marketable title to such Units, free and clear of any Liens as of immediately prior to the Closing. The Units listed on Schedule A opposite such Unitholder's name constitute all of the equity interests of the Company legally and beneficially owned by such Unitholder as of immediately prior to the Closing, and such Unitholder neither holds nor has any legal or beneficial ownership in any other equity interest in the Company. Except pursuant to this Agreement and as set forth in the Operating Agreement, no Person has any contractual or other right or obligation to purchase or otherwise acquire any of such Unitholder's Units and such Unitholder is not party to any Contract obligating such Unitholder to sell, transfer, pledge or otherwise dispose of any interest in any of such Unitholder's Units. Upon the Closing, valid title to the Units will pass to Buyer, free and clear of any Liens.

Section III.4 Voting Power. Such Unitholder has full voting power, with respect to such Unitholder's Voting Units (as defined in the Operating Agreement) and full power of disposition, full power to issue instructions with respect to the matters set forth herein and full power to agree to all of the matters set forth in this Agreement, in each case with respect to all of such Unitholder's Units. Other than as set forth in the Operating Agreement and the Unitholder Option Agreement, none of such Unitholder's Units are subject to any proxy, voting trust or other agreement or arrangement with respect to the voting of such Units (including any preemptive right, right of participation, right of maintenance or any similar right; any right of first refusal or similar right; or relating to the registration of, or restricting any Person from purchasing, selling, pledging or otherwise disposing of the Units).

Section III.5 Reliance. Such Unitholder has had the opportunity to review this Agreement with counsel of such Unitholder's own choosing. Such Unitholder understands and acknowledges that Buyer is

entering into this Agreement in reliance upon such Unitholder's execution, delivery and performance of this Agreement.

Section III.6 Absence of Litigation. As of the date hereof, there is no Action pending against, or, to the knowledge of such Unitholder, threatened against such Unitholder or any of such Unitholder's properties or assets (including such Unitholder's Units) that could reasonably be expected to prevent or materially delay or impair the consummation by such Unitholder of the transactions contemplated by this Agreement or any other Transaction Documents to which such Unitholder is a party or otherwise adversely impact such Unitholder's ability to perform its obligations hereunder or thereunder. To such Unitholder's knowledge, there are no Actions pending or threatened against or by the Company affecting any of the Company's properties or assets (or by or against such Unitholder or any Affiliate thereof and relating to the Company) or against or by the Company, such Unitholder or any Affiliate of such Unitholder that challenges or seeks to prevent, enjoin or otherwise delay the transactions contemplated by this Agreement or any other Transaction Documents to which such Unitholder is a party.

Section III.7 Brokers. Neither such Unitholder nor any Person acting on its behalf has employed, paid or entered into any Contract which has or will result in an obligation to pay any fee or commission to any broker, finder or similar intermediary for or on account of the transactions contemplated by the Unitholder Option Agreement or this Agreement.

ARTICLE IV
REPRESENTATIONS AND WARRANTIES OF BUYER

To induce the Company and the Unitholders to enter into the Transactions Documents and consummate the transactions contemplated thereby, Buyer represents and warrants to the Company and the Unitholders as follows:

Section IV.1 Organization; Authorization. Buyer is a corporation duly organized, validly existing and in good standing under the laws of Delaware and has full corporate power and authority to enter into this Agreement and the other Transaction Documents to which Buyer is a party.

Section IV.2 Authorization; No Conflict.

(a) Buyer has full corporate power and authority to execute, deliver and perform its obligations under this Agreement and the other Transaction Documents to which it is a party. The execution, delivery and performance by Buyer of this Agreement and any other Transaction Documents to which Buyer is a party (together with the other instruments, documents and agreements contemplated hereby or thereby or to be executed in connection with the transactions contemplated hereby or thereby) have been duly authorized by all necessary corporate or organizational actions on the part of Buyer. This Agreement has been duly authorized, executed and delivered by Buyer and is the legal, valid and binding agreement of Buyer enforceable in accordance with its terms, except as enforceability may be limited by applicable bankruptcy, insolvency, reorganization, moratorium or similar Laws affecting the enforcement of creditors' rights generally and by the effect of general principles of equity (regardless of whether enforcement is considered in a proceeding in equity or at law).

(b) Neither the execution and delivery by of this Agreement or any other Transaction Documents to which Buyer is a party, nor the consummation of any of the transactions contemplated

hereby or thereby, nor compliance with or fulfillment of the terms, conditions and provisions hereof or thereof, in each case by Buyer, will:

(i) (A) conflict with, result in a breach of the terms, conditions or provisions of, or constitute a Default, an event of default or an event creating rights of acceleration, termination or cancellation or a loss of rights under the certificate of incorporation or by-laws of Buyer, or under any contract to which Buyer is a party, or (B) violate any Court Order or material Laws applicable to Buyer, in each case, solely to the extent that such Default or violation would have a material adverse impact on Buyer's ability to consummate the transactions contemplated hereby; or

(ii) require the approval, consent, authorization or act of, or the making by Buyer of any declaration, filing or registration with, any Person.

Section IV.3 Brokers and Agents. Neither Buyer nor any Person acting on its behalf has employed, paid or entered into any Contract which has or will result in an obligation to pay any fee or commission to any broker, finder or similar intermediary for or on account of the transactions contemplated by the Unitholder Option Agreement or this Agreement.

Section IV.4 Independent Investigation. Buyer has conducted its own independent investigation, review and analysis of the Business, the Company Intellectual Property, results of operations, condition (financial or otherwise) and assets of the Company, and acknowledges that it has been provided adequate access to the personnel, assets, books and records, and other documents and data of the Company for such purpose. Notwithstanding such investigation, Buyer acknowledges and agrees that: (a) in making its decision to enter into this Agreement and to consummate the transactions contemplated hereby, Buyer has relied solely upon the express representations and warranties of the Company and the Unitholders set forth in this Agreement; and (b), neither the Company nor the Unitholders have made any representation or warranty to Buyer except as expressly set forth in this Agreement.

ARTICLE V
CLOSING DELIVERIES

Section V.1 By the Company. At the Closing, the Company shall deliver to Buyer each of the following, duly executed by the Company to the extent applicable:

(a) Escrow Agreement by and among Buyer, the Representative, and the Escrow Agent, in a customary form reasonably satisfactory to Buyer and the Company (the "Escrow Agreement"), duly executed by the Company and the Representative;

(b) Fully executed payoff letters from all holders of Closing Date Company Indebtedness being repaid at Closing (as set forth on Section 5.1(c) of the Disclosure Schedule), which payoff letters shall, among other things, be in form and substance reasonably acceptable to Buyer, and include a complete release of all Liens with respect to such Closing Date Company Indebtedness and wire instructions for such holder of Closing Date Company Indebtedness;

(c) A list of each Person owed any Transaction Costs being paid at the Closing pursuant to Section 1.4(a)(ii), setting forth the amount of Transaction Costs owed to such Person and wire instructions for each such Person;

(d) Evidence that any Contract or arrangement set forth on Section 5.1(e) of the Disclosure Schedule to which, on the one hand, the Company, and, on the other hand, any current or former (A) Related Party, (B) officer, director or employee of the Company, or (C) Affiliate of any of the foregoing, is a party, has been terminated, with no Liability to the Company;

(e) Resignations (effective as of the Closing) from all directors of the Company and such officers of the Company as Buyer may have requested prior to the Closing, in each case solely in their capacity as directors or officers (and not in their capacity as employees, as applicable).

(f) Each Unitholder that is a U.S. person shall provide Buyer with a Non-Foreign Person Affidavit in accordance with Section 1446(f) of the Code and Treasury Regulation Section 1.1445-2(b)(2). Each Unitholder that is a non-U.S. person shall cause the Company to provide to Buyer (i) a certificate in accordance with Treasury Regulations Section 1.1445-11T(d)(2) and (ii) a certificate pursuant to Proposed Treasury Regulations Section 1.1446(f)-2(c)(2)(ii)(C) (or at the election of Buyer, Section 7.03 of Internal Revenue Service Notice 2018-29), dated as of the Closing Date certifying each such Unitholder's share of partnership liabilities in the Company.

Section V.2 By Buyer. At the Closing, Buyer shall deliver to the Company the following items each duly executed by Buyer to the extent applicable:

(a) Escrow Agreement, duly executed by Buyer;

(b) the Estimated Initial Purchase Consideration, and other payments due pursuant to Section 1.4, in immediately available funds.

ARTICLE VI
POST-CLOSING COVENANTS

Section VI.1 Further Assurances. From time to time after the Closing Date, upon the reasonable request of any party, each party hereto shall execute, acknowledge and deliver all such other instruments and documents and shall take all such other actions required to consummate and make effective the transactions contemplated by the Transaction Documents; provided, that Buyer shall not be required to pay any further consideration or amounts therefor.

Section VI.2 Confidentiality.

(a) Each party hereunder agrees that a party (the "Receiving Party") receiving Confidential Information of another party before or after the Effective Date (the "Disclosing Party") shall: (a) maintain in confidence such Confidential Information using not less than the efforts such Receiving Party uses to maintain in confidence its own proprietary information of similar kind and value, but in no event less than reasonable efforts; (b) not disclose such Confidential Information to any Third Party without the prior written consent of the Disclosing Party, except for disclosures expressly permitted below; and (c) not use such Confidential Information for any purpose except those permitted by the

Transaction Documents. For purposes of this Section 6.2, capitalized terms used but not defined herein shall have the meanings ascribed to them in the Program Agreement

(b) For purposes of this Agreement, "Confidential Information" means all proprietary non-public information that is disclosed by one party or its Affiliates or its representatives to another party or its Affiliates or its representatives, provided by or on behalf of one party to another party in writing, orally, visually or observed by a party while on the premises of another party, including processes, formulae, data, Know-How, improvements, inventions, chemical or biological materials, chemical structures, techniques, marketing plans, strategies, customer lists or other information that has been created, discovered or developed or otherwise made by or on behalf of a party, or has otherwise become known to a party, or to which rights have been assigned to a party, as well as any other information and materials that are deemed confidential or proprietary to or by a party or that a reasonable person would understand to be confidential or proprietary due to the context of its disclosure or its scope, content, or nature (including all information and materials of a party's customers and any other Third Party and their consultants), in each case regardless of whether any of the foregoing are marked "confidential" or "proprietary" or communicated to the other by the Disclosing Party in oral, written, graphic or electronic form.

(c) Exceptions. The obligations in Section 6.2(a) shall not apply with respect to any portion of the Confidential Information that the Receiving Party can show by competent written evidence:

(i) is publicly disclosed by the Disclosing Party, either before or after it is disclosed to the Receiving Party hereunder;

(ii) was known to the Receiving Party or any of its Affiliates, without any obligation to keep it confidential or any restriction on its use, prior to disclosure by the Disclosing Party (and the applicable evidence thereof shall be contemporaneous);

(iii) is subsequently disclosed to the Receiving Party or any of its Affiliates by a Third Party rightfully in possession thereof and without any obligation to keep it confidential or any restriction on its use;

(iv) is published by a Third Party or otherwise becomes publicly available or enters the public domain through no fault (whether by action or inaction) of the Receiving Party, either before or after it is disclosed to the Receiving Party; or

(v) is independently developed by or for the Receiving Party or its Affiliates without reference to or reliance upon the Disclosing Party's Confidential Information.

(d) Authorized Disclosures. The Receiving Party may disclose Confidential Information belonging to the Disclosing Party and Confidential Information deemed to belong to both Buyer and the Company under Section 6.2(a), to the extent (and only to the extent) such disclosure is reasonably necessary in the following instances:

(i) subject to Section 7.5 of the Program Agreement, complying with Applicable Laws (including the rules and regulations of the Securities and Exchange Commission or any

national securities exchange) and with judicial process, if in the reasonable opinion of the Receiving Party's counsel, such disclosure is necessary for such compliance;

(ii) Prosecution and Maintenance of Prosecuted Patents in accordance with the Program Agreement;

(iii) in connection with the enforcement of any of the Transaction Documents; and

(iv) disclosure, solely on a "need to know basis," to Affiliates, Service Providers, potential or actual research and Development collaborators, licensees, subcontractors, shareholders or equity-holders, or other potential financial partners, and each of the Parties' respective directors, officers, employees, consultants, stockholders or members, attorneys and contractors, each of whom, prior to disclosure, must be bound by written obligations of confidentiality and non-use or binding professional responsibility standards containing confidentiality obligations no less restrictive than the obligations set forth in this Section 6.2 (but of shorter duration if customary); provided, however, that, in each of the above situations, the Receiving Party shall remain responsible for any failure by any Person who receives Confidential Information pursuant to this Section 6.2(c)(iv) to treat such Confidential Information as required under this Section 6.2.

