

Contacts Ultragenyx Pharmaceutical Inc.
Investors
Joshua Higa
ir@ultragenyx.com

Media
Jess Rowlands
media@ultragenyx.com

Ultragenyx Reports Fourth Quarter and Full Year 2025 Financial Results and Corporate Update

*2025 total revenue of \$673 million,
Crysvita® revenue of \$481 million and Dojolvi® revenue of \$96 million*

2026 total revenue from current products expected to be between \$730 million to \$760 million

Initiated a strategic restructuring plan to significantly reduce and focus expenses and headcount, reiterate path to profitability in 2027

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

NOVATO, Calif. – February 12, 2025 – 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 its financial results for the quarter and year ended December 31, 2025.

“The year ahead marks an important turning point for the company, as we approach two potential product launches and a pivotal data readout that, together, could significantly accelerate our commercial revenue trajectory,” said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. “We are implementing a strategic restructuring plan to reduce our operating expenses and ensure our resources are squarely aligned with our highest-impact opportunities, while leading the future of rare disease with multiple first ever treatments.”

Fourth Quarter 2025 Selected Financial Data Tables and Financial Results

Revenues (dollars in millions), (unaudited).

	Three Months Ended December 31,		Year Ended December 31,	
	2025	2024	2025	2024
Crysvita				
Product sales - Latin America and Türkiye	\$ 40	\$ 22	\$ 177	\$ 135
Royalty revenue - U.S. and Canada	97	87	275	249
Royalty revenue - Europe	8	7	29	26
Total Crysvita Revenue	145	116	481	410
Dojolvi	32	31	96	88
Evkeeza	17	10	59	32
Mepsevii	13	8	37	30
Total revenues	\$ 207	\$ 165	\$ 673	\$ 560

Revenues

Ultragenyx reported \$207 million in total revenue for the fourth quarter 2025, which represents 25% growth compared to the same period in 2024. Crysvita revenue in the fourth quarter 2025 was \$145 million, which includes product sales of \$40 million from Latin America and Türkiye. Dojolvi revenue in the fourth quarter 2025 was \$32 million. Evkeeza revenue in the fourth quarter 2025 was \$17 million.

Total revenue for the year ended December 31, 2025 was \$673 million, which represents 20% growth compared to the prior year. Full year 2025 Crysvita revenue was \$481 million, which represents 17% growth compared to the prior year. This includes product sales of \$177 million from Latin America and Türkiye, which represents 31% growth compared to the prior year. Dojolvi revenue in 2025 was \$96 million, which represents 9% growth compared to the prior year. Evkeeza revenue in 2025 was \$59 million, which represents 84% growth compared to the prior year, as demand continues to build following launches in the company’s territories outside of the United States.

Selected Financial Data (dollars in millions, except per share amounts), (unaudited).

	Three Months Ended December 31,		Year Ended December 31,	
	2025	2024	2025	2024
Total revenues	\$ 207	\$ 165	\$ 673	\$ 560
Operating expenses:				
Cost of sales	29	17	109	77
Research and development	203	188	750	698
Selling, general and administrative	89	82	349	321
Total operating expenses	321	287	1,208	1,096
Net loss	\$ (129)	\$ (133)	\$ (575)	\$ (569)
Net loss per share, basic and diluted	\$ (1.29)	\$ (1.39)	\$ (5.83)	\$ (6.29)

Operating Expenses

Total operating expenses for the fourth quarter 2025 were \$321 million. Total operating expenses for the year ended December 31, 2025 were \$1.2 billion, including \$153 million of non-cash stock-based compensation.

Net Loss

Net loss for the fourth quarter 2025 was \$129 million, or \$1.29 per share basic and diluted, compared with a net loss for the fourth quarter 2024 of \$133 million, or \$1.39 per share basic and diluted. Net loss for the year ended December 31, 2025 was \$575 million, or \$5.83 per share basic and diluted, compared with a net loss the prior year of \$569 million, or \$6.29 per share, basic and diluted.

Cash Balance and Net Cash Used in Operations

Cash, cash equivalents, and marketable securities were \$737 million as of December 31, 2025. For the three months ended December 31, 2025, net cash used in operations was \$100 million and for the year ended December 31, 2025 was \$466 million.

Financial Guidance and Strategic Restructuring Plan

2026 revenue guidance

- Total revenues, excluding potential revenue from new product launches, in the range of \$730 million to \$760 million, an increase of 8% to 13% compared to 2025
- Crysvita revenue in the range of \$500 million to \$520 million, reflecting growing underlying global demand partially offset by expected timing of ordering patterns in Brazil
- Dojolvi revenue in the range of \$100 million to \$110 million

Strategic restructuring plan and path to profitability in 2027

Ultragenyx has initiated a strategic restructuring plan designed to reduce its headcount and expenses and focus resources on its largest value drivers. The significant reduction and partial reinvestment of expenses, and the planned growth in revenue from current and new product launches, are designed to keep the company on its path to profitability in 2027.

