Ultragenyx Reports Preliminary 2020 Revenue and 2021 Revenue Guidance for Crysvita in Ultragenyx Territories

January 11, 2021

Preliminary 2020 Crysvita revenue in Ultragenyx Territories of $137 million to $139 million

2021 Crysvita Ultragenyx revenue expected in the range of $180 million to $190 million

Year-end 2020 cash balance of approximately $1.2 billion

NOVATO, Calif., Jan. 11, 2021 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel products for serious rare and ultra-rare genetic diseases, today reported preliminary unaudited 2020 Crysvita revenue in Ultragenyx territories and cash and investments at year end 2020 and provided 2021 revenue guidance for Crysvita in Ultragenyx territories.

“The strong performance of Crysvita and our other two commercial therapies across four indications have propelled our growth as a commercial rare disease company,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “This success has enabled us to rapidly advance our diversified pipeline with six programs in the clinic across multiple modalities, including multiple larger rare disease opportunities that could be first time treatments or major advances for patients and significant drivers of growth.”

Financial Update

2020 Preliminary Crysvita[1] Revenue (unaudited)

Crysvita revenue in Ultragenyx territories for the year ended December 31, 2020 is approximately $137 million to $139 million. This is at the top end of the guidance range of $130 million to $140 million that was provided during the third quarter financial results and at the beginning of 2020, despite COVID-19 impact.

2021 Crysvita[1] Guidance

For 2021, Crysvita revenue in the Ultragenyx territories is estimated to be between $180 million and $190 million.

2020 Ending Cash Position (unaudited)

Cash, cash equivalents, and available-for-sale investments were approximately $1.2 billion as of December 31, 2020, which includes net proceeds of approximately $435 million received from the underwritten public offering of its common stock and net proceeds of approximately $80 million from the sale of a portion of the company’s investment in Arcturus Therapeutics.

The 2020 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 preliminary revenue results are based on management's initial analysis of operations for the quarter and year ended December 31, 2020. The Company expects to issue full financial results for the fourth quarter and fiscal year 2020 in February 2021.

Recent Updates and 2021 Milestones

DTX401 for Glycogen Storage Disease Type Ia (GSDIa): Positive longer-term data from Phase 1/2 study; Phase 3 study expected to initiate in the first half of 2021

- In the Phase 1/2 study, all nine patients in the first three cohorts continue to respond to treatment and demonstrate continued glucose control while significantly tapering cornstarch glucose replacement therapy. All three patients in a fourth and final cohort, which utilizes prophylactic steroids, have been dosed at the same dose as Cohorts 2 and 3, are doing well and have demonstrated early reduction in daily cornstarch intake.
- The company has held Scientific Advice and End of Phase 2 meetings with the European Medicines (EMA) and U.S. Food and Drug Administration (FDA), respectively. This feedback is being incorporated into the Phase 3 design, and finalization

is pending.

- The Phase 3 study is expected to initiate in the first half of 2021. The planned 48-week study will enroll approximately 50 patients, randomized 1:1 to DTX401 or placebo. Ultragenyx intends to study glycemic control by assessing the reduction in cornstarch requirements while maintaining or improving glucose control.

DTX301 for Ornithine Transcarbamylase (OTC) Deficiency: Strong, durable responses in Phase 1/2 study; Phase 3 study expected to start in second half 2021

- In the Phase 1/2 study, all six responders in the first three cohorts demonstrated durable metabolic control, including greater than two-year sustained responses in the earliest treated patients. Two of three patients in a fourth and final cohort, which utilizes prophylactic steroids, have been dosed at the same dose as Cohort 3. Both patients are doing well clinically with good metabolic control and without any safety issues. The third patient in the cohort, who has not yet been dosed due to delays related to COVID-19, is expected to be dosed this month.
- Ultragenyx has received Scientific Advice from the EMA and has an End of Phase 2 meeting scheduled with the FDA late in the first quarter of 2021. These discussions and prior discussions are being incorporated into the Phase 3 plans.
- A Phase 3 study is expected to begin in the second half of 2021. The proposed 48-week study will include approximately 50 patients, randomized 1:1 to DTX301 or placebo. The change in 24 hour ammonia levels is the expected primary endpoint, supported by change in the rate of ureagenesis as a key secondary endpoint.