(v) If and whenever any Confidential Information is disclosed in accordance with this Section 6.2, such disclosure shall not cause any such information to cease to be Confidential Information except to the extent that such disclosure results in a public disclosure of such information (otherwise than by breach of this Agreement). The Receiving Party shall notify the Disclosing Party of the Receiving Party's intent to make any disclosures pursuant to Section 6.2(c)(i) or Section 6.2(c)(iii) sufficiently prior to making such disclosure so as to allow the Disclosing Party adequate time to take whatever action it may deem appropriate to protect the confidentiality of the information (including seeking a confidential treatment order or protective or limiting order, as applicable), and the Receiving Party shall provide reasonable assistance to the Disclosing Party with respect thereto as allowed by law; provided, that, in any event, the Receiving Party shall use reasonable measures to ensure confidential treatment of such information and shall only disclose such Confidential Information of the Disclosing Party as is necessary to comply with such Applicable Laws or judicial process.

Section VI.3 Tax Matters.

(a) Cooperation. The Representative, the Unitholders and Buyer shall (and Buyer shall cause the Company to) cooperate fully, as and to the extent reasonably requested by the other parties, to (i) assist in the preparation and timely filing of any Tax Return of the Company; and (ii) assist in any audit or other proceeding with respect to the Tax Returns or Taxes of the Company, except as to TAMUS, to the extent allowed by the Laws of the State of Texas and approved by the Attorney General of the State of Texas. Buyer and the Company shall not destroy or dispose of any Tax workpapers, schedules or other materials and documents supporting Tax Returns of the Company for Pre-Closing Tax Periods until the earlier of (i) the seventh anniversary of the Closing Date and (ii) the expiration of the applicable statute of limitations, without the prior written consent of the Representative.

(b) Transfer Taxes. Fifty percent (50%) of all Transfer Taxes arising from the transactions contemplated hereunder (as well as any reasonable costs associated with the filing of Tax Returns for such Transfer Taxes) will be paid by each of Buyer and the Representative (on behalf of the Unitholders). Each Tax Return for Transfer Taxes will be prepared by the party that customarily has primary responsibility for filing such Tax Return, with the non-preparing party to reimburse the preparing party for the non-preparing party's share of Transfer Taxes associated with such Tax Return.

(c) Tax Matters. Buyer shall promptly notify the Representative upon receipt by Buyer, the Company or any subsidiary of Buyer or the Company of any notice of any inquiries, assessments, proceedings or similar events received from any Governmental Authority with respect to Taxes of the Company imposed in respect of Pre-Closing Tax Periods or notice of a claim by a Third Party, in either case for which Unitholders are required to indemnify any Buyer Indemnified Party pursuant to this Agreement (any such inquiry, assessment, proceeding or similar event, a "Tax Matter"); provided, however, that Buyer's failure to give timely notice to the Representative shall not prevent Buyer from making an indemnity claim under this Agreement, except to the extent (if any) such failure actually and materially prejudiced the Unitholders. The Representative shall have the right to control, at the Unitholders' expense, the conduct and resolution of any Tax Matter for a Pre-Closing Tax Period solely with respect to Tax Returns that reflect income that flows through to the Unitholders, except, as to TAMUS, the Representative must obtain written approval of TAMUS; provided, however, the Representative shall consult with Buyer before taking any significant action in connection with such Tax Matter, and the Buyer shall have the right to participate in the conduct and resolution of any Tax Matter at its expense. The Representative shall use commercially reasonable efforts to timely keep the Buyer apprised of any material developments in any such Tax Matter and shall not enter into any settlement of or otherwise compromise any such Tax Matter without the prior written consent of Buyer, which consent shall not be unreasonably conditioned, withheld or delayed. Buyer shall have the right to control the conduct and resolution of any other Tax Matter relating to a Pre-Closing Tax Period or Straddle Period at the Unitholders' expense, except, as to TAMUS, the Representative must obtain written approval of TAMUS; provided, however, Buyer shall consult with the Representative before taking any significant action in connection with such Tax Matter, and the Representative shall have the right to participate in the conduct and resolution of any Tax Matter at its expense. Buyer shall use commercially reasonable efforts to timely keep the Representative apprised of any material developments in any such Tax Matter and shall not enter into any settlement of or otherwise compromise any such Tax Matter without the prior written consent of the Representative, which consent shall not be unreasonably conditioned, withheld or delayed. With respect to any audit of a Pre-Closing Tax Period of the Company that is subject to the partnership audit and procedures of the Bipartisan Budget Act of 2015 to the extent the Audit Opt-Out Election is not permitted under applicable Law to be made with respect to the Company for any applicable jurisdiction (federal, state or local), the Company (i) will elect the application of Section 6226 of the Code (or any similar provisions under state or local law) or (at the Representative's sole discretion unless an election under Section 6226 of the Code is unavailable or ineffective) require the Unitholders to either file amended tax returns in accordance with Section 6225(c)(2)(A) of the Code (or any similar provisions under state or local law) or follow the procedures in Section 6225(c)(2)(B) (or any similar provisions under state or local law) and (ii) will not elect for the Company to pay any "imputed underpayment" within the meaning of Code Section 6225 (or any corresponding or similar provision of state, local or foreign tax law). In the event of any conflict between the provisions of this Section 6.3(c) and the provisions of Section 7.6, the provisions of this Section 6.3(c) shall control.

(d) Preparation of Tax Returns. The parties acknowledge and agree that as a consequence of the transactions contemplated hereunder, the Company shall terminate as a partnership for U.S. federal and applicable state and local income tax purposes pursuant to Revenue Ruling 99-6 on the Closing Date. The Representative shall have an IRS Form 1065, U.S. Return of Partnership Income, and any state and local income Tax Returns that are pass-through Tax Returns with respect to the Company prepared for the taxable year ending on the Closing Date (the "Final Income Tax Returns"). All such Final Income Tax Returns shall be prepared in a manner consistent with the past practices of the Company, except as otherwise required by this Agreement or applicable Law, and provided that any income of the Company from deferred revenue or prepaid amounts received on or prior to the Closing Date shall be reported on the Final Income Tax Returns for the taxable period ending on the Closing Date. The Company shall, to the maximum extent permitted under applicable Law, make the election described in Code Section 6221(b) on such Tax Returns (and, for state and local tax jurisdictions, any similar or corresponding election) (collectively, the "Audit Opt-Out Election"). The Representative shall provide Buyer with a draft of each Final Income Tax Return at least [***] days prior to the due date for filing thereof for Buyer's review, comment and approval (not to be unreasonably withheld, conditioned or delayed). The Representative shall incorporate all reasonable comments of the Buyer to such Final Income Tax Returns so long as such comments are consistent with the foregoing principles. Buyer shall prepare and file, or cause to be prepared and filed, all Tax Returns (other than the Final Income Tax Returns) of the Company for periods ending on or before the Closing Date and Straddle Periods that are due after the Closing Date, in each case in a manner consistent with the past practice of the Company unless otherwise required by applicable Tax Law. Buyer shall deliver any such Tax Return that is an income tax return for a Straddle Period to the Representative at least [***] days before the due date of such Tax Return for review and comment by the Representative (unless such Tax Return is due within forty (40) days of the Closing Date, in which case Buyer shall use reasonable efforts to provide the Representative a draft of such Tax Return prior to the filing thereof). The Representative shall have [***] days to review and comment on such draft Tax Returns (unless such Tax Return is due within thirty (30) days of the Closing Date, in which case the Representative shall use reasonable efforts to provide comments as promptly as practicable after the receipt of any draft from Buyer). Buyer shall consider such comments in good faith. Notwithstanding the foregoing, nothing in this Section 6.3(d) shall be deemed to require Buyer or the Company to delay timely filing any such Tax Return on the date any such Tax Return is due (taking into account any applicable extensions available with respect thereto). Buyer shall cause any amounts shown to be due on such Tax Returns to be timely remitted to the applicable Governmental Authority.

(e) Straddle Period. In the case of any taxable period of the Company that includes, but does not end on, the Closing Date ("Straddle Period"), (i) the amount of any Taxes based on or measured by income or receipts, sales or use taxes, employment taxes, or withholding taxes of such for the Pre-Closing Tax Period shall be determined based on an interim closing of the books as of the close of business on the Closing Date (and for such purpose, the taxable period of any partnership, or other pass-through entity or any non-U.S. entity in which such Person holds a beneficial interest shall be deemed to terminate at such time) and (ii) the amount of any other Taxes for a Straddle Period that relates to the Pre-Closing Tax Period shall be deemed to be the amount of such Tax for the entire taxable period multiplied by a fraction the numerator of which is the number of days in the taxable period ending on and including the Closing Date and the denominator of which is the number of days in such Straddle Period.

Section VI.4 Public Disclosures. None of the parties shall make any public disclosure or permit any of their respective Affiliates to make any disclosure (whether or not in response to an inquiry) of the subject matter of this Agreement or the other Transaction Documents unless previously approved by

Buyer and the Representative in writing, which approval shall not be unreasonably withheld or delayed, except as such release or announcement may be required by Applicable Law.

Section VI.5 No Solicitation of Employees. Each Unitholder, other than TAMUS, shall not, and shall cause its agents, representatives, Affiliates, employees, stockholders, officers and directors to not, for a period of [***] from the date hereof, directly or indirectly, solicit for employment or employ or cause to leave the employ of Buyer or any of its Affiliates any individual serving as an officer or employee of Buyer or any of its Affiliates, without obtaining the prior written consent of Buyer.

Section VI.6 Competition. For a period of [***] from the date hereof, each Unitholder (other than TAMUS) shall not conduct, participate in, or fund (directly or indirectly) any research, Development or Manufacturing activities of any antisense oligonucleotide that is intended to modulate (or that otherwise modulates) UBE3A for the treatment or diagnosis of Angelman Syndrome, other than a Product, or facilitate any of the foregoing, other than on behalf of the Company. For the avoidance of doubt, a Unitholder may provide services to a Person who conducts, participates in, or funds (directly or indirectly) research, Development or Manufacturing activities of an antisense oligonucleotide intended to modulate (or that otherwise modulates) UBE3A for the treatment or diagnosis of Angelman Syndrome provided that such Unitholder recuses itself from such activities. For the further avoidance of doubt, but without limiting Section 6.2, if a Third Party approaches a Unitholder for advice on endpoints, information about the patient population, or assistance with disseminating information about a Clinical Study, such Unitholder shall be permitted to provide such assistance.

ARTICLE VII
REMEDIES

Section VII.1 Indemnification by All Unitholders. Subject to the limitations set forth in this Article VII, from and after the Closing, the Unitholders covenant and agree to indemnify, defend, protect and hold harmless Buyer and its Affiliates (including, after the Closing, the Company) and each of Buyer's and its Affiliates' respective officers, directors, control persons, employees, stockholders, representatives, permitted assigns and successors (the "Buyer Indemnified Parties"), severally on a pro rata basis, from, against and in respect of all Damages that may be suffered, sustained, incurred or paid by any Buyer Indemnified Party to the extent resulting from or arising out of:

(a) any misrepresentation, breach or inaccuracy of any representation or warranty of the Company set forth in Article II of this Agreement or in the Disclosure Schedule (or any claim by any Third Party that, if true, would constitute such a misrepresentation, breach, or inaccuracy) or under any other Transaction Document;

(b) any nonfulfillment or breach of any covenant or agreement on the part of the Company set forth in this Agreement or any other Transaction Document (or any claim by a Third Party that, if true, would constitute such a nonfulfillment or breach);

(c) any (i) Company Indebtedness or (ii) Transaction Costs, in each case, to the extent not deducted from the Definitive Initial Purchase Consideration (including after taking into account the adjustment thereto made pursuant to Section 1.3);

(d) (i) any Taxes of the Company (or for which the Company may otherwise be liable, including for the avoidance of doubt, pursuant to the Bipartisan Budget Act of 2015 (or any corresponding provisions of state or local law) attributable to any Pre-Closing Tax Period except to the extent such Taxes were specifically identified as included in the calculation of the Definitive Net Working Capital Adjustment or were included in Indebtedness, (ii) any Taxes resulting from or attributable to the consummation of the transactions contemplated by this Agreement (including Taxes for which Unitholders are responsible pursuant to Section 6.3(b)), (iii) any Taxes imposed on any Unitholder for any taxable period, (iv) any Taxes of any member of an affiliated, consolidated, combined or unitary group of which the Company is or was a member prior to the Closing Date, including Taxes imposed on the Company under Treasury Regulations Section 1.1502-6 or under any comparable, analogous or similar provision of state, local or non-U.S. Law, and (v) any Taxes of any Person imposed on the Company or for which the Company may otherwise be liable) as a transferee or successor, or pursuant to any contractual obligation or otherwise, or pursuant to any law, rule or regulations, which Taxes relate to an event, agreement or transaction occurring prior to the Closing; and

(e) any inaccuracy, omission, misstatement or error in the Allocation Schedule or any certificate, schedule, exhibit or other similar document delivered by the Company or any Unitholder, or the failure to allocate amounts paid to the Payments Administrator in accordance with the Allocation Schedule or Operating Agreement (including any claim or allegation made by or on behalf of any current or former holder of Units or alleged holder of Units challenging, disputing or objecting to the amount of the portion of any payment to Unitholders hereunder).

