Ultragenyx Reports First Quarter 2019 Financial Results and Corporate Update

May 6, 2019

Strong Crysvita® (burosumab) Launch Continues with Approximately 730 Patients on Reimbursed Commercial Therapy in the United States

NOVATO, Calif., May 06, 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 March 31, 2019.

"We are seeing strong, sustained momentum from the Crysvita launch in the United States, driven by increased breadth and depth of prescribers treating both adults and children with XLH," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "We are also on track to submit UX007 for LC-FAOD for regulatory review in the coming months, and are advancing our gene therapy platform with key data readouts expected from two clinical programs. In addition, we plan to move three preclinical programs into the clinic in 2020."

First Quarter 2019 Financial Results

Net Revenues

For the first quarter of 2019, Ultragenyx reported $18.2 million in total revenue. Ultragenyx recognized $14.5 million in total Crysvita revenue. This includes $11.9 million in collaboration revenue in the U.S. profit share territory and $2.0 million in royalty revenue in the European territory from the collaboration and license agreement with Kyowa Hakko Kirin. Net product sales for Crysvita in other regions were $0.6 million. Mepsevii® (vestronidase alfa) product revenue for the first quarter of 2019 was $2.7 million, and UX007 named patient revenue was $0.7 million. Ultragenyx recognized $0.3 million in revenue from its research agreement with Bayer.

Operating Expenses

Total operating expenses for the first quarter of 2019 were $117.4 million compared with $107.2 million for the same period in 2018, including non-cash stock-based compensation of $20.2 million and $18.8 million in the first quarter 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 first quarter of 2019, Ultragenyx reported a net loss of $96.8 million, or $1.82 per share, basic and diluted, compared with a net income for the first quarter of 2018 of $30.3 million, or $0.63 per basic share and $0.62 per diluted share. The income for the first quarter of 2018 included the $130.0 million gain from the sale of the priority review voucher (PRV). The net loss for the first quarter of 2019 reflected cash used in operations of $95.8 million compared to $89.5 million for the same period in 2018.

Cash, Cash Equivalents and Investments

Cash, cash equivalents and investments were $715.3 million as of March 31, 2019.

Recent Updates and Upcoming Milestones

Crysvita in X-Linked Hypophosphatemia (XLH)

- **Strong launch continues in the United States,** with approximately 730 patients on reimbursed commercial therapy at the end of the first quarter 2019.
- **Crysvita was approved in Brazil for the treatment of XLH in adults and pediatric patients one year of age and older.** Crysvita is approved in three key regions of the world including North America, Europe, and now the first country in Latin America.

UX007 in Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD)

- **Fast Track Designation and Rare Pediatric Disease Designation granted by U.S. Food and Drug Administration**

(FDA). Fast Track designation allows for early and frequent communication with the FDA, and also enables eligibility for Priority Review, if relevant criteria are met.
- **Ultragenyx is on track to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) in mid-2019.** The submission will include data from a company-sponsored Phase 2 study of UX007 in 29 patients, data from a long-term safety and efficacy extension study in 75 patients, a retrospective medical record review of 20 original compassionate use patients, data from 70 patients treated through expanded access, and a randomized controlled investigator-sponsored study of 32 patients showing an effect of triheptanoin on cardiac function.

DTX301 Gene Therapy in Ornithine Transcarbamylase (OTC) Deficiency

- **DTX301 data from first two dose cohorts demonstrate long-term normalization of ureagenesis.** The two responders in Cohort 1 and 2 have now maintained ureagenesis levels above normal for 52 and 78 weeks.
- **DTX301 Phase 1/2 data from the third-dose cohort are expected in mid-2019.**

DTX401 Gene Therapy in Glycogen Storage Disease Type Ia (GSDIa)

- **Positive longer-term results from the first cohort of the Phase 1/2 clinical study of DTX401 gene therapy in GSDIa.** After 24 weeks of treatment, all three patients have either maintained or further increased their time to hypoglycemia during the controlled fasting challenge compared to baseline. All three patients continue to show a clinical response with additional improvements in glucose control reflected by prolonged time to hypoglycemia during a controlled fasting challenge. Patients have currently reduced cornstarch intake by approximately 50 to 90% and continue to maintain normal glucose levels throughout the day and overnight. There were no infusion-related adverse events and no treatment-related serious adverse events reported.
- **DTX401 Phase 1/2 data from the second-dose cohort are expected in mid-2019.**

Corporate

- **Equity financing of approximately $330 million:** In March 2019, we completed an underwritten public offering, with net proceeds of approximately $330 million. We intend to use a portion of the net proceeds to build our own GMP gene therapy manufacturing facility to reduce the costs and increase the speed with which we can execute on our gene therapy programs.
- **Analyst and Investor Day:** In April 2019, we held an Analyst and Investor Day that provided an update on recent corporate and pipeline updates, including a deeper dive on the gene therapy platform and three preclinical programs with near-term INDs expected in 2020.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Monday, May 6, 2019, at 2 p.m. PT/ 5 p.m. ET to discuss first 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 3666589. 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 1 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 Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 20, 2019, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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

	Three Months Ended March 31,	
	2019	**2018**
Statement of Operations Data:		
Revenues:		
Collaboration and license	$ 14,238	$ 9,362
Product sales	3,934	1,315
Total revenues	18,172	10,677
Operating expenses:		
Cost of sales	452	225
Research and development	78,105	75,504
Selling, general and administrative	38,829	31,435
Total operating expenses	117,386	107,164
Loss from operations	(99,214)	(96,487)
Gain from sale of priority review voucher	—	130,000
Other income (expense), net	2,674	(3,221)
Income (loss) before income taxes	(96,540)	30,292
Provision for income taxes	(216)	(39)
Net income (loss)	$ (96,756)	$ 30,253
Net income (loss) per share:		
Basic	$ (1.82)	$ 0.63
Diluted	$ (1.82)	$ 0.62
Shares used in computing net income (loss) per share:		
Basic	53,209,215	48,190,511
Diluted	53,209,215	49,077,742

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

	March 31, 2019	December 31, 2018
Balance Sheet Data:		
Cash, cash equivalents and investments	$ 715,316	$ 459,706
Working capital	720,007	447,644
Total assets	1,007,759	719,558
Total stockholders' equity	886,729	608,908

Source: Ultragenyx Pharmaceutical Inc.