Today, in connection with the restructuring plan, the company announced a 10% workforce reduction, impacting approximately 130 employees.

Based on the progression of the business and the reductions from the restructuring plan:

- In 2026, combined R&D and SG&A expenses are expected to be flat to down low-single digits versus 2025. This includes the impact of spend reductions and approximately \$50 million for severance, manufacturing, and other non-recurring restructuring charges
- In 2027, R&D expenses are expected to decrease from 2025 levels by 38%, or approximately \$280 million, driven by the completion of multiple phase 3 studies and reduction of early-stage research efforts. SG&A expenses are expected to increase in support of new product launches and existing approved products. On a combined basis, 2027 R&D and SG&A expenses are expected to decrease at least 15% versus 2025.

2026 Clinical and Regulatory Catalysts

- DTX401 (pariglasgene brecaparvovec) AAV8 gene therapy for glycogen storage disease type Ia (GSDIa): Biologics License Application (BLA) rolling submission completed in December 2025, with an anticipated Prescription Drug User Fee Act (PDUFA) action date in the third quarter of 2026.
- UX111 (rebisufligene etispavovec) AAV9 gene therapy for Sanfilippo syndrome type A (MPS IIIA): The BLA was resubmitted in January 2026 and included substantial longer-term data, that were recently presented at the 2026 *WORLDSymposium*, on multiple measures of neurologic benefit to support an intermediate clinical endpoint for accelerated approval supported further by CSF heparan sulfate and other biomarker data, as agreed with the FDA during the last clinical review.

Earlier today the company received an Incomplete Response Letter (IRL) regarding its resubmitted BLA. The IRL requests additional supportive documentation related to its CRL CMC responses, which the company will provide in a resubmission.

- 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. Enrollment in the Phase 2/3 *Aurora* study is also underway in other genotypes and ages, with the first patient dosed in October 2025.

- UX701 (rivunatpagene miziparvovec) AAV9 gene therapy for Wilson disease: Enrollment is complete for the fourth cohort in the ongoing, dose-finding stage of the pivotal *Cyprus2+* study. Data from this stage are expected in 2026.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Thursday, February 12, 2026, at 2 p.m. PT/5 p.m. ET to discuss the fourth quarter and full year 2025 financial results and provide a corporate update. The live and replayed webcast of the call will be available through the company's website at <https://ir.ultragenyx.com/events-presentations>. The replay of the call will be available for three months.

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, including the company's expectations for profitability in 2027, anticipated cost or expense reductions, including the company's expectations related to benefits and savings from the strategic restructuring plan, the timing, progress and plans for its clinical programs and clinical studies, future regulatory interactions, the components and timing of regulatory submissions, the company's ability to provide the requested documentation and address the comments in the CRL for UX111 to the satisfaction of the FDA, the timing of FDA review of the company's BLA submissions, the timing and outcome of any FDA inspections related to UX111 or other clinical product candidates, the timing of future regulatory interactions related to the company's clinical product candidates 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, delays or unexpected costs and other adverse effects related to the strategic restructuring plan, 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 5, 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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Ultragenyx Pharmaceutical Inc.
Selected Statement of Operations Financial Data
(in millions, except per share amounts)
(unaudited)

	Three Months Ended December 31,		Year Ended December 31,	
	2025	2024	2025	2024
Statement of Operations Data:				
Revenues:				
Product sales	\$ 102	\$ 72	\$ 369	\$ 285
Royalty revenue	105	93	304	275
Total revenues	207	165	673	560
Operating expenses:				
Cost of sales	29	17	109	77
Research and development	203	188	750	698
Selling, general and administrative	89	82	349	321
Total operating expenses	321	287	1,208	1,096
Loss from operations	(114)	(122)	(535)	(536)
Non-cash interest expense on liabilities for sales of future royalties	(19)	(15)	(62)	(63)
Other income, net	5	4	26	32
Loss before income taxes	(128)	(133)	(571)	(567)
Provision for income taxes	(1)	—	(4)	(2)
Net loss	\$ (129)	\$ (133)	\$ (575)	\$ (569)
Net loss per share, basic and diluted	\$ (1.29)	\$ (1.39)	\$ (5.83)	\$ (6.29)
Shares used in computing net loss per share, basic and diluted	99.9	95.7	98.6	90.5

Ultragenyx Pharmaceutical Inc.
Selected Activity included in Operating Expenses
(in millions)
(unaudited)

	Three Months Ended December 31,		Year Ended December 31,	
	2025	2024	2025	2024
Non-cash stock based compensation	\$ 38	\$ 40	\$ 153	\$ 158
GTX-102 clinical milestone	—	\$ 30	—	\$ 30

Ultragenyx Pharmaceutical Inc.
Selected Balance Sheet Financial Data
(in millions)
(unaudited)

	December 31, 2025	December 31, 2024
Balance Sheet Data:		
Cash, cash equivalents, and marketable securities	\$ 737	\$ 745
Working capital	567	473
Total assets	1,532	1,503
Total stockholders' equity (deficit)	(80)	255