March 25, 2015

Ultragenyx Reports Fourth Quarter and Full-Year 2014 Financial Results and Corporate Update

NOVATO, Calif., March 25, 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 fourth quarter and full-year ended December 31, 2014.

"This past year was transformative for Ultragenyx in our mission to advance novel therapeutics for patients with rare and ultra-rare diseases," said Emil D. Kakkis, Ph.D., M.D., Chief Executive Officer and President of Ultragenyx. "The completion of our initial public offering early in 2014 has provided us with the capital to build the development capabilities necessary to rapidly advance our pipeline into the late stages of clinical trials. We are now in or entering Phase 3 for two of our six clinical programs, and we expect to see Phase 2 data for all four of the others in 2015 or early 2016. In tandem with that development progress, we will also now begin to build out our sales and marketing capabilities to support our transition to a commercial rare disease company and enhance our earlier stage translational research to drive future pipeline growth."

Fourth Quarter and Full-Year 2014 Financial Results

For the fourth quarter of 2014, Ultragenyx reported a net loss attributable to common stockholders of $16.7 million, or $0.52 per share, basic and diluted, compared with a net loss attributable to common stockholders for the fourth quarter of 2013 of $18.7 million, or $4.98 per share, basic and diluted.

For the year ended December 31, 2014, Ultragenyx reported a net loss attributable to common stockholders of $64.6 million, or $2.25 per share, basic and diluted, compared with a net loss attributable to common stockholders for the year ended December 31, 2013 of $50.3 million, or $14.87 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 2014 were $56.8 million compared with $32.3 million for 2013. Total operating expenses for the fourth quarter of 2014 were $16.9 million compared with $9.5 million for the same period in 2013. The increase in total operating expenses is due to the increase in clinical trial, manufacturing, and other development activities for the company's clinical development pipeline, in addition to increased headcount in support of both research and development and general and administration activities.

Cash, cash equivalents, and short-term investments were $187.5 million as of December 31, 2014. Based on current operating levels, the company expects that existing cash, cash equivalents and short-term investments, including $174.5 million in net proceeds received from the underwritten public offering in February 2015, will be sufficient to fund operations into 2018.

Recent Highlights & Upcoming Milestones

Corporate Update

- **New board member appointed.** In February 2015, Ultragenyx appointed Michael Narachi, the President, Chief Executive Officer, and a member of the board of directors of Orexigen Therapeutics, Inc., to its board of directors.

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

- **Pediatric Phase 2 16-week phosphate and safety data in 36 patients expected in first half of 2015.** Enrollment in the Phase 2 study of KRN23 in pediatric patients completed ahead of schedule and exceeded the target number of patients. Pediatric Phase 2 40-week data, including radiographic assessments of rickets, are expected in late 2015 or early 2016.

- **Data presented at ENDO highlight significant disease burden in 165 adult XLH patients.** Commonly reported features of XLH included short stature, motion and gait issues, joint stiffness, bone and joint pain, fractures, extraosseous

calcification, and quality of life questionnaire scores below the general population. The majority of patients (approximately 70%) reported taking medications for their underlying disease or its complications.

- **Initiation of an adult randomized, placebo-controlled study expected in second half of 2015.** Details of the trial design are currently being finalized.

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

- **Interim Phase 2 data expected late 2015 or early 2016.** In January 2015, Ultragenyx announced a new program for KRN23 targeting TIO, a disease characterized by benign tumors that produce excess levels of fibroblast growth factor-23 (FGF23), which can lead to a severe XLH-like disease. The Phase 2 study of KRN23 in TIO is currently enrolling patients.

rhGUS in Mucopolysaccharidosis 7 (MPS 7)

- **Data from 12-patient pivotal Phase 3 study in MPS 7 expected in 2016.** In December 2014, the company enrolled the first patient in its pivotal Phase 3 study of 4 mg/kg of rhGUS every other week. Enrollment is ongoing.

- **Agreement reached with FDA and EMA for single pivotal Phase 3 study design.** The US Food and Drug Administration (FDA) stated that their evaluation of the pivotal Phase 3 study will be based on the totality of the data on a patient-by-patient basis and advised against the declaration of a primary clinical endpoint in order to allow for more flexibility in the overall efficacy evaluation. The European Medicines Agency (EMA) has agreed that approval under exceptional circumstances could be possible based on urinary glycosaminoglycans (GAG) levels as a surrogate primary endpoint, provided the data are strongly supportive of a favorable benefit/risk ratio and that some evidence or trend in improvement in clinical endpoints is observed.

