Ultragenyx Reports First Quarter 2018 Financial Results and Corporate Update

May 7, 2018

NOVATO, Calif., May 07, 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 March 31, 2018.

“With the recent approvals and launches of Crysvita in the United States and Europe, we have transformed into a commercial stage company with two medicines now available for patients,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “We continue to advance our clinical and preclinical programs and expect significant progress across our two clinical-stage gene therapy programs as well as our small molecule and biologics programs this year.”

First Quarter 2018 Financial Results

For the first quarter of 2018, Ultragenyx reported net income of $30.3 million, or $0.63 per basic share and $0.62 per diluted share, compared with a net loss for the first quarter of 2017 of $68.3 million, or $1.63 per share, basic and diluted. The income for the first quarter of 2018 includes the $130 million gain from the sale of the priority review voucher (PRV). The net income for the first quarter of 2018 reflected cash used in operations of $89.5 million compared to $61.2 million reflected in the net loss for the same period in 2017.

For the first quarter of 2018, Ultragenyx reported $10.7 million in total revenue, which includes $1.3 million in product revenue from Mepsevii™ (vestronidase alfa) and UX007, and $9.4 million in collaboration and license revenue, primarily from our research agreement with Bayer. Total operating expenses for the first quarter of 2018 were $107.2 million compared with $70.0 million for the same period in 2017, including non-cash stock-based compensation of $18.8 million and $14.5 million in the first quarter of 2018 and 2017, respectively. The increase in total operating expenses is due to the increase in development, commercial, and general and administrative costs as the company commercializes, grows and advances its pipeline.

Cash, cash equivalents, and investments were $571.3 million as of March 31, 2018.

Recent Highlights

Crysvita® (burosumab) in X-Linked Hypophosphatemia (XLH)

- **In the U.S., Crysvita was approved on April 17, 2018 and is now commercially available to adults and children with X-linked hypophosphatemia (XLH).** In April, the U.S. Food and Drug Administration (FDA) approved Crysvita for the treatment of XLH in adult and pediatric patients one year of age and older. The first patient has now received commercial treatment with Crysvita.

- **In Europe, burosumab received conditional marketing authorization for the treatment of XLH with radiographic evidence of bone disease in children 1 year of age and older and adolescents with growing skeletons.**

DTX301 Gene Therapy in ornithine transcarbamylase (OTC) Deficiency

- **Data from Phase 1/2 study of DTX301, our AAV8 vector in patients with OTC, showed positive topline results including normalization of ureagenesis in one patient in the first, lowest-dose cohort.** The first patient’s rate of ureagenesis was normalized, maintained and then substantially increased over 24 weeks. The second and third patients did not show a clinically meaningful change in rate of ureagenesis over 20 weeks and 12 weeks, respectively. There have been no infusion-related adverse events and no serious adverse events reported. All adverse events have been Grade 1 or 2 and have resolved. Two patients have been enrolled in the higher dose Cohort 2 portion of the study, and data from the second cohort are expected in the second half of 2018.

DTX401 Gene Therapy in glycogen storage disease type Ia (GSDIa)

- **The U.S. FDA cleared the Investigational New Drug (IND) application for DTX401 for the treatment of patients with GSDIa.** Enrollment in the Phase 1/2 study is expected to begin in the first half of 2018, with data from the first cohort in the second half of 2018.

Corporate

- **PRV sold for $130 million**: In January 2018, we completed the sale of the PRV that we received at the time of the approval of Mepsevii.

- **Equity financing of approximately $271.0 million:** In January 2018, we completed an underwritten public offering, with net proceeds of approximately $271.0 million.

Upcoming Key Milestones

Crysvita (burosumab) in XLH

- **Data from the Phase 3 study in pediatric patients expected in the second half of 2018.** The ongoing Phase 3 randomized open-label clinical study is comparing the efficacy and safety of burosumab to oral phosphate and active vitamin D therapy in pediatric patients with XLH. This study will serve as a confirmatory study in Europe.

