August 13, 2015

Ultragenyx Reports Second Quarter 2015 Financial Results and Corporate Update

NOVATO, Calif., Aug. 13, 2015 (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, 2015.

"The second quarter included important data releases on two programs, including data from an investigator study of triheptanoin in Glut1 DS patients with movement disorder and KRN23 in pediatric XLH patients," commented Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "We expect to see continued clinical and regulatory progress this year as we report additional data on triheptanoin and KRN23, advance our Phase 3 programs for rhGUS, Ace-ER, and KRN23, and file our first MAA for Ace-ER."

Second Quarter 2015 Financial Results

For the second quarter of 2015, Ultragenyx reported a net loss attributable to common stockholders of $29.8 million, or $0.83 per share, basic and diluted, compared with a net loss attributable to common stockholders for the second quarter of 2014 of $13.6 million, or $0.45 per share, basic and diluted. For the six months ended June 30, 2015, net loss attributable to common stockholders was $51.2 million, or $1.46 per share, basic and diluted, compared with a net loss attributable to common stockholders for the same period in 2014 of $32.0 million, or $1.25 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 2015 were $30.1 million compared with $13.7 million for the same period in 2014. Non-cash stock-based compensation accounted for $5.1 million and $0.9 million of total operating expenses in the second quarter of 2015 and 2014, respectively. Total operating expenses for the six months ended June 30, 2015 were $51.6 million compared with $24.0 million for the same period in 2014. Non-cash stock-based compensation accounted for $7.5 million and $1.7 million of total operating expenses in the first half of 2015 and 2014, respectively. The increase in total operating expenses is due to the increase in clinical trial, manufacturing, other development activities, and supportive general and administrative costs as the company's pipeline continues to advance to the late stages of development.

Cash, cash equivalents, and short-term investments were $326.0 million as of June 30, 2015. Subsequent to the end of the quarter, the company completed a public offering of 2,530,000 shares of common stock at a price to the public of $120.00 per share, which includes the exercise in full by the underwriters of their option to purchase 330,000 additional shares of common stock. Net proceeds to Ultragenyx from the offering were approximately $286.6 million.

Recent Highlights & Upcoming Milestones

Corporate Update

- **Multiple additions to executive leadership team.** Ultragenyx recently announced the appointment of three new members to the company's executive leadership team. Dennis Huang was appointed Chief Technical Operations Officer, Jayson Dallas, M.D., was appointed Chief Commercial Officer, and John Pinion was appointed Chief Quality Operations Officer. Ultragenyx also announced that Daniel Welch has been elected Chairman of the Board.

- **Research & Development Day to be held in December 2015.** The company plans to hold its first Research & Development Day for analysts and investors on the afternoon of December 3, 2015 in New York. An update on the company's pipeline will be provided.

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

- **Increases in serum phosphorus in all 36 patients in pediatric Phase 2 16-week analysis.** The majority of patients in both the biweekly and monthly dosing groups reached the normal serum phosphorus range. Comparable increases were seen in TmP/GFR and Vitamin D. There were no serious adverse events or discontinuations, and the most common adverse events were injection-site related.

- **Interim analysis of 12 pediatric patients showed improvement in mean rickets score at 40 weeks.** The eleven patients with evidence of rickets at baseline as measured by the Thacher Rickets Severity Scoring method demonstrated a mean 58% reduction in rickets score. Patients in the biweekly dosing group demonstrated a mean 80% reduction in rickets score, with all five patients improving, and three showing no sign of rickets at week 40. The safety and tolerability results were consistent with the 16-week analysis in all patients.

- **40-week data in 36 pediatric patients expected in fourth quarter of 2015.** Additional data, including rickets scores, from 36 patients in the pediatric Phase 2 study are expected later in 2015. The study is also being expanded to enroll a total of up to 50 patients, and 40-week results for the fully expanded study are expected in mid-2016.

- **Potential for conditional approval pathway in Europe.** In discussions with the European Medicines Agency (EMA), the agency indicated that a filing for conditional approval for adult and pediatric patients may be possible following the pediatric Phase 2 40-week data.

- **Phase 3 program in adult XLH patients expected to start in the second half of 2015.** Ultragenyx intends to initiate a Phase 3 randomized, double-blind, placebo-controlled study in approximately 120 adult XLH patients. The primary endpoint will be serum phosphorus levels at 24 weeks, with the Brief Pain Inventory (BPI) patient-reported outcome as a key secondary endpoint. The company will also initiate a 48-week open-label bone biopsy study in approximately ten patients evaluating the effect of KRN23 on osteomalacia. Based on meetings with FDA and EMA, we believe that a combination of these data, if positive, would be acceptable to support approval.

- **Data presented at International Conference on Children's Bone Health (ICCBH) in June 2015 highlight significant disease burden in 71 pediatric XLH patients.** Commonly reported features of XLH included short stature, bowing of the legs, intoeing, functional limitations, bone and joint pain, and various quality of life questionnaire scores below those of the general population.

