Ultragenyx Reports Second Quarter 2018 Financial Results and Corporate Update

August 2, 2018

NOVATO, Calif, Aug. 02, 2018 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, today reported its financial results and corporate update for the quarter ended June 30, 2018.

“Enthusiasm from the XLH community has resulted in promising commercial uptake of Crysvita in the United States, and momentum continues to grow among both pediatric and adult patients,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “In the second half of the year we will continue to focus on the launches of both Crysvita and Mepsevii, and advance the rest of our pipeline including our clinical-stage gene therapy programs with key data readouts expected for both programs.”

Financial Results

For the second quarter of 2018, Ultragenyx reported a net loss of $52.7 million, or $1.06 per share, basic and diluted, compared with a net loss for the second quarter of 2017 of $72.9 million, or $1.72 per share, basic and diluted. The loss for the second quarter 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® (burosumab) approval. For the six months ended June 30, 2018, net loss was $22.5 million, or $0.46 per share, basic and diluted, compared with a net loss for the same period in 2017 of $141.2 million, or $3.35 per share, basic and diluted. In addition to the Crysvita PRV, the loss from the first six months also includes the sale of the Mepsevii™ (vestronidase alfa) PRV in January 2018 for net proceeds of $130.0 million. The net loss for the first six months of 2018 reflected cash used in operations of $165.6 million compared to $110.0 million for the same period in 2017.

Net Revenues

For the second quarter of 2018, Ultragenyx reported $12.8 million in total revenue. Ultragenyx recognized $8.9 million in revenue from the research agreement with Bayer. For Crysvita, Ultragenyx recognized $1.6 million in profit sharing and royalty revenue from the collaboration and license agreement with Kyowa Hakko Kirin. This includes $1.1 million in collaboration revenue in the U.S profit share territory, where Crysvita became commercially available on April 27, 2018, as well as $0.5 million in royalty revenue in the European territory, where Crysvita received conditional marketing authorization on February 23, 2018. There were nominal net product sales for Crysvita in other regions. Mepsevii product revenue for the second quarter of 2018 was $2.0 million, and UX007 named patient revenue was $0.2 million.

Operating Expenses

Total operating expenses for the second quarter of 2018 were $107.7 million compared with $78.4 million for the same period in 2017, including non-cash stock-based compensation of $19.6 million and $16.8 million in the second quarter of 2018 and 2017, respectively. Total operating expenses for the six months ended June 30, 2018 were $214.9 million compared with $148.4 million for the same period in 2017, including non-cash stock-based compensation of $38.4 million and $31.3 million in the first six months of 2018 and 2017, 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.

Cash, cash equivalents, and investments

Cash, cash equivalents, and investments were $547.1 million as of June 30, 2018.

Recent Highlights

Crysvita in X-Linked Hypophosphatemia (XLH)

- **Positive data from the Phase 3 pediatric study demonstrated that Crysvita was superior to oral phosphate and active vitamin D (conventional therapy) in improving rickets in children with XLH after 40 weeks of treatment.**

Mepsevii in mucopolysaccharidosis VII (MPS VII)

- **In Europe, Mepsevii received a positive opinion from the Committee for Medicinal Products for Human Use (CHMP), recommending the marketing authorization under exceptional circumstances of Mepsevii for the treatment of non-neurological manifestations of MPS VII**. A decision from the European Commission, which has the authority to approve medicines for the European Union, is expected in the third quarter of 2018.

UX007 in long-chain fatty acid oxidation disorders (LC-FAOD)

- **Discussions are ongoing with FDA and EMA to determine the acceptability of filing UX007 for the treatment of LC-FAOD based on the totality of currently available data.** The data from the Phase 2 study for UX007 show a significant reduction in major clinical events; however, the FDA continues to believe that the data are confounded and are

not sufficient to support a New Drug Application (NDA). We continue to pursue potential filings with FDA and EMA based on the current data and expect to conclude discussions in the second half of 2018. These discussions also should provide further clarity regarding whether an additional study would be required for approval.

DTX401 gene therapy in glycogen storage disease type Ia (GSDIa)

- **The first patient has been dosed in the Phase 1/2 study of DTX401, our adeno-associated virus 8 (AAV8) gene therapy program for the treatment of patients with GSDIa.** Data from the three-patient first dose cohort are expected in the second half of 2018.
- **The U.S. FDA granted fast-track designation to DTX401 for the treatment of GSDIa.** This designation is designed to facilitate the development and expedite the review of drugs that are intended to treat serious conditions and fill an unmet medical need, and it allows for more frequent interaction with the FDA review team. It also enables eligibility for priority review if relevant criteria are met and the potential for a rolling review of the Biologic License Application (BLA) as data become available.