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

- **Data from all patients in Phase 2 study in TIO expected in mid-2018.** This is an open label Phase 2 study evaluating the safety and efficacy of burosumab in 17 adult patients with TIO.

Mepsevii (vestronidase alfa) in mucopolysaccharidosis VII (MPS VII)

- **In Europe, an opinion from the Committee for Medicinal Products for Human Use (CHMP) is expected in mid-2018.**

UX007 in long-chain fatty acid oxidation disorders (FAOD) and glucose transporter type-1 deficiency syndrome (Glut1 DS)

- **Completing study design of Phase 3 study in patients with FAOD; providing additional data to FDA for consideration of early filing based on Phase 2 data.** Following an end-of-phase 2 meeting, we are providing additional information to submit to FDA for consideration of an early filing based on the results from the Phase 2 study. While the FDA still prefers that a randomized controlled trial be completed before filing, it left open the possibility of filing on the current data. We are simultaneously completing the design of a Phase 3 study that could be used for registration or confirmatory purposes. We expect that a decision on a potential filing for approval based on Phase 2 data will be made in mid-2018.

- **Data from the Phase 3 movement disorder study in patients with Glut1 DS.** Enrollment is complete and data are expected in the second half of 2018.

Conference Call & Webcast Information

Ultragenyx will host a conference call today, Monday, May 7, 2018 at 5pm ET to discuss first 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 3748439. 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 product candidates with the potential to address diseases for which the unmet medical need is high, the biology for treatment is clear, and 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.

Ultragenyx is conducting Phase 2 and Phase 3 studies of burosumab in patients with XLH and TIO, both rare diseases that impair bone mineralization; a Phase 2 clinical study of UX007 in patients severely affected by LC-FAOD, a genetic disorder in which the body is unable to convert long chain fatty acids into energy; a Phase 3 study for UX007 in patients with Glut1 DS, a brain energy deficiency, who are experiencing movement disorders; a Phase 1/2 study of DTX301 gene therapy in OTC deficiency, a urea cycle disorder that causes ammonia to accumulate in the blood; and a Phase 1/2 study of DTX401 gene therapy in GSDIa, a disease that arises from a defect in G6Pase, an essential enzyme in glycogen and glucose metabolism.

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 design of clinical studies, the demonstrated impact of clinical data and other information to support approval of product candidates, potential indications for its product candidates, discussions with regulatory authorities, the potential issuance of a priority review voucher, sufficiency for, and timing of, regulatory submissions and approvals, and the timing and locations of commercialization efforts 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 Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 21, 2018, 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 March 31,	
	2018	**2017**
Statement of Operations Data:		
Revenues:		
Collaboration and license	$ 9,362	$ —
Product sales	1,315	—
Total revenues	10,677	—
Operating expenses:		
Cost of goods sold	225	—
Research and development	75,504	51,269
Selling, general and administrative	31,435	18,685
Total operating expenses	107,164	69,954
Loss from operations	(96,487)	(69,954)
Gain from sale of priority review voucher	130,000	—
Other income (expense), net	(3,221)	1,664
Income (loss) before income taxes	30,292	(68,290)
Income tax provision	(39)	—
Net income (loss)	$ 30,253	$ (68,290)
Net income (loss) per share:		
Basic	$ 0.63	$ (1.63)
Diluted	$ 0.62	$ (1.63)
Shares used in computing net income (loss) per share:		
Basic	48,190,511	41,841,612
Diluted	49,077,742	41,841,612

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

	March 31, 2018	**December 31, 2017**
Balance Sheet Data:		
Cash, cash equivalents and investments	$ 571,253	$ 244,468
Working capital	523,997	198,569
Total assets	817,350	490,753
Total stockholders' equity	723,657	383,454

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

Source: Ultragenyx Pharmaceutical Inc.