July 27, 2017

Ultragenyx Reports Second Quarter 2017 Financial Results and Corporate Update

Burosumab US BLA submission planned for August 2017

NOVATO, Calif., July 27, 2017 (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, 2017.

"We look forward to submitting our biologics license application (BLA) for burosumab and working with the FDA to bring this potential treatment to adult and pediatric patients," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "We believe the first bone biopsy data from our ongoing Phase 3 bone quality study underscore burosumab's impact on the underlying bone disease in adults with XLH."

Second Quarter 2017 Financial Results

For the second quarter of 2017, Ultragenyx reported a net loss of $72.9 million, or $1.72 per share, basic and diluted, compared with a net loss of $56.9 million, or $1.46 per share, basic and diluted in the second quarter of 2016. For the six months ended June 30, 2017, net loss was $141.2 million, or $3.35 per share, basic and diluted, compared with a net loss for the same period in 2016 of $109.7 million, or $2.81 per share, basic and diluted. This reflected cash used in operations of $110.0 million for the six months ended June 30, 2017 compared to $84.6 million for the same period in 2016.

Total operating expenses for the second quarter of 2017 were $78.4 million compared with $58.1 million for the same period in 2016, including non-cash stock-based compensation of $16.8 million and $10.9 million for the second quarter of 2017 and 2016, respectively. Total operating expenses for the six months ended June 30, 2017 were $148.4 million compared with $111.7 million for the same period in 2016, including non-cash stock-based compensation of $31.3 million and $21.1 million in the first six months of 2017 and 2016, respectively. The increase in total operating expenses is due to the increase in development, commercial, and general and administrative costs as the company grows and advances its pipeline.

Cash, cash equivalents, and investments were $457.5 million as of June 30, 2017.

Recent Highlights

Burosumab (KRN23) anti-FGF23 Monoclonal Antibody in X-Linked Hypophosphatemia (XLH)

- **Submission of a US BLA for burosumab for the treatment of pediatric and adult XLH patients planned in August 2017.** In June 2017, we announced that we had reached agreement with the FDA at a pre-Biologics License Application (pre-BLA) meeting on the clinical package to support the burosumab BLA filing for XLH. At the meeting, the FDA agreed that the BLA could be submitted based on available clinical data and confirmed that both pediatric and adult indications would be included in the review. Specifically for pediatric patients, the filing will include the 64-week data from our Phase 2 study in 5-12 year olds and 24-week data from the Phase 2 study in 1-4 year olds. The FDA confirmed that data from the ongoing pediatric Phase 3 study would not be required for the BLA filing. To support the adult indication, the submission will include the 24-week placebo-controlled data from the adult Phase 3 study. Additionally, FDA agreed to accept any available bone biopsy data from the 48-week open label bone quality study in adults as supportive evidence.
- **Two new pieces of data on adult patients to be included in the burosumab BLA.** Bone biopsy results from the first two adults in the bone quality study will be included in the burosumab BLA**.** In this study, baseline biopsies obtained from 11 patients confirmed that a majority of the patients had severe osteomalacia with a mean osteoid volume/bone volume of 26% vs. normal range of 0.3%-3.1%. These data verify that adult XLH patients have severe underlying bone disease even many years past puberty. Follow-up biopsies after 48 weeks of burosumab treatment are available from the first two patients. For these two patients, osteoid volume/bone volume was decreased from 24% and 29% to 9% and 7%, respectively. Osteomalacia was characterized by the pathologist as improving from severe to mild disease. Additionally, a post-hoc statistical analysis of bone fracture healing in the recently announced adult Phase 3 placebo-controlled study showed an odds ratio of 7.76 for complete healing of fractures and pseudofractures in the burosumab group compared to the placebo group (p=0.0004) at 24 weeks.

- **Burosumab designated a drug for a "rare pediatric disease".** The FDA's Office of Orphan Drug Development (OOPD) has recently designated burosumab for the treatment of XLH as a drug for a "rare pediatric disease". Under FDA's Rare Pediatric Disease Priority Review Voucher program, companies who receive approval for a new drug application or BLA for a rare pediatric disease may be eligible to receive a voucher for a Priority Review of a subsequent marketing application for a different product. The Priority Review Voucher may be used by the company or sold to a third party.
- **European Conditional Marketing Authorization Application (MAA) to be focused on pediatric indication.** To ensure the expeditious availability of burosumab for pediatric XLH patients in Europe and to avoid any potential delays in the review procedure due to the large amount of recent data from the adult XLH Phase 3 study to be filed, our partners Kyowa Hakko Kirin Co., Ltd. (Kyowa Hakko Kirin) and Kyowa Kirin International PLC have decided to separate the adult and pediatric indications. A filing for the adult indication is planned after a decision is first reached on the pediatric indication.
- **Turkey agreement with Kyowa Kirin.** In May 2017, Ultragenyx signed an agreement with a wholly owned subsidiary of Kyowa Kirin, pursuant to which Ultragenyx was granted the right to commercialize burosumab in Turkey. This subsidiary will have the option to take over commercialization efforts after a certain minimum period.

