Ultragenyx Reports Second Quarter 2019 Financial Results and Corporate Update

August 1, 2019

Strong Crysvita® (burosumab) Launch Continues with Approximately 960 Patients on Reimbursed Commercial Therapy in the United States

Ultragenyx Submits New Drug Application to FDA for UX007 (triheptanoin) for Treatment of Long-chain Fatty Acid Oxidation Disorders

NOVATO, Calif., Aug. 01, 2019 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for serious rare and ultra-rare genetic diseases, today reported its financial results and corporate update for the quarter ended June 30, 2019.

“The U.S. launch of Crysvita continues to be strong with the number of patients on reimbursed therapy increasing significantly this quarter and the number of unique prescribers continuing to grow steadily,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “Earlier this week we submitted the New Drug Application for UX007, which marks significant progress in bringing this important therapy to patients, and we are advancing our gene therapy platform with updates expected from the two clinical-stage programs in the third quarter.”

Second Quarter 2019 Financial Results

Net Revenues

For the second quarter of 2019, Ultragenyx reported $24.1 million in total revenue, including $20.2 million in Crysvita revenue. Crysvita revenue includes $17.3 million in collaboration revenue in the North American profit share territory, $1.9 million in royalty revenue in the European territory from Kyowa Kirin Co., and product revenue in other regions of $1.0 million. Mepsevii® (vestronidase alfa) product revenue for the second quarter of 2019 was $3.2 million and UX007 named patient revenue was $0.6 million. Ultragenyx also recognized $0.1 million in revenue from its research agreement with Bayer.

Revenue for the six months ended June 30, 2019 was $42.3 million, including $34.7 million in Crysvita revenue. Crysvita revenue includes $29.2 million in collaboration revenue in the North American profit share territory, $3.9 million in royalty revenue in the European territory from Kyowa Kirin Co., and product revenue for Crysvita in other regions of $1.6 million. Mepsevii product revenue for the six months ended June 30, 2019 was $5.9 million and UX007 named patient revenue was $1.3 million. Ultragenyx also recognized $0.4 million in revenue from its research agreement with Bayer in the first half of 2019.

Operating Expenses

Total operating expenses for the second quarter of 2019 were $136.6 million compared with $107.7 million for the same period in 2018, including a $15.6 million research and development expense in the second quarter of 2019 from the Arcturus collaboration amendment, and non-cash stock-based compensation of $22.2 million and $19.6 million in the second quarter of 2019 and 2018, respectively. Total operating expenses for the six months ended June 30, 2019 were $254.0 million compared with $214.9 million for the same period in 2018, including a $15.6 million research and development expense from the Arcturus collaboration amendment in the second quarter of 2019, and non-cash stock-based compensation of $42.4 million and $38.4 million in the first half of 2019 and 2018, respectively. The increase in total operating expenses is due to the increase in commercial, development, and general and administrative costs as the company commercializes, grows, and advances its pipeline.

For the second quarter of 2019, Ultragenyx reported a net loss of $99.2 million, or $1.72 per share, basic and diluted, compared with a net loss for the second quarter of 2018 of $52.7 million, or $1.06 per share, basic and diluted. For the six months ended June 30, 2019, net loss was $195.9 million, or $3.54 per share, basic and diluted, compared with a net loss for the same period in 2018 of $22.5 million, or $0.46 per share, basic and diluted. The loss for the second quarter of 2019 and for the six months ended June 30, 2019 is net of a $9.8 million unrealized gain from the fair value adjustment on the investment in Arcturus equity. The loss for the second quarter of 2018 is net of a $40.3 million gain from Ultragenyx’s portion of the sale of the priority review voucher (PRV) received with the Crysvita U.S. Food and Drug Administration (FDA) approval. In addition to the Crysvita PRV, the loss for the first six months of 2018 also is net of the $130.0 million gain from the sale of the PRV received with the Mepsevii FDA approval. The net loss for the first six months of 2019 reflected cash used in operations of $184.8 million compared to $165.6

million for the same period in 2018.

Cash, Cash Equivalents and Investments

Cash, cash equivalents and available-for-sale investments were $618.3 million as of June 30, 2019, which factors in the $30 million paid in the Arcturus collaboration agreement.

Recent Updates and Upcoming Milestones

Crysvita Commercial Progress in X-Linked Hypophosphatemia (XLH)

- **Strong U.S. launch continues,** with approximately 960 patients on reimbursed commercial therapy in the United States at the end of the second quarter 2019.

UX007 NDA submitted to FDA for the treatment of Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD)

- **Ultragenyx submitted a New Drug Application (NDA) to the FDA earlier this week.** The FDA previously granted Rare Pediatric Disease Designation and Fast Track designation, which enables eligibility for Priority Review, if relevant criteria are met. The company expects to hear back from FDA on submission acceptance and review designation within 60 days.

