November 7, 2016

Ultragenyx Reports Third Quarter 2016 Financial Results and Corporate Update

NOVATO, Calif., Nov. 07, 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 September 30, 2016.

"Based on recent discussions with the FDA, we now plan to file for US approval of KRN23 in the second half of 2017," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "We expect continued clinical and regulatory progress across all of our programs, including filings for rhGUS and the European filing for KRN23."

Third Quarter 2016 Financial Results

For the third quarter of 2016, Ultragenyx reported a net loss of $64.9 million, or $1.64 per share, basic and diluted, compared with a net loss for the third quarter of 2015 of $39.2 million, or $1.03 per share, basic and diluted. For the nine months ended September 30, 2016, net loss was $174.6 million, or $4.46 per share, basic and diluted, compared with a net loss for the same period in 2015 of $90.4 million, or $2.51 per share, basic and diluted. This reflected cash used in operations of $113.3 million for the nine months ended September 30, 2016 compared to $65.5 million for the same period in 2015.

Total operating expenses for the third quarter of 2016 were $65.9 million compared with $39.9 million for the same period in 2015, including non-cash stock-based compensation of $13.7 million and $7.9 million in the third quarter of 2016 and 2015, respectively. Total operating expenses for the nine months ended September 30, 2016 were $177.6 million compared with $91.6 million for the same period in 2015, including non-cash stock-based compensation of $34.8 million and $15.4 million in the first nine months 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 $472.6 million as of September 30, 2016.

Recent Highlights

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

- **Ultragenyx now plans to submit a biologics license application (BLA) to the US FDA for KRN23 in the second half of 2017.** Based on discussions with the FDA, the pediatric Phase 3 study is currently not expected to be required for a US filing. The company continues to discuss the details of the planned submission with FDA. In June, FDA granted breakthrough therapy designation to KRN23 for the treatment of X-linked hypophosphatemia (XLH) in pediatric patients one year of age and older.
- **Phase 3 study in pediatric XLH patients initiated.** The randomized, open-label clinical study will enroll approximately 60 patients and will compare the efficacy and safety of KRN23 compared to oral phosphate and active vitamin D therapy. The study will evaluate changes in rickets, growth velocity and height, pharmacodynamic assessments, walking ability, patient reported outcomes assessing pain, fatigue and physical function, and safety.
- **Positive interim data from the ongoing Phase 2 XLH and TIO studies of KRN23 were presented at ASBMR.** Data from the pediatric XLH study demonstrated that serum phosphorus levels, rickets, growth rates and other functional outcomes improved with continued KRN23 treatment. Data from the adult XLH study demonstrated a significant increase in serum phosphorus levels and evidence of clinical improvement in walking, mobility, pain and stiffness at 24 weeks of treatment. In patients with TIO, KRN23 improved serum phosphorus levels and bone metabolism measures at 24 weeks in the first eight patients enrolled in the study. Adverse events were consistent with what has been previously observed for KRN23 for the treatment of XLH and TIO.

rhGUS in MPS 7

- **In October, Ultragenyx was issued a composition of matter patent and a method of treatment patent in the US.** These patents are expected to expire in 2035 without any patent term extension.

Corporate Updates

- As part of the strategic collaboration with Takeda Pharmaceutical Company Limited, Ultragenyx exercised its option in October whereby Takeda made a second equity purchase of 352,530 shares of the Ultragenyx common stock for $25 million.

Upcoming Milestones

KRN23 in XLH

- **Ultragenyx and Kyowa Hakko Kirin plan to file for conditional marketing authorization in the European Union for XLH around the end of 2016.**
- **Data from the Phase 3 study in adult XLH patients expected in the first half of 2017.** The fully-enrolled Phase 3 study will evaluate change in serum phosphorus levels, pain, stiffness, physical function, and safety of monthly KRN23 compared with placebo over 24 weeks in 134 adult XLH patients.

rhGUS in MPS 7

- **Ultragenyx has met with the FDA and the EMA and plans to submit regulatory filings in the first half of 2017, based on Phase 3 study results**. In Europe, the primary endpoint is the percent reduction in urinary glycosaminoglycans (GAG) excretion after 24 weeks of treatment. The European Medicines Agency (EMA) has indicated that some evidence or trend in improvement in clinical endpoints would also be necessary for approval. In the US, there is no primary endpoint declared; the FDA will consider the totality of data on a per-patient basis.

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 2016.** Data will include a comparison of major medical event rates approximately 18 months before and after UX007 treatment. Long-term safety and exercise tolerance data will also be provided.
- **Phase 3 movement disorder study in Glut1 DS patients expected to initiate by the end 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.
- **Phase 2 seizure study data in Glut1 DS patients expected in the first quarter of 2017.** The ongoing placebo-controlled study is evaluating frequency of generalized and partial tonic-clonic seizures by patient diary, absence seizures by EEG, and cognitive function. The last patient visit has taken place and the data are being prepared for analysis.

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

- **CHMP opinion on conditional marketing authorization application in Europe expected by the end of 2016, and a decision is expected in the first half of 2017.** The company is seeking conditional marketing authorization from the 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.
- **Data from the pivotal Phase 3 study in GNE myopathy expected in the second half of 2017.** The fully-enrolled randomized, double-blind, placebo-controlled international study in 89 patients is evaluating the efficacy and safety of Ace-ER compared with placebo over 48 weeks.

Conference Call & Webcast Information

Ultragenyx will host a conference call today, Monday, November 7, 2016 at 5pm ET to discuss third 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 7847134. 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 has completed a Phase 3 study of recombinant human beta-glucuronidase (rhGUS) in patients with mucopolysaccharidosis 7 (MPS 7), a rare lysosomal storage disease, and is conducting a Phase 3 study of aceneuramic acid extended-release (Ace-ER) in patients with GNE myopathy, a progressive muscle-wasting disorder; a Phase 2 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 pediatric and adult 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, expectations regarding the adequacy of clinical data to support approval of product candidates, 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), whether the Phase 3 results for Ace-ER will in fact confirm or mirror the results from the prior Phase 2 study, whether the FDA will accept the planned BLA submission for KRN23, 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 9, 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 September 30,		Nine Months Ended September 30,	
	2016	**2015**	**2016**	**2015**
Statements of Operations Data:				
Revenue	$ 111	$ -	$ 128	$ -
Operating expenses:				
Research and development	48,711	29,704	132,458	70,172
General and administrative	17,183	10,232	45,128	21,408
Total operating expenses	65,894	39,936	177,586	91,580
Loss from operations	(65,783)	(39,936)	(177,458)	(91,580)
Other income, net	876	704	2,871	1,182
Net loss	$ (64,907)	$ (39,232)	$ (174,587)	$ (90,398)
Net loss per share, basic and diluted	$ (1.64)	$ (1.03)	$ (4.46)	$ (2.51)
Shares used in computing net loss per share, basic and diluted	39,551,923	38,268,632	39,184,994	36,086,598

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

	September 30, 2016	December 31, 2015
Balance Sheet Data:		
Cash, cash equivalents and investments	$ 472,641	$ 536,256
Working capital	366,354	422,289
Total assets	515,166	559,569
Total stockholders' equity	455,890	531,090

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