Ultragenyx Reports Preliminary 2019 Revenue and Provides 2020 Crysvita Revenue Guidance

January 13, 2020

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Preliminary 2019 Crysvita revenue to Ultragenyx of approximately $86 million to $88 million

2020 Crysvita revenue in Ultragenyx territories is expected to be in the range of $125 million to $140 million excluding EU royalty revenue

Year-end 2019 cash balance of greater than $750 million

NOVATO, Calif., Jan. 13, 2020 (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 preliminary unaudited 2019 revenue and cash and investments at year end 2019, and provided 2020 revenue guidance for Crysvita in Ultragenyx territories.

"We passed the $100 million revenue threshold in 2019 based on the strong launches of Crysvita and Mepsevii. We aim to build on this success in 2020 with the potential approvals of UX007 in LC-FAOD and Crysvita in TIO," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "This growing commercial foundation will continue to enable significant pipeline advancement as we execute on our gene therapy clinical studies and initiate multiple new clinical programs."

2019 Preliminary Revenue, 2020 Crysvita Revenue Guidance, and 2019 Ending Cash Position

2019 Preliminary Revenue (unaudited)

Total revenue for the year ended December 31, 2019 is approximately $102 million to $104 million. Total Crysvita revenue to Ultragenyx is approximately $86 million to $88 million in 2019, which includes collaboration revenue in the North American profit share territory, royalty revenue in the European territory from Kyowa Kirin Co. Ltd., and product revenue for Crysvita in other regions.

2020 Crysvita Guidance

For 2020, Crysvita revenue in the Ultragenyx territories is estimated to be between $125 million and $140 million. Ultragenyx territories include the North American profit share territory and other regions where collaboration revenue and product sales are recognized by Ultragenyx. The 2020 Crysvita revenue guidance excludes the European territory royalty revenue, the rights to which were sold to Royalty Pharma. Beginning January 1, 2020, the company will no longer receive cash payments from the EU territory royalty until the respective threshold amount is met; however, the company will continue to record the royalty as "non-cash" revenue.

The company is not providing total 2020 revenue guidance at this time. The preliminary revenue results are based on management's initial analysis of operations for the quarter and year ended December 31, 2019. The 2019 revenue and cash position included in this release are preliminary and prior to the completion of review and audit procedures by Ultragenyx's external auditors, and are therefore subject to adjustment. The Company expects to issue full financial results for the fourth quarter and fiscal year 2019 in February 2020.

2019 Ending Cash Position (unaudited) and 2020 Expected Net Cash Burn Rate

Cash, cash equivalents, and available-for-sale investments were greater than $750 million as of December 31, 2019, including proceeds of $320 million received from the sale of the company's royalty interest in Crysvita in the European territory. The company also expects a more than 20 percent reduction in net cash burn (net cash used in operations plus capital expenditures) in 2020 compared to 2019.

Recent Updates and Upcoming Milestones

Crysvita for X-linked Hypophosphatemia: Non-dilutive $320 million royalty sale of future European royalties

- In December, Ultragenyx sold to Royalty Pharma for $320 million its royalty interest in Crysvita in the European territory, where it is being commercialized by Kyowa Kirin Co., Ltd.

Crysvita for Tumor-Induced Osteomalacia (TIO): Supplemental Biologics License Application (sBLA) submitted

- Ultragenyx submitted the sBLA to the U.S. FDA on December 18, 2019 and expects to hear back from FDA on submission acceptance and review designation in February 2020.

UX007 for Long-Chain Fatty Acid Oxidation Disorders (LC-FAOD): NDA under review by U.S. FDA

- The U.S. FDA accepted for review the New Drug Application (NDA) and has set a Prescription Drug User Fee Act (PDUFA) date of July 31, 2020. The FDA has indicated that it is not currently planning to hold an advisory committee meeting to discuss the application.

DTX301 for Ornithine Transcarbamylase (OTC) Deficiency: Positive data from higher cohort of Phase 1/2 study; data from prophylactic steroid cohort in second half 2020

- Recent positive data from Cohort 3 indicated two confirmed responders and a potential third responder out of three total patients, as well as a

new responder in Cohort 2. There are currently up to six responders of the nine dosed to date with a more consistent response at higher doses.

- Ultragenyx is initiating a fourth cohort (n=3) using prophylactic steroids at the same dose as Cohort 3. The first patient is expected to be enrolled in the first half of 2020, and data from the prophylactic steroid cohort are expected in the second half of 2020.

DTX401 for Glycogen Storage Disease Type Ia (GSDIa): Positive data from Phase 1/2 study; enrollment complete in confirmatory cohort and data expected in first half 2020

- Enrollment is complete in the confirmatory cohort of three patients at the second dose cohort, with data expected in the first half of 2020. Following the results of the confirmatory cohort, a Phase 3 study could begin in the second half of 2020.

GTX-102 for Angelman Syndrome: Partnered program with GeneTx; IND expected in first half 2020

- An investigational new drug (IND) application is expected in the first half of 2020. In August 2019, Ultragenyx and GeneTx announced a partnership to develop GTX-102 with Ultragenyx receiving an exclusive option to acquire GeneTx.

UX701 for Wilson Disease: IND expected in second half 2020

- An IND application is expected in the second half of 2020 for a new gene therapy for Wilson disease, a larger rare metabolic disease. UX701 will be the company's second clinical program to utilize its HeLa manufacturing system. The Hemophilia A program partnered with Bayer uses the HeLa system and has released its first clinical data.

Ultragenyx to Present at 38th Annual J.P. Morgan Healthcare Conference

- Dr. Kakkis will present on Tuesday, January 14, 2020 at 12:00 p.m. PT in San Francisco. The live and archived webcast of the presentation will be accessible from the company's website at http://ir.ultragenyx.com/events.cfm

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.

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 and projections regarding its future operating results and financial performance, anticipated cost or expense reductions, the timing, progress and 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, collaboration with third parties, 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, Ultragenyx's reliance on its third party partner, Kyowa Kirin Co., Ltd., for the supply of Crysvita, smaller than anticipated market opportunities for the company's products and product candidates, the company's evolving commercial infrastructure, uncertainties related to insurance coverage and reimbursement approval for the company's products, manufacturing risks, the uncertainties inherent in the clinical drug development process, including the potential for substantial delays and the risk that earlier study results may not be predictive of future study results, the lack of predictability in the regulatory approval process, the timing of regulatory filings and approvals (including whether such approvals can be obtained), and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations, the company's future operating results and financial performance and the availability or commercial potential of Ultragenyx's 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, 2019, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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Source: Ultragenyx Pharmaceutical Inc.