November 10, 2014

Ultragenyx Reports Third Quarter 2014 Financial Results and Corporate Update

NOVATO, Calif., Nov. 10, 2014 (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 third quarter ended September 30, 2014.

"In the third quarter, we reported positive clinical data in three programs, including long-term data for KRN23 in adults with XLH and for SA-ER in HIBM patients, as well as 12-week data for rhGUS in MPS 7," said Emil D. Kakkis, Ph.D., M.D., Chief Executive Officer and President of Ultragenyx. "We are making great progress with our pipeline and look forward to initiating later-stage development in multiple programs in 2014 and 2015."

Third Quarter 2014 Financial Results

For the third quarter of 2014, Ultragenyx reported a net loss attributable to common stockholders of $15.8 million, or $0.50 per share, basic and diluted, compared with a net loss attributable to common stockholders for the third quarter of 2013 of $12.6 million, or $3.48 per share, basic and diluted. For the nine months ended September 30, 2014, net loss attributable to common stockholders was $47.9 million, or $1.73 per share, basic and diluted, compared with a net loss attributable to common stockholders for the same period in 2013 of $31.6 million, or $9.70 per share, basic and diluted. Net loss attributable to common stockholders differs from net loss due to dividends and other charges related to outstanding preferred stock, which was converted into common stock upon the company's initial public offering.

Total operating expenses for the third quarter of 2014 were $15.8 million compared with $7.8 million for the same period in 2013. Total operating expenses for the nine months ended September 30, 2014 were $39.8 million compared with $22.8 million for the same period in 2013. The increase in total operating expenses is due to the initiation of new programs for glucose transporter type-1 deficiency syndrome (Glut1 DS) and X-linked hypophosphatemia (XLH), as well as the initiation of the Phase 2 clinical study of triheptanoin in fatty acid oxidation disorders (FAOD), the continued advancement of the recombinant human beta-glucuronidase (rhGUS) and sialic acid-extended release (SA-ER) programs, higher general and administrative costs related to being a public company, and increased headcount.

Cash, cash equivalents, and short-term investments were $201.2 million as of September 30, 2014. Based on the current operating plan, the company expects that its cash, cash equivalents, and short-term investments will be sufficient to fund operations through 2016.

Recent Highlights

Finance Update

- In July 2014, the company received net proceeds of $60.2 million from a public offering of common stock.

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

- **EU Orphan Drug Designation granted**. In October 2014, Ultragenyx announced that the European Commission had granted orphan drug designation for KRN23 for the treatment of XLH. KRN23 is being developed under a license and collaboration agreement between Ultragenyx and Kyowa Hakko Kirin., Ltd (KHK).

- **Positive 16-month Adult Phase 1/2 data released**. Data from the long-term Phase 1/2 study of KRN23, conducted by KHK, in adult patients with XLH were presented in September 2014. Improvements were generally observed over approximately 16 months in serum phosphate levels, renal tubular reabsorption of phosphate, serum vitamin D levels, and markers of bone remodeling. The most common treatment-related adverse events were injection-site reaction, arthralgia, and gastrointestinal discomfort. No anti-KRN23 antibodies were detected.

- **Pediatric Phase 2 enrollmenttarget achieved ahead of schedule.** In November 2014, Ultragenyx reached its target enrollment of 30 patients in the Phase 2 study of KRN23 in pediatric patients with XLH. The study is evaluating the dose, dosing regimen, and safety of KRN23 as well as radiographic assessments, growth, muscle strength, and motor function.

The company expects that interim data from this study will be available in 2015.

rhGUS in Mucopolysaccharidosis 7 (MPS 7)

- **Positive 12-week Phase 1/2 data released**. Interim data from the Phase 1/2 study of rhGUS in three patients with MPS 7 were presented in September 2014. Results from the 12-week primary analysis phase showed a decline in urinary GAG excretion and reduction in excess liver size in the two patients with enlarged livers at baseline. No serious adverse events were observed in up to 28 total weeks of treatment. The most common adverse events reported to date were infections and gastrointestinal disorders. No infusion-associated reactions were observed after a total of 38 infusions to date in these three subjects.

