May 9, 2016

Ultragenyx Reports First Quarter 2016 Financial Results and Corporate Update

NOVATO, Calif., May 09, 2016 (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, 2016.

"We are continuing to make progress with our pipeline, including our recent interim data from the Phase 2 study of KRN23 showing serum phosphorus increases and a preliminary look on bone health in patients with tumor-induced osteomalacia," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "As a company we are gratified by the opportunity to advance a first ever treatment for a severe disease like TIO, and we expect clinical data or regulatory milestones from all of our programs in the next 12 months."

First Quarter 2016 Financial Results

For the first quarter of 2016, Ultragenyx reported a net loss of $52.8 million, or $1.35 per share, basic and diluted, compared with a net loss for the first quarter of 2015 of $21.4 million, or $0.63 per share, basic and diluted. This reflected cash used in operations of $44.9 million in the first quarter of 2016 compared to $17.7 million in the first quarter of 2015.

Total operating expenses for the first quarter of 2016 were $53.6 million compared with $21.5 million for the same period in 2015. Non-cash stock-based compensation accounted for $10.2 million and $2.4 million of total operating expenses in the first quarter of 2016 and 2015, 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 $487.8 million as of March 31, 2016.

Recent Highlights & Upcoming Milestones

KRN23 anti-FGF23 Monoclonal Antibody in X-Linked Hypophosphatemia (XLH) and Tumor-Induced Osteomalacia (TIO)

- **Positive interim data from Phase 2 study in tumor-induced osteomalacia (TIO).** Initial data from the first eight patients in the study demonstrated that KRN23 improved serum phosphorus levels and other bone metabolism measures. After KRN23 treatment began, six of the eight patients achieved normalization of their serum phosphorus levels. The dose continues to be titrated up in one of two patients whose serum phosphorus levels increased but had not yet entered the normal range. The two patients who completed 24 weeks of treatment showed an improvement in bone mineral density, and one of these patients showed early evidence of fracture resolution, determined via bone scan. Safety data were consistent with other studies of KRN23 in XLH patients. Additional bone data will be available in the second half of 2016.

- **40-week data in 52 patients and 64-week data in 36 patients in the Phase 2 pediatric XLH study expected in the second half of 2016.** Safety and efficacy data, including rickets scores (RSS and RGI-C), from 52 patients at 40 weeks in the pediatric Phase 2 study will be available. In addition, 64 week data from 36 patients, including data on height growth velocity, are expected in the second half of 2016. Ultragenyx and Kyowa Hakko Kirin plan to file for conditional marketing authorization in Europe around the end of 2016.

- **Phase 3 study in pediatric XLH patients expected to initiate in mid-2016.** The study will utilize RGI-C as the primary endpoint and will include a standard of care reference arm. This study is expected to be required for potential approval in the US, and could also serve as a confirmatory study in the EU if conditional marketing authorization is granted. The study could be required for approval in the EU if conditional marketing authorization is not granted.

rhGUS in Mucopolysaccharidosis 7 (MPS 7)

- **Phase 3 study data expected in mid-2016.** The pivotal blinded placebo-controlled 48-week study is fully enrolled, and data are expected in mid-2016. Outside of the US, the primary endpoint is the percent reduction in urinary glycosaminoglycans (GAG) excretion after 24 weeks of treatment. In the US, there is no primary endpoint declared;

the Food and Drug Administration (FDA) will consider the totality of data on a per-patient basis.

- **Patients continue to be treated in the Phase 2 study of patients under five years of age and on an expanded access basis.** Multiple patients with MPS 7, including some with non-immune hydrops fetalis, a severe infantile presentation of the disease, continue to receive rhGUS treatment via these studies.

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

- **Data at 78 weeks from the Phase 2 study in LC-FAOD expected in the second half of 2016.** Data will include a comparison of major medical event rates approximately 18 months before and after UX007 treatment.

- **Phase 3 study in LC-FAOD patients expected to initiate in 2017.** Based on the interim 24-week results, Ultragenyx continues to plan for a Phase 3 study in LC-FAOD. The Phase 3 trial design and endpoints continue to be optimized prior to discussion with regulators. Further details are expected to be provided after discussions with regulatory authorities.