Upcoming Key Milestones

Crysvita (burosumab) in tumor-induced osteomalacia (TIO)

- **Data from all patients in the Phase 2 study in TIO are expected in the second half of 2018.** This is an open label Phase 2 study evaluating the safety and efficacy of burosumab in adult patients with TIO.

UX007 in LC-FAOD and glucose transporter type-1 deficiency syndrome (Glut1 DS)

- **In LC-FAOD, additional clarity from FDA and EMA on the regulatory pathway is expected in the second half of 2018.**
- **The fully-enrolled Phase 3 movement disorder study in patients with Glut1 DS is on track and data are expected in second half of 2018.**

DTX301 gene therapy in ornithine transcarbamylase (OTC) Deficiency

- **Results from the fully-enrolled cohort 2 of the Phase 1/2 study are expected in second half of 2018.**

DTX401 Gene Therapy in GSDIa

- **Data from the first, lowest dose cohort are expected in the second half of 2018.**

Conference Call & Webcast Information

Ultragenyx will host a conference call today, Thursday, August 2, 2018 at 5pm ET to discuss second quarter 2018 financial results and to 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 2895644. 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 rare and ultra-rare diseases, with a focus on serious, debilitating genetic diseases. Founded in 2010, the company has rapidly 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 no approved therapies.

Crysvita® (burosumab) is approved by the U.S. FDA 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 by the U.SFDA for the treatment of children and adults with MPS VII, and has received a positive opinion from the CHMP in Europe recommending the marketing authorization under exceptional circumstances of Mepsevii for the treatment of non-neurological manifestations of MPS VII.

In addition to the approved treatments for XLH and MPS VII, Ultragenyx has six 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 Glut1 DS, a brain energy deficiency, who are experiencing movement disorders; it is also being studied in patients severely affected by LC-FAOD, a genetic disorder in which the body is unable to convert long chain fatty acids into energy. The company has three gene therapy pipeline candidates: DTX 301 is an AAV8 gene therapy product candidate in development for the treatment of patients with OTC deficiency, the most common urea cycle disorder; DTX 401 is an AAV8 gene therapy product candidate for the treatment of patients with GSDIa; and DTX201 is an FVIII gene therapy clinical candidate for the treatment of hemophilia A that is being developed with Bayer.

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 regarding Ultragenyx's expectations regarding ongoing or additional studies for its product candidates and timing regarding these studies, the demonstrated impact of clinical data and other information to support approval of product candidates, discussions with regulatory authorities, and sufficiency for, and timing of, regulatory submissions and approvals 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 our 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 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 the company in general, see Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on May 8, 2018, and its subsequent periodic reports filed with the Securities and Exchange Commission.

Contact Ultragenyx Pharmaceutical Inc.
Investors & Media
Danielle Keatley
415-475-6876

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,	
	2018	**2017**	**2018**	**2017**
Statement of Operations Data:				
Revenues:				
Collaboration and license	$ 10,519	$ —	$ 19,881	$ —
Product sales	2,275	—	3,590	—
Total revenues	12,794	—	23,471	—
Operating expenses:				
Cost of sales	141	—	366	—
Research and development	76,835	58,436	152,339	109,705
Selling, general and administrative	30,718	20,005	62,153	38,690
Total operating expenses	107,694	78,441	214,858	148,395
Loss from operations	(94,900)	(78,441)	(191,387)	(148,395)
Gain from sale of priority review vouchers	40,322	—	170,322	—
Other income (expense), net	1,952	5,564	(1,269)	7,228
Loss before income taxes	(52,626)	(72,877)	(22,334)	(141,167)
Provision for income taxes	(102)	(14)	(141)	(14)
Net loss	$ (52,728)	$ (72,891)	$ (22,475)	$ (141,181)
Net loss per share, basic and diluted	$ (1.06)	$ (1.72)	$ (0.46)	$ (3.35)
Shares used in computing net loss per share, basic and diluted	49,819,528	42,346,830	49,046,838	42,095,750

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

	June 30, 2018	**December 31, 2017**
Balance Sheet Data:		
Cash, cash equivalents and investments	$ 547,132	$ 244,468
Working capital	538,694	198,569
Total assets	806,022	490,753
Total stockholders' equity	703,971	383,454

Primary Logo

Source: Ultragenyx Pharmaceutical Inc.