August 11, 2014

Ultragenyx Reports Second Quarter 2014 Financial Results and Corporate Update

NOVATO, Calif., Aug. 11, 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 second quarter ended June 30, 2014.

"Ultragenyx has made multiple significant advances over the last few months," commented Emil D. Kakkis, Ph.D., M.D., Chief Executive Officer and President of Ultragenyx. "We reported positive Phase 1/2 adult data for KRN23 and initiated the Phase 2 pediatric study for that program. We secured new intellectual property rights for triheptanoin to extend the value of the product. We completed a follow-on offering that raised approximately $60 million for the company, and we hired a new Chief Medical Officer who will be instrumental in the continued execution of our clinical development strategy."

Second Quarter 2014 Financial Results

For the second quarter of 2014, Ultragenyx reported a net loss attributable to common stockholders of $13.6 million, or $0.45 per share, basic and diluted, compared with a net loss attributable to common stockholders for the second quarter of 2013 of $10.8 million, or $3.32 per share, basic and diluted. For the six months ended June 30, 2014, net loss attributable to common stockholders was $32.0 million, or $1.25 per share, basic and diluted, compared with a net loss attributable to common stockholders for the same period in 2013 of $19.0 million, or $6.19 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 second quarter of 2014 were $13.7 million compared with $8.2 million for the same period in 2013. Total operating expenses for the six months ended June 30, 2014 were $24.0 million compared with $15.0 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, as well as the initiation of the Phase 2 clinical study of triheptanoin in fatty acid oxidation disorders (FAOD), higher general and administrative costs related to being a public company, and increased headcount.

Cash, cash equivalents, and short-term investments were $153.3 million as of June 30, 2014. Subsequent to the end of the quarter, the company completed a public offering of 2,319,951 shares of common stock at a price to the public of $40.00 per share, which includes the exercise in full by the underwriters of their option to purchase up to 302,602 additional shares of common stock. Ultragenyx sold a total of 1,613,879 shares in the offering, and certain existing stockholders sold 706,072 shares. Net proceeds to Ultragenyx from the offering were approximately $60.2 million. 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

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

- In June 2014, results from a Phase 1/2 study with up to four escalating doses of KRN23 were presented at the 2014 ICE/ENDO joint meeting of The Endocrine Society and the International Congress on Endocrinology. The data demonstrated that repeat doses of KRN23 over four months led to an increase in serum phosphate in 100% of patients, with approximately 89% of patients reaching the low end of the normal range. Increases in bone remodeling markers of bone formation and bone resorption, as well as increases in quality of life and disability measures, were also observed in the Phase 1/2 study.
- There were no significant changes in parathyroid hormone, serum calcium, or urinary calcium excretion in the Phase 1/2 study. The most common adverse events were nasopharyngitis, joint pain, diarrhea, back pain, and restless legs syndrome. There were no serious adverse events related to treatment or renal or cardiac tissue calcification. One patient discontinued treatment due to an injection-site reaction. No anti-KRN23 antibodies were observed.
- Data from the long-term Phase 1/2 study evaluating KRN23 for an additional 12 doses are expected to be presented at the American Society of Bone and Mineral Research (ASBMR) Annual Meeting on September 14, 2014.
- In July 2014, Ultragenyx announced the initiation of a Phase 2 study evaluating the dose, dosing regimen and safety profile of KRN23 in approximately 30 pediatric patients with XLH. Preliminary clinical effects of KRN23 treatment on bone health and deformity as measured by radiographic assessments, growth, muscle strength, and motor function will also be

assessed. The company expects that interim data from this study will be available in 2015.
- Ultragenyx also announced that in a recent meeting, the Food and Drug Administration (FDA) agreed that blinded radiographic assessments of bone abnormalities and changes in growth may be used as primary endpoint measures in the pediatric development program. The FDA also indicated that a Phase 3 study in pediatric patients could be open-label, but recommended inclusion of a standard-of-care control arm for comparison on a non-inferiority basis. The final design of a pediatric Phase 3 study will be determined once sufficient safety and efficacy data are available and after further consultation with the FDA.

rhGUS in Mucopolysaccharidosis 7 (MPS 7)

- The Phase 1/2 study of rhGUS is ongoing and interim 12-week data are expected to be presented at the Society for the Study of Inborn Errors of Metabolism (SSIEM) Annual Symposium on September 3, 2014.
- If the Phase 1/2 results are supportive, Ultragenyx plans to initiate a pivotal Phase 3 study.