- **Phase 2 seizure study data in Glut1 DS patients expected in the second half of 2016.** The placebo-controlled Phase 2 study in Glut1 DS patients with seizures continues to enroll patients. The study is evaluating frequency of generalized and partial tonic-clonic seizures by patient diary, absence seizures by EEG, and cognitive function.

- **Phase 3 movement disorder study in Glut1 DS patients expected to initiate in the second half of 2016.** The study is expected to enroll approximately 40 patients and be a randomized, double-blind, placebo-controlled, double cross-over study. The study is designed to assess the impact of UX007 on movement disorder events as recorded by a patient diary. Ultragenyx continues to have discussions with the FDA regarding the primary endpoint. The company is working on further substantiating the clinical meaningfulness of Glut1 DS movement disorder events prior to finalizing the study design.

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

- **CHMP opinion on conditional marketing authorization application in Europe expected in the second half of 2016.** The company is seeking conditional marketing authorization from the European Medicines Agency (EMA) for Ace-ER in the treatment of adults with GNE myopathy based on positive data from the Phase 2 randomized, double-blind, placebo-controlled study. In October 2015 we announced the filing and acceptance of an MAA seeking conditional marketing authorization from the EMA for the use of six grams per day of Ace-ER tablets in the treatment of GNE myopathy.

- **Enrollment in pivotal Phase 3 study in GNE Myopathy ongoing.** The randomized, double-blind, placebo-controlled international 48-week study in approximately 80 patients is evaluating the efficacy of Ace-ER with the primary endpoint being a composite of upper extremity muscle strength. Data from the study are expected in 2017.

Corporate Updates

- **Research collaboration established with Saint Louis University (SLU) for facioscapulohumeral muscular dystrophy (FSHD).** Ultragenyx entered into three-year sponsored research and option agreements with SLU's Center for World Health and Medicine to collaborate on the development of small molecule therapeutics for the potential treatment of FSHD. Ultragenyx has an exclusive option to exclusively license existing and future intellectual property arising from the collaboration.

- **New board member appointed.** In March 2016, Ultragenyx appointed Lars Ekman, M.D., Ph.D., to its board of directors. Dr. Ekman is an Executive Partner at Sofinnova Ventures.

Conference Call & Webcast Information

Ultragenyx will host a conference call today, Monday, May 9, 2016 at 5pm ET to discuss first quarter 2016 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 95581110. 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.

Ultragenyx is currently conducting a Phase 3 study of aceneuramic acid extended-release (Ace-ER) in patients with GNE myopathy, a progressive muscle-wasting disorder; a Phase 3 study of recombinant human beta-glucuronidase (rhGUS) in patients with mucopolysaccharidosis 7 (MPS 7), a rare lysosomal storage disease; a Phase 2 clinical study for UX007 in patients with glucose transporter type-1 deficiency syndrome (Glut1 DS), a brain energy deficiency; 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; and Phase 2 and Phase 3 studies of KRN23, an antibody targeting fibroblast growth factor 23 (FGF23), in patients with X-linked hypophosphatemia (XLH) and tumor induced osteomalacia (TIO), both rare diseases that impair bone mineralization.

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 the timing of release of additional data for its product candidates, plans to initiate additional studies for its product candidates and timing regarding these studies, plans regarding ongoing studies for existing programs, its intent to file for conditional approval and its expectations regarding timing of receiving potential approval of its product candidates, 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 (including with respect to the MAA we filed seeking conditional approval from EMA with respect to Ace-ER), 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 26, 2016, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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

	Three Months Ended March 31,	
	2016	**2015**
Statements of Operations Data:		
Operating expenses:		
Research and development	$ 40,415	$ 17,364
General and administrative	13,207	4,138
Total operating expenses	53,622	21,502
Loss from operations	(53,622)	(21,502)
Other income, net	865	123
Net loss	$ (52,757)	$ (21,379)
Net loss per share, basic and diluted	$ (1.35)	$ (0.63)
Shares used to compute net loss per share, basic and diluted	38,970,151	34,008,830

Ultragenyx Pharmaceutical Inc.

Selected Balance Sheets Financial Data
(in thousands)
(unaudited)

	March 31, 2016	December 31, 2015
Balance Sheet Data:		
Cash, cash equivalents and investments	$ 487,787	$ 536,256
Working capital	400,354	422,289
Total assets	524,184	559,569
Total stockholders' equity	489,824	531,090

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