Ultragenyx Reports Preliminary 2021 Revenue and 2022 Revenue Guidance for Crysvita® in Ultragenyx Territories* And Dojolvi® Globally; Provides Pipeline Updates and 2022 Milestones

January 10, 2022

Preliminary 2021 Crysvita revenue in Ultragenyx territories of $191 million to $193 million and Dojolvi revenue of $38 million to $40 million

2022 Crysvita revenue in Ultragenyx territories expected in the range of $250 million to $260 million and Dojolvi revenue of $55 million to $65 million

Year-end 2021 cash balance of approximately $1.0 billion

NOVATO, Calif., Jan. 10, 2022 (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 2021 Crysvita® revenue in Ultragenyx territories and Dojolvi® global revenue, cash and investments at year end 2021, and provided 2022 revenue guidance for Crysvita in Ultragenyx territories and Dojolvi globally.

"We are seeing strong growth for Crysvita multiple years out from launch and expect similar progress for Dojolvi heading into year two," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "2022 will be a year of execution and momentum for us, both on the commercial side as we gear up for the launch of Evkeeza® in Europe and other key ex-US geographies, and on the clinical side as we progress our large and late-stage pipeline that includes four pivotal programs."

Ultragenyx will present at the 40th annual J.P. Morgan Healthcare Conference on Monday, January 10, 2022 at 3:45 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.

Financial Update

Crysvita: 2021 Preliminary Revenue (unaudited) and 2022 Guidance

Crysvita revenue in Ultragenyx territories* for the year ended December 31, 2021 is approximately $191 million to $193 million. This is above the guidance range of $180 million to $190 million that was provided at the beginning of 2021, notwithstanding disruptions and effects from the COVID-19 pandemic. For 2022, Crysvita revenue in the Ultragenyx territories is estimated to be between $250 million and $260 million, representing growth of 33% year-over-year at the mid-point of our guidance.

Dojolvi: 2021 Preliminary Revenue (unaudited) and 2022 Guidance

Dojolvi revenue for the year ended December 31, 2021 is approximately $38 million to $40 million. For 2022, Dojolvi revenue is estimated to be between $55 million and $65 million, representing growth of 60% year-over-year at the mid-point of our guidance in the second year of launch.

2021 Ending Cash Position (unaudited)

Cash, cash equivalents, and available-for-sale investments were approximately $1.0 billion as of December 31, 2021.

The 2021 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, 2021. The Company expects to issue full financial results for the fourth quarter and fiscal year 2021 in February 2022.

Recent Updates and 2022 Milestones

Evkeeza® for Homozygous Familial Hypercholesterolemia (HoFH): Submission of reimbursement dossiers expected in European countries in 2022

Ultragenyx and Regeneron announced a license and collaboration agreement for Ultragenyx to commercialize and distribute Evkeeza (evinacumab) in countries outside of the U.S. This includes the European Economic Area where Evkeeza was approved in June 2021 as a first-in-class monoclonal antibody targeting ANGPTL3 for use together with diet and other low-density lipoprotein-cholesterol (LDL-C) lowering therapies to treat adults and adolescents aged 12 years and older with HoFH.

Ultragenyx plans to submit reimbursement dossiers with national health authorities in Europe in 2022.

GTX-102 for Angelman Syndrome: Phase 1/2 study is titrating patients in Canada and the U.K. with data anticipated mid-year 2022

The first four patients in the Phase 1/2 study have received multiple doses of GTX-102 and regular assessments for safety. To date three have also received a preliminary assessment of clinical response. There have been no treatment-related serious adverse events of any type nor adverse events related to lower extremity weakness observed in these patients, and initial assessments have shown early signs of clinical activity.

The data safety monitoring board (DSMB) met to discuss the assessments for the first two patients in Cohort 4 (ages 4 to <8 years) and recommended that dose escalation may proceed as planned and the study may enroll the remaining four patients in this cohort. Since then, both patients in Cohort 4 met the criteria to increase their doses and have each escalated to the 5 mg dose, and Cohort 4 has been expanded and an additional patient has received their first dose. The DSMB for Cohort 5 (ages 8 to <18 years) is expected to meet soon and confirm whether enrollment for the remaining four patients in that group may commence. Data on full Cohorts 4 and 5 in the Canada/U.K. arm of the study is anticipated in mid 2022 after completing Day 128 of the protocol.

UX143 (setrusumab) for Osteogenesis Imperfecta (OI): Pivotal Phase 2/3 Orbit study expected to initiate in first half of 2022; Phase 2 study in children under age 5 planned for second half of 2022

Ultragenyx expects to initiate the seamless Phase 2/3 Orbit study of UX143 in pediatric and adult patients ages 5 to <26 in the first half of 2022. A dosing update in the Phase 2 portion and transition to Phase 3 is expected in the second half of 2022. In addition, Ultragenyx intends to initiate a Phase 2 study in children under age 5 with OI in the second half of 2022.

