Ultragenyx Provides Financial and Business Updates at J.P. Morgan Annual Healthcare Conference

January 12, 2026

Preliminary 2025 total revenue of $672 million to $674 million, exceeding top end of guidance

Preliminary cash and investments of approximately $735 million as of December 31, 2025

Anticipated 2026 catalysts include two potential approvals and pivotal Phase 3 data from the GTX-102 Phase 3 Aspire study for Angelman syndrome

NOVATO, Calif., Jan. 12, 2026 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel therapies for serious rare and ultra-rare genetic diseases, today reported preliminary unaudited 2025 revenue results, cash and investments at year end 2025, and provided a corporate update.

“In 2025, we delivered another year of significant revenue growth, with preliminary revenue exceeding the top end of guidance," said Emil Kakkis, chief executive officer and president of Ultragenyx. "The year ahead will be transformational for our company and the rare disease communities we serve, with two potential gene therapy approvals of first-ever treatments, and a pivotal data readout from our Phase 3 program in Angelman syndrome, setting the stage for our next phase of growth."

Financial Update
2025 Preliminary Revenue (unaudited)
Total revenue for 2025 is estimated to be $672 million to $674 million, which exceeds the top end of the guidance range, and represents approximately 20% growth versus 2024. Crysvita® revenue for 2025 is estimated to be $480 million to $482 million, which exceeds the top end of the guidance range, and represents approximately 17% growth versus 2024. Dojolvi® revenue for 2025 is estimated to be $95 million to $97 million, at the midpoint of the guidance range, and represents approximately 9% growth versus 2024.

2025 Ending Cash Position (unaudited)
Cash and investments were approximately $735 million as of December 31, 2025.

The 2025 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, 2025. The Company expects to issue financial guidance for fiscal year 2026, including details on previously announced planned expense reductions, and full financial results for the fourth quarter and fiscal year 2025, in February 2026.

2026 Clinical and Regulatory Catalysts

- **DTX401 (pariglasgene brecaparvovec) AAV gene therapy for glycogen storage disease type Ia (GSDIa):** Biologics License Application (BLA) rolling submission completed in December 2025, with an anticipated PDUFA date in the third quarter of 2026.
- **UX111 (rebisufligene etisparvovec) AAV gene therapy for Sanfilippo syndrome type A (MPS IIIA):** Resubmission of the BLA is on track for early in 2026 and will be followed by an up to 6-month review per FDA regulations.
- **GTX-102 (apazunersen) antisense oligonucleotide (ASO) for the treatment of Angelman syndrome (AS):** Data from the fully enrolled, pivotal, Phase 3 Aspire study in patients with a genetically confirmed diagnosis of *UBE3A* deletion is expected in the second half of 2026. The Phase 2/3 Aurora study is also underway in other genotypes and ages, with first patient dosed in October 2025.
- **UX701 (rivunatpagene miziparvovec) AAV gene therapy for Wilson disease:** Enrollment is complete for the fourth cohort in the ongoing, dose-finding stage of the pivotal *Cyprus2+* study. Data are expected in the first half of 2026.

Data from the Phase 3 Orbit and Cosmic studies of UX143 (setrusumab) in osteogenesis imperfecta, including data on bone mineral density, vertebral fractures, and patient reported outcomes on pain and physical function, will be presented at the J.P. Morgan Healthcare Conference.

The company presentation is scheduled for Monday, January 12, 2026 at 10:30 AM PT. The live and archived webcast of the presentation will be accessible from the company’s website at https://ir.ultragenyx.com/events-presentations.

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, the components and timing of regulatory submissions and the timing of results from ongoing clinical studies 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 commercialization of Crysvita in certain major markets, including the U.S. and Canada, and for our commercial supply of Crysvita in those markets, fluctuations in buying or distribution patterns from distributors and specialty pharmacies, , smaller than anticipated market opportunities for the company's products and product candidates, manufacturing risks, our ability to successfully manage the expansion of our company, competition from other therapies or products, regulatory scrutiny of the company's products and product candidates, the company's limited experience as a company in operating its own manufacturing facility, market acceptance of our products, uncertainty related to insurance coverage and reimbursement, 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 4, 2025, 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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