Vestronidase alpha or rhGUS in Mucopolysaccharidosis 7 (MPS 7)

- **BLA and MAA filings accepted for review.** The FDA has granted vestronidase alpha Priority Review Status, and the BLA has a PDUFA goal date for a decision of November 16, 2017. The Committee for Medicinal Products for Human Use (CHMP) opinion is expected in the first half of 2018.

Upcoming Key Milestones

Burosumab in XLH

- **Ultragenyx plans to submit a BLA for both adult and pediatric patients to the U.S. FDA for burosumab in August 2017.** Based on follow-up discussions with the FDA on the content and structure of the filing, the BLA filing is in final assembly and will be submitted in August. Given that the product is designated as a breakthrough therapy, it is expected that burosumab would obtain priority review after the filing which would indicate an 8-month review period.
- **Opinion from CHMP on the burosumab Conditional MAA for pediatric XLH expected around the end of 2017.** The MAA was submitted and accepted for review by the European Medicines Agency (EMA) in December 2016.

Vestronidase alpha in MPS 7

- **PDUFA goal date and CHMP opinion.** The PDUFA goal date for a decision is November 16, 2017 and no advisory committee meeting is expected in the US at this time. The CHMP opinion is expected in the first half of 2018.

Aceneuramic Acid Extended Release (Ace-ER) in GNE Myopathy

- **Data from the pivotal Phase 3 study in GNE myopathy expected in the second half of 2017.** The fully enrolled randomized, double-blind, placebo-controlled international study in 89 patients is evaluating the efficacy and safety of Ace-ER compared with placebo over 48 weeks. We plan to submit an NDA and MAA based on the Phase 3 data, if positive.

UX007 in Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD) and Glut1 Deficiency Syndrome (Glut1 DS)

- **Phase 3 study in FAOD patients**. We continue to plan for discussions with regulators regarding the Phase 3 study in FAOD patients.
- **Phase 3 movement disorder study in Glut1 DS patients ongoing**. The randomized, double-blind, placebo-controlled, cross-over study is enrolling approximately 40 patients. The primary endpoint compares the frequency of disabling paroxysmal movement disorder events with UX007 to placebo, as recorded by a daily electronic diary.

Conference Call & Webcast Information

Ultragenyx will host a conference call today, Thursday, July 27, 2017 at 5pm ET to discuss second quarter 2017 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 59665992. The replay of the call will be available for one year.

About Ultragenyx

Ultragenyx is a clinical-stage biopharmaceutical company committed to bringing to market 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.

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.

Ultragenyx Product Candidates

Ultragenyx has completed a Phase 3 study of vestronidase alpha (rhGUS) in patients with mucopolysaccharidosis 7 (MPS 7), a rare lysosomal storage disease, and is conducting a Phase 3 study of aceneuramic acid extended-release (Ace-ER) in patients with GNE myopathy, a progressive muscle-wasting disorder; Phase 2 and Phase 3 studies of burosumab, an antibody targeting fibroblast growth factor 23 (FGF23), in pediatric and adult patients with X-linked hypophosphatemia (XLH) and a Phase 2 study in tumor induced osteomalacia (TIO), both rare diseases that impair bone mineralization; a Phase 3 study for UX007 in patients with glucose transporter type-1 deficiency syndrome (Glut1 DS), a brain energy deficiency, who are experiencing movement disorders; a Phase 2 study of UX007 in Glut1 DS patients with seizures, and a Phase 2 clinical study of UX007 in patients severely affected by long-chain fatty acid oxidation disorders (LC-FAOD), a genetic disorder in which the body is unable to convert long chain fatty acids into energy.

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 the timing of release, demonstrated impact and adequacy of clinical data and other information to support approval of product candidates, its intent to make regulatory submissions and its expectations regarding timing of receiving potential approval of its product candidates, ongoing or additional studies for its product candidates and timing regarding these studies, the design of clinical studies, the extent of its translational research program, potential indications for its product candidates, discussions with regulatory authorities, and sufficiency for, 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 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 5, 2017, 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,	
	2017	2016	2017	2016
Statement of Operations Data:				
Revenue	$ -	$ 17	$ -	$ 17
Operating expenses:				
Research and development	58,436	43,332	109,705	83,747
General and administrative	20,005	14,738	38,690	27,945
Total operating expenses	78,441	58,070	148,395	111,692
Loss from operations	(78,441)	(58,053)	(148,395)	(111,675)
Other income, net	5,564	1,130	7,228	1,995
Loss before income taxes	(72,877)	(56,923)	(141,167)	(109,680)

Income tax provision	(14)	-	(14)	-
Net loss	$ (72,891)	$ (56,923)	$ (141,181)	$ (109,680)
Net loss per share, basic and diluted	$ (1.72)	$ (1.46)	$ (3.35)	$ (2.81)
Shares used in computing net loss per share, basic and diluted	42,346,830	39,028,701	42,095,750	38,999,439

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

	June 30, 2017	December 31, 2016
Balance Sheet Data:		
Cash, cash equivalents and investments	$457,450	$ 498,111
Working capital	357,119	341,436
Total assets	497,542	540,626
Total stockholders' equity	429,453	473,974

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