Ultragenyx Reports Third Quarter 2018 Financial Results and Corporate Update

November 5, 2018

NOVATO, Calif., Nov. 05, 2018 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for serious rare and ultra-rare genetic diseases, today reported its financial results and corporate update for the quarter ended September 30, 2018.

"We're encouraged by the growing demand for Crysvita from both children and adults with XLH in the United States, and we are making significant progress reaching doctors and helping them get patients on commercially available therapy in this early launch phase," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "Next year, we expect to file for potential approval of our third therapy, UX007 in fatty acid oxidation disorders, and we continue to advance our two gene therapy clinical programs with additional data expected around the end of this year and in 2019."

Financial Results

For the third quarter of 2018, Ultragenyx reported a net loss of $87.3 million, or $1.74 per share, basic and diluted, compared with a net loss for the third quarter of 2017 of $79.2 million, or $1.87 per share, basic and diluted. For the nine months ended September 30, 2018, net loss was $109.8 million, or $2.22 per share, basic and diluted, compared with a net loss for the same period in 2017 of $220.4 million, or $5.22 per share, basic and diluted. The loss from the first nine months was reduced by the sale of the Mepsevii™ (vestronidase alfa) priority review voucher (PRV) in January 2018 for net proceeds of $130.0 million and a $40.3 million gain from Ultragenyx's portion of the sales of the PRV received with the Crysvita® (burosumab) approval. The net loss for the first nine months of 2018 reflected cash used in operations of $234.7 million compared to $172.0 million for the same period in 2017.

Net Revenues

For the third quarter of 2018, Ultragenyx reported $11.8 million in total revenue. For Crysvita, Ultragenyx recognized $5.4 million in profit sharing and royalty revenue from its collaboration and license agreement with Kyowa Hakko Kirin. This includes $4.4 million in collaboration revenue in the U.S. profit share territory and $1.0 million in royalty revenue in the European territory. Net product sales for Crysvita in other regions were nominal. Mepsevii product revenue for the third quarter of 2018 was $2.1 million, and UX007 named patient revenue was $0.4 million. Ultragenyx recognized $3.6 million in revenue from its research agreement with Bayer.

Operating Expenses

Total operating expenses for the third quarter of 2018 were $101.4 million compared with $83.9 million for the same period in 2017, including non-cash stock-based compensation of $20.7 million and $17.2 million in the third quarter of 2018 and 2017, respectively. Total operating expenses for the nine months ended September 30, 2018, were $316.3 million compared with $232.3 million for the same period in 2017, including non-cash stock-based compensation of $59.0 million and $48.5 million in the first nine months of 2018 and 2017, respectively. The increase in total operating expenses is due to the increase in commercial, development, and general and administrative costs as the company commercializes, grows and advances its pipeline.

Cash, Cash Equivalents and Investments

Cash, cash equivalents and investments were $503.1 million as of September 30, 2018.

Recent Updates

Crysvita in X-Linked Hypophosphatemia (XLH)

- **Ultragenyx submitted regulatory filings in Canada, Brazil and Colombia.** We anticipate regulatory decisions in these markets over the course of 2019. Reimbursed named patient treatment has already begun in Argentina in response to physician requests for multiple patients.

Crysvita in Tumor-Induced Osteomalacia (TIO)

- **Positive longer-term 48-week and 72-week data from the Phase 2 study of Crysvita in adults with TIO were presented at the American Society for Bone and Mineral Research (ASBMR) 2018 Annual Meeting in Montreal**. In adults with TIO, Crysvita was associated with increases in serum phosphorus and serum 1,25 dihydroxy vitamin D levels; improvements in osteomalacia, mobility and vitality; and reductions in fatigue. Adverse events (AE) generally reflected the patients' underlying diseases, and there were no serious treatment-related AEs during the study. Discussions with the U.S. Food and Drug Administration (FDA) regarding the regulatory pathway are ongoing.

Mepsevii in Mucopolysaccharidosis VII (MPS VII)

- **The European Commission (EC) approved the Marketing Authorization Application (MAA) for Mepsevii under exceptional circumstances for the treatment of non-neurological manifestations of MPS VII.** Mepsevii is now approved for use in all 28 EU countries as well as in Iceland, Liechtenstein and Norway, and has launched in Germany.
- **Brazil's National Health Surveillance Agency approved Mepsevii for the treatment of MPS VII for patients of all ages.** Additional regulatory decisions for patients in Columbia and Chile are anticipated over the course of 2019.

UX007 in Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD) and Glucose Transporter Type 1 Deficiency Syndrome (Glut1 DS)

- **The FDA accepted Ultragenyx's proposal to submit a New Drug Application (NDA) for UX007 for the treatment of LC-FAOD based on currently available data.** Further details will be forthcoming following a pre-NDA meeting, which is scheduled to take place by the end of 2018. Additionally, discussions are progressing with the European Medicines Agency regarding a potential conditional marketing authorization in Europe.

- **In October 2018, Ultragenyx announced that the Phase 3 study evaluating UX007 in patients with Glut1 DS did not achieve its primary endpoint compared to placebo.** The safety profile observed in this study was consistent with what has been previously reported with UX007. Ultragenyx plans to discontinue further Glut1 DS clinical development for UX007, and expects no impact on plans for the LC-FAOD indication.

