Ultragenyx Reports Fourth Quarter and Full Year 2017 Financial Results and Corporate Update

February 20, 2018

NOVATO, Calif., Feb. 20, 2018 (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, 2017.

“2017 was a transformative year for Ultragenyx with our first product approval and launch of Mepsevii™, significant clinical and regulatory progress, and the addition of gene therapy development and manufacturing technology through the acquisition of Dimension Therapeutics,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “The momentum will continue in 2018 with the potential for approval and launch of burosumab in the U.S. and Europe, and significant progress expected with our clinical, gene therapy and translational research programs.”

Fourth Quarter and Full Year 2017 Financial Results

For the fourth quarter of 2017, Ultragenyx reported a net loss of $81.7 million, or $1.89 per share, basic and diluted, compared with a net loss for the fourth quarter of 2016 of $71.3 million, or $1.75 per share, basic and diluted. For the year ended December 31, 2017, net loss was $302.1 million, or $7.12 per share, basic and diluted, compared with a net loss for the same period in 2016 of $245.9 million, or $6.21 per share, basic and diluted. The net loss for the fourth quarter and for the full year of 2017 includes a non-recurring tax benefit from the Dimension acquisition of $16.2 million as a result of the change in the U.S. corporate tax rate from 34% to 21% on the deferred tax liability. The net loss for the full year 2017 reflected cash used in operations of $253.8 million for the year ended December 31, 2017 compared to $161.0 million for the same period in 2016.

The U.S. Food and Drug Administration (FDA) approved Mepsevii for the treatment of children and adults with Mucopolysaccharidosis VII (MPS VII) on November 15, 2017. For the fourth quarter and full year of 2017, Ultragenyx reported $0.3 million and $0.5 million respectively in Mepsevii revenues. Total operating expenses for the fourth quarter of 2017 were $99.2 million compared with $70.6 million for the same period in 2016, including non-cash stock-based compensation of $19.5 million and $13.5 million in the fourth quarter of 2017 and 2016, respectively. Total operating expenses for the year ended December 31, 2017 were $331.6 million compared with $248.1 million for the same period in 2016, including non-cash stock-based compensation of $68.0 million and $48.3 million in 2017 and 2016, respectively. The increase in total operating expenses is due to the increase in development, commercial, and general and administrative costs as the company commercializes, grows and advances its pipeline.

Cash, cash equivalents, and investments were $244.5 million as of December 31, 2017. In January 2018, Ultragenyx completed the sale of its Rare Pediatric Disease Priority Review Voucher for $130.0 million and also raised approximately $271.0 million in net proceeds through an equity offering.

Recent Highlights

Mepsevii (vestronidase alfa) for MPS VII

- **On November 15, the U.S. FDA, approved our first product, Mepsevii, the first medicine approved for the treatment of children and adults with MPS VII.** The Mepsevii launch has been progressing well with start forms received and patients on treatment.

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

- **In Europe, burosumab received a positive CHMP opinion for the treatment of XLH with radiographic evidence of bone disease in children 1 year of age and older and adolescents with growing skeletons.** A final decision on the conditional marketing authorization from the European Commission (EC) is expected in February 2018.

- **Bone biopsy data from adult patients in the bone quality study demonstrated continued improvement in osteomalacia.** At 48 weeks, all ten patients with evaluable paired bone biopsies demonstrated meaningful improvements from baseline in mean osteoid volume/bone volume. The mean decrease from 26.1% to 11.2% among these patients represents a 57% improvement from baseline in mean osteoid volume/bone volume which is the gold standard for the evaluation of osteomalacia. The patients also demonstrated mean improvements of 32% and 26% in osteoid thickness and osteoid surface/bone surface parameters respectively. These patients also experienced a meaningful improvement in mineralization lag time. These results, including safety, are consistent with the data provided to the FDA.

- **Continued improvement in rickets and bowing observed in 64-week data from pediatric XLH patients under 5 years old.** Longer term data from the Phase 2 study in 1-4 year olds demonstrated that treatment with Crysvita was consistent with and further improved from what was seen at 40 weeks. These included sustained improvements in serum phosphorus levels and a progressive reduction into the normal range of alkaline phosphatase. There were continued improvements in bowing and rickets scores at 64 weeks. The safety profile observed in this study was consistent with other burosumab studies.

- **Positive 48-week data from adult Phase 3 study of burosumab.** Data showed sustained maintenance of normal serum phosphorus levels, increased rate of fracture healing and further improvement in stiffness, physical function and pain and a substantial decrease in pain medicine usage over 48 weeks. The safety profile was consistent with what has been previously observed in this study and in other open label studies of burosumab in adults and children.

DTX301 Gene Therapy in Ornithine Transcarbamylase (OTC) Deficiency

- **Initial data from Phase 1/2 study with our AAV8 vector in OTC patients showed activity in the first, lowest-dose cohort.** There were no infusion-related adverse events and no serious adverse events reported. The only treatment-related adverse events were mild, clinically asymptomatic and manageable elevations in alanine aminotransferase (ALT) in two patients, who both completed a standard tapering course of corticosteroids to treat the ALT elevations. One patient's rate of ureagenesis was normalized and maintained with a 30% increase in the rate of ureagenesis from baseline to week 12 to reach 87% of normal. The second patient did not show a clinically meaningful change in the rate of ureagenesis over the 12-week period, and the third patient showed a modest increase in ureagenesis from baseline over the first six weeks of treatment, with 12-week data not yet available.

Corporate

- **Chief Medical Officer Appointed:**Camille L. Bedrosian, M.D., was appointed Executive Vice President and Chief Medical Officer. Most recently, Dr. Bedrosian was Senior Vice President and Chief Medical Officer at Alexion Pharmaceuticals, Inc.

