Ultragenyx Reports Fourth Quarter and Full Year 2018 Financial Results and Corporate Update

February 19, 2019

Strong Launch Continues With More than 550 Patients on Reimbursed Commercial Crysvita® (burosumab) Therapy in the United States at End of 4th Quarter 2018, 80 Percent Increase Versus End of 3rd Quarter

NOVATO, Calif., Feb. 19, 2019 (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 and full year ended December 31, 2018.

“This last year was important for Ultragenyx as we successfully launched two therapies internationally and validated clinical data from our gene therapy platform,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “In 2019 we look forward to expanding the global commercial reach of our approved therapies, submitting a New Drug Application for a third, and advancing our gene therapy platform toward pivotal studies.”

Fourth Quarter and Full Year 2018 Financial Results

Net Revenues

For the fourth quarter of 2018, Ultragenyx reported $16.3 million in total revenue. Ultragenyx recognized $11.6 million in total Crysvita revenue. This includes $9.9 million in collaboration revenue in the U.S. profit share territory and $1.3 million in royalty revenue in the European territory from the collaboration and license agreement with Kyowa Hakko Kirin. Net product sales for Crysvita in other regions were $0.4 million. Mepsevii™ (vestronidase alfa) product revenue for the fourth quarter of 2018 was $2.7 million, and UX007 named patient revenue was $0.5 million. Ultragenyx recognized $1.6 million in revenue from its research agreement with Bayer.

Net revenue for the year ended December 31, 2018 totaled $51.5 million. Since launching Crysvita on April 27, 2018 through the end of the year, Ultragenyx recognized $18.9 million in total Crysvita revenue. This includes $15.3 million in collaboration revenue in the U.S. profit share territory and $2.9 million in royalty revenue in the European territory from the collaboration and license agreement with Kyowa Hakko Kirin. Net product sales for Crysvita in other regions totaled $0.6 million. Mepsevii product revenue for the year ended December 31, 2018 was $7.9 million and UX007 named patient revenue was $1.3 million. Ultragenyx recognized $23.5 million in revenue from its research agreement with Bayer in the year ended December 31, 2018.

Operating Expenses

Total operating expenses for the fourth quarter of 2018 were $106.6 million compared with $99.2 million for the same period in 2017, including non-cash stock-based compensation of $21.1 million and $19.5 million in the fourth quarter of 2018 and 2017, respectively. Total operating expenses for the year ended December 31, 2018, were $422.9 million compared with $331.6 million for the same period in 2017, including non-cash stock-based compensation of $80.1 million and $68.0 million in the full year 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.

For the fourth quarter of 2018, Ultragenyx reported a net loss of $87.8 million, or $1.73 per share, basic and diluted, compared with a net loss for the fourth quarter of 2017 of $81.7 million, or $1.89 per share, basic and diluted. For the year ended December 31, 2018, net loss was $197.6 million, or $3.97 per share, basic and diluted, compared with a net loss for the same period in 2017 of $302.1 million, or $7.12 per share, basic and diluted. The loss from the full year 2018 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 full year 2018 reflected cash used in operations of $290.6 million compared to $253.8 million for the same period in 2017.

Cash, Cash Equivalents and Investments

Cash, cash equivalents and investments were $459.7 million as of December 31, 2018.

Recent Updates

Crysvita in X-Linked Hypophosphatemia (XLH)

- **Longer-term data from the Phase 3 pediatric study demonstrated superior efficacy of Crysvita compared with conventional oral phosphate treatment.** After 64 weeks of treatment, patients treated with Crysvita continued to show significantly greater improvement in healing of rickets, growth, and bowing of the legs compared with patients treated with oral phosphate and active Vitamin D, which was the standard of care for over 30 years. The 64-week safety profile was similar to that observed at 40 weeks and in other Crysvita pediatric XLH studies.
- **Crysvita was approved and launched in Canada for the treatment of XLH in adults and pediatric patients one year of age and older.**Canada is included in the North American profit share agreement with our partner Kyowa Hakka Kirin.