Section VII.2 Indemnification by Each Unitholder. Each Unitholder covenants and agrees to indemnify, defend, protect and hold harmless the Buyer Indemnified Parties severally and not jointly from and against all Damages that may be suffered, sustained, incurred or paid by any Buyer Indemnified Party to the extent resulting from or arising out of:

(a) any misrepresentation, inaccuracy or breach of any representation or warranty of such Unitholder set forth in Article III of this Agreement (or any claim by any Third Party that, if true, would constitute such a misrepresentation, breach, or inaccuracy); and

(b) any nonfulfillment or breach of any covenant or agreement on the part of such Unitholder set forth in this Agreement (or any claim by a Third Party that, if true, would constitute such a nonfulfillment or breach).

Section VII.3 Indemnification by Buyer. Buyer covenants and agrees to indemnify, defend, protect and hold harmless each Unitholder and each Unitholder's respective officers, directors, control persons, employees, equity holders, representatives, permitted assigns and successors (the "Unitholder Indemnified Parties") from and against all Damages that may be suffered, sustained, incurred or paid by any Unitholder Indemnified Party to the extent resulting from or arising out of:

(a) any misrepresentation, inaccuracy or breach of any representation or warranty of Buyer set forth in Article IV of this Agreement (or any claim by any Third Party that, if true, would constitute such a misrepresentation, breach, or inaccuracy); and

(b) any nonfulfillment or breach of any covenant or agreement on the part of Buyer set forth in this Agreement (or any claim by a Third Party that, if true, would constitute such a nonfulfillment or breach).

Section VII.4 Limitations on Indemnification Obligations.

(a) Notwithstanding the above, there shall be no liability for indemnification under Section 7.1(a), Section 7.2(a) or Section 7.3(a) or, as the case may be, unless the aggregate amount of Damages with respect to all Claims thereunder exceeds [***] Dollars ($[***]) (the "Deductible"), at which point the Indemnifying Party(ies) will only be obligated to indemnify the Indemnified Parties for the amount of Damages described in Section 7.1(a), Section 7.2(a) or Section 7.3(a) (as applicable) exceeding the Deductible; provided that the Deductible shall not apply to the misrepresentation, breach or inaccuracy of any representation or warranty made by the Unitholders in Article III and any of the following Sections: Section 2.1 (due organization), Section 2.2 (authorization; no conflict), Section 2.3 (capitalization), Section 2.9 (assets), Section 2.10 (taxes), Section 2.16 (intellectual property), Section 2.19 (brokers and agents), Section 3.1 (due organization, authorization), Section 3.3 (ownership of units), Section 3.7 (brokers), Section 4.1 (organization and authorization), Section 4.2 (authorization; no conflict), and Section 4.3 (brokers and agents) (collectively, the "Fundamental Reps"). With respect to any claim as to which an Indemnified Party may be entitled to indemnification hereunder, the Indemnifying Party shall not be liable for any Damages arising out of any individual or related set of facts and circumstances that do not exceed thirty-seven thousand five hundred Dollars ($37,500) (which Damages shall not be counted towards the Deductible).

(b) The indemnification obligations of each of the parties under Section 7.1(a), Section 7.2(a) and Section 7.3(a), respectively, shall not exceed on a cumulative basis for the Unitholders, on the one hand, or Buyer on the other hand, an amount equal to the Cap (as defined below), determined as of the date of assertion of any Claim for indemnification hereunder, provided, that the Cap shall not apply with respect to any misrepresentation, breach or inaccuracy of any Fundamental Rep, which shall be capped at an amount equal to the Total Purchase Consideration (determined as of the date of assertion of any Claim for indemnification hereunder). For purposes of this Agreement, the "Cap" shall be equal to the Escrow Amount.

(c) The amount of Damages recoverable by the Buyer and Buyer Indemnified Parties under this Article VII shall be reduced, on a dollar-for-dollar basis, by any amounts actually recovered (after deducting therefrom the full amount of costs, Taxes and expenses incurred in procuring such recovery, including any increase in premiums and any deductible or retention associated therewith) by the Buyer and Buyer Indemnified Parties under insurance policies or other collateral sources (such as contractual indemnities of any Person which are contained outside of this Agreement). Buyer agrees to use commercially reasonable efforts to make a claim under any insurance policy, or against any other collateral source, available to it with respect to the facts giving rise to the right to indemnification hereunder. For clarity, the making of any such claim shall not impact the timing or amount of any indemnification that may be due from the Unitholders to the Buyer Indemnified Parties. To the extent that any Buyer Indemnified Party receives a payment under any such insurance policy or collateral source corresponding to an indemnification payment made by the Unitholders with respect a Claim for indemnification hereunder, such Buyer Indemnified Parties shall reimburse the Unitholders for such indemnification payment up to the amount of such insurance policy or collateral source payment.

(d) The right to indemnification or other remedy based on the representations, warranties, covenants and agreements herein shall not be affected or deemed waived by reason of any investigation made or conducted by or on behalf of the party seeking indemnification (including by any of their advisors or representatives) or by reason of the fact that the party seeking indemnification or any of its advisors or representatives knew, or should have known, that such representation or warranty is or might be inaccurate or that any fact, event or circumstance had or had not occurred.

(e) Notwithstanding anything herein to the contrary, for purposes of (i) determining whether a breach of the applicable representation or warranty has occurred and (ii) calculating Damages under this Section 7.4, the representations and warranties set forth in Article II, Article III, and Article IV shall be read without regard to any limitation as to materiality or Material Adverse Effect contained therein.

Section VII.5 Survival and Expiration of Representations, Warranties, Covenants and Agreements.

(a) The representations and warranties of the Unitholders and Buyer contained in this Agreement shall survive the Closing and shall expire on the date that is [***] months after the Closing Date (the "Survival Date"); provided, that (i) the Fundamental Reps shall survive until the expiration of [***] plus [***] Business Days. Notwithstanding the preceding sentence, the indemnification obligations with respect to any representation or warranty in respect of which indemnity may be sought under this Agreement shall survive the time at which it would otherwise terminate pursuant to the preceding sentence if an Indemnified Party gives written notice of a claim for indemnification with respect to such representation or warranty in accordance with the procedures set forth in Section 7.6 prior to such time.

(b) All covenants and agreements to be performed in their entirety prior to the Closing Date shall terminate upon the Closing except as expressly provided herein. All covenants and agreements to be performed in whole or in part after the Closing Date shall survive in accordance with their terms.

Section VII.6 Indemnification Procedures. Subject to Section 6.3(c), which shall control with respect to Tax Matters, all claims for indemnification under this Section 7.6 ("Claims") shall be asserted and resolved as follows:

(a) Buyer Indemnified Parties who make a Claim against the Unitholders shall be required to deliver such Claim to the Representative and not to each Unitholder. Any dispute in connection with such Claim shall be handled by the Buyer Indemnified Parties who made such Claim and the Representative.

(b) In the event that any Person entitled to indemnification hereunder (the "Indemnified Party") has a Claim against any party obligated to provide indemnification pursuant to Section 7.1, Section 7.2 or Section 7.3 hereof (the "Indemnifying Party") which has been asserted against an Indemnified Party by a Third Party (a "Third Party Claim"), the following provisions shall apply:

(i) With reasonable promptness after the Indemnified Party receives a written notice of a Third Party Claim and determines that such Third Party Claim would reasonably be expected to give rise to a right of indemnification hereunder, the Indemnified Party shall notify the Indemnifying Party of such Third Party Claim (the "Claim Notice"). The Indemnified Party's

failure to give such notice of any Third Party Claim pursuant to the foregoing sentence shall not relieve the Indemnifying Party of any indemnification obligations it may have to the Indemnified Party, except and only to the extent the failure to give such notice actually and materially prejudices the Indemnifying Party with respect to such Third Party Claim.

(ii) If any Indemnified Party asserts a Claim involving a Third Party Claim, the Indemnifying Party shall, within [***] days from delivery of the Claim Notice (the "Notice Period"), notify the Indemnified Party (A) whether or not such Indemnifying Party disputes its indemnification obligation to the Indemnified Party hereunder with respect to such Third Party Claim and (B) if such Indemnifying Party does not dispute such indemnification obligation, whether or not the Indemnifying Party desires, at the sole cost and expense of the Indemnifying Party, to defend against such Third Party Claim, provided that the Indemnified Party is hereby authorized (but not obligated) prior to and during the Notice Period to file any motion, answer or other pleading and to take any other action which the Indemnified Party shall deem necessary or appropriate to protect the Indemnified Party's interests. If, and for so long as, (I) the Indemnifying Party notifies the Indemnified Party within the Notice Period that the Indemnifying Party agrees to provide full indemnification with respect to such Third Party Claim (without regard to the limitations in this Section 7.6) and desires to defend the Indemnified Party against such Third Party Claim, and (II) the Third Party Claim does not (1) involve criminal liability or any admission of wrongdoing, (2) seek equitable relief or any other non-monetary remedy against the Indemnified Party, (3) involve any Governmental Authority as a party thereto or (4) involve Taxes, then except as hereinafter provided, such Indemnifying Party shall have the right to defend against such Third Party Claim by appropriate proceedings with legal counsel reasonably acceptable to the Indemnified Party, which proceedings shall be promptly settled or diligently prosecuted by such party to a final conclusion; provided that, unless the Indemnified Party otherwise agrees in writing, the Indemnifying Party may not settle any matter (in whole or in part) unless such settlement (w) includes a complete and unconditional release of the Indemnified Party and its Affiliates in respect of the Third Party Claim, (x) involves no admission of wrongdoing by the Indemnified Party or its Affiliates, (y) excludes any injunctive or non-monetary relief applicable to the Indemnified Party or its Affiliates and (z) the monetary relief contemplated by such settlement is fully covered by the Indemnifying Party pursuant to this Section 7.6. If the Indemnified Party desires to participate in (but not control) any such defense or settlement, the Indemnified Party may do so at its sole cost and expense; provided, that, if the Indemnified Party reasonably concludes, based on advice of counsel, that there are issues that raise actual or potential conflicts of interest between the Indemnifying Party and the Indemnified Party, the Indemnifying Party shall be responsible for the reasonable fees and expenses of one counsel to such Indemnifying Party in connection with such defense. For the avoidance of doubt, the assumption of the conduct and control of any Third Party Claim includes posting bonds or other security required by the court or adjudicative body before which such proceeding is taking place.

(iii) If (A) the Indemnifying Party elects not to defend the Indemnified Party against such Third Party Claim, whether by failure of the Indemnifying Party to give the Indemnified Party timely notice as provided above or otherwise, (B) the terms of this Agreement do not permit the Indemnifying Party to defend the Indemnified Party against such Third Party Claim, or (C) the Indemnified Party, based on advice of counsel, has different or additional defenses available to it that present a conflict with the interests of the Indemnifying Party, then the Indemnified Party shall be entitled, subject to the terms and limitations contained in this

Section 7.6, to its own counsel with respect to the participation in or defense of such Third Party Claim, at the sole cost and expense of the Indemnified Party.

(iv) In the event that the Indemnifying Party or the Indemnified Party (the "Defending Party") undertakes any such defense against any such Third Party Claim (to the extent that such party is permitted to undertake such defense pursuant to the terms and conditions of this Section 7.6), the other party (the "Non-Defending Party") shall reasonably cooperate with the Defending Party in such defense and make available to the Defending Party all witnesses, pertinent records, materials and information in the Non-Defending Party's possession or under the Non-Defending Party's control related thereto as is reasonably required by the Defending Party. The Defending Party shall also have the right to receive from the Non-Defending Party copies of all pleadings, notices and communications with respect to such Third Party Claim that are in the possession of the Non-Defending Party.

Section VII.7 Fraud, etc.. If any Buyer Indemnified Party asserts an indemnification claim based on fraud, willful breach or intentional misrepresentation, no limitations contained in this Agreement (including those set forth in Section 7.4 and Section 7.5) shall apply to such claim.

Section VII.8 Manner of Payment; Release of Escrow.

(a) Subject to the terms of this Article VII, any indemnification obligations of the Unitholders to any Buyer Indemnified Parties pursuant to this Article VII shall be satisfied (i) first, to the extent there is a sufficient Escrow Amount, by release of funds to the Buyer Indemnified Parties from the Escrow Amount by the Escrow Agent pursuant to the Escrow Agreement by the joint instructions of Representative and Buyer, which release shall accordingly reduce the Escrow Amount, (ii) following depletion or release of the Escrow Amount to the Unitholders, by the Unitholders on a pro rata basis, provided that Buyer may, in its sole discretion at any time, choose to set-off any indemnification obligations of the Unitholders to any Buyer Indemnified Parties pursuant to this Section against Milestone Payments, Priority Review Voucher Payments, or Royalty Payments payable hereunder.