Clinical-stage Gene Therapy Programs Advance

- **DTX301 Phase 1/2 study in ornithine transcarbamylase (OTC) deficiency:** Previous data from Cohorts 1 and 2 demonstrated that the two responders maintained ureagenesis levels above normal for 78 and 52 weeks, respectively. There have been no infusion-related adverse events and no treatment-related serious adverse events reported.
- **DTX401 Phase 1/2 Study in glycogen storage disease type Ia (GSDIa):** Previous data from Cohort 1 demonstrated that all three patients showed improvements in glucose control reflected by prolonged time to hypoglycemia during a controlled fasting challenge. All patients have sustained their reductions in cornstarch compared to baseline, and continue to maintain normal glucose levels throughout the day and overnight. There have been no infusion-related adverse events and no treatment-related serious adverse events reported.
- **Ultragenyx plans to provide updates from the third dose cohort of the DTX301 study in OTC and the second dose cohort of the DTX 401 study in GSDIa in the third quarter of 2019.**

Corporate Updates

- **Expanded collaboration with Arcturus Therapeutics to develop additional nucleic acid therapies.** The research collaboration and license agreement with Arcturus Therapeutics now includes the discovery and development of mRNA, DNA, and siRNA therapeutics for up to 12 rare disease targets.
- **Erik Harris named Chief Commercial Officer.** In June 2019, Ultragenyx promoted Erik Harris to Executive Vice President and Chief Commercial Officer. Mr. Harris joined Ultragenyx in 2017 as Senior Vice President, Head of North American Commercial Operations, leading the launches of Crysvita and Mepsevii. In his new role, Mr. Harris will be responsible for all commercial operations in North America, Europe, and Latin America.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Thursday, August 1, 2019, at 2 p.m. PT/ 5 p.m. ET to discuss second quarter 2019 financial results and provide a corporate update. The live and replayed webcast of the call will be available through the company's website at http://ir.ultragenyx.com/events.cfm. To participate in the live call by phone, dial (855) 797-6910 (USA) or (262) 912-6260 (international) and enter the passcode 6298416. The replay of the call will be available for one year.

About Ultragenyx

Ultragenyx is a biopharmaceutical company committed to bringing to patients novel products for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved therapies and product candidates aimed at addressing diseases with high unmet medical need and clear biology for treatment, for which there are typically no approved therapies treating the underlying disease.

Crysvita® (burosumab) is approved in the United States, Canada and Brazil for the treatment of XLH in adult and pediatric patients one year of age and older and has received conditional marketing authorization in Europe for the treatment of XLH with radiographic evidence of bone disease in children one year of age and older and adolescents with growing skeletons. Mepsevii® (vestronidase alfa) is approved in the United States and Brazil for the treatment of children and adults with MPS VII. In Europe, Mepsevii is approved under exceptional circumstances for the treatment of non-neurological manifestations of MPS VII.

In addition to the approved treatments for XLH and MPS VII, Ultragenyx has four clinical development programs. Crysvita is being

studied for the treatment of TIO, a rare disease that impairs bone mineralization. UX007 is being studied in patients with LC-FAOD, a genetic disorder in which the body is unable to convert long chain fatty acids into energy. The company has two gene therapy pipeline candidates: DTX301 is an AAV8 gene therapy product candidate in development for the treatment of patients with OTC deficiency, the most common urea cycle disorder; and DTX401 is an AAV8 gene therapy product candidate for the treatment of patients with GSDIa.

The company is led by a management team experienced in the development and commercialization of rare disease therapeutics. Ultragenyx's strategy is predicated upon time- and cost-efficient drug development, with the goal of delivering safe and effective therapies to patients with the utmost urgency.

For more information on Ultragenyx, please visit the company's website at www.ultragenyx.com.

Forward-Looking Statements

Except for the historical information contained herein, the matters set forth in this press release, including statements related to Ultragenyx's expectations regarding plans for its clinical programs and clinical studies, future regulatory interactions, and the components and timing of regulatory submissions are forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development programs, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the uncertainties inherent in the clinical drug development process, such as the regulatory approval process, the timing of regulatory filings, and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations and the availability or commercial potential of our products and drug candidates. Ultragenyx undertakes no obligation to update or revise any forward looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of Ultragenyx in general, see Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on May 7, 2019, and its subsequent periodic reports filed with the Securities and Exchange Commission.

Ultragenyx Pharmaceutical Inc.
Selected Statement of Operations Financial Data
(in thousands, except share and per share amounts)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	**2018**	**2019**	**2018**
Statement of Operations Data:				
Revenues:				
Collaboration and license	$ 19,247	$ 10,519	$ 33,485	$ 19,881
Product sales	4,902	2,275	8,836	3,590
Total revenues	24,149	12,794	42,321	23,471
Operating expenses:				
Cost of sales	766	141	1,218	366
Research and development	96,045	76,835	174,150	152,339
Selling, general and administrative	39,812	30,718	78,641	62,153
Total operating expenses	136,623	107,694	254,009	214,858
Loss from operations	(112,474)	(94,900)	(211,688)	(191,387)
Gain from sale of priority review vouchers	—	40,322	—	170,322
Change in fair value of investment in Arcturus equity securities	9,828	—	9,828	—
Other income (expense), net	3,687	1,952	6,361	(1,269)
Loss before income taxes	(98,959)	(52,626)	(195,499)	(22,334)
Provision for income taxes	(213)	(102)	(429)	(141)
Net loss	$ (99,172)	$ (52,728)	$ (195,928)	$ (22,475)
Net loss per share, basic and diluted	$ (1.72)	$ (1.06)	$ (3.54)	$ (0.46)
Shares used in computing net loss per share, basic and diluted	57,519,308	49,819,528	55,376,336	49,046,838

Ultragenyx Pharmaceutical Inc.
Selected Balance Sheet Financial Data
(in thousands)
(unaudited)

	June 30, 2019	December 31, 2018
Balance Sheet Data:		
Cash, cash equivalents, and available-for-sale investments	$ 618,286	$ 459,706
Working capital	624,425	447,644
Total assets	958,927	719,558
Total stockholders' equity	819,787	608,908

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