February 16, 2017

Ultragenyx Reports Fourth Quarter and Full Year 2016 Financial Results and Corporate Update

NOVATO, Calif., Feb. 16, 2017 (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 and full year ended December 31, 2016.

"In 2016 we advanced each program in our clinical pipeline and continued to build our earlier stage pipeline through two new partnerships and ongoing progress with our translational research program," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "In 2017 we expect to drive two products through the regulatory process with two more in Phase 3 studies."

Fourth Quarter and Full Year 2016 Financial Results

For the fourth quarter of 2016, Ultragenyx reported a net loss of $71.3 million, or $1.75 per share, basic and diluted, compared with a net loss for the fourth quarter of 2015 of $55.2 million, or $1.42 per share, basic and diluted. For the year ended December 31, 2016, net loss was $245.9 million, or $6.21 per share, basic and diluted, compared with a net loss for the same period in 2015 of $145.6 million, or $3.96 per share, basic and diluted. This reflected cash used in operations of $161.0 million for the year ended December 31, 2016 compared to $106.0 million for the same period in 2015.

Total operating expenses for the fourth quarter of 2016 were $70.6 million compared with $56.2 million for the same period in 2015, including non-cash stock-based compensation of $13.5 million and $9.5 million in the fourth quarter of 2016 and 2015, respectively. Total operating expenses for the year ended December 31, 2016 were $248.1 million compared with $147.7 million for the same period in 2015, including non-cash stock-based compensation of $48.3 million and $24.9 million in 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 $498.1 million as of December 31, 2016.

Recent Highlights

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

- **KRN23 Conditional Marketing Authorization Application (MAA) for XLH filed and accepted by the European Medicines Agency (EMA) in December 2016.** An opinion from the Committee for Medicinal Products for Human Use is expected in the second half of 2017.

UX007 in Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD)

- **Positive 78-week data from the Phase 2 study in LC-FAOD showed a reduction in frequency and total duration of major medical events.** We continue to further develop the Phase 3 study design and endpoints before meeting with regulators and initiating the study in 2017.

Upcoming Key Milestones

KRN23 in XLH

- **Data from the Phase 3 study in adult XLH patients expected in the first half of 2017.** The fully-enrolled Phase 3 study of monthly KRN23 compared with placebo over 24 weeks in 134 adult XLH patients will evaluate change in serum phosphorus levels as the primary endpoint, and pain, stiffness and physical function as key secondary endpoints.
- **Ultragenyx plans to submit a biologics license application (BLA) to the U.S. FDA for KRN23 in the second**

half of 2017. The company continues to discuss the details of the planned submission with FDA, and expects to submit both pediatric Phase 2 data and adult Phase 3 data, if positive. Based on discussions with the FDA, the pediatric Phase 3 study is currently not expected to be required for a U.S. filing**.** In June 2016, the FDA granted breakthrough therapy designation to KRN23 for the treatment of X-linked hypophosphatemia (XLH) in pediatric patients one year of age and older.

rhGUS in MPS 7

- **Ultragenyx has met with FDA and 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 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 Glut1 Deficiency Syndrome (Glut1 DS)

- **Phase 3 movement disorder study in Glut1 DS patients expected to initiate imminently.** 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 disabling 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

- **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. We plan to submit an NDA and MAA based on the Phase 3 data, if positive.

Conference Call & Webcast Information

Ultragenyx will host a conference call today, Thursday, February 16, 2017 at 5pm ET to discuss fourth quarter and full year 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 67687697. 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 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, 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 November 8, 2016, and its subsequent periodic reports filed with the Securities and Exchange Commission.

Ultragenyx Pharmaceutical Inc.
Selected Statement of Operations Financial Data
(in thousands, except share and per share amounts)

(unaudited)

	Three Months Ended December 31,		Year Ended December 31,	
	2016	**2015**	**2016**	**2015**
Statement of Operations Data:				
Revenue	$ 5	$ -	$ 133	$ -
Operating expenses:				
Research and development	50,746	44,565	183,204	114,737
General and administrative	19,808	11,593	64,936	33,001
Total operating expenses	70,554	56,158	248,140	147,738
Loss from operations	(70,549)	(56,158)	(248,007)	(147,738)
Other income (expense), net	(703)	938	2,168	2,120
Loss before income taxes	(71,252)	(55,220)	(245,839)	(145,618)
Income tax provision	(35)	-	(35)	-
Net loss	$ (71,287)	$ (55,220)	$ (245,874)	$ (145,618)
Net loss per share, basic and diluted	$ (1.75)	$ (1.42)	$ (6.21)	$ (3.96)
Shares used in computing net loss per share, basic and diluted	40,783,829	38,847,922	39,586,908	36,782,603

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

	December 31, 2016	December 31, 2015
Balance Sheet Data:		
Cash, cash equivalents and investments	$ 498,111	$ 536,256
Working capital	341,436	422,289
Total assets	540,626	559,569
Total stockholders' equity	473,974	531,090

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