Ultragenyx Reports First Quarter 2026 Financial Results and Corporate Update

May 5, 2026

First quarter total revenue of $136 million, Crysvita® revenue of $93 million and Dojolvi® revenue of $18 million

Reaffirm 2026 financial guidance, including total revenue of $730 million to $760 million and combined R&D and SG&A expenses to be flat to slightly down versus 2025; remain on path to profitability in 2027

GTX-102 for Angelman syndrome: New longer-term data from Phase 1/2 clinical study support durable and improving effects across multiple domains; Phase 3 data expected in the second half of 2026

NOVATO, Calif., May 05, 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 its financial results for the quarter ended March 31, 2026 and reaffirmed its financial guidance for 2026.

“This is an important year for Ultragenyx with two BLAs under review and our Angelman Phase 3 readout coming soon. We have the opportunity to meaningfully accelerate our consistent commercial revenue growth over the last few years as we prepare for two potential gene therapy approvals and launches in two urgent diseases without any approved therapies,” said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. “The latest long-term GTX-102 Phase 1/2 data further support the potential of the program, as it heads toward Phase 3 results later this year.”

First Quarter 2026 Revenue Highlights and 2026 Revenue Guidance

- **Total revenue** in the first quarter of 2026 was $136 million. The company reaffirms its full year 2026 total revenue guidance of $730 million to $760 million, which excludes revenue from potential new product launches.
- **Crysvita** revenue in the first quarter of 2026 was $93 million, consistent with expected seasonality in the U.S. and Canada and ordering patterns in Brazil. The company reaffirms its full year 2026 Crysvita revenue guidance of $500 million to $520 million.
- **Dojolvi** revenue in the first quarter 2026 was $18 million. The company reaffirms its full year 2026 Dojolvi revenue guidance of $100 million to $110 million.
- **Evkeeza®** revenue in the first quarter 2026 was $18 million, driven by increased demand from new country launches and early access.
- **Mepsevii®** revenue in the first quarter 2026 was $7 million.

Recent Clinical Milestones and 2026 Catalysts

- **GTX-102 (apazunersen) antisense oligonucleotide (ASO) for the treatment of Angelman syndrome (AS):** As of a March 2026 Phase 1/2 data cut-off date, a total of 74 patients had been treated with GTX-102, with 66 patients continuing in the long-term extension (LTE) study. Phase 1/2 patients have been on continuous treatment for an average of more than three years, with some patients now in their fifth year, generally receiving the 14 mg quarterly maintenance dose. Patients have continued to show positive improvements across multiple domains and continued to gain ground developmentally. GTX-102 has maintained a consistent safety profile, sustained over multiple years of chronic treatment, while demonstrating no new cases of transient lower extremity weakness nor any other recurring drug-related serious adverse events. These updated efficacy and safety data are planned to be presented at a future scientific meeting.

  The Phase 3 *Aspire* study, in patients with a full maternal *UBE3A* gene deletion, enrolled 129 patients, randomized 1:1 to GTX-102 or sham. Data from this study are expected in the second half of 2026.

  Enrollment in the open-label Phase 2/3 *Aurora* study, evaluating GTX-102 in other genotypes and ages, began enrollment in October 2025 and is expected to complete enrollment in the second half of 2026.

- **DTX401 (pariglasgene brecaparvovec) AAV8 gene therapy for the treatment of glycogen storage disease type Ia (GSDIa):** In February 2026, the U.S. Food and Drug Administration (FDA) accepted for review the Biologics License Application (BLA) seeking approval of DTX401 as a treatment for GSDIa, granted the BLA Priority Review, and assigned a Prescription Drug User Fee Act (PDUFA) action date of August 23, 2026. The FDA also recently informed the company that an Advisory Committee meeting is not anticipated at this time.

- **UX111 (rebisufligene etisparvovec) AAV9 gene therapy for the treatment of Sanfilippo syndrome type A (MPS IIIA):** In April 2026, the FDA accepted for review the resubmitted BLA seeking accelerated approval for UX111 as a treatment for MPS IIIA. The resubmitted BLA included substantial longer-term data that were presented in February at the WORLD*Symposium*™2026 and included up to eight years of follow-up. These data demonstrated further clinical improvement relative to the decline observed in natural history studies, and showed a durable treatment effect across clinical evaluations and multiple biomarkers, while maintaining an acceptable safety profile. In February 2025, the FDA granted the BLA Priority Review and, in April 2026, assigned a PDUFA action date of September 19, 2026.