(b) Promptly following the date that is fifteen (15) months after the Closing Date (the "Escrow Release Date"), following receipt of a joint instruction letter from Buyer and Representative, the Escrow Agent shall release, in accordance with such joint instructions and the Escrow Agreement, the then remaining balance of the Escrow Amount (to the extent not utilized to pay any Buyer Indemnified Parties for any indemnification claim) to the Representative for further distribution to the Unitholders in accordance with the Allocation Schedule, except that the Escrow Agent shall retain an amount (up to the total amount then-held by the Escrow Agent) equal to the amount of claims for indemnification under this Article VII asserted prior to the date that is fifteen (15) after the Closing Date but which are not yet resolved ("Unresolved Claims"). The amount of the Escrow Amount retained for Unresolved Claims shall be released by the Escrow Agent (to the extent not utilized to pay any Buyer Indemnified Parties for any such claims resolved in favor of such Buyer Indemnified Parties) upon their final and binding resolution in accordance with this Article VII and the Escrow Agreement.

Section VII.9 Tax Treatment of Indemnity Payments. Each of the Representative, the Unitholders and Buyer agree to treat any indemnity payment made pursuant to this Article VII as an adjustment to the Definitive Initial Purchase Consideration for all Tax purposes to the extent permitted by Applicable Law.

Section VII.10 Exclusive Remedy. Except with respect to claims resulting from fraud, willful breach or intentional misrepresentation and other remedies that cannot be waived as a matter of law and injunctive relief (including specific performance), and except as set forth in Section 1.8, each Unitholder and Buyer agree that, from and after the date of this Agreement, this Article VII shall be the exclusive remedy with respect to any misrepresentation, breach or inaccuracy of the representations or warranties, or the nonfulfillment or breach of the covenants and agreements set forth in this Agreement.

Section VII.11 Release.

(a) As an inducement to Buyer to enter into this Agreement and consummate the transactions contemplated hereby and for other good and sufficient consideration, each of the Unitholders, with the intention of binding himself, herself or itself and each of such Unitholder's officers, directors, managers, employees, representatives, Affiliates, heirs, executors, administrators and assigns (the "Releasors"), hereby release, acquit and forever discharge Buyer, the Company, the Representative and each of their past and present Affiliates, officers, directors, managers, employees, representatives, and all Persons acting by, through, under, or in concert with such Persons (the "Releasees"), of and from any and all, expect from those explicitly granted under this Agreement, manner of action or actions, cause or causes of action, suits, arbitrations, demands, debts, contracts, agreements, promises, liability, Damages, or loss of any nature whatsoever, known or unknown, suspected or unsuspected, fixed or contingent, direct, derivative, vicarious or otherwise, whether based in contract, tort, or other legal, statutory, or equitable theory of recovery, each as though fully set forth at length herein, (hereinafter, a "Released Claim"), which the Releasors now have or may hereafter have against the Releasees, or any of them, by reason of any matter, cause, act, omission or thing whatsoever in any way arising out of, based upon, or relating to Unitholders ownership of Securities or other equity interest in the Company (the "Released Matters"); provided, however, that nothing set forth in this Section 7.11 shall affect the ability of any of such Unitholders to bring a Released Claim under this Agreement.

(b) Each Unitholder represents and warrants to the Company and Buyer that there has been no assignment or other transfer of any interest in any Released Claim arising out of or based upon any of the Released Matters which such Unitholder may have against any of the Releasees.

(c) Each Unitholder represents and warrants to the Company and Buyer that is has not filed, nor has as of the date of this Agreement, any Released Claims arising out of or based upon any of the Released Matters against any of the Releasees.

Section VII.12 TAMUS Limitations. Notwithstanding anything in this Agreement to the contrary, any obligations in this Agreement for TAMUS to indemnify, defend, protect, and hold harmless any Person, party, Affiliate, Third Party, or any other entity, such obligation is subject to the extent allowed by the laws of the State of Texas. Notwithstanding anything in this Agreement to the contrary, any obligation of TAMUS to represent and warrant, including variations thereof, in this Agreement is limited to only the obligation to represent. If there is a conflict between any other section of this Agreement and this Section 7.12, this Section 7.12 will control.

ARTICLE VIII
DEFINITIONS

Section VIII.1 Specific Definitions. Terms in this Agreement with initial letters capitalized, whether used in the singular or the plural, if not otherwise defined herein shall have the meaning ascribed to such term in the Unitholder Option Agreement. As used in this Agreement, the following capitalized terms shall have the meanings set forth below:

"Accounting Firm" has the meaning set forth in Section 1.3(d).

"Action" means any action, suit, arbitration, demand, claim, complaint, hearing, examination, audit, investigation, demand letter, warning letter, proceeding or request for information of any nature, civil, criminal, regulatory, administrative or otherwise, whether at law or in equity, by or before a Governmental Authority.

"Actual Adjustment" means (a) the Definitive Initial Purchase Consideration as set forth on the Final Statement of Purchase Consideration minus (b) the Estimated Initial Purchase Consideration as set forth on the Estimated Initial Purchase Consideration Calculation.

"Affiliate" means, with respect to any specified Person, any other Person which, at the time of determination, directly or indirectly Controls, is Controlled by or is under common Control with such Person.

"Agreement" has the meaning set forth in the preamble.

"Allocation Schedule" has the meaning set forth in Section 1.4(b).

"Angelman Syndrome" means a rare genetic and neurological disorder characterized by severe developmental delay and learning disabilities; absence or near absence of speech and inability to coordinate voluntary movements (ataxia) caused by deletion or abnormal expression of the maternal UBE3A.

"Antitrust Authority" means any Governmental Authority with valid jurisdiction over applicable antitrust matters.

"Antitrust Filings" means any filings which are necessary under the HSR Act or any other applicable antitrust Law.

"Applicable Law" means any applicable federal, state, local, municipal, foreign or other law, order, judgment, rule, code, statute, regulation, constitution, resolution, principle of common law, requirement, variance, decree, writ, injunction, award, ruling notice, or ordinance issued, enacted, adopted, promulgated, implemented or otherwise put into effect by or under the authority of any Governmental Authority.

"Assets" means all tangible, intangible and other assets, Contracts, rights and properties used, held for use, owned or purportedly owned by the Company, to the extent used or held for use in the Business.

"Base Consideration" has the meaning set forth in Section 1.3(a)(ii).

"Biomarker" means a defined characteristic that is measured as an indicator of normal biological processes, pathogenic processes, or responses to an exposure or intervention, including therapeutic interventions.

"Books and Records" means any material business records, financial books and records, minute books, stock record books, sales order files, purchase order files, engineering order files, warranty and repair files, supplier lists, customer lists, dealer, representative and distributor lists, studies, surveys, analyses, strategies, plans, forms, designs, diagrams, drawings, specifications, technical data, and production and quality control records and formulations.

"Business" or "business" means the business of the Company as conducted as of the Closing Date, including all activities conducted by or on behalf of the Company as of the Closing Date.

"Business Day" means any day that is not a Saturday or a Sunday or a day on which banks located in San Francisco, California are authorized or required to be closed.

"Buyer" has the meaning set forth in the preamble.

"Buyer Indemnified Parties" has the meaning set forth in Section 7.1.

"Buyer Options" means the options granted to Buyer pursuant to the Unitholder Option Agreement.

"CA Approval" has the meaning set forth in Section 1.6(c)(1).

"Calendar Year" means each successive period of twelve (12) months commencing on January 1 and ending on December 31.

"Cap" has the meaning set forth in Section 7.4(b).

"Cessation" means each Selling Party has ceased to Develop, Manufacture and Commercialize all Royalty-Bearing Products; *provided that* pauses in Development, Manufacturing or Commercialization due to a [***] shall not satisfy this definition of Cessation.

"Chosen Courts" has the meaning set forth in Section 9.5.

"Claim Notice" has the meaning set forth in Section 7.6(b)(i).

"Claims" has the meaning set forth in Section 7.6.

"Class A Common Units" has the meaning set forth in the recitals.

"Class B Common Units" has the meaning set forth in the recitals.

"Clinical Studies" means human clinical trials anywhere in the world, including any Phase 1 Clinical Studies, Phase 2 Clinical Studies, Phase 3 Clinical Studies, or variations or subsets of such trials (for example, a Phase 2/3 clinical trial or Phase 1B Clinical Study).

"Clinical Study Report" means the final clinical study report for a Clinical Study of a Product.

"Closing" has the meaning set forth in Section 1.2.

"Closing Cash" means, as of the Closing, the amount of (a) cash held on hand or in deposit, checking or other similar accounts, including money market accounts, by or for the benefit of the Company, and (b) cash equivalents (including marketable securities) held by or for the benefit of the Company, in each case, determined in accordance with GAAP; provided, that Closing Cash shall (i) not include any deposits in transit or checks issued to the Company that have not been cashed as of immediately prior to Closing and (ii) be calculated net of any restricted cash or bank overdrafts.

"Closing Date" has the meaning set forth in Section 1.2.

"Closing Date Calculations" has the meaning set forth in Section 1.3(b).

"Closing Date Cash Payment" has the meaning set forth in Section 1.4(a)(iii).

"Closing Date Company Indebtedness" means the Company Indebtedness as of immediately prior to the Closing.

"Code" means the U.S. Internal Revenue Code of 1986, as amended.

"Combination Product" means a product that includes any Royalty-Bearing Product and [***] other than such Royalty-Bearing Product. Pharmaceutical dosage form vehicles, adjuvants, and excipients shall not be deemed to be "active ingredients," except in the case where such vehicle, adjuvant, or excipient is recognized by the U.S. Food and Drug Administration, or any successor agency thereto as an active ingredient in accordance with 21 C.F.R. 210.3(b)(7).

"Commercialization" or "Commercialize" means all activities undertaken relating to the marketing, offering for sale or selling of a product to physicians and their patients, including packaging, labelling, advertising, education, planning, marketing, promotion, market and product support, sales representative detailing, medical support and post-marketing commitments.

"Commercially Reasonable Efforts" means, with respect to a party, the efforts and resources typically used by biotechnology or pharmaceutical companies similar in size and scope to such party to perform the obligation at issue, which efforts shall not be less than those efforts made with respect to other products at a similar stage of development or in a similar stage of product life, with similar developmental risk profiles, of similar market and commercial potential, taking into account the proprietary position of the products, the regulatory structure involved, Regulatory Authority-approved labeling, the profitability of the applicable products, issues of safety and efficacy, the likely timing of the product's entry into the market, the likelihood of receiving Regulatory Approval, and other relevant scientific, technical and commercial factors but without regard to any payments owed under this Agreement.

"Company" has the meaning set forth in the preamble.

"Company Benefit Plan" means each benefit or compensation plan, program, policy, arrangement, agreement, obligation, custom or practice, whether or not legally enforceable, including employment or consulting agreements, severance agreements or pay policies, stay or retention bonuses or compensation, executive or bonus or incentive compensation programs or arrangements, sick leave, vacation pay, patent award programs, salary continuation for disability, consulting, or other compensation arrangements, workers' compensation, retirement, deferred compensation, bonus, stock option or purchase plans or programs, equity or equity-based arrangements, hospitalization, medical insurance, life insurance, tuition reimbursement or scholarship programs, employee discount programs, meals, travel, or vehicle allowances, any plans providing benefits or payments in the event of a change of control, change in ownership or effective control, and any other benefit plan, agreement, program, policy, commitment or other arrangement (including any related funding mechanism now in effect or required in the future), whether formal or informal, oral or written, insured or self-insured, funded or unfunded or domestic or foreign, relating to any present, former or retired employee, officer, director, member or other service provider, or any dependent or beneficiary of any of the foregoing individuals, (a) that is sponsored or maintained by the Company, any of its subsidiaries or any of their respective ERISA Affiliates, (b) to which the Company, any of its subsidiaries or any of their respective ERISA Affiliates is required to contribute or has an obligation to contribute, or (c) under or with respect to which the Company or any of its subsidiaries has any current or potential Liability, whether contingent or otherwise.

"Company Employee Indebtedness" means, without duplication, the aggregate amount of any compensation or benefits owed to any current or former consultant, director, officer, employee or other service provider of the Company related to the transactions contemplated by this Agreement (including severance, retention, change of control and similar payment obligations or bonuses), even if further contingent on a cessation of employment or the provision of additional services, plus any payroll Taxes of the Company attributable to such compensation and benefits, together with any interest and penalties.

"Company In-Licenses" has the meaning set forth in Section 2.15(a)(xi).

