November 2, 2017

Ultragenyx Reports Third Quarter 2017 Financial Results and Corporate Update

NOVATO, Calif., Nov. 02, 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 ended September 30, 2017.

"Burosumab and vestronidase alfa regulatory submissions have been accepted and regulatory review is progressing well in both the US and European Union. We are preparing for a near-term potential launch of vestronidase alfa with a PDUFA date of November 16," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "We are continuing to build a next-generation rare disease company and we expect the pending acquisition of Dimension Therapeutics to provide a unique opportunity to approach treatment of more rare diseases with gene therapy technology."

Third Quarter 2017 Financial Results

For the third quarter of 2017, Ultragenyx reported a net loss of $79.2 million, or $1.87 per share, basic and diluted, compared with a net loss of $64.9 million, or $1.64 per share, basic and diluted in the third quarter of 2016. For the nine months ended September 30, 2017, net loss was $220.4 million, or $5.22 per share, basic and diluted, compared with a net loss for the same period in 2016 of $174.6 million, or $4.46 per share, basic and diluted. This reflected cash used in operations of $172.0 million for the nine months ended September 30, 2017 compared to $113.3 million for the same period in 2016.

Total operating expenses for the third quarter of 2017 were $83.9 million compared with $65.9 million for the same period in 2016, including non-cash stock-based compensation of $17.2 million and $13.7 million for the third quarter of 2017 and 2016, respectively. Total operating expenses for the nine months ended September 30, 2017 were $232.3 million compared with $177.6 million for the same period in 2016, including non-cash stock-based compensation of $48.5 million and $34.8 million in the first nine months of 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 grows and advances its pipeline.

Cash, cash equivalents, and investments were $396.0 million as of September 30, 2017.

Recent Highlights

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

- **US BLA for burosumab accepted and PDUFA action date set for April 17, 2018.** The U.S. Food and Drug Administration (FDA) has 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 issuance of a priority review voucher if burosumab is approved.
- **Bone biopsy data from the first six adult patients demonstrate substantial improvement in severe osteomalacia and are included in FDA review.** We now have six total available paired bone biopsies from the 48-week open label bone quality study, and they will be included in the BLA review. The bone biopsy data demonstrated substantial improvement in osteomalacia from severe to mild disease. The six patients had mean osteoid volume/bone volume decreases from 24.3 percent to 7.0 percent, representing a 71 percent improvement. In our past discussions with FDA, they emphasized that improvement in osteomalacia could also be considered an important outcome for adults with XLH.
- **Significant improvement in rickets and bowing observed in early 40-week data from pediatric XLH patients under 5 years old.** At ASBMR in September, data from the Phase 2 under 5 study demonstrated that treatment with burosumab significantly improved serum phosphorus levels, bowing, and rickets scores. Burosumab increased mean serum phosphorus levels into the low normal range after one week of treatment, and these levels were maintained through week 40. The patients had more severe rickets at baseline than the older children that we have previously studied, and every patients showed substantial healing of rickets at week 40. These younger

patients also showed a significant improvement in bowing, which was readily visible on X-rays of some patients in only 40 weeks. The safety profile observed in this study was consistent with other burosumab studies. The data support the concept that early treatment could have a more profound impact on the bone deformities that would typically affect XLH patients throughout their lives.

Burosumab (KRN23) anti-FGF23 Monoclonal Antibody in tumor-induced osteomalacia (TIO)

- **Treatment of TIO patients shows improved serum phosphorus levels and effect on bone disease and physical function.** Data from the open-label Phase 2 study presented at ASBMR showed increases in mean serum phosphorus levels, significant increases in bone turnover markers, improvements in histomorphometric indices of osteomalacia in 3 of the 4 patients who completed 48 weeks of treatment with bone biopsy, and statistically significant improvements in patient-reported outcomes. The safety profile was consistent with other burosumab studies.