- **Phase 3 initiation upcoming**. Based on the Phase 1/2 results, Ultragenyx intends to initiate a pivotal Phase 3 study in the coming months.

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

- **Glut1 DS US Orphan Drug Designation granted**. In October 2014, Ultragenyx announced that the FDA had granted orphan drug designation for triheptanoin for the treatment of Glut1 DS.

- **Glut1 DS Phase 2 study enrollment ongoing**. Based on recently published results and in order to potentially accelerate enrollment, the company is planning to amend the inclusion criteria to also enroll patients with only absence seizures. The company expects to release interim data from this study in 2015 and, based on investigator feedback, continues to explore additional clinical studies of triheptanoin in patients on the ketogenic diet or who have additional disease manifestations.

- **LC-FAOD Phase 2 study enrollment continues**. The company expects that interim data from this study should be available in 2015.

- **Investigator-sponsored trials ongoing**. The company continues to support multiple investigator-sponsored clinical studies testing triheptanoin in FAOD, Glut1 DS, and a variety of other potential indications.

Sialic Acid Extended-Release (SA-ER) in Hereditary Inclusion Body Myopathy (HIBM)

- **Positive 2-year Phase 2 extension study data released.** Interim data from the Phase 2 extension study of SA-ER in HIBM were presented in October 2014. Over approximately two years, treatment with SA-ER appeared to slow the progression of upper extremity disease when compared to the 24-week placebo group extrapolated to two years. The 12-gram dose of SA-ER did not appear to demonstrate any clinically meaningful advantage over the 6-gram dose. The rate of mild to moderate gastrointestinal adverse events did appear to be greater with the higher dose.

- **Agreement reached with FDA on primary endpoint for Phase 3**. The company recently completed an End-of-Phase 2 meeting with the FDA regarding a potential randomized placebo-controlled 48-week single pivotal study of SA-ER in HIBM patients. The FDA agreed with the proposed Phase 3 study design, including the primary endpoint of a composite of upper extremity muscle strength, with supportive secondary endpoint data from a patient-reported outcome such as the GNEM-FAS (GNE myopathy-functional activity scale), both of which were studied in the Phase 2 trial. The company plans to initiate the Phase 3 trial by mid-2015.

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.

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 sufficiency of existing cash, cash equivalents and short-term investments to fund

operations for projected periods of time, timing of release of additional data for its product candidates, timing of initiation of additional studies for its product candidates, timing of commencement of treatment of patients enrolled in clinical studies and plans regarding ongoing studies for existing programs, 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, including 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 August 11, 2014, 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 September 30,		Nine Months Ended September 30,	
	2014	**2013**	**2014**	**2013**
Statements of Operations Data:				
Operating expenses:				
Research and development	$ 12,854	$ 6,762	$ 32,446	$ 19,625
General and administrative	2,981	999	7,389	3,130
Total operating expenses	15,835	7,761	39,835	22,755
Loss from operations	(15,835)	(7,761)	(39,835)	(22,755)
Other income (expense), net	(14)	(667)	(3,229)	(998)
Net loss	$ (15,849)	$ (8,428)	$ (43,064)	$ (23,753)
Net loss attributable to common stockholders	(15,849)	(12,590)	(47,872)	(31,624)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.50)	$ (3.48)	$ (1.73)	$ (9.70)
Shares used to compute net loss per share attributable to common stockholders, basic and diluted	31,631,385	3,621,654	27,697,137	3,260,484

Ultragenyx Pharmaceutical Inc.

Selected Balance Sheets Financial Data

(in thousands)

(unaudited)

	September 30, 2014	December 31, 2013
Balance Sheet Data:		
Cash, cash equivalents and short-term investments	$ 201,194	$ 53,377
Working capital	195,669	49,304
Total assets	210,931	59,649
Convertible preferred stock warrant liability	—	3,419
Convertible preferred stock	—	124,930
Accumulated deficit	(122,302)	(74,836)
Total stockholders' equity (deficit)	199,326	(74,821)

CONTACT: Ultragenyx Pharmaceutical Inc.

844-758-7273

For Media, Bee Nguyen

For Investors, Robert Anstey