Ultragenyx Reports Third Quarter 2019 Financial Results and Corporate Update

November 5, 2019

Strong Crysvita® (burosumab) Launch Continues with Approximately 1,130 Patients on Reimbursed Commercial Therapy in the United States as of the end of the Third Quarter

UX007 (triheptanoin) NDA for Treatment of Long-chain Fatty Acid Oxidation Disorders Accepted for Review by FDA

NOVATO, Calif., Nov. 05, 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 September 30, 2019.

"The U.S. launch of Crysvita continues to be strong with the number of patients on reimbursed therapy continuing to increase, which will be further supported by the recently expanded label for both pediatric and adult patients," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "The New Drug Application for UX007 is now being reviewed by FDA, and our second indication for Crysvita for tumor-induced osteomalacia will be submitted in the coming months, potentially giving us approved treatments for four different diseases in 2020."

Third Quarter 2019 Financial Results

Net Revenues

For the third quarter of 2019, Ultragenyx reported $25.8 million in total revenue, including $22.6 million in Crysvita revenue. Crysvita revenue includes $19.5 million in collaboration revenue in the North American profit share territory, $2.0 million in royalty revenue in the European territory from Kyowa Kirin Co., and product revenue in other regions of $1.1 million. The Company recognized $2.4 million in Mepsevii® (vestronidase alfa) product revenue, $0.7 million in UX007 named patient revenue, and also $0.1 million in revenue from its research agreement with Bayer.

Revenue for the nine months ended September 30, 2019 was $68.1 million, including $57.3 million in Crysvita revenue. Crysvita revenue includes $48.7 million in collaboration revenue in the North American profit share territory, $5.9 million in royalty revenue in the European territory from Kyowa Kirin Co., and product revenue for Crysvita in other regions of $2.7 million. The Company recognized $8.3 million in Mepsevii product revenue, $2.0 million in UX007 named patient revenue, and also $0.5 million in revenue from its research agreement with Bayer.

Operating Expenses

Total operating expenses for the third quarter of 2019 were $143.8 million compared with $101.4 million for the same period in 2018, including a $20.0 million research and development expense in the third quarter of 2019 from the GeneTx agreement, and non-cash stock-based compensation of $19.9 million and $20.7 million in the third quarter of 2019 and 2018, respectively. Total operating expenses for the nine months ended September 30, 2019 were $397.8 million compared with $316.3 million for the same period in 2018, including a $15.6 million research and development expense from the Arcturus collaboration amendment, a $20.0 million research and development expense from the GeneTx agreement, and non-cash stock-based compensation of $62.3 million and $59.0 million in the first nine months 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 third quarter of 2019, Ultragenyx reported a net loss of $113.0 million, or $1.96 per share, basic and diluted, compared with a net loss for the third quarter of 2018 of $87.3 million, or $1.74 per share, basic and diluted. For the nine months ended September 30, 2019, net loss was $308.9 million, or $5.50 per share, basic and diluted, compared with a net loss for the same period in 2018 of $109.8 million, or $2.22 per share, basic and diluted. The loss for the third quarter of 2019 and for the nine months ended September 30, 2019 includes unrealized gains of $2.2 million and $12.0 million, respectively, from the fair value adjustment on the investment in Arcturus equity securities. The loss for the first nine months of 2018 includes 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 and includes a $130.0 million gain from the sale of the PRV received with the Mepsevii FDA approval. The net loss for the first nine months of 2019 reflected cash used in operations of $273.3 million compared to $234.7

million for the same period in 2018.

Cash, Cash Equivalents and Investments

Cash, cash equivalents and available-for-sale investments were $527.1 million as of September 30, 2019.

Recent Updates and Upcoming Milestones

Crysvita Commercial Progress in X-Linked Hypophosphatemia (XLH)

- **Strong U.S. launch continues,** with approximately 170 new patients on reimbursed commercial therapy in the United States in the third quarter 2019.
- **U.S. Crysvita label expanded.** The U.S. FDA approved a label expansion for Crysvita, which now includes clinical data demonstrating superiority of treatment with Crysvita versus conventional therapy in pediatric patients, and improvement in stiffness and maintenance of efficacy in adult patients with longer-term treatment. The indication has also been expanded to include infants as young as six months of age.