DTX401 for Glycogen Storage Disease Type Ia (GSDIa): Positive longer-term data from Phase 1/2 study presented at ICIEM; Phase 3 GlucoGene study initiated and screening patients

Data presented at the 14th International Congress of Inborn Errors of Metabolism (ICIEM) in November demonstrate that across all 12 patients in the Phase 1/2 study the mean reduction in daily cornstarch intake was 69.9% ($p < 0.0001$), ranging from 19-100% when comparing baseline to the most recent visit. The 12 patients in the study continued to demonstrate improved glucose control while tapering or discontinuing oral glucose replacement therapy with cornstarch up to 3 years after receiving DTX401.

The Phase 3 GlucoGene study of DTX401 in patients with GSDIa is currently screening patients with the first patient randomized and expected to receive a first dose later this month. The planned 48-week study will enroll approximately 50 patients, randomized 1:1 to DTX401 or placebo. The primary endpoint focuses on glycemic control by assessing the reduction in cornstarch requirements while maintaining or improving glucose control. Secondary endpoints include time to hypoglycemia during a controlled fasting challenge and change from baseline in the Glycogen Storage Disease Functional Assessment Diary (GSD FAD) Signs and Symptoms Scale.

DTX301 for Ornithine Transcarbamylase (OTC) Deficiency: Durable metabolic control and sustained responses in Phase 1/2 study presented at ICIEM; Phase 3 Enh3ance study expected to initiate in first half of 2022

Data presented at ICIEM in November show that the six patients who previously demonstrated a response remain clinically and metabolically stable. The longest treated responders have demonstrated a durable response up to 4 years after dosing and up to 3.5 years after discontinuing ammonia-scavenger medications and liberalizing protein-restricted diets.

Ultragenyx expects to initiate the Phase 3 Enh3ance study of DTX301 in patients with OTC in the first half of 2022. The 64-week study will include approximately 50 patients, randomized 1:1 to DTX301 or placebo. The primary endpoints are response as measured by change in baseline disease management and change in 24-hour ammonia levels, supported by change in the rate of ureagenesis as a key secondary endpoint.

UX701 for Wilson Disease: Cyprus2+ pivotal Phase 1/2/3 study currently enrolling

Ultragenyx is currently screening and enrolling patients with Wilson disease into the baseline monitoring period prior to dosing in its pivotal, seamless Phase 1/2/3 Cyprus2+ study of UX701. During the first stage of the study, the safety and efficacy of up to three dose levels of UX701 will be evaluated and a dose will be selected for further evaluation in stage 2. In stage 2, a new cohort of patients will be randomized 2:1 to receive the selected dose of UX701 or placebo. The primary efficacy endpoints are change in 24-hour urinary copper concentration and percent reduction in standard of care medication by Week 52.

UX053 for Glycogen Storage Disease Type III (GSDIII) Debrancher Deficiency: Phase 1/2 study currently dosing patients; Preliminary data from first part of study and initiation of second part of study anticipated in second half of 2022

Ultragenyx has begun to dose patients in the two-part Phase 1/2 clinical trial evaluating the safety, tolerability and efficacy of UX053 in adults age 18 and older with GSDIII. Part 1 is open label and will enroll up to 10 patients who will receive a single ascending dose of UX053 administered via intravenous infusion. Part 2 is double-blind and will evaluate five repeat doses at escalating dose levels in up to 16 patients across four cohorts randomized 3:1 to UX053 or placebo. The primary endpoints are treatment-emergent adverse events (TEAEs), serious TEAEs, and related TEAEs in both parts of the study. Secondary endpoints include pharmacokinetic parameters. Exploratory endpoints include clinician- and patient-reported outcomes, muscle strength, blood sugar, and biomarkers of liver, cardiac and muscle health.

Preliminary data from the Part 1 single ascending dose phase of the study and initiation of the Part 2 repeat dosing phase of the study is anticipated in the second half of the year.

*Ultragenyx territories for Crysvita 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) pursuant to the company's collaboration and license agreement with Kyowa Kirin Co., Ltd. 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 Pharmaceutical Inc.

Ultragenyx is a biopharmaceutical company committed to bringing novel therapies to patients for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved medicines and treatment candidates aimed at addressing diseases with high unmet medical need and clear biology, 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 and Use of Digital Media

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 and commercial activities and 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, the company's ability to achieve its projected development goals in its expected timeframes, risks and uncertainties related to the regulatory approval process, 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 (SEC) on November 3, 2021, and its subsequent periodic reports filed with the SEC.

In addition to its SEC filings, press releases and public conference calls, Ultragenyx uses its investor relations website and social media outlets to publish important information about the company, including information that may be deemed material to investors, and to comply with its disclosure obligations under Regulation FD. Financial and other information about Ultragenyx is routinely posted and is accessible on Ultragenyx's investor relations website (https://ir.ultragenyx.com/) and LinkedIn website (https://www.linkedin.com/company/ultragenyx-pharmaceutical-inc-/mycompany/).

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