Ultragenyx Reports Third Quarter 2026 Financial Results, Announces Substantial Expense Reductions, and Reaffirms Profitability in 2027

November 3, 2026

Third quarter total revenue of $173 million, CRYSVITA® revenue of $109 million and DOJOLVI® revenue of $30 million

GENGLYCOS™ and FAYUVI™ BLAs approved by FDA in the third quarter

Two Priority Review Vouchers (PRVs) under agreement for combined sales price of $413 million

Substantial reduction of expenses and headcount underway, expected to drive at least a 50% reduction in 2027 R&D expenses vs 2025

NOVATO, Calif., Nov. 03, 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 September 30, 2026, announced substantial expense reductions, and reaffirmed its guidance for 2026 revenue and 2027 profitability.

"We received two BLA approvals from the FDA within one month for our first gene therapy treatments and the launches are progressing well with growing demand for these products," said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. "With substantial reductions in headcount and expenses, combined with two launches and continued growth in our base business, we expect meaningful profitability in 2027 and going forward."

Third Quarter 2026 Revenue Highlights and 2026 Revenue Guidance

- **Total revenue** in the third quarter 2026 was $173 million. The company reaffirms its full year 2026 total revenue guidance of $730 million to $760 million, which excludes revenue from new product launches.
- **CRYSVITA** revenue in the third quarter 2026 was $109 million, consistent with expected ordering patterns. The company reaffirms its full year 2026 Crysvita revenue guidance of $500 million to $520 million.
- **DOJOLVI** revenue in the third quarter 2026 was $30 million. The company reaffirms its full year 2026 Dojolvi revenue guidance of $100 million to $110 million.
- **EVKEEZA®** revenue in the third quarter 2026 was $24 million, driven by increased demand from new country launches and early access.
- **MEPSEVII®** revenue in the third quarter 2026 was $10 million.

Summary of Third Quarter 2026 Financial Results

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Total revenues	$ 173	$ 160	$ 523	$ 466
Operating expenses:				
Cost of sales	32	28	96	80
Research and development	154	216	508	547
Selling, general and administrative	97	87	273	261
Total operating expenses	283	331	877	888
Net loss	$ (118)	$ (180)	$ (395)	$ (446)
Net loss per share, basic and diluted	$ (1.16)	$ (1.81)	$ (3.89)	$ (4.55)

Operating Expenses

Total operating expenses for the third quarter 2026 were $283 million, including $34 million of non-cash stock-based compensation.

Net Loss

Net loss for the third quarter 2026 was $118 million, or $1.16 per share basic and diluted, compared with a net loss for the third quarter 2025 of $180 million, or $1.81 per share basic and diluted.

Cash Balance and Net Cash Used in Operations
Cash, cash equivalents, and marketable securities were $401 million as of September 30, 2026. For the three months ended September 30, 2026, net cash used in operations was $43 million.

In October 2026, the Company entered into two definitive agreements to sell the PRVs received upon the approval of GENGLYCOS and FAYUVI for a combined price of $413 million. The sale of the GENGLYCOS PRV closed last week and the FAYUVI PRV sale is expected to close later this month. Net proceeds from both sales are expected to be approximately $350 million.

Initiation of substantial expense reductions and reaffirming profitability in 2027

With the recent completion of late-stage development programs, including first-ever FDA approvals in glycogen storage disease type Ia (GSDIa) and Sanfilippo syndrome type A, Ultragenyx is entering a new stage focused on commercial execution and a smaller, more targeted R&D footprint. To support this transition, the company has initiated substantial headcount and expense reductions and expects to reduce 2027 R&D expenses to less than $350 million and 2027 SG&A expenses to less than $375 million. Savings actions include:

- Reducing headcount by approximately 20%, resulting in a revised workforce of approximately 1,000 employees
- Completing wind-down of all ongoing studies for GTX-102 for Angelman syndrome
- Terminating UX701 for Wilson disease based on not achieving study goals
- Reducing expenses across the company, including reduction of real estate footprint

In addition to executing these critical expense reductions, the company is focused on driving successful launches for GENGLYCOS and FAYUVI and continuing to grow current product revenues. R&D investments will be focused on delivering primary data from the responder endpoint of the ongoing Phase 3 study for DTX301 for ornithine transcarbamylase deficiency, determining if there is a viable path forward for UX143 (setrusumab) for osteogenesis imperfecta, and measured spending to progress early-stage programs for future INDs.

Fourth quarter 2026 financial results are expected to include approximately $25 million of severance and other non-recurring restructuring charges, and the reduction in force is expected to be substantially completed by the end of 2026. The company continues to anticipate achieving full-year GAAP profitability in 2027 and going forward.