- **DTX301 (avalotcagene ontaparvovec) AAV8 gene therapy for the treatment of Ornithine Transcarbamylase, or OTC, deficiency:** As announced in March 2026, at Week 36 in the randomized, double-blind placebo-controlled period of the Phase 3 study, DTX301 patients (n=18) demonstrated a statistically significant and clinically meaningful 18% (p=0.018) reduction in 24-hour plasma ammonia (AUC_{0-24}) into the normal range compared to placebo (n=19). Eight of nine patients with abnormal ammonia AUC_{0-24} at baseline also reached normal ammonia levels rapidly, which were generally maintained during this treatment period. At Week 24, patient global impression scale (PGIC) for overall OTC symptoms (n=15) showed 71% of DTX301 patients were much improved (equivalent to +3), compared to 0% of placebo patients. DTX301 was well tolerated with an acceptable safety profile.

  Per the protocol, the study is continuing to its second primary endpoint, which evaluates reduction in treatment burden, including use of

ammonia scavengers and dietary management, across both the treatment and placebo-crossover groups following treatment with DTX301 through 64 weeks of follow-up. Data are expected in the first half of 2027.

- **UX701 (rivunatpagene miziparvovec) AAV9 gene therapy for the treatment of 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.
- **UX016 novel prodrug for sialic acid used as a substrate replacement therapy for the treatment of GNE myopathy:** The FDA cleared the Investigational New Drug (IND) application for UX016 and an externally funded Phase 1/2 study is expected to begin in the second half of 2026.

Summary of First Quarter 2026 Financial Results

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

	Three Months Ended March 31,	
	2026	**2025**
Total revenues	$ 136	$ 139
Operating expenses:		
Cost of sales	30	29
Research and development	187	166
Selling, general and administrative	88	87
Total operating expenses	305	282
Net loss	$ (185)	$ (151)
Net loss per share, basic and diluted	$ (1.84)	$ (1.57)

Operating Expenses

Total operating expenses for the first quarter 2026 were $305 million, including $30 million of non-cash stock-based compensation and $30 million of expense related to the restructuring announced last quarter. The company reaffirms its full year 2026 and 2027 guidance for combined R&D and SG&A operating expenses: compared to 2025, combined R&D and SG&A expenses in 2026 are expected to be flat to down low-single digits, and combined R&D and SG&A expenses in 2027 are expected to decrease by at least 15%.

Net Loss

Net loss for the first quarter 2026 was $185 million, or $1.84 per share basic and diluted, compared with a net loss for the first quarter 2025 of $151 million, or $1.57 per share basic and diluted.

Cash Balance and Net Cash Used in Operations

Cash, cash equivalents, and marketable securities were $534 million as of March 31, 2026. For the three months ended March 31, 2026, net cash used in operations was $197 million and includes the payment of annual bonuses and $38 million of payments related to UX143 manufacturing activities.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Tuesday, May 5, 2026, at 2 p.m. PT/5 p.m. ET to discuss the first quarter 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 Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on February 18, 2026, 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-/).

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

Media
Jess Rowlands
media@ultragenyx.com

Ultragenyx Pharmaceutical Inc.
Selected Revenue Data
(in millions)
(unaudited)

	Three Months Ended March 31,	
	2026	**2025**
Crysvita		
Product sales - Latin America and Türkiye	$ 46	$ 55
Royalty revenue - U.S. and Canada	39	41
Royalty revenue - Europe	8	7
Total Crysvita Revenue	93	103
Dojolvi	18	17
Evkeeza	18	11
Mepsevii	7	8
Total revenues	$ 136	$ 139

Ultragenyx Pharmaceutical Inc.
Selected Statement of Operations Financial Data
(in millions, except per share amounts)
(unaudited)

	Three Months Ended March 31,	
	2026	**2025**
Statement of Operations Data:		
Revenues:		
Product sales	$ 89	$ 91
Royalty revenue	47	48
Total revenues	136	139
Operating expenses:		
Cost of sales	30	29
Research and development	187	166
Selling, general and administrative	88	87
Total operating expenses	305	282
Loss from operations	(169)	(143)
Non-cash interest expense on liabilities for sales of future royalties	(21)	(14)
Other income, net	6	7
Loss before income taxes	(184)	(150)
Provision for income taxes	(1)	(1)
Net loss	$ (185)	$ (151)
Net loss per share, basic and diluted	$ (1.84)	$ (1.57)

Shares used in computing net loss per share, basic and diluted	100.6	96.3

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

	Three Months Ended March 31,	
	2026	**2025**
Non-cash stock-based compensation	$ 30	$ 40
Restructuring expense	$ 30	—

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

	March 31, 2026	**December 31, 2025**
Balance Sheet Data:		
Cash, cash equivalents, and marketable securities	$ 534	$ 737
Working capital	332	567
Total assets	1,296	1,532
Total stockholders' equity (deficit)	(236)	(80)