"Company Indebtedness" means, without duplication, the aggregate amount of (a) any obligations of the Company for borrowed money, or with respect to deposits or advances of any kind to the Company, and any prepayment premiums, transaction premiums, penalties and any other fees and expenses paid to satisfy such indebtedness, (b) any obligations of the Company evidenced by bonds, debentures, notes or similar instruments, (c) any obligations of the Company upon which interest charges are customarily paid (excluding trade accounts payable), (d) any obligations of the Company under conditional sale or other title retention agreements, (e) any obligations of the Company issued or assumed as the deferred purchase price of property or services (excluding obligations of the Company to creditors for goods and services incurred in the ordinary course of such Person's business), (f) any capitalized lease obligations of the Company, (g) any deferred revenue obligations of the Company, (h) any obligations of others secured by any Lien (other than a Permitted Lien) on property or assets owned or acquired by the Company, whether or not the obligations secured thereby have been assumed, (i) any obligations of the Company under interest rate or currency swap transactions (valued at the termination value thereof), (j) any amounts owed with respect to drawn letters of credit issued for the account of the Company, (k) any obligations of the Company to purchase securities (or other property) which arise out of or in connection with the sale of the same or substantially similar securities or property, (l) any guaranties or arrangements having the economic effect of a guaranty by the Company of any indebtedness of any other Person, (m)

any accrued interest or penalties on any of the foregoing and (n) unpaid income Taxes relating to any Pre-Closing Tax Period; provided, that for the sake of clarity, "Company Indebtedness" shall in no event include any Transaction Costs, and shall only include accounts payable to the extent aged at least thirty (30) days.

"Company Intellectual Property" means all (i) Company Owned Intellectual Property and (ii) Company Licensed IP.

"Company Licensed IP" means any Intellectual Property that is licensed to the Company from, or otherwise permitted to be used by the Company, including all Intellectual Property that is subject to a Company In-License that is set forth in Schedule 2.16(a)(i).

"Company Out-Licenses" has the meaning set forth in Section 2.15(a)(xi).

"Company Owned Intellectual Property" means all Intellectual Property owned or purported to be owned (in each case, solely or jointly) by the Company, including the Intellectual Property that is set forth on Schedule 2.16(a)(i).

"Company Pass-Through Expenses" means all outstanding reimbursable amounts due from Buyer to the Company for mutually agreed costs and expenses of work performed by the Company or its vendors on back-up compound program activities, expansion cohorts, and other costs and expenses that may be agreed in the performance of the Development Program, in each case for which Ultragenyx has agreed to reimburse the Company.

"Company Registrations" has the meaning set forth in Section 2.16(a)(i).

"Company Representative" means the Company's officers, directors, controlling persons, unitholders, employees, representatives, agents, advisors and Affiliates.

"Confidential Information" has the meaning set forth in Section 6.2(a).

"Confidentiality Agreement" means that certain Mutual Nondisclosure Agreement, dated as of February 14, 2019, by and between the Company and Buyer, as the same may be amended from time to time.

"Contract" means any contract, plan, undertaking, arrangement, concession, understanding, agreement, agreement in principle, franchise, permit, instrument, license, lease, sublease, note, bond, indenture, deed of trust, mortgage, loan agreement or other binding commitment, whether written or oral.

"Control" means, as to any Person, (a) the power to directly or indirectly cause the direction of the management and policies of such Person, whether through the direct or indirect ownership of voting securities, by contract or otherwise or (b) the ownership of at least twenty-five percent (25%) of the voting securities or other voting interest of any Person. The terms "Controlled by," "under common Control with" and "Controlling" shall have correlative meanings. "Control" means, as to any Asset or any item of or rights in or to Intellectual Property, ownership or other legal authority or right of a Person (whether by license, other than pursuant to this Agreement, or otherwise) to grant the right to use such item or a license or sublicense to such Asset or under such Intellectual Property rights to any other Person, or to

otherwise disclose proprietary or trade secret information to such other Person, without breaching the terms of any agreement with a Third Party.

"Copyright" means any copyrights in copyrightable works, including all rights of authorship, use, publication, reproduction, distribution, performance, transformation, moral rights and rights of ownership of copyrightable works, all registrations, applications for registration and renewals of any of the foregoing anywhere in the world, and all rights to register and obtain renewals and extensions of registrations, together with all other interests accruing by reason of copyright law anywhere in the world.

"Court Order" means any judgment, decision, writ, consent decree, injunction, ruling or order of any federal, state, local or other domestic or foreign court or Governmental Authority that is binding on any Person or its property.

"Cover", "Covering" or "Covered" means (a) with reference to a Patent, that the Manufacture, use, offer for sale, sale, importation or exportation of a product or practice of a method would infringe a Valid Claim of such Patent in the country in which such activity occurs absent a license thereto (or ownership thereof), and, in the case of a Patent application, that the Manufacture, use, offer for sale, sale, importation or exportation of a product or practice of a method would infringe a pending claim thereof, considering such pending claim for the time period specified in the definition of "Valid Claim" assuming that such pending claim were to issue in such scope and form, and (b) with reference to Know-How, that the Manufacture, Development or Commercialization of a product or practice of a method incorporates, embodies or otherwise makes use of such Know-How.

"Damages" means all Liabilities, losses, claims, damages, diminution, lost profits, causes of action, lawsuits, administrative proceedings (including informal proceedings), investigations, audits, demands, assessments, adjustments, judgments, settlement payments, deficiencies, Taxes, penalties, fines, interest (including interest from the date of such damages) and costs and expenses (including amounts paid in settlement, interest, court costs, costs of investigations, fees and expenses of attorneys, accountants, financial advisors and other experts, and other expenses of litigation).

"Deductible" has the meaning set forth in Section 7.4(a).

"Default" means (a) any actual breach, violation or default, (b) the existence of circumstances or the occurrence of an event that with the passage of time or the giving of notice or both would constitute a breach, violation or default or (c) the existence of circumstances or the occurrence of an event that, with or without the passage of time or the giving of notice or both, would give rise to a right of termination, renegotiation or acceleration.

"Defending Party" has the meaning set forth in Section 7.6(b)(iv).

"Definitive Initial Purchase Consideration" has the meaning set forth in Section 1.3(b)(i).

"Definitive Net Working Capital" has the meaning set forth in Section 1.3(a).

"Definitive Net Working Capital Adjustment" has the meaning set forth in Section 1.3(a).

"Development" means Pre-Clinical Development and clinical drug development activities reasonably relating to the development of technology (including a compound or product) and submission

of information regarding technology (including a compound or product) to a Regulatory Authority after Development, including Clinical Studies (including pre- and post-Regulatory Approval studies and statistical analysis), including Manufacturing of Product for the purpose of Development activities, but excluding Commercialization activities. When used as a verb, "Develop" means to engage in Development.

"Development Plan" has the meaning set forth in the Program Agreement.

"Disclosing Party" has the meaning set forth in Section 6.2(a).

"Disclosure Schedule" has the meaning set forth in the preamble to Article II.

"Dispute" has the meaning set forth in Section 9.5.

"Dollars" or "$" means the legal tender of the United States of America.

"Early Option Deadline" has the meaning set forth in the Unitholder Option Agreement.

"Effective Date of the Unitholder Option Agreement" means August 13, 2019.

"EMA" means the European Medicines Agency, or any successor thereto.

"Environment" means any surface water, ground water, drinking water supply, land surface or subsurface strata, or ambient air.

"Environmental Law" means, whenever in effect, any Law or Permit relating to protection of the Environment, human or worker health and safety, pollution or exposure of Persons or property to Hazardous Materials, including any Law (including administrative decisions or orders) pertaining to: (i) the presence of or the treatment, storage, disposal, generation, transportation, handling, distribution, manufacture, processing, use, import, export, labeling, recycling, registration, investigation or remediation of Hazardous Materials or documentation related to the foregoing; (ii) air, water and noise pollution; (iii) groundwater and soil contamination; (iv) the Release, threatened Release, or accidental Release into the Environment, the workplace or other areas of Hazardous Materials, including emissions, discharges, injections, spills, escapes or dumping of Hazardous Materials; (v) transfer of interests in, or control of, real property which may be contaminated; (vi) community or worker right-to-know disclosures with respect to Hazardous Materials; (vii) the protection of wild life, marine life and wetlands, and endangered and threatened species; (viii) storage tanks, vessels, containers, abandoned or discarded barrels and other closed receptacles; (ix) indoor air quality; and (x) health and safety of employees and other persons.

"Environmental Matters" mean any Liability of the Company, or its predecessors (or any other Person for whose conduct any of them are or may be responsible), arising under any Environmental Law or relating to Hazardous Materials, whether arising under theories of contract, tort, negligence, successor or enterprise liability, strict liability or other legal or equitable theory, including (i) any failure to comply with Environmental Laws and (ii) any Liabilities arising from the presence of, Release or threatened Release of, or exposure of Persons or property to, Hazardous Materials.

"Equity Participation" means any (a) share, security, participation right and any other present or future right entitling the holder, absolutely or contingently (through the exercise of any subscription,

conversion, exchange, option or similar right), to participate in the equity ownership, dividends or equity appreciation of another Person, including capital shares, membership interests, units, performance units, options, warrants, company appreciation rights, interests in "phantom" stock plans, restricted or contingent shares or profits interests, voting securities, share appreciation rights or equivalents, share loan purchase plans, convertible debentures or share bonus plans and (b) commitments to issue any of the foregoing.

"ERISA" means the Employee Retirement Income Security Act of 1974, as amended.

"ERISA Affiliate" means, with respect to any entity, trade or business (whether or not incorporated), any other entity, trade or business (whether or not incorporated) that is, or within the past six years was, a member of a group described in Section 414(b), (c), (m) or (o) of the Code or Section 4001(b)(1) of ERISA that includes the first entity, trade or business (whether or not incorporated), or that is, or within the past six years was, a member of the same "controlled group" as the first entity, trade or business (whether or not incorporated) pursuant to Section 4001(a)(14) of ERISA.

"Escrow Agent" means Acquiom Clearinghouse LLC, a Delaware limited liability company.

"Escrow Agreement" has the meaning set forth in Section 5.1(a).

"Escrow Amount" equals, if this Agreement is entered into as a result of Buyer's delivery of a Notice of Interest (x) on or before the Early Option Deadline, an amount equal to [***] Dollars ($[***]), and (y) after the Early Option Deadline but on or before the Interim Option Deadline, an amount equal to [***] Dollars ($[***]), and (z) after the Interim Option Deadline, an amount equal to [***] Dollars ($[***]), as the same may be increased by interest accrued thereon from time to time.

"Estimated Initial Purchase Consideration" has the meaning set forth in Section 1.3(a)(i).

"Estimated Initial Purchase Consideration Calculation" has the meaning set forth in Section 1.3(a).

"Estimated Net Working Capital" has the meaning set forth in Section 1.3(a)(iv).

"Estimated Net Working Capital Adjustment" has the meaning set forth in Section 1.3(a)(iii).

"[***]" means, with respect to a Royalty-Bearing Product, the earlier to occur of (i) [***], and (ii) [***].

"FDA" means the United States Food and Drug Administration, or any successor agency thereto.

"[***]" means, with respect to a Royalty-Bearing Product, [***] in accordance with Applicable Law.

"FD&C Act" has the meaning set forth in Section 2.20(a).

"Field" means the cure, mitigation, treatment, palliation, diagnosis or prevention of Angelman Syndrome.

"Final Statement of Purchase Consideration" has the meaning set forth in Section 1.3(d).

"Financial Statements" has the meaning set forth in Section 2.4.

"First Commercial Sale" means, on a Royalty-Bearing Product-by- Royalty-Bearing Product and country-by-country basis, the first commercial sale for end use or consumption in an arms' length transaction of a Royalty-Bearing Product to a Third Party by Buyer or any of its Affiliates or Licensees in such country following receipt of applicable Regulatory Approval (including, where applicable, pricing or reimbursement approval) of such Royalty-Bearing Product in such country. First Commercial Sale will not include any distribution or other sale solely for patient assistance, named patient use, compassionate use, or other patient access programs, or test marketing programs or non-registrational studies or similar programs or studies where the Royalty-Bearing Product is supplied without charge or at the actual Manufacturing cost thereof (with only nominal indirect costs or markup).

"Full Royalty Product" means the Lead Product alone or as a Combination Product.

"Fundamental Reps" has the meaning set forth in Section 7.4(a).

"GAAP" means U.S. generally accepted accounting principles.

"GDPR" has the meaning set forth in Section 2.25(e).

"Generic Competition" means, with respect to a Royalty-Bearing Product (the "Reference Product") in a particular country on a form by form and strength by strength basis, when the Generic Products have, in the aggregate, achieved more than [***] of the market share in such country by [***] (based on data provided by a reliable Third Party data source mutually agreed to by Buyer and the Payments Administrator) of [***].