GTX-102 for Angelman Syndrome: Phase 1/2 data demonstrate substantial clinical activity; Study expected to resume first half 2021 with additional data anticipated in second half 2021

- All five patients in the Phase 1/2 study demonstrated improvements in at least 3 disease domains and scores of ‘much improved’ or ‘very much improved’ in 2 disease domains (mean global change +2.4 out of +3). Disease domains include communication, behavior, sleep, gross motor function, and fine motor function as measured by the Clinical Global Impression of Improvement Scale for Angelman Syndrome (CGI-I-AS). All five patients had a grade 1 or 2 serious adverse event (SAE) of lower extremity weakness associated with local inflammation in the region of intrathecal administration in the lower back at the higher doses of GTX-102. Dosing was paused after the first SAE onset was observed and the study is on clinical hold. The SAE has fully resolved in all five patients, and clinical improvements have been sustained beyond resolution of the SAE. There have been no other safety events following discontinuation of treatment reported to date.
- Ultragenyx has submitted a substantial information amendment to the IND including follow-up safety information for the five patients dosed and toxicology data in nonhuman primates that demonstrate no evidence of this safety issue at higher repeat dosing. A safety evaluation of the SAEs included in the amendment supports causality due to local contact toxicity. An amended dosing and administration plan has been proposed to the FDA. These changes are expected to reduce the local contact time and concentration, and the new dosing plan is within the observed range of clinical activity but below doses associated with SAEs. The study is expected to resume enrollment and dosing in the first half of 2021, following resolution of FDA requests and approval to proceed.
- A Clinical Trial Application (CTA) has already been submitted in Canada and a protocol and information amendment similar to what has been proposed to FDA will be submitted. The company is in the process of expanding the study to other countries using the amended dosing and administration plan.
- Additional interim data from the study are expected in the second half 2021.

UX701 for Wilson Disease: Phase 1/2/3 IND submitted; study start expected first half 2021

- Ultragenyx has submitted an Investigational New Drug (IND) application for UX701, an AAV9 gene therapy for the treatment of Wilson Disease. UX701 has received orphan drug designation by the FDA and the EMA. The company expects to initiate a seamless single-protocol Phase 1/2/3 study in the first half of 2021.

UX053 for Glycogen Storage Disease Type III (GSDIII) Debrancher Deficiency: IND first half 2021 and study initiation second half 2021

- Ultragenyx plans to submit an IND application for UX053, an mRNA/lipid nanoparticle (LNP) for the treatment of GSDIII debrancher deficiency, in the first half of 2021 and to begin the Phase 1/2 study in the second half of 2021.

Setrusumab (UX143) for Osteogenesis Imperfecta (OI): Pediatric Phase 2/3 study expected to begin in second half 2021

- Ultragenyx is currently planning a pediatric Phase 2/3 study of setrusumab, a monoclonal antibody for the treatment of OI, a large genetic bone disorder. A separate pivotal study is also being planned for adults with OI.
- Ultragenyx announced that it entered into a collaboration and license agreement with Mereo BioPharma for setrusumab in

December 2020. The completion of the transaction is subject to Hart-Scott-Rodino Antitrust Improvements Act of 1976 (HSR) review and the satisfaction of other customary closing conditions. Mereo has previously completed a Phase 2b study of setrusumab in adults with OI that demonstrated a dose-dependent increase in bone formation, density, and strength.

Ultragenyx to Present at 39th Annual J.P. Morgan Healthcare Conference

- Dr. Kakkis will present on Tuesday, January 12, 2021 at 2:50 p.m. ET. The live and archived webcast of the presentation will be accessible from the company's website at http://ir.ultragenyx.com/events.cfm

1: Ultragenyx territories include the collaboration revenue from the North American profit share territory (U.S. and Canada) and other regions where revenue from product sales are recognized by Ultragenyx (Latin America, Turkey). This excludes the European territory revenue, which is recognized as non-cash royalty revenue since the rights were sold to Royalty Pharma in December 2019.

About Ultragenyx

Ultragenyx is a biopharmaceutical company committed to bringing novel products to patients 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, the effects from the COVID-19 pandemic on the company's clinical activities, business and operating results, risks related to reliance on third party partners to conduct certain activities on the company's behalf, uncertainty and potential delays related to clinical drug development, smaller than anticipated market opportunities for the company's products and product candidates, manufacturing risks, competition from other therapies or products, 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, the timing of clinical trial activities and reporting results from same, 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 October 27, 2020, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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