Corporate

- **Definitive merger agreement signed for the acquisition of Dimension Therapeutics.** We have entered into a definitive merger agreement with Dimension under which Ultragenyx will acquire all of the outstanding shares of common stock of Dimension for $6.00 per share, or approximately $151 million in cash, based on shares outstanding as of the date of the merger agreement. The transaction is structured as a tender offer and will be funded through our existing cash resources. The transaction could close as early as November 7, 2017, depending on the satisfaction of the conditions for the merger.

Upcoming Key Milestones

Burosumab in XLH

- **PDUFA action date of April 17, 2018.** The BLA was accepted for priority review in October 2016.
- **Opinion from CHMP on the burosumab Conditional MAA for pediatric XLH expected around the end of 2017.** The MAA was submitted and accepted for review by the European Medicines Agency (EMA) in December 2016.
- **48-week data from the Phase 3 study in adult XLH patients expected by the end of 2017.** Data will include change in serum phosphorus levels as well as pain, stiffness and physical function.
- **Bone biopsy results from all 11 patients in bone quality study expected in early 2018.**

Vestronidase alfa or rhGUS in Mucopolysaccharidosis 7 (MPS 7)

- **PDUFA goal date of November 16, 2017 and CHMP opinion in first half of 2018.** The FDA has granted vestronidase alfa Priority Review Status, and the BLA has a PDUFA action date for a decision of November 16, 2017. The Committee for Medicinal Products for Human Use (CHMP) opinion is expected in the first half of 2018.

UX007 in Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD) and Glut1 Deficiency Syndrome (Glut1 DS)

- **Phase 3 study in FAOD patients.** We continue to plan for discussions with regulators regarding the Phase 3 study in FAOD patients by the end of 2017.
- **Phase 3 movement disorder study in Glut1 DS patients ongoing.** The randomized, double-blind, placebo-controlled, cross-over study is enrolling approximately 40 patients. The primary endpoint compares the frequency of disabling paroxysmal movement disorder events with UX007 to placebo, as recorded by a daily electronic diary.

Corporate

- **Analyst Day to be held at 8:00 am ET on December 4, 2017 in New York**. The company will review development progress and commercial readiness, and outside experts will share their perspectives during the Analyst Day.

Conference Call & Webcast Information

Ultragenyx will host a conference call today, Thursday, November 2, 2017 at 5pm ET to discuss third quarter 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 3976859. 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). The Company is conducting Phase 2 and Phase 3 studies of burosumab, 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; 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 an ongoing 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.

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 the pending acquisition of Dimension Therapeutics, Inc. and potential benefits of the acquisition, 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, and sufficiency for, and timing of, regulatory submissions and approvals, 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 July 27, 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 September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Statement of Operations Data:				
Revenue	$ 198	$ 111	$ 198	$ 128
Operating expenses:				
Research and development	60,412	48,711	170,117	132,458
General and administrative	23,499	17,183	62,189	45,128
Total operating expenses	83,911	65,894	232,306	177,586
Loss from operations	(83,713)	(65,783)	(232,108)	(177,458)
Other income, net	4,490	876	11,718	2,871
Loss before income taxes	(79,223)	(64,907)	(220,390)	(174,587)
Income tax provision	(4)	-	(18)	-
Net loss	$ (79,227)	$ (64,907)	$ (220,408)	$ (174,587)

Net loss per share, basic and diluted	$ (1.87)	$ (1.64)	$ (5.22)	$ (4.46)
Shares used in computing net loss per share, basic and diluted	42,471,606	39,551,923	42,222,413	39,184,994

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

	September 30, 2017	December 31, 2016
Balance Sheet Data:		
Cash, cash equivalents and investments	$ 396,030	$ 498,111
Working capital	326,642	341,436
Total assets	435,047	540,626
Total stockholders' equity	365,558	473,974

Contact Ultragenyx Pharmaceutical Inc.
Investors & Media
Danielle Keatley
415-475-6876