KRN23 anti-FGF23 Monoclonal Antibody in Tumor-Induced Osteomalacia (TIO)

- **Phase 2 study in TIO continues to enroll patients.** Interim data for the first few patients are expected in late 2015 or early 2016.

rhGUS in Mucopolysaccharidosis 7 (MPS 7)

- **Phase 3 study in MPS 7 fully enrolled.** The pivotal blinded placebo-controlled 48-week study completed enrollment of 12 patients in June 2015. The primary endpoint is to determine efficacy of rhGUS as determined by reduction in urinary glycosaminoglycans (GAG) excretion after 24 weeks of treatment. Safety and efficacy data from the study are expected to be released in mid-2016.

- **First patient enrolled in Phase 2 study in patients under five years old with MPS 7.** In August 2015, Ultragenyx initiated a study in up to seven pediatric patients under five years of age, potentially including patients with non-immune hydrops fetalis, a severe infantile presentation of the disease. Interim data are expected by the end of 2016.

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

- **Data from compassionate use of triheptanoin in infants with cardiomyopathy due to LC-FAOD to be presented in September 2015.** Case reports from five patients with moderate or severe life-threatening cardiomyopathy in early infancy treated with triheptanoin will be presented at the Society for the Study of Inborn Errors of Metabolism (SSIEM) Annual Symposium in France.

- **Phase 2 data from the company-sponsored triheptanoin study in LC-FAOD are expected in the second half of 2015.** The study is a "learning" study and will evaluate triheptanoin in different subsets of patients with skeletal muscle, liver/hypoglycemia, and cardiac disease, to help define the right patient subsets and clinical endpoints for confirmation in a Phase 3 study.

- **An interim analysis from the Phase 2 Glut1 DS seizure study is expected around the end of 2015 or early 2016.** This placebo-controlled study is currently enrolling patients and is designed to evaluate generalized and partial tonic-clonic seizures by patient diary, absence seizures by EEG, and cognitive function.

- **Planning continues for clinical study of triheptanoin in Glut1 DS movement disorders.** Discussions with the FDA regarding a randomized controlled study of triheptanoin in Glut1 DS patients with movement disorders are expected in the second half of 2015. The planned study follows positive data from an investigator-sponsored trial in this phenotype presented at the American Academy of Neurology annual meeting in April 2015.

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

- **Pivotal Phase 3 study in GNE Myopathy initiated in May 2015.** The randomized, double-blind, placebo-controlled 48-week study in approximately 80 patients is evaluating the efficacy of Ace-ER with the primary endpoint of a composite of upper extremity muscle strength. Data from the study are expected in late 2016 or early 2017. Enrollment is ongoing internationally.

- **MAA filing in Europe is expected the second half of 2015.** The MAA filing will seek conditional marketing authorization by the EMA based on feedback to date from the authorities.

- **Collaboration and License Agreement with Nobelpharma amended.** In August 2015, Ultragenyx entered into an amendment to the agreement that reduced the effective royalty rate payable to Nobelpharma from the high-single digits to mid-single digits based on a mutual reassessment of the partnership since initiation in 2010, and included other clarifications regarding the supply of sialic acid drug substance.

- **Aceneuramic acid is the United States Adopted Name (USAN) and International Nonproprietary Name (INN) for sialic acid.** GNE Myopathy is the new name for Hereditary Inclusion Body Myopathy (HIBM) agreed upon by patients and physicians in the disease community.

Conference Call & Webcast Information

Ultragenyx will host a conference call today, Thursday, August 13, 2015 at 5pm ET to discuss second quarter 2015 financial results and 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 94596921. 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.

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 and intent to file for conditional approval, 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 May 12, 2015, 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,	
	2015	2014	2015	2014
Statements of Operations Data:				
Operating expenses:				
Research and development	$ 23,104	$ 11,239	$ 40,468	$ 19,592
General and administrative	7,038	2,422	11,176	4,408
Total operating expenses	30,142	13,661	51,644	24,000
Loss from operations	(30,142)	(13,661)	$ (51,644)	$ (24,000)
Other income (expense)	355	76	478	(3,215)
Net loss	$ (29,787)	$ (13,585)	$ (51,166)	$ (27,215)
Net loss attributable to common stockholders	$ (29,787)	$ (13,585)	$ (51,166)	$ (32,023)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.83)	$ (0.45)	$ (1.46)	$ (1.25)
Shares used to compute net loss per share attributable to common stockholders, basic and diluted	35,937,442	30,055,943	34,977,498	25,697,407

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Selected Balance Sheets Financial Data

(in thousands)

(unaudited)

	June 30, 2015	December 31, 2014
Balance Sheet Data:		
Cash, cash equivalents and short-term investments	$ 326,029	$ 187,487
Working capital	313,488	180,899
Total assets	343,004	197,967
Total stockholders' equity	319,278	184,945

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Investors & Media

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