Triheptanoin in Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD)

- Ultragenyx continues to enroll and treat patients in the ongoing Phase 2 study of triheptanoin in severely affected LC-FAOD patients. The company expects that interim data from this study should be available in 2015.
- In August 2014, Ultragenyx announced that a United States patent had been issued with claims directed to compositions of triheptanoin above a certain level of purity. The patent term expires in October 2025 (not including potential patent term extension of up to five additional years).

Triheptanoin in Glucose Transporter Type-1 Deficiency Syndrome (Glut1 DS)

- Ultragenyx continues to enroll and treat patients in the ongoing Phase 2 study of triheptanoin in Glut1 DS patients. The company expects to release interim data from this study in 2015.
- In August 2014, Ultragenyx announced a license agreement with UniQuest Pty Limited for intellectual property rights related to the treatment of refractory epilepsy and other seizure-related and neurologic disorders with triheptanoin. The intellectual property originated from research on epilepsy and other neurological models conducted at The University of Queensland.
- The company continues to support multiple investigator-sponsored clinical studies testing triheptanoin in Glut1 DS and a variety of other potential indications.

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

- Data from the randomized, double-blind, placebo-controlled Phase 2 study of SA-ER in 47 HIBM patients were presented at the 13th International Congress on Neuromuscular Diseases (ICNMD) in July 2014.
- The data, which were previously presented at the American Academy of Neurology (AAN) Annual Meeting in April 2014, showed a statistically significant difference in an upper extremity composite of muscle strength at 48 weeks in the higher dose group compared to the lower dose group.
- SA-ER appeared to be well tolerated with no serious adverse events observed to date, and no dose-dependent treatment-emergent adverse events identified. Most adverse events were mild to moderate and most commonly gastrointestinal in nature.
- Ultragenyx continues to treat patients in an extension study evaluating a 12 gram dosage of sialic acid based on the dose dependent response observed at weeks 24 and 48 of the Phase 2 study.
- Preliminary data from the extension study are expected to be presented at the International Congress of the World Muscle Society (WMS) on October 11, 2014.

Corporate Update

- In July 2014, Ultragenyx announced the appointment of Sunil Agarwal, M.D. as Chief Medical Officer and Senior Vice President. Dr. Agarwal most recently served as Senior Vice President and Global Head of Clinical Development for Immunology and Infectious Diseases, Metabolism, Neuroscience, and Ophthalmology at Genentech.

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, design of studies for its product candidates, plans regarding ongoing studies for existing programs and potential benefits of its products under development 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 for the quarter ended March 31, 2014, filed with the Securities and Exchange Commission on May 12, 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 June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
Statements of Operations Data:				
Operating expenses:				
Research and development	$ 11,239	$ 7,199	$ 19,592	$ 12,863
General and administrative	2,422	1,048	4,408	2,131
Total operating expenses	13,661	8,247	24,000	14,994
Loss from operations	(13,661)	(8,247)	(24,000)	(14,994)
Other income (expense), net	76	(343)	(3,215)	(331)
Net loss	$ (13,585)	$ (8,590)	$ (27,215)	$ (15,325)
Net loss attributable to common stockholders	$ (13,585)	$ (10,829)	$ (32,023)	$ (19,034)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.45)	$ (3.32)	$ (1.25)	$ (6.19)
Shares used to compute net loss per share attributable to common stockholders, basic and diluted	30,055,943	3,257,806	25,697,407	3,076,907

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

	June 30, 2014	December 31, 2013
Balance Sheet Data:		
Cash, cash equivalents and short-term investments	$ 153,271	$ 53,377
Working capital	149,273	49,304
Total assets	162,586	59,649

Convertible preferred stock warrant liability	—	3,419
Convertible preferred stock	—	124,930
Accumulated deficit	(106,454)	(74,836)
Total stockholders' equity (deficit)	153,083	(74,821)

CONTACT: Ultragenyx Pharmaceutical Inc.

844-758-7273

For Media, Bee Nguyen

For Investors, Robert Anstey