- **Added gene therapy development and manufacturing technology:** In November, we completed the acquisition of Dimension Therapeutics, Inc., for approximately $152.3 million in cash.

- **Sold Rare Pediatric Disease Priority Review Voucher (PRV) for $130 million**: In January 2018 we completed the sale of the PRV that we received at the time of the approval of Mepsevii.

- **Equity financing of approximately $271.0 million:** In January 2018, we completed an underwritten public offering, with net proceeds of approximately $271.0 million.

Upcoming Key Milestones

Burosumab in XLH

- **PDUFA action date of April 17, 2018.** The U.S. FDA accepted the Biologics License Application (BLA) for burosumab to treat pediatric and adult patients with X-Linked Hypophosphatemia (XLH) and has granted Priority Review status. The Agency has indicated that it is not currently planning to hold an advisory committee meeting to discuss the BLA. The FDA has designated burosumab as a drug for a "rare pediatric disease", enabling the issuance of a priority review voucher if burosumab is approved.

- **Data from the Phase 3 study in pediatric patients expected in the second half of 2018.** The ongoing Phase 3 randomized open-label clinical study is comparing the efficacy and safety of burosumab to oral phosphate and active vitamin D therapy in pediatric patients with XLH. This study will not be required to support a US approval and will serve as a confirmatory study in Europe.

Burosumab in tumor-induced osteomalacia (TIO)

- **Data from all patients in Phase 2 study in TIO expected first half of 2018.** This is an open label Phase 2 study evaluating the safety and efficacy of burosumab in 17 adult patients with TIO.

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

- **Completing study design of Phase 3 study in FAOD patients; providing additional data to FDA for consideration of early filing based on Phase 2 data.** Following an end-of-phase 2 meeting, we are working to provide additional information to submit to FDA for consideration of an early filing based on the results from the Phase 2 study. While the FDA still prefers that a randomized controlled trial be completed before filing, it left open the possibility of filing on the current data. We are simultaneously completing the design of a Phase 3 study that could be used for registration or confirmatory purposes. We expect that a decision on a potential filing for approval based on Phase 2 data will be made in mid-2018.

- **Data from the Phase 3 movement disorder study in Glut1 DS patients.** Enrollment has gone well in this study and data is expected in the second half of 2018.

DTX301 Gene Therapy in Ornithine Transcarbamylase (OTC) Deficiency

- **Full data from Cohort 1 of Phase 1/2 study in OTC patients expected in March.** The higher dose Cohort 2 is expected to initiate in March followed by data in the second half of 2018.

DTX401 Gene Therapy in Glycogen storage disease type Ia (GSDIa)

- **IND submission for the Phase 1/2 study on track for the first half 2018.** Data from the first cohort is expected in the second half of 2018.

DTX201 Gene Therapy in Hemophilia A

- **IND submission on track for second half 2018.**

Conference Call & Webcast Information

Ultragenyx will host a conference call today, Tuesday, February 20, 2018 at 5pm ET to discuss fourth quarter and full year 2017 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 1987066. The replay of the call will be available for one year.

About Ultragenyx

Ultragenyx is a biopharmaceutical company committed to bringing to patients 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.

Mepsevii™ (vestronidase alfa) is approved by the U.S. FDA for the treatment of children and adults with mucopolysaccharidosis VII (MPS VII). Ultragenyx is conducting Phase 2 and Phase 3 studies of burosumab, in pediatric and adult patients with XLH and tumor induced osteomalacia (TIO), both rare diseases that impair bone mineralization; 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; a Phase 3 study for UX007 in patients with glucose transporter type-1 deficiency syndrome (Glut1 DS), a brain energy deficiency, who are experiencing movement disorders; and a Phase 1/2 study of DTX301 Gene Therapy in Ornithine Transcarbamylase (OTC) Deficiency.

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 ongoing or additional studies for its product candidates and timing regarding these studies, the design of clinical studies, the demonstrated impact of clinical data and other information to support approval of product candidates, potential indications for its product candidates, discussions with regulatory authorities, the potential issuance of a priority review voucher, sufficiency for, and timing of, regulatory submissions and approvals, and the timing and locations of commercialization efforts 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, 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 3, 2017, 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,	
	2017	**2016**	**2017**	**2016**
Statement of Operations Data:				
Revenues:				
Collaboraion and license	$ 2,136	$ -	$ 2,136	$ -
Product sales	278	5	476	133
Total revenues	2,414	5	2,612	133
Operating expenses:				
Cost of sales	1	-	1	-
Research and development	61,527	50,746	231,644	183,204
Selling, general and administrative	37,720	19,808	99,909	64,936
Total operating expenses	99,248	70,554	331,554	248,140
Loss from operations	(96,834)	(70,549)	(328,942)	(248,007)
Other income (expense)	(1,114)	(703)	10,604	2,168
Loss before income taxes	(97,948)	(71,252)	(318,338)	(245,839)
Benefit from (provision for) income taxes	16,217	(35)	16,199	(35)
Net loss	$ (81,731)	$ (71,287)	$ (302,139)	$ (245,874)
Net loss per share, basic and diluted	$ (1.89)	$ (1.75)	$ (7.12)	$ (6.21)
Shares used in computing net loss per share, basic and diluted	43,137,679	40,783,829	42,453,135	39,586,908

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

	December 31, 2017	December 31, 2016
Balance Sheet Data:		
Cash, cash equivalents and investments	$ 244,468	$ 498,111
Working capital	198,569	341,436
Total assets	490,753	540,626
Total stockholders' equity	383,454	473,974

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