"Generic Product" means, with respect to the Reference Product in a particular country, any pharmaceutical product that (a) is marketed for sale by a Third Party not authorized by Buyer or any of its Affiliates, (b) receives Regulatory Approval (with or without pricing or reimbursement approval) in such country in full or partial reliance on the Regulatory Approval (but not necessarily pricing or reimbursement approval) of the Reference Product, and (c) is determined by a Regulatory Authority to be therapeutically equivalent to and substitutable with the Reference Product, it being acknowledged that the foregoing standard is intended to be generally consistent with the standard set forth in the introduction to the "Orange Book," as amended from time to time, or any analogous or comparable standard in any country outside of the United States. For avoidance of doubt, in the United States, a "Generic Product" as defined herein includes one approved under Section 505(j) of the FD&C Act.

"Governmental Authority" means any (a) nation, state, commonwealth, province, territory, county, municipality, district or other jurisdiction of any nature; (b) international, multinational, federal, state, local, municipal, foreign or other government, agency or authority; or (c) governmental or quasi-governmental authority of any nature (including any governmental division, department, agency, division (including political subdivision), Regulatory Authority, commission, instrumentality, official, organization, unit, board, body or Person and any court or other tribunal).

"Hazardous Material" means any material, substance or waste that is subject to regulation, standards of conduct or liability under any Environmental Law, or has been designated by any Governmental Authority or by any applicable Environmental Law to be radioactive, toxic, a pollutant or

contaminant, hazardous or otherwise a danger to health or the environment, including PCBs, asbestos, oil, petroleum and petroleum products (including fractions thereof), urea-formaldehyde, mold and all substances listed as hazardous substances pursuant to the Comprehensive Environmental Response, Compensation, and Liability Act of 1980, or defined as a solid or hazardous waste pursuant to the Resource Conservation and Recovery Act of 1976, regulated under the Occupational Safety and Health Act, or the regulations promulgated pursuant to said Laws, or pursuant to analogous state Laws or regulations.

"Hazardous Materials Activities" means the treatment, distribution, transport, storage, use, management, handling, manufacture, sale, disposal or release of, arrangement or permission for the disposal of, or exposure of its employees or any Persons to, Hazardous Materials or any products containing a Hazardous Material.

"Healthcare Laws" means all Laws relating to patient care or human health and safety, including, as amended from time to time, any such Law pertaining to the research (including preclinical, nonclinical, and clinical research or studies), development, testing, production, manufacture, transfer, storing, distribution, approval, labeling, marketing, pricing, third-party reimbursement or sale of drugs, biological products and medical devices, including (i) the FD&C Act and the United States Public Health Service Act; (ii) any federal health care program (as such term is defined in 42 U.S.C. § 1320a-7b(f)), including those pertaining to providers of goods or services that are paid for by any federal health care program (as such term is defined in 42 U.S.C. § 1320a-7b(f)), including the federal Anti-Kickback Statute (42 U.S.C. § 1320a-7b(b)), the civil False Claims Act (31 U.S.C. § 3729 et seq.), the administrative False Claims Law (42 U.S.C. § 1320a-7b(a)), Sections 1320a-7 and 1320a-7a of Title 42 of the United States Code, Medicare (Title XVIII of the Social Security Act) and Medicaid (Title XIX of the Social Security Act); and (iii) all related rules and regulations of (i) and (ii) and equivalent applicable Laws of other Governmental Authorities.

"HIPAA" means, collectively, the Health Insurance Portability and Accountability Act of 1996, as amended by the HITECH Act and the HIPAA Final Omnibus Rule issued on January 17, 2013 and all associated rules and regulations.

"HSR Act" means the Hart Scott Rodino Antitrust Improvements Act of 1976, as amended.

"IND" means (a) an Investigational New Drug Application to the FDA as described within 21 C.F.R. § 312.23, or (b) the equivalent application or submission to a Regulatory Authority in any other country, the filing of which is necessary to initiate or conduct clinical testing of a pharmaceutical product in humans in such jurisdiction.

"IND Acceptance" means, with respect to the first Clinical Study of a Royalty-Bearing Product to be conducted in the United States, determination from the FDA that allows the IND and the commencement of such Clinical Study in the United States, provided that such determination will be deemed to have been made and given if the applicable statutory or regulatory response period has elapsed following confirmation by the FDA of its receipt of such IND (as confirmed either via a letter or fax) without imposition of a clinical hold by the FDA or any other notification by the FDA that the applicable Clinical Study cannot so proceed as contemplated by such IND and as a consequence, the Clinical Study may lawfully proceed as described in the applicable IND.

"Indemnified Party" has the meaning set forth in Section 7.6(b).

"Indemnifying Party" has the meaning set forth in Section 7.6(b).

"Insurance Policies" has the meaning set forth in Section 2.21.

"Intellectual Property" means any of the following in any jurisdiction throughout the world and all rights associated therewith: (a) Patents and other indicia of ownership of an invention recognized or issued by or filed with any Governmental Authority; (b) Trademarks; (c) Internet domain names; (d) Copyrights; (e) Know-How; (f) software (including source code, executable code, systems, network tools, data, databases, applications, firmware and all related documentation); and (g) all other intellectual property and proprietary rights.

"Intellectual Property Registrations" means issued Patents, registered Trademarks, registered copyrights and designs, mask work registrations, Internet domain name registrations, and applications for each of the foregoing.

"Know-How" means all commercial, technical, scientific and other data, results, know-how and information, trade secrets, technology, methods, processes, practices, formulae, instructions, skills, techniques, procedures, knowledge, experiences, ideas, technical assistance, designs, drawings, assembly procedures, computer programs, specifications and confidential and proprietary information (including biological, chemical, pharmacological, toxicological, clinical, safety, assay, study designs and protocol and related know-how and trade secrets, and manufacturing data, pre-clinical and clinical data, specifications of ingredients, manufacturing processes, formulation, specifications, sourcing information, quality control and testing procedures and related know-how and trade secrets), in all cases, whether or not confidential, proprietary, patented or patentable, in written, electronic or any other form now known or hereafter developed.

"Knowledge of the Company" means, with respect to the Company, the actual knowledge of Paula Evans, Allyson Berent, Deborah A. Guagliardo, Scott Dindot, Jennifer Panagoulias (or, in each case, any successor holding substantially comparable authority to any of the foregoing) and the knowledge that they would have if they had made reasonable and diligent inquiry of those employees and consultants and other persons who reasonably would be expected to have knowledge as to the relevant matter. "Know" and "Known" shall have correlative meanings.

"Lead Product" means the Royalty-Bearing Product that is the subject of the following clinical trial: "A Phase 1/2 Open-label, Multiple-dose, Dose-escalating Clinical Trial of the Safety and Tolerability of GTX-102 in Pediatric Patients With Angelman Syndrome (AS)."

"Leased Real Property" means all leasehold or subleasehold estates and other rights to use or occupy any land, buildings, structures, improvements, fixtures or other interest in real property.

"Liability" means any direct or indirect liability, debt, Company Indebtedness, Company Employee Indebtedness, guaranty, endorsement, claim, loss, damage, deficiency, cost, expense, obligation or responsibility, whether accrued, absolute, contingent, mature, unmature or otherwise and whether known or unknown, fixed or unfixed, choate or inchoate, liquidated or unliquidated, secured or unsecured.

"Licensee" means a Third Party and any Affiliates to whom Buyer or its Affiliate grants rights to Develop, Manufacture, or Commercialize any Product, or any subsequent sublicensee and any Affiliates of such rights.

"Lien" means any adverse claim, mortgage, security interest, pledge, hypothecation, assignment, deposit arrangement, encumbrance, lien (statutory or otherwise), charge, preference, priority or other security agreement, option, warrant, attachment, right of first refusal, preemption, conversion, put, call or other claim or right, restriction on transfer, or preferential arrangement of any kind or nature whatsoever (including any restriction on the transfer of any assets), any conditional sale or other title retention agreement and any financing lease involving substantially the same economic effect as any of the foregoing.

"MAA" means a marketing authorization application for approval to market a pharmaceutical product for human use in a country or group of countries outside the U.S., as defined in the applicable Laws and filed with the applicable Regulatory Authority of a given country or group of countries.

"[***]" means [***].

"Manufacturing" means all activities directed to sourcing of necessary raw materials, manufacturing, producing, processing, testing, filling, finishing, packaging, labeling, quality assurance testing, release, shipping and holding of a product (or any intermediate or component of such product). When used as a verb, "Manufacture" means to engage in Manufacturing.

"Material Adverse Effect" means any result, occurrence, fact, change, development, condition, event, effect or other matter that, individually or in the aggregate with any other results, occurrences, facts, changes, developments, conditions, events, effects or other matter, has had, has or could reasonably be expected to have or give rise to a material adverse effect on or material adverse change to (a) the Company's Development of Products, assets, liabilities or condition (financial or otherwise) of the Company or (b) the ability of the Company or the Unitholders to consummate the transactions contemplated hereby or to perform their respective obligations hereunder in a timely manner. Notwithstanding the foregoing, solely for purposes of the foregoing clause (a), no result, occurrence, fact, change, development, condition, event, effect or other matter shall be taken into account in determining whether a Material Adverse Effect has occurred to the extent resulting from (i) conditions generally affecting the United States economy as a whole or the economy of any jurisdiction in which the Company has material operation or (ii) changes in GAAP or changes in any Laws.

"Material Contracts" has the meaning set forth in Section 2.15(a).

"Milestone Payments" has the meaning set forth in Section 1.6(a).

"NDA" means a New Drug Application to market a pharmaceutical product in the United States, as defined in 21 C.F.R. § 314.3 and filed with the FDA.

"Negotiation Period" means the [***] period beginning on the date on which Buyer gives Representative written notice of Cessation.

"Net Sales" means, with respect to the Royalty-Bearing Products, the gross amount invoiced for all commercial sales of such Royalty-Bearing Products by Buyer and its Affiliates (including, after the Closing, the Company) or Licensees (each, a "Selling Party") to a Third Party (other than another Selling Party), less the following deductions actually incurred or paid or otherwise accrued, allowed, reserved or allocated in accordance with GAAP and reported in Buyer's financial statements:

(i) normal and customary trade, cash and quantity discounts, allowances and credits for such Royalty-Bearing Products;

(ii) fees paid, reserves, and allowances to distributors and discounts (including cash, quantity and patient program discounts), charge-back payments, rebates and similar payments granted to customers, wholesalers, distributors, buying groups, retailers, health care insurance carriers, pharmacy benefit management companies, health maintenance organizations, managed health care organizations or to federal, state and local governments, their agencies, and purchasers and reimbursers or to trade customers;

(iii) credits or allowances actually granted for price adjustments (including retroactive price adjustments), damaged goods, spoiled product, claims, recalls, rejections or returns of such Royalty-Bearing Products, including such Royalty-Bearing Products returned in connection with withdrawals;

(iv) freight out, postage, customs charges, shipping and insurance charges for delivery of such Royalty-Bearing Products; and

(v) taxes or duties levied on, absorbed or otherwise imposed on the sale of such Royalty-Bearing Products, including value-added taxes, or other governmental charges otherwise imposed upon the billed amount, as adjusted for rebates and refunds, to the extent not paid by the Third Party, and annual fees due under Section 9008 of the United States Patient Protection and Affordable Care Act of 2010 (Pub. L. No. 111-48) and other successor or comparable laws.

Sales and other transfer of Royalty-Bearing Products between Selling Parties and any dispositions of any Royalty-Bearing Products for pre-clinical or clinical testing required in connection with obtaining Regulatory Approval of any Royalty-Bearing Products shall not give rise to Net Sales. Net Sales shall be calculated in accordance with GAAP so as to arrive at "net sales" under GAAP as reported by such Selling Party, as applicable, in such Person's financial statements.

"Non-Defending Party" has the meaning set forth in Section 7.6(b)(iv).

"Notice of Exercise" has the meaning set forth in the Unitholder Option Agreement.

"Notice Period" has the meaning set forth in Section 7.6(b)(ii).

"NWC Target Amount" means $[***].

"Operating Agreement" has the meaning set forth in Section 1.4(b).

"Option" has the meaning set forth in the recitals.

"Patents" means (a) all patents and patent applications (provisional and non-provisional) anywhere in the world, including PCT applications, (b) all divisionals, continuations, continuations in-part thereof, or any other patent application claiming priority, or entitled to claim priority, directly or indirectly to (i) any such patents or patent applications or (ii) any patent or patent application from which such patents or patent applications claim, or is entitled to claim, direct or indirect priority, and (c) all patents issuing on any of the foregoing anywhere in the world (including from PCT applications), together with all registrations, reissues, re-examinations, patents of addition, utility models or designs, renewals, supplemental protection certificates, or extensions of any of the foregoing and counterparts thereof anywhere in the world.

"Permit" means all certifications (including those of standards-setting organizations), licenses, permits, franchises, approvals, authorizations, exemptions, certificates, accreditations, notices to, consents or orders of, filings with, or any similar rights from any trade association, any standards-setting organization or any Governmental Authority required for the operation of the Business.