Crysvita Supplemental Biologics License Application (sBLA) for Tumor-Induced Osteomalacia (TIO)

- **Ultragenyx plans to submit a sBLA to the U.S. FDA for Crysvita for the treatment of TIO in the first half of 2020.**

UX007 NDA for the Treatment of Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD) Accepted for Review by U.S. FDA

- **The U.S. FDA accepted for review the New Drug Application (NDA) and has set a Prescription Drug User Fee Act (PDUFA) date of July 31, 2020.** The FDA has indicated that it is not currently planning to hold an advisory committee meeting to discuss the application.

Clinical-stage Gene Therapy Programs Advance

- **DTX301 Phase 1/2 study in ornithine transcarbamylase (OTC) deficiency:** Data from Cohorts 1 and 2 demonstrated that the two responders maintained ureagenesis levels above normal and normal ammonia levels for 78 and 52 weeks, respectively. There have been no infusion-related adverse events and no treatment-related serious adverse events reported. Ultragenyx plans to provide an update from the ongoing third dose cohort around the end of the year. The company also plans to proceed to an additional cohort using prophylactic steroids.
- **DTX401 Phase 1/2 Study in glycogen storage disease type Ia (GSDIa):** Data from the first two dose cohorts demonstrated that all patients showed clinical responses, including improvements in glucose control shown by time to hypoglycemia and reductions in cornstarch requirements. In the second dose cohort, all three patients showed a more meaningful reduction in glycogen storage as measured by liver fat fraction and improvements in metabolism as measured by lactate levels. Based on these data, Ultragenyx is conducting a confirmatory expansion cohort of three patients at the second cohort dose of 6.0 × 10^12 GC/kg, with data from this cohort expected in the first half of 2020.

Corporate Updates

- **Agreement with GeneTx Biotherapeutics to advance treatment for Angelman Syndrome**. Ultragenyx and GeneTx are collaborating on the development of GeneTx's GTX-102, an antisense oligonucleotide (ASO) for the treatment of Angelman syndrome, a debilitating and rare neurogenetic disorder. GTX-102 has been granted Orphan Drug Designation and Rare Pediatric Disease Designation, and the Investigational New Drug (IND) timing is on track. Ultragenyx made an upfront payment of $20 million which included an exclusive option to acquire GeneTx. This option may be exercised any time prior to 30 days following FDA acceptance of the IND for GTX-102. The option period may be extended by paying an option extension payment of $25 million.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Tuesday, November 5, 2019, at 2 p.m. PT/ 5 p.m. ET to discuss third 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 9946526. 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 for the treatment of XLH in adult and pediatric patients six months of age and older, and in Canada and Brazil for the treatment of XLH in adult and pediatric patients one year of age and older. Crysvita 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 August 2, 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 September 30,		Nine Months Ended September 30,	
	2019	**2018**	**2019**	**2018**
Statement of Operations Data:				
Revenues:				
Collaboration and license	$ 21,585	$ 9,015	$ 55,070	$ 28,896
Product sales	4,215	2,748	13,051	6,338
Total revenues	25,800	11,763	68,121	35,234
Operating expenses:				
Cost of sales	2,683	273	3,901	639
Research and development	100,144	70,041	274,294	222,380
Selling, general and administrative	41,006	31,095	119,647	93,248
Total operating expenses	143,833	101,409	397,842	316,267
Loss from operations	(118,033)	(89,646)	(329,721)	(281,033)
Gain from sale of priority review vouchers	—	—	—	170,322

Change in fair value of investment in Arcturus equity securities	2,166	—	11,994	—
Other income (expense), net	3,166	2,583	9,527	1,314
Loss before income taxes	(112,701)	(87,063)	(308,200)	(109,397)
Provision for income taxes	(293)	(247)	(722)	(388)
Net loss	$ (112,994)	$ (87,310)	$ (308,922)	$ (109,785)
Net loss per share, basic and diluted	$ (1.96)	$ (1.74)	$ (5.50)	$ (2.22)
Shares used in computing net loss per share, basic and diluted	57,707,694	50,319,772	56,161,996	49,447,889

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

	September 30, 2019	December 31, 2018
Balance Sheet Data:		
Cash, cash equivalents, and available-for-sale investments	$ 527,058	$ 459,706
Working capital	523,179	447,644
Total assets	879,944	719,558
Total stockholders' equity	727,265	608,908

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