May 4, 2017

Ultragenyx Reports First Quarter 2017 Financial Results and Corporate Update

Additional Phase 3 data on burosumab shows increased healing of fractures in adult XLH

NOVATO, Calif, May 04, 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 March 31, 2017.

"We have started the year with positive data from our pediatric and adult studies of burosumab and look forward to moving this key program through the regulatory process in both the US and Europe this year," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "We believe the additional data on fracture healing from our Phase 3 study support burosumab's value in promoting bone healing in adults with XLH."

First Quarter 2017 Financial Results

For the first quarter of 2017, Ultragenyx reported a net loss of $68.3 million, or $1.63 per share, basic and diluted, compared with a net loss of $52.8 million, or $1.35 per share, basic and diluted in the first quarter of 2016. This reflected cash used in operations of $61.2 million for the quarter ended March 31, 2017 compared to $44.9 million for the same period in 2016.

Total operating expenses for the first quarter of 2017 were $70.0 million compared with $53.6 million for the same period in 2016, including non-cash stock-based compensation of $14.5 million and $10.2 million for the first three 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 $506.1 million as of March 31, 2017.

Recent Highlights

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

- **Positive Phase 3 study in adult XLH patients met primary endpoint of serum phosphorus response and secondary endpoint of stiffness improvement**. Twenty-four week data from the randomized, double-blind, placebo-controlled study demonstrated a statistically significant improvement in serum phosphorus levels among patients in the burosumab arm, a statistically significant improvement in stiffness and strong trends in improvements in physical function and pain. Adverse events were consistent with what has been previously observed in open label studies in adults and children.
- **Burosumab treatment resulted in increased healing of fractures compared to placebo.** As a pre-specified additional endpoint of the adult phase 3 XLH study, fractures were identified at baseline and followed at week 12 and week 24. X-rays were scored by two radiologists and an adjudicator in a blinded evaluation process. At study entry, 52% of patients (comprising 48% of patients randomized to burosumab and 56% of patients randomized to placebo) presented with either active fractures (12%) or pseudofractures (47%) or both. At week 24, 37% of active fractures or pseudofractures in patients treated with burosumab were completely healed compared to 10% on placebo. Additionally, at week 24, 3% of existing active fractures or pseudofractures treated with burosumab worsened compared to 11% on placebo. We look forward to discussing these data with regulators.
- **Positive data from Phase 2 pediatric studies of burosumab demonstrated sustained reduction in bone disease and improvement in growth through 64 weeks of treatment, and a sustained effect on bone mineral metabolites in patients under 5 years old.** Additional data from a study in children aged five to 12 showed that the previously reported positive treatment effects on serum phosphorus levels, rickets, growth rates, and functional outcomes were sustained over 64 weeks. Interim 24-week data from a separate study in patients aged one to five years demonstrated that burosumab increased serum phosphorus levels into the low normal range. Adverse events were consistent with what has been previously observed for burosumab for the treatment of XLH.

UX007 in Glut1 Deficiency Syndrome (Glut1 DS)

- **Phase 3 movement disorder study in Glut1 DS patients initiated.** 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.
- **Data from Phase 2 study in Glut1 DS patients with seizures showed a decrease in absence seizures, however overall seizures were not significantly reduced.** When evaluating each seizure type independently, treatment with UX007 showed a reduction in absence seizures captured on EEG. Based on these data, Ultragenyx is continuing to evaluate plans in the seizure indication.

Upcoming Key Milestones

Burosumab in XLH

- **Ultragenyx plans to submit a biologics license application (BLA) to the U.S. FDA for burosumab in the second half of 2017.** The company will discuss the details of the planned submission with FDA at a pre-BLA meeting, and expects to submit both pediatric and adult data. The FDA has granted breakthrough therapy designation to burosumab for the treatment of XLH in pediatric patients one year of age and older.
- **Opinion from Committee for Medicinal Products for Human Use (CHMP) on the burosumab Conditional Marketing Authorization Application (MAA) for XLH expected by the end of 2017.** The MAA was submitted and accepted for review by the European Medicines Agency (EMA) in December 2016.

rhGUS in MPS 7

- **Ultragenyx is on track for regulatory filings in the U.S. and Europe in the first half of 2017, based on Phase 3 study results**. In Europe, the primary endpoint is the percent reduction in urinary glycosaminoglycans (GAG) excretion after 24 weeks of treatment. The EMA has indicated that some evidence or trend in improvement in clinical endpoints would also be necessary for approval. In the US, there is no primary endpoint declared; the FDA will consider the totality of data on a per-patient basis.

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.

Conference Call & Webcast Information

Ultragenyx will host a conference call today, Thursday, May 4, 2017 at 5pm ET to discuss first 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 12422215. 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 recombinant human beta-glucuronidase (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 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 Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 17, 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 March 31,	
	2017	**2016**
Statement of Operations Data:		
Operating expenses:		
Research and development	$ 51,269	$ 40,415
General and administrative	18,685	13,207
Total operating expenses	69,954	53,622
Loss from operations	(69,954)	(53,622)
Other income, net	1,664	865
Net loss	$ (68,290)	$ (52,757)
Net loss per share, basic and diluted	$ (1.63)	$ (1.35)
Shares used in computing net loss per share, basic and diluted	41,841,612	38,970,151

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

	March 31, 2017	December 31, 2016
Balance Sheet Data:		
Cash, cash equivalents and investments	$ 506,052	$ 498,111
Working capital	392,930	341,436
Total assets	545,822	540,626

Total stockholders' equity	488,478	473,974

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