DTX301 Gene Therapy in Ornithine Transcarbamylase (OTC) Deficiency

- **Positive topline data from the first two dose cohorts of the Phase 1/2 study demonstrate normalization of ureagenesis in two patients and further support proof-of-concept.** The first responder (Cohort 1) showed a further increased level of ureagenesis at 52 weeks and has been clinically stable for more than eight months after discontinuing alternate pathway medications and also after liberalizing a protein-restricted diet near the 52 week time point. The second responder (Cohort 2) achieved normalization of ureagenesis at 24 weeks, and has been clinically stable for more than one month after also discontinuing all alternate pathway medications. The remaining four patients in Cohorts 1 and 2 continue to demonstrate no clinically meaningful change in rate of ureagenesis. All patients have remained clinically and metabolically stable, with only modest rises in ALT well treated by a reactive, tapering steroid regimen. The first patient in cohort 3 has been dosed. Data from higher dose Cohort 3 are expected in mid-2019.

DTX401 Gene Therapy in Glycogen Storage Disease Type Ia (GSDIa)

- **All three patients in the lowest-dose Cohort 1 of the Phase 1/2 study have been dosed.** Data from this cohort are expected around the end of 2018.

CDKL5 Deficiency Disorder (CDD)

- **Ultragenyx exercised its option with REGENXBIO Inc. to develop a gene therapy to treat patients with CDD using REGENXBIO's adeno-associated virus (AAV) vectors, including AAV9.** CDD is a severe and debilitating neurological disorder that shares many features of Rett Syndrome. It was commonly identified as an atypical Rett Syndrome until improved genetic testing led to more accurate diagnosis and an increasing prevalence of diagnosed CDD-affected patients.

Corporate

- **Ultragenyx appointed a Chief Operating Officer,** Wladimir (Vlad) Hogenhuis, M.D., on September 28, 2018. In this newly created role, Dr. Hogenhuis oversees the Global Commercial, Business Development, and Manufacturing organizations.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Monday, November 5, 2018, at 2 p.m. PT/ 5 p.m. ET to discuss third quarter 2018 financial results and 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 1290627. 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 serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved therapies and product candidates aimed at addressing diseases with high unmet medical need and clear biology for treatment, for which there are no approved therapies.

Crysvita® (burosumab) is approved by the U.S. FDA for the treatment of XLH in adult and pediatric patients one year of age and older, and has received conditional marketing authorization in Europe for the treatment of XLH with radiographic evidence of bone disease in children 1 year of age and older and adolescents with growing skeletons. Mepsevii™ (vestronidase alfa) is approved inthe United States for the treatment of children and adults with MPS VII. In Europe, Mepsevii is approved under exceptional circumstances for the treatment of non-neurological manifestations of MPS VII.

In addition to the approved treatments for XLH and MPS VII, Ultragenyx has four clinical development programs. Crysvita is being studied for the treatment of TIO, a rare disease that impairs bone mineralization. UX007 is being studied in patients severely affected by LC-FAOD, a genetic disorder in which the body is unable to convert long chain fatty acids into energy. The company has two gene therapy pipeline candidates: DTX 301 is an AAV8 gene therapy product candidate in development for the treatment of patients with OTC deficiency, the most common urea cycle disorder; and DTX 401 is an AAV8 gene therapy product candidate for the treatment of patients with GSDIa.

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 relating to Ultragenyx's plans for its clinical programs and future regulatory filings and interactions, 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 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 products and 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 Ultragenyx in general, see Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on August 3, 2018, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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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,	
	2018	**2017**	**2018**	**2017**
Statement of Operations Data:				
Revenues:				
Collaboration and license	$ 9,015	$ —	$ 28,896	$ —
Product sales	2,748	198	6,338	198
Total revenues	11,763	198	35,234	198
Operating expenses:				
Cost of sales	273	—	639	—
Research and development	70,041	60,412	222,380	170,117
Selling, general and administrative	31,095	23,499	93,248	62,189
Total operating expenses	101,409	83,911	316,267	232,306
Loss from operations	(89,646)	(83,713)	(281,033)	(232,108)
Gain from sale of priority review vouchers	—	—	170,322	—
Other income (expense), net	2,583	4,490	1,314	11,718
Loss before income taxes	(87,063)	(79,223)	(109,397)	(220,390)
Provision for income taxes	(247)	(4)	(388)	(18)
Net loss	$ (87,310)	$ (79,227)	$ (109,785)	$ (220,408)
Net loss per share, basic and diluted	$ (1.74)	$ (1.87)	$ (2.22)	$ (5.22)
Shares used in computing net loss per share, basic and diluted	50,319,772	42,471,606	49,447,889	42,222,413

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

	September 30, 2018	December 31, 2017
Balance Sheet Data:		
Cash, cash equivalents and investments	$ 503,098	$ 244,468
Working capital	500,799	198,569
Total assets	765,145	490,753
Total stockholders' equity	662,596	383,454

Source: Ultragenyx Pharmaceutical Inc.