Ultragenyx Reports Preliminary 2022 Revenue; Guidance for 2023 Revenue and Cash Usage; Pipeline Updates and 2023 Milestones

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Preliminary 2022 Total Product Revenue of $352 million to $356 million, Crysvita® Revenue in Ultragenyx Territories[1] of $257 million to $258 million and Dojolvi® revenue of $55 million to $56 million

2023 expected Total Product Revenue guidance between $425 million to $450 million, Crysvita revenue of $325 million to $340 million, and Dojolvi revenue of $65 million to $75 million

Year-end 2022 cash balance of approximately $900 million and 2023 guidance for net cash used in operations expected to be less than $400 million

NOVATO, Calif., Jan. 06, 2023 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel products for serious rare and ultrarare genetic diseases, today reported preliminary unaudited 2022 revenue results, cash and investments at year end 2022, and provided 2023 guidance on select key financial metrics including net cash used in operations.

"We have made substantial investments in our pipeline and manufacturing capabilities over the last two years that put us in a special position to advance multiple transformative treatments in late-stage development while continuing to drive revenue growth, delivering approved medicines globally for rare disease patients," said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. "We have also made a substantial effort to focus on key value drivers within the pipeline and reduce cash spending to maintain our strong financial position through 2023."

Ultragenyx will present at the 41th annual J.P. Morgan Healthcare Conference on Tuesday, January 10, 2023 at 9:45 a.m. PT. The live and archived webcast of the presentation will be accessible from the company's website at https://ir.ultragenyx.com/events-presentations.

Financial Update

2022 Revenue (unaudited) and 2023 Revenue Guidance ($ in millions)

	2022 Preliminary Revenue	2023 Revenue Guidance
Crysvita in Ultragenyx Territories[1]	$257 - $258	
Total Crysvita	$277 - $279	$325 - $340
Dojolvi	$55 - $56	$65 - $75
Total Product Revenue	$352 - $356	$425 - $450

In 2023, Crysvita guidance is provided for all regions where Ultragenyx will recognize revenue, including the royalties in Europe, which have been ongoing, and the royalties in North America, which will begin in April 2023. The mid-point of the Total Crysvita guidance range represents a 20% year-over-year growth rate, as compared to the same regions in 2022.

2022 Ending Cash Position (unaudited) and 2023 Cash Used in Operations Guidance
Cash, cash equivalents, and available-for-sale investments were approximately $900 million as of December 31, 2022. Cash uses in 2022 included non-recurring items of approximately $90 million for capital expenses for the gene therapy manufacturing facility, which is nearing completion, $75 million for the acquisition of GeneTx and $30 million upfront fee related to the Evkeeza® license and collaboration agreement.

In 2023 with increased focus on key pipeline value drivers, net cash use is projected to be meaningfully reduced. Net Cash Used in Operations for 2023 is expected to be less than $400 million.

The 2022 revenues and cash position included in this release are preliminary and are therefore subject to adjustment. The preliminary revenue results are based on management's initial analysis of operations for the year ended December 31, 2022. The Company expects to issue full financial results for the fourth quarter and fiscal year 2022 in February 2023.

Recent Updates and 2023 Clinical Milestones

UX143 (setrusumab) monoclonal antibody for Osteogenesis Imperfecta (OI): Phase 2/3 study dosing patients; Phase 2 data expected in mid-2023
Ultragenyx is currently dosing patients in the Phase 2/3 Orbit study of UX143 in pediatric and adult patients with OI aged five to <26 years. Enrollment completion is anticipated in early 2023 and data from the Phase 2 portion of the study is expected in mid-2023, including two-month changes in bone biomarkers response that will be used to establish the dosing algorithm for the Phase 3 portion of the study. Optimizing the dosing across the age range of the study will support meaningful benefit in the reduction of clinically evident fractures.

In addition, in the first half of 2023, Ultragenyx intends to initiate a randomized study in OI in children under age five with severe bone disease, comparing bisphosphonates to setrusumab. Younger pediatric patients with OI often have a much higher fracture rate than other age groups and a greater medical need, driving clinical urgency for better treatment options. Total fractures is expected to be the primary endpoint in the study.

GTX-102 an antisense oligonucleotide for Angelman Syndrome: Phase 1/2 continues dose exploration
Ultragenyx continues to explore the dose of GTX-102 in cohorts of younger and older patients. Expansion of the dosing cohort population is expected in the first half of 2023.

DTX401 AAV gene therapy for Glycogen Storage Disease Type Ia (GSDIa): Last patient in the Phase 3 study has entered the baseline

screening period. Phase 3 data readout expected in the first half of 2024.
The Phase 3 study has a 48-week primary efficacy analysis period, and the company has enrolled approximately 50 patients eight years of age and older, randomized 1:1 to DTX401 or placebo. The primary endpoint is the reduction in oral glucose replacement with cornstarch while maintaining glucose control. The last patient has entered the baseline screening phase of the Phase 3 study of DTX401, with Phase 3 data expected in the first half of 2024.

UX701 AAV gene therapy for Wilson Disease: Stage 1 of pivotal clinical study dosing patients; expect interim Stage 1 enrollment completion in mid-2023
The company is dosing patients in the first stage of the *Cyprus2*+ study of UX701 under a recently amended protocol that removes placebo from the dose finding stage and enrolls five patients per cohort. During this stage of the study, 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. Completion of Stage 1 enrollment is expected in mid-2023 with data on safety and initial signs of clinical activity expected in early 2024.

DTX301 AAV gene therapy for Ornithine Transcarbamylase (OTC) Deficiency: Phase 3 study expected to initiate in the first quarter of 2023
Ultragenyx expects to initiate the Phase 3 study of DTX301 in patients with OTC in the first quarter of 2023. The 64-week study will include approximately 50 patients, randomized 1:1 to DTX301 or placebo. The primary endpoints are response as measured by removal of ammonia-scavenger medications and protein-restricted diet and change in 24-hour ammonia levels.

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 is 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 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, including under our collaboration agreement with Kyowa Kirin, our limited experience in generating revenue from product sales, risks related to product liability lawsuits, our dependence on Kyowa Kirin for the commercial supply of Crysvita, fluctuations in buying or distribution patterns from distributors and specialty pharmacies, the transition back to Kyowa Kirin of our exclusive rights to promote Crysvita in the United States and Canada and unexpected costs, delays, difficulties or adverse impact to revenue related to such transition, 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, 2022, 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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