Ultragenyx Reports Preliminary 2023 Revenue; Guidance for 2024 Revenue and Cash Usage; Pipeline Updates and 2024 Milestones

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Preliminary 2023 Total Revenue of $430 million to $435 million, Crysvita® Revenue of $325 million to $330 million and Dojolvi® revenue of $70 million to $71 million

2024 expected Total Revenue guidance between $500 million to $530 million, Crysvita revenue of $375 million to $400 million, and Dojolvi revenue of $75 million to $80 million

Year-end 2023 cash balance of approximately $776 million and 2024 guidance for net cash used in operations expected to be less than $400 million

NOVATO, Calif., Jan. 07, 2024 (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 2023 revenue results, cash and investments at year end 2023, and provided 2024 guidance on select key financial metrics including net cash used in operations.

“2024 is poised to be a year of significant momentum for Ultragenyx, with clinical catalysts across multiple value-driving programs, meaningful revenue growth from our commercial products, and continued focus on financial discipline,” said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. “We anticipate several important milestones in the first half of the year, including completing enrollment of our Phase 3 studies in osteogenesis imperfecta, interim data on a substantive portion of patients enrolled in our Phase 1/2 study in Angelman syndrome, Stage 1 data from our pivotal Phase 1/2/3 study in Wilson disease, and topline Phase 3 data from our GSDIa gene therapy program.”

Ultragenyx will present at the 42th annual J.P. Morgan Healthcare Conference on Monday, January 8, 2024 at 3:00 p.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

2023 Revenue (unaudited) and 2024 Revenue Guidance

($ in millions)	2023 Preliminary Revenue	2024 Revenue Guidance
Crysvita	$325 - $330	$375 - $400
Dojolvi	$70 - $71	$75 - $80
Total Revenue	$430 - $435	$500 - $530

2023 Ending Cash Position (unaudited) and 2024 Cash Used in Operations Guidance
Cash, cash equivalents, and available-for-sale investments were approximately $776 million as of December 31, 2023. Cash uses in 2023 included the completion of construction of our gene therapy manufacturing facility. With forecasted ~20% increases in revenue and continued focus on key pipeline value drivers, 2024 net cash use is projected to be less than $400 million.

The 2023 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, 2023. The Company expects to issue full financial results for the fourth quarter and fiscal year 2023 in February 2024.

Recent Updates and 2024 Clinical Milestones

UX143 (setrusumab) monoclonal antibody for Osteogenesis Imperfecta (OI): Phase 3 portion of Orbit study expected to be fully enrolled in the first quarter of 2024

Patients are being dosed in the late-stage clinical trials, *Orbit* and *Cosmic*, which evaluate setrusumab in pediatric and young adult patients with OI. The randomized, placebo-controlled Phase 3 portion of the *Orbit* study is expected to be fully enrolled around the end of the first quarter of 2024. Additional longer-term Phase 2 data from the *Orbit* study are expected in 2024. The Phase 3 *Cosmic* study is an active-controlled study evaluating the effect of setrusumab compared to intravenous bisphosphonate (IV-BP) therapy on annualized total fracture rate in patients aged 2 to <5 years. *Cosmic* is targeting to enroll approximately 65 patients at more than 20 global sites and is expected to complete enrollment in the first half of 2024.

GTX-102 an antisense oligonucleotide for Angelman Syndrome: Phase 1/2 fully enrolled; expansion data expected in the first half of 2024
Enrollment in the expansion cohorts was completed in December 2023 with a total of 53 patients enrolled. There are a total of 74 patients enrolled in the Phase 1/2 study including the dose escalation/extension study patients. The expansion cohorts will evaluate many of the same safety, pharmacokinetic, and efficacy measures as the previously enrolled dose escalation cohorts plus some new evaluations. The next data update is expected in the first half of 2024 and is planned to include at least 20 expansion cohort patients with a minimum of Day 170 data.

UX701 AAV gene therapy for Wilson Disease: Last patient in Cohort 3 on track to be dosed soon; expect interim Stage 1 data in the first half of 2024
Four of five patients in the third of three dose escalation cohorts in the pivotal study have been dosed, with the fifth patient scheduled to be dosed soon. During Stage 1, the safety and efficacy of UX701 will be evaluated and a dose will be selected for further evaluation in Stage 2, the pivotal, randomized, placebo-controlled stage of the study. Data from Stage 1 are expected in the first half of 2024, which will be followed by dose selection and initiation of Stage 2 in the second half of 2024.

UX111 AAV gene therapy for Sanfilippo syndrome (MPS IIIA): Updated data from pivotal Transfer A study to be presented at

WORLDSymposium™ in February 2024
Ultragenyx will present new data from the ongoing pivotal *Transfer A* study evaluating the efficacy and safety of UX111 in children with MPS IIIA at the 20th Annual WORLD*Symposium™*. The presentation will show that reductions of heparan sulfate exposure in cerebrospinal fluid correlate with improved long-term cognitive function in patients with MPS IIIA following treatment with UX111. Discussions with FDA seeking an accelerated review path are ongoing.

DTX401 AAV gene therapy for Glycogen Storage Disease Type Ia (GSDIa): Dosing in Phase 3 study complete; Phase 3 data readout expected in the first half of 2024
In May 2023, Ultragenyx announced the last patient had been dosed in the Phase 3 study. The 48-week study has fully enrolled 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. Phase 3 data are expected in the first half of 2024.

DTX301 AAV gene therapy for Ornithine Transcarbamylase (OTC) Deficiency: Phase 3 study dosing patients; expect enrollment to be completed in the first half of 2024
Ultragenyx is randomizing and dosing patients in the ongoing Phase 3 study. The pivotal, 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. Enrollment is currently expected to be completed in the first half of 2024.

About Ultragenyx

Ultragenyx is a biopharmaceutical company committed to bringing novel products to patients for the treatment of serious rare and ultrarare 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 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 the Company's clinical development programs, commercial success of its products and product candidates, continued 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 uncertainty of clinical drug development and unpredictability and lengthy process for obtaining regulatory approvals, risks related to serious or undesirable side effects of our product candidates, the company's ability to achieve its projected development goals in its expected timeframes, risks related to reliance on third party partners to conduct certain activities on the company's behalf, 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 candidate . 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, 2023, 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-/).

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