"Permitted Lien" means any (a) landlord's, mechanic's, carrier's, workmen's, repairmen's or other similar statutory Liens arising or incurred in the ordinary course of business for amounts which are not due and payable but which are accounted for in the calculation of Net Working Capital, (b) statutory Lien for Taxes that are not yet due and payable or which are being contested in good faith by appropriate proceedings and are reserved for in full on the most recent Financial Statements in accordance with GAAP, and (c) any other condition, easement and reservation of rights (including any easement and reservation of, or rights of others for, rights of way, sewers, electric lines, telegraph and telephone lines and other similar purposes), encroachment, covenant or restriction that does not detract in any material respect from the use of the Assets in the operation of the Business.

"Person" means any individual, a limited liability company, a joint venture, a corporation, a company, a partnership, an association, a business trust, a trust, a Governmental Authority, an agency of the State of Texas, an institution of higher education, a division or operating group of any of the foregoing or any other entity or organization.

"Personal Information" has the meaning set forth in Section 2.25(a).

"Phase 1 Clinical Study" means a human clinical trial of a product designed to satisfy the requirements of 21 C.F.R. § 312.21(a) and is intended to determine the metabolism and pharmacologic actions of the drug in humans, the side effects associated with increasing doses and, if possible, to gain early evidence of efficacy, or any comparable trial under Applicable Laws in the United States.

"Phase 1A Clinical Study" means a human clinical trial of a product, the principal purpose of which is a preliminary determination of safety, pharmacokinetic, and pharmacodynamic parameters in healthy individuals or patients, or a similar clinical study prescribed by the Regulatory Authorities in the United States.

"Phase 1B Clinical Study" means a human clinical trial of a product: (a) the principal purpose of which is to evaluate safety and tolerability of the drug following repeat dosing in patients and (b) the secondary purpose of which is to evaluate biomarker-based and clinical endpoint-based trends of efficacy, conducted after the initiation of an initial Phase 1 Clinical Study or Phase 1A Clinical Study of such Product, prior to commencement of Phase 2 Clinical Studies or Phase 3 Clinical Studies, and that is designed to

provide (itself or together with other available data) evidence of sufficient safety and clinical activity to enable the decision to proceed to a Phase 2 Clinical Study.

"Phase 2 Clinical Study" means a human clinical trial of a product designed to satisfy the requirements of 21 C.F.R. § 312.21(b) and intended to explore dose response and duration of effect, and to generate data on side effects and clinical efficacy for a particular indication or indications in a target patient population, or any comparable trial under Applicable Laws of the United States.

"Phase 3 Clinical Study" means a human clinical trial of a product designed to satisfy the requirements of 21 C.F.R. § 312.21(c) and is intended to (a) establish that the product is safe and efficacious for its intended use, (b) define contraindications, warnings, precautions and adverse reactions that are associated with the product in the dosage range to be prescribed, and (c) support Regulatory Approval for such product, or any comparable trial under Applicable Laws of the United States.

"Pre-Clinical Development" means activities reasonably relating to the discovery, research and pre-clinical development of a compound or product, including toxicology, pharmacology and other discovery, optimization and pre-clinical efforts, test method development and stability testing, Manufacturing process development, formulation development, delivery system development, and quality assurance and quality control development, but excluding Clinical Studies (including pre- and post-Regulatory Approval studies and statistical analysis) and Commercialization activities.

"Pre-Closing Tax Period" means any taxable period or portion thereof ending on or before the Closing Date.

"Priority Review Voucher" means a priority review voucher awarded by the FDA pursuant to Section 529(a) of the FD&C Act to Buyer in connection with a rare pediatric disease product application for a Royalty-Bearing Product, or an equivalent voucher under a superseding law related to an FDA Approval.

"Priority Review Voucher Payments" has the meaning set forth in Section 1.6(a).

"Product" means (a) the Lead Product, (b) any other antisense oligonucleotides capable of modulating UBE3A expression, (c) any pharmaceutical composition or preparation that constitutes, incorporates, comprises, or contains the Lead Product or any oligonucleotide referenced in clause (b) of this definition, alone or as a Combination Product, and (d) any pharmaceutical composition that is Covered by the Company Intellectual Property, in each case in any presentation, form or formulation (including different dosage strengths) for any use.

"Profits Interests" has the meaning set forth in the recitals.

"Purchase Consideration Dispute Notice" has the meaning set forth in Section 1.3(d).

"Real Property" means all interests in real property including fee estates, leaseholds and subleaseholds, purchase options, easements, licenses, rights to access, and rights of way, and all buildings and other improvements thereon, together with any additions thereto or replacements thereof.

"Receiving Party" has the meaning set forth in Section 6.2(a).

"Reduced Royalty Product" shall mean any Royalty-Bearing Product that is not a Full Royalty Product.

"Regulatory Approval" means all approvals necessary for the Manufacture and Commercialization of a product in a country or regulatory jurisdiction (including an NDA or MAA), which may include satisfaction of all applicable regulatory and notification requirements.

"Regulatory Authority" means, with respect to a jurisdiction, any national (e.g., the FDA or the EMA), supra-national, regional, state or local regulatory agency, department, bureau, commission, council or other Governmental Authority regulating or otherwise exercising authority with respect to the development, manufacture, commercialization and sale of drug products, including the FDA and EMA.

"Regulatory Materials" means: (i) regulatory applications, submissions and approvals (including all INDs, NDAs, BLAs and foreign counterparts thereof) (and any supplements or amendments thereto) that relate to the Business or the Product; (ii) any notifications, communications, correspondence, minutes of meetings or telephone conversations, registrations, letters of authorization for master files, or other filings made to, received from or otherwise conducted with FDA and other Governmental Authorities; (iii) records and other materials maintained to comply with applicable Healthcare Laws (e.g., regarding good laboratory practice, good clinical practice and good manufacturing practice, as applicable); and (iv) records that are necessary in order to obtain Permits from Governmental Authorities under applicable Healthcare Laws for the research (including preclinical, nonclinical and clinical research or studies), development, testing, production, manufacture, transfer, distribution, approval, labeling, marketing, pricing, third-party reimbursement or sale of drugs, biological products and medical devices and all amendments, supplements, supporting files, data, studies, and reports relating thereto (in hard and electronic form) and all technical and other information contained therein, and all correspondence with the FDA and other Governmental Authorities relating to the foregoing, that, in each case, are in the possession of or controlled by the Company, whether generated, filed or held by or for the Company.

"Related Party" means any Affiliate of the Company or any current or former officer, director, Unitholder or employee of the Company or an Affiliate or immediate family member of such a Person.

"Release" means any spilling, leaking, pumping, pouring, emitting, emptying, discharging, injecting, escaping, leaching, dumping or disposing into the Environment (including the abandonment or discarding of barrels, containers and other closed receptacles containing any Hazardous Materials).

"Released Claim" has the meaning set forth in Section 7.11(a).

"Released Matters" has the meaning set forth in Section 7.11(a).

"Releasees" has the meaning set forth in Section 7.11(a).

"Releasors" has the meaning set forth in Section 7.11(a).

"Representative" has the meaning set forth in the preamble.

"Representative's Fund" has the meaning set forth in Section 1.7(e).

"Representative Losses" has the meaning set forth in Section 1.7(d).

"Review Period" has the meaning set forth in Section 1.3(d).

"Royalty-Bearing Product" means any Product (a) whose manufacture, use, sale, offer for sale or import is Covered by a Valid Claim of a Patent within the Company Intellectual Property, where such Patent (i) is in existence as of the Closing Date, (ii) claims priority to a Patent in existence within the Company Intellectual Property as of the Closing Date, or (iii) is the subject of an invention disclosure provided to the Company's patent counsel prior to the Closing Date and is shared with Buyer's patent counsel promptly following the Closing Date, (b) that was developed using Know-How in existence as of the Closing Date contained within the Company Intellectual Property, (c) that is manufactured using Know-How in existence as of the Closing Date contained within the Company Intellectual Property, or (d) that, when used, practices Know-How in existence as of the Closing Date contained within the Company Intellectual Property.

"Royalty Payments" has the meaning set forth in Section 1.6(a).

"Royalty Term" has the meaning set forth in Section 1.6(f)(iii).

"Selling Parties" has the meaning set forth in the definition of "Net Sales."

"Series A Preferred Units" has the meaning set forth in the recitals.

"Straddle Period" has the meaning set forth in Section 6.3(e).

"Survival Date" has the meaning set forth in Section 7.5(a).

"TAMUS" has the meaning set forth in the Unitholder Option Agreement.

"Tax" (including with correlative meaning the terms "Taxes" and "Taxable") means (a) any taxes, charges, fees, duties, contributions, levies or other similar assessments or liabilities, including income, gross receipts, corporation, ad valorem, premium, value-added, net worth, capital stock, capital gains, documentary, recapture, alternative or add-on minimum, disability, registration, recording, excise, real property, personal property, sales, use, license, lease, service, service use, transfer, withholding, imputed underpayment, employment, unemployment, insurance, social security, national insurance, business license, production, goods and services, business organization, environmental, workers compensation, payroll, profits, severance, stamp, occupation, escheat, windfall profits, customs duties, franchise, estimated and other taxes, customs, duties or government fee, or other like assessment or charge of any kind whatsoever imposed by any state, local or foreign government, or any agency or political subdivision thereof, and any interest, fines, penalties, assessments or additions to tax imposed with respect to such items or any contest or dispute thereof and (b) any liability of any Person for the payment of amounts of the type described in clause (a) as a transferee, successor or payable pursuant to a contractual obligation.

"Tax Matter" has the meaning set forth in Section 6.3(c).

"Tax Return" means any reports, returns (including information returns), declarations, claim for refund or statements relating to Taxes, including any schedule or attachment thereto and any related or supporting workpapers or information with respect to any of the foregoing, including any amendment thereof required to be filed with or submitted to any Governmental Authority in connection with the

determination, assessment, collection or payment of Taxes or in connection with the administration, implementation or enforcement of or compliance with any legal requirement relating to any Tax.

"Third Party" means any Person other than any of the Parties hereto or their respective Affiliates.

"Third Party Claim" has the meaning set forth in Section 7.6(b).

"Total Purchase Consideration" means the Definitive Initial Purchase Consideration, Milestone Payments, Priority Review Voucher Payments, and Royalty Payments actually paid to the Unitholders.

"Trademarks" means trademarks, service marks, trade dress, trade names, logos, slogans, corporate names, doing business designations, and all other indicia of origin, and all registrations, applications for registration and renewals of the foregoing anywhere in the world, and all goodwill associated with the foregoing.

"Transaction Costs" means the fees, costs, expenses and disbursements of the Company to its respective agents, representatives, brokers, finders, financial advisors, accountants and counsel, incurred in connection with (i) the process by which the Company or the Unitholders solicited, discussed and negotiated strategic alternatives or this Agreement, the Unitholder Option Agreement and the other Transaction Documents or the transactions contemplated hereby, (ii) the preparation and submission of any filing or notice required to be made by the Company in connection with the transactions contemplated by the Transaction Documents, or (iii) the obtaining of any consent, waiver or approval required to be obtained by the Company in connection with the transactions contemplated hereby and under the Transaction Documents, but, in each case of clauses (i) - (iii) solely to the extent such fees, expenses and disbursements remain unpaid as of the Closing and to the extent that Buyer has not agreed to bear such fees, expenses and disbursements. In the event the Representative elects to have the Escrow Amount invested in an interest bearing account, Transaction Costs shall also include all incremental fees and expenses payable to the Escrow Agent above what such expenses would have been for a non-interest bearing account.

"Transaction Documents" means this Agreement, the Unitholder Option Agreement, the Escrow Agreement, the Program Agreement and each of the other agreements and instruments contemplated hereby and thereby to be executed by the Company, Buyer, a Unitholder, the Representative or any of their respective Affiliates.

"Transfer Taxes" means all transfer, stock transfer, documentary, sales, use, value-added stamp, registration and other similar Taxes and fees, including stamp duty (including any penalties and interest).

"UBE3A" means the gene encoding for the ubiquitin protein ligase E3A, which is also known as E6AP ubiquitin-protein ligase (E6AP).

"UK Approval" has the meaning set forth in Section 1.6(c)(2).

"Units" has the meaning set forth in the recitals.

"Unitholders" has the meaning set forth in the preamble.

"Unitholder Indemnified Parties" has the meaning set forth in Section 7.3.

"Unitholder Option Agreement" has the meaning set forth in the recitals.

"Unresolved Claims" has the meaning set forth in Section 7.8(b).