- **Positive Phase 1/2 36-week data reported at WORLD Symposium.** Updated long-term data from the study indicated that the 4 mg/kg dose of rhGUS led to the greatest reduction in urinary GAG excretion, with a mean urinary GAG reduction of approximately 60%. Sustained decreases in liver size occurred in the two patients who had enlarged livers at baseline, and an improvement in pulmonary function was observed in the one patient who was able to perform the evaluations. No serious adverse events or infusion-associated reactions were observed in the study. The most common adverse events were consistent with the symptoms of MPS 7 or related to intravenous administration of the investigational therapy.

- **Positive data published on patient receiving rhGUS under emergency IND.** Data after 24 weeks of treatment of a single patient being treated with rhGUS under an emergency investigational new drug (eIND) application were published in *Molecular Genetics and Metabolism* in October 2014. The data showed a reduction in urinary GAG of more than 50%, a reduction in liver and spleen size to normal levels, and an improvement in pulmonary function. No serious adverse events or infusion-associated reactions were observed. Patients continue to be treated under eINDs.

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

- **Interim data from Phase 2 study in Glut1 DS anticipated in the second half of 2015.** The inclusion criteria for the study were recently broadened to allow for patients suffering only from absence seizures, which has led to a higher rate of study enrollment.

- **Interim data from Phase 2 study in FAOD expected in second half of 2015.** The last patient in the ongoing study of triheptanoin in FAOD was enrolled in December 2014. Interim data focused on acute effects of the disease, particularly related to exercise tolerance, are expected later this year.

- **License agreement for intellectual property in Huntington's disease executed.** A license agreement with Inserm Transfert SA and Institut du Cerveau et de la Moelle Epiniere (ICM) was entered into based on a pilot clinical study in ten patients with early-stage Huntington's disease. Triheptanoin appeared to impact brain energy metabolism and the Unified Huntington's Disease Rating Scale motor score. Ultragenyx is supporting a second investigator-sponsored clinical study being planned by ICM in Huntington's disease.

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

- **Filing for conditional approval in Europe planned in the second half of 2015.** Based on Scientific Advice received from the EMA, the company intends to file for conditional marketing authorization for stabilization or slowing of decline in upper extremity muscle strength.

- **Phase 3 study design accepted by FDA and EMA with initiation expected in mid-2015.** Ultragenyx intends to initiate a global, randomized, double-blind, placebo-controlled Phase 3 study of six grams per day of SA-ER in 80 patients with HIBM in mid-2015. A composite of upper extremity muscle strength will be the primary endpoint. Key secondary

endpoints include GNE myopathy-functional activity scale (GNEM-FAS) (including patient-reported outcome scores of mobility and upper extremity function), and several measures of lower extremity muscle strength including the lower extremity muscle strength composite (LEC). Successful completion of the single Phase 3 study is expected to be sufficient for full approval of SA-ER by the FDA and EMA.

- **First US patent issued for SA-ER in HIBM.** In February 2015, Ultragenyx received a notice of allowance for a US patent application with claims directed to methods of treating HIBM by orally administering free sialic acid. The patent will expire no earlier than 2028 and may expire later after patent term adjustment is provided by the US Patent and Trademark Office.

Conference Call & Webcast Information

Ultragenyx will host a conference call today, Wednesday, March 25, 2015 at 5pm ET, to discuss the fourth quarter and full-year 2014 financial results and 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 12430845. 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 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, plans with respect to supporting investigator-sponsored studies, plans regarding ongoing studies for existing programs, intent to file for conditional approval and potential for patent-term extension, 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 November 10, 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 December 31,		Year Ended December 31,	
	2014	**2013**	**2014**	**2013**
Statements of Operations Data:				
Operating expenses:				

Research and development	$ 13,521	$ 8,204	$ 45,967	$ 27,829
General and administrative	3,422	1,321	10,811	4,451
Total operating expenses	16,943	9,525	56,778	32,280
Loss from operations	(16,943)	(9,525)	(56,778)	(32,280)
Other income (expense), net	205	(1,792)	(3,024)	(2,790)
Net loss	$ (16,738)	$ (11,317)	$ (59,802)	$ (35,070)
Net loss attributable to common stockholders	$ (16,738)	$ (18,665)	$ (64,610)	$ (50,289)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.52)	$ (4.98)	$ (2.25)	$ (14.87)
Shares used to compute net loss per share attributable to common stockholders, basic and diluted	31,897,138	(3,744,525)	28,755,758	3,382,489

Ultragenyx Pharmaceutical Inc.

Selected Balance Sheets Financial Data

(in thousands)

(unaudited)

	December 31, 2014	December 31, 2013
Balance Sheet Data:		
Cash, cash equivalents and short-term investments	$ 187,487	$ 53,377
Working capital	180,899	49,304
Total assets	197,967	59,649
Convertible preferred stock warrant liability	—	3,419
Convertible preferred stock	—	124,930
Total stockholders' equity (deficit)	184,945	(74,821)

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Investors & Media

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