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

- **Positive topline data from ongoing long-term extension study of UX007 for the treatment of LC-FAOD showed sustained reductions in the duration and frequency of major clinical events (MCEs), primarily hospitalizations for hypoglycemia, cardiomyopathy, and rhabdomyolysis.** Patients who had previously been enrolled in the Company-sponsored Phase 2 study (n=24) demonstrated sustained reductions in MCEs after an additional 78 weeks of treatment, and 20 additional patients who had not previously received UX007 demonstrated similar substantial reductions in these MCEs with treatment, further corroborating prior results. The safety profile was consistent with what has been previously observed with UX007.

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

- **Positive topline results from the first cohort of the Phase 1/2 clinical study of DTX401 gene therapy in GSDIa.** All three patients demonstrated a clinical response, reflected by improved glucose control, increased time to hypoglycemia during fasting and reductions in necessary cornstarch dosing. Two patients demonstrated a clinically meaningful improvement in time to hypoglycemia, making it possible to sleep through the night. Typically, for GSDIa patients, cornstarch or tube feeding is required during the night to prevent severe hypoglycemia, which can cause seizures or death. There were no infusion-related adverse events and no treatment-related serious adverse events reported.

Upcoming Key Milestones

UX007 in LC-FAOD

- **Ultragenyx is on track to submit a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) in mid-2019.** The submission will include data from the company-sponsored Phase 2 study in 29 patients, data from the long-term efficacy and safety extension study in 75 patients, a retrospective medical record review of 20 original compassionate use patients, data from 70 patients treated through expanded access, and a randomized controlled investigator-sponsored study of 32 patients showing an effect of UX007 on cardiac function.

DTX301 Gene Therapy in Ornithine Transcarbamylase (OTC) Deficiency

- **Enrollment is ongoing in the third-dose cohort of the Phase 1/2 study.** Cohort 3 data expected in mid-2019.

DTX401 Gene Therapy in GSDIa

- **Enrollment is ongoing in the second-dose cohort of the Phase 1/2 study.** Cohort 2 data expected in mid-2019.

Corporate

- **Ultragenyx plans to host an Analyst Day on Wednesday, April 17.** The event will provide updates and expert commentary on commercial, clinical stage and early pipeline programs. Additional details to come.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Tuesday, February 19, 2018, at 2 p.m. PT/ 5 p.m. ET to discuss fourth quarter and full year 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 6689186. 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 typically no approved therapies treating the underlying disease.

Crysvita® (burosumab) is approved by the U.S. FDA and Health Canada 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 and Brazil 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 with 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: DTX301 is an AAV8 gene therapy product candidate in development for the treatment of patients with OTC deficiency, the most common urea cycle disorder; and DTX401 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 related to Ultragenyx's expectations regarding plans for its clinical programs and clinical studies, future regulatory interactions, and the components and timing of regulatory submissions 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 November 6, 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 December 31,		Year Ended December 31,	
	2018	2017	2018	2017
Statement of Operations Data:				
Revenues:				
Collaboration and license	$ 12,797	$ 2,136	$ 41,693	$ 2,136
Product sales	3,464	278	9,802	476
Total revenues	16,261	2,414	51,495	2,612
Operating expenses:				
Cost of sales	507	1	1,146	1
Research and development	71,618	61,527	293,998	231,644
Selling, general and administrative	34,476	37,720	127,724	99,909
Total operating expenses	106,601	99,248	422,868	331,554
Loss from operations	(90,340)	(96,834)	(371,373)	(328,942)
Gain from sale of priority review vouchers	—	—	170,322	—
Other income (expense), net	2,640	(1,114)	3,954	10,604
Loss before income taxes	(87,700)	(97,948)	(197,097)	(318,338)
Benefit from (provision for) income taxes	(126)	16,217	(514)	16,199
Net loss	$ (87,826)	$ (81,731)	$ (197,611)	$ (302,139)
Net loss per share, basic and diluted	$ (1.73)	$ (1.89)	$ (3.97)	$ (7.12)
Shares used in computing net loss per share, basic and diluted	50,694,007	43,137,679	49,775,223	42,453,135

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

	December 31, 2018	December 31, 2017
Balance Sheet Data:		
Cash, cash equivalents and investments	$ 459,706	$ 244,468
Working capital	447,644	198,569
Total assets	719,558	490,753
Total stockholders' equity	608,908	383,454

Source: Ultragenyx Pharmaceutical Inc.