"Valid Claim" means a claim of (a) an issued Patent that has not expired, lapsed, abandoned, dedicated to the public or been held permanently unenforceable, invalid or revoked by a Governmental Authority of competent jurisdiction in an order or decision from which no appeal has been taken within the time allowed for appeal or can be taken, including through opposition, reexamination, reissue, disclaimer, post grant review (PGR) or inter partes review (IPR); or (b) a pending Patent application that has not expired or been irrevocably abandoned or irrevocably rejected and which has been pending for no more than ten (10) years from the date of filing of the earliest priority Patent application to which such pending Patent application is entitled to claim benefit.

Section VIII.2 Usage. The defined terms herein shall apply equally to both the singular and plural forms of the terms defined, and if a word or phrase is defined, the other grammatical forms of such word or phrase have a corresponding meaning. "Will" has the same meaning and effect as the word "shall" and vice versa. References to the word "any" mean "any and all" unless otherwise clearly indicated by context. References to this "Agreement" or any other agreement or document shall be construed as references to this Agreement or, as the case may be, such other agreement or document as the same may have been, or may from time to time be, amended, varied, novated or supplemented. Whenever the context may require, any pronoun shall include the corresponding masculine, feminine and neuter forms. All references herein to "Articles", "Sections" and "Exhibits" shall be deemed to be references to Articles and Sections of and Exhibits to this Agreement unless the context shall otherwise require. The words "include", "includes" and "including" shall be deemed to be followed by the phrase "without limitation." The word "or" is not exclusive. The words "hereof", "herein" and "hereunder" and words of similar import when used in this Agreement shall refer to this Agreement as a whole and not to any particular provision of this Agreement. Unless otherwise expressly provided herein, any statute defined or referred to herein means such statute as from time to time amended, modified or supplemented, including by succession of comparable successor statutes. Any reference to any federal, state, local or foreign statute or law shall be deemed also to refer to all rules and regulations promulgated thereunder, unless the context requires otherwise. Titles to Articles and headings of Sections are inserted for convenience of reference only and shall not be deemed a part of or to affect the meaning or interpretation of this Agreement. Unless otherwise expressly provided, wherever the consent of any Person is required or permitted herein, such consent may be withheld in such Person's sole and absolute discretion.

ARTICLE IX
GENERAL

Section IX.1 Notices. Any notice, request, claim, demand, waiver, consent, approval or other communication which is required or permitted hereunder shall be in writing and shall be deemed given if delivered personally or sent by e-mail (with confirmation of receipt), by registered or certified mail, postage prepaid, or by nationally recognized overnight courier service, as follows:

If to Buyer or the Company:

Ultragenyx Pharmaceuticals, Inc.
60 Leveroni Court
Novato, CA 94949
Attn: Chief Business Officer
Telephone: [***]
Email: [***]

With a required copy to (which shall not constitute notice):

Goodwin Procter LLP
100 Northern Ave.
Boston, MA 02110
Attention: Kingsley Taft
Telephone: [***]
Email: [***]

If to the Unitholder Representative:

GeneTx Biotherapeutics, LLC
Information to be provided under separate cover

With a required copy to (which shall not constitute notice):

Saul Ewing Arnstein & Lehr LLP
1919 Pennsylvania Avenue N.W., Suite 550
Washington, DC 20006
Attention: Jay G. Reilly
Telephone: [***]
Email: [***]

If to the Unitholders: to the address, phone or e-mail opposite each Unitholder's name on such Unitholder's signature page hereto;

or to such other address as the person to whom notice is to be given may have specified in a notice duly given to the sender as provided herein. Such notice, request, claim, demand, waiver, consent, approval or other communication shall be deemed to have been given (a) as of the date so delivered or e-mailed, (b) one Business Day after it is sent for next Business Day delivery via a reputable nationwide overnight courier service, (c) four Business Days after it is sent by registered or certified mail, and (d) if given by any other means, shall be deemed given only when actually received by the addressees.

Section IX.2 Entire Agreement. This Agreement (which includes the Disclosure Schedule, the other Schedules hereto and the Exhibits hereto), the other Transaction Documents, and all other agreements contemplated hereby sets forth the entire understanding of the parties hereto with respect to the transactions contemplated hereby. Any previous agreements and understandings between or among the parties regarding the subject matter hereof, whether written or oral, including the Confidentiality Agreement, are superseded by this Agreement and any information disclosed under the Confidentiality Agreement shall be deemed to have been disclosed under this Agreement. Each of the Disclosure Schedule, Schedules and Exhibits is incorporated into this Agreement by this reference and expressly made a part hereof.

Section IX.3 Successors and Assigns. This Agreement and the rights of the parties hereunder may not be assigned without the prior written consent of the other parties hereto (except by operation of Law) and shall be binding upon and shall inure to the benefit of the parties hereto, and their respective successors, heirs and legal representatives; provided that notwithstanding the foregoing, Buyer may assign any or all of its rights, obligations or Liabilities hereunder to any of its Affiliates following advance written notice of such assignment to the Representative; provided, further, that no assignment shall

relieve any party of its obligations hereunder. Any attempted assignment in violation of the provisions hereof shall be null and void and have no effect.

Section IX.4 Counterparts; Delivery by Electronic Means or E-mail. This Agreement may be executed in multiple counterparts and any party hereto may execute any such counterpart, each of which when executed and delivered shall be deemed to be an original and all of which counterparts taken together shall constitute but one and the same instrument. For purposes of this Agreement, .PDF or other electronic signatures shall be deemed originals.

Section IX.5 Governing Law; Jurisdiction; Waiver of Jury Trial.

(a) This Agreement, and all claims or causes of action (whether in contract, tort or otherwise) that may be based upon, arise out of or relate to this Agreement or the negotiation, execution or performance of this Agreement (including any claim or cause of action based upon, arising out of or related to any representation or warranty made in or in connection with this Agreement or as an inducement to enter into this Agreement), shall be governed by and construed in accordance with the internal Laws of the State of Delaware, except for any claims or causes of action brought by or against TAMUS arising out of or relating to this Agreement as such claims must be governed by the internal Laws of the State of Texas.

(b) Each of the Parties hereby irrevocably and unconditionally consents to submit any dispute arising under or in connection with this Agreement, any agreement, document or instrument entered into pursuant to this Agreement, or the transactions contemplated hereby, with respect to any provision of this Agreement or any agreement entered into pursuant to this Agreement (a "Dispute") to the sole and exclusive jurisdiction of any state or federal courts located in the State of Delaware (the "Chosen Courts"). Each party agrees not to commence any litigation relating to any Dispute except in the Chosen Courts, waives any objection to the laying of venue of any such litigation in the Chosen Courts, agrees not to plead or claim in any Chosen Court that such litigation brought therein has been brought in any inconvenient forum. Each of the Parties hereto agrees that service of process may also be made on such party by prepaid certified mail with a proof of mailing receipt. Service made pursuant to the preceding sentence above shall have the same legal force and effect as if served upon such party personally within the State of Delaware. Notwithstanding the dispute resolution procedures set forth in this Section 9.5, in the event of an actual or threatened breach of this Agreement, the aggrieved party may seek equitable relief (including restraining orders, specific performance or other injunctive relief), without first submitting to any dispute resolution procedures hereunder. Further, notwithstanding the foregoing, this Section 9.5(b) shall not apply to TAMUS.

(c) EXCEPT AS LIMITED BY APPLICABLE LAW, EACH PARTY HERETO (OTHER THAN TAMUS) HEREBY IRREVOCABLY WAIVES ALL RIGHT TO TRIAL BY JURY IN ANY ACTION, PROCEEDING OR COUNTERCLAIM (WHETHER BASED ON CONTRACT, TORT OR OTHERWISE) ARISING OUT OF OR RELATING TO THIS AGREEMENT OR THE ACTIONS OF ANY PARTY HERETO IN THE NEGOTIATION, ADMINISTRATION, PERFORMANCE AND ENFORCEMENT HEREOF.

Section IX.6 Specific Performance. Each party hereto acknowledges that the parties hereto will be irreparably harmed and that there will be no adequate remedy at law for any violation by any party of any of the covenants or agreements contained in the Transaction Documents. It is accordingly agreed that, in addition to any other remedies which may be available upon the breach of any such covenants or

agreements, each of the parties hereto shall have the right, prior to any termination of this Agreement, to injunctive relief to restrain a breach or threatened breach of, or otherwise to seek to obtain specific performance of, any other party's covenants and agreements contained in the Transaction Documents, in the Chosen Courts, in addition to any other remedy to which it may be entitled, at law or in equity, and each party hereto waives any requirement for the securing or posting of any bond or security in connection with any such remedy.

Section IX.7 Severability. If any term or other provision of this Agreement is invalid, illegal or incapable of being enforced by any rule of law or public policy, all other conditions and provisions of this Agreement shall nevertheless remain in full force and effect so long as the economic or legal substance of the transactions contemplated hereby is not affected in any manner materially adverse to any party. Upon such determination that any term or other provision is invalid, illegal or incapable of being enforced, the parties shall negotiate in good faith to modify this Agreement so as to effect the original intent of the parties as closely as possible to the end that the transactions contemplated hereby are fulfilled to the greatest extent possible.

Section IX.8 Amendment; Waiver. Any provision of this Agreement may be modified, supplemented or waived only by an instrument in writing duly executed by (a) the Representative and (b) Buyer. Any extension or waiver by any party of any provision hereto shall be valid only if set forth in an instrument in writing signed on behalf of such party. As to TAMUS, the Representative must obtain written approval from TAMUS for any modification or waiver to or of this Agreement before it becomes effective against TAMUS.

Section IX.9 Absence of Third Party Beneficiary Rights. No provision of this Agreement is intended, nor will be interpreted, to provide or to create any Third Party beneficiary rights or any other rights of any kind in any client, customer, Affiliate, stockholder, officer, director, employee or partner of any party hereto or any other Person, other than (a) the parties hereto and (b) the Buyer Indemnified Parties (who shall be Third Party beneficiaries of Section 7.6).

Section IX.10 Mutual Drafting. This Agreement is the mutual product of the parties hereto, and each provision hereof has been subject to the mutual consultation, negotiation and agreement of each of the parties, and shall not be construed for or against any party hereto.

Section IX.11 Further Representations. Each party to this Agreement acknowledges and represents that it has been represented by its own legal counsel in connection with the transactions contemplated hereby, with the opportunity to seek advice as to its legal rights from such counsel. Each party further represents that it is being (or has had the opportunity to be) independently advised as to the tax consequences of the transactions contemplated hereby and is not relying on any representation or statements made by any other party as to such tax consequences.

[SIGNATURE PAGES TO FOLLOW**]**

IN WITNESS WHEREOF, the parties hereto have executed this Unit Purchase Agreement as of the day and year first written above.

BUYER: ULTRAGENYX PHARMACEUTICAL INC.

By: /s/ Emil D. Kakkis, M.D., Ph.D.
Name: Emil D. Kakkis, M.D., Ph.D.
Title: President and Chief Executive Officer

COMPANY: GENETX BIOTHERAPEUTICS LLC

By: /s/ Paula Evans
Name: Paula Evans
Title: Chief Executive Officer

REPRESENTATIVE: Deborah A. Guagliardo
solely in her capacity as the Representative

/s/ Deborah Guagliardo

UNITHOLDERS:

[***]	/s/ [***] By: [***] Title: [***]
[***]	/s/ [***] By: [***] Title: [***]
[***]	/s/ [***] By: [***] Title: [***]
[***]	/s/ [***] By: [***] Title: [***]
[***]	/s/ [***] By: [***] Title: [***]
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[***]	/s/ [***] By: [***] Title: [***]
[***]	/s/ [***] By: [***] Title: [***]
[***]	/s/ [***] By: [***] Title: [***]

Exhibit 31.1

CERTIFICATION PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Emil D. Kakkis, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Ultragenyx Pharmaceutical Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of registrant's Board of Directors (or persons performing the equivalent functions):

   a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Dated: July 28, 2022

/s/ Emil D Kakkis
Emil D. Kakkis, M.D., Ph.D.
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Mardi C. Dier, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Ultragenyx Pharmaceutical Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of registrant's Board of Directors (or persons performing the equivalent functions):

   a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Dated: July 28, 2022

/s/ Mardi C. Dier
Mardi C. Dier
Executive Vice President and Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002 (18 U.S.C. SECTION 1350)

In connection with the accompanying Quarterly Report of Ultragenyx Pharmaceutical Inc. (the "Company") on Form 10-Q for the quarter ended June 30, 2022 (the "Report"), I, Emil D. Kakkis, M.D., Ph.D., as President and Chief Executive Officer of the Company, and Mardi C. Dier, as Executive Vice President and Chief Financial Officer of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: July 28, 2022

/s/ Emil D. Kakkis
Emil D. Kakkis, M.D., Ph.D.
President and Chief Executive Officer
(Principal Executive Officer)

Dated: July 28, 2022

/s/ Mardi C. Dier
Mardi C. Dier
Executive Vice President and Chief Financial Officer
(Principal Financial Officer)