Recent Corporate Updates and Upcoming Milestones

- **GENGLYCOS (pariglasgene brecaparvovec) AAV8 gene therapy for the treatment of glycogen storage disease type Ia (GSDIa):** In August 2026, the U.S. Food and Drug Administration (FDA) granted accelerated approval for GENGLYCOS as a treatment for adult and pediatric patients eight years and older with GSDIa. The company received a PRV upon this approval and sold it for $210 million.

- **FAYUVI (rebisufligene etisparvovec) AAV9 gene therapy for the treatment of Sanfilippo syndrome type A (MPS IIIA):** In September 2026, the FDA granted standard full approval for FAYUVI as a treatment of pediatric patients with Sanfilippo syndrome Type A. The company received a PRV upon this approval and, in October 2026, entered into a definitive agreement to sell the PRV for $203 million.

- **UX701 (rivunatpagene miziparvovec) AAV9 gene therapy for the treatment of Wilson disease:** Additional data in the dose-finding stage of the *Cyprus2+* study of UX701 for the treatment of Wilson disease, including data from the fourth dosing cohort, demonstrated clinical activity in some patients, but did not meet the criteria that the majority of patients achieve biochemical response and elimination of standard of care, to advance the program to the pivotal Phase 3, stage of development. UX701 demonstrated a favorable safety profile across all cohorts dosed. Data from the completed study is expected to be presented at an upcoming medical or scientific congress.

  The company will terminate further development of this program and is working with clinical sites and patient advocacy groups to support the patient community through this transition.

- **UX016 novel prodrug for sialic acid used as a substrate replacement therapy for the treatment of GNE myopathy:** The Phase 1/2 study is evaluating safety and dose of UX016 and its impact on muscle strength in adults with GNEM. The UX016 program is fully funded through clinical proof-of-concept by an external venture philanthropy agreement with a patient group, which supports all costs for the Phase 1/2 study and dosing will be initiated soon.

- **DTX301 (avalotcagene ontaparvovec) AAV8 gene therapy for the treatment of Ornithine Transcarbamylase, or OTC, deficiency:** The Phase 3 *Enh3ance* placebo-controlled study showed substantial improvement in ammonia control and reduction in the need for scavenger medicines along with profound improvement in global impression scale. The study continues with patients in both treatment and cross-over groups progressing through 64 weeks of follow-up to evaluate the second primary endpoint of the reduction in the use of ammonia scavengers and dietary management. Data are expected in the first half of 2027.

- **UX143 (setrusumab) monoclonal antibody for the treatment of osteogenesis imperfecta:** Phase 3 data with pre- and post-hoc analyses were presented at the American Society for Bone and Mineral Research 2026 Annual Meeting showing a meaningful benefit on bone mineral density, fractures, and other endpoints in the two Phase 3 studies. The company will continue to engage regulatory agencies to determine the necessary data to support a regulatory filing and if there is a potential path forward for UX143.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Tuesday, November 3, 2026, at 2 p.m. PT/5 p.m. ET to discuss the third 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.

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 regarding Ultragenyx's expectations and projections concerning its future operating results and financial performance, including its 2026 revenue guidance for total revenue, Crysvita and Dojolvi, its anticipated R&D and SG&A expenses in 2027, the expected timing of closing and net proceeds from the sales of its PRVs, the anticipated scope, timing, costs, benefits and savings of its headcount and expense reductions, including expected severance and other restructuring charges and the expected timing of completion of the reduction in force, and the timing and sustainability of profitability, including expected full-year GAAP profitability in 2027 and going forward; the timing, progress, results and plans for its clinical programs and studies, including the wind-down of the GTX-102 studies, the termination of the UX701 program, the anticipated timing of data from the Phase 3 Enh3ance study of DTX301, engagement with regulatory agencies regarding UX143 and whether there is a potential path forward for UX143, and initiation of dosing in the UX016 study;

Ultragenyx's ability to successfully launch and commercialize GENGLYCOS and FAYUVI, demand for these products, and Ultragenyx's ability to satisfy post-marketing requirements, including confirmatory study requirements associated with the accelerated approval of GENGLYCOS; and the potential patient impact, commercial opportunity and growth associated with Ultragenyx's products and 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, the risk that the FDA may withdraw the accelerated approval of GENGLYCOS if confirmatory studies do not verify clinical benefit, risks related to serious or undesirable side effects of our products and 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 launching and commercializing gene therapy products, 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, the risk that the PRV sales may not close on the expected timeline or at all, delays or unexpected costs and other adverse effects related to the headcount and expense reductions, including disruption to our operations and our ability to retain key employees, 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 marketable securities 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 product 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 August 5, 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-/).

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	**2025**	**2026**	**2025**
CRYSVITA				
Product sales - Latin America and Türkiye	$ 41	$ 47	$ 141	$ 137
Royalty revenue - U.S. and Canada	61	57	194	177
Royalty revenue - Europe	7	8	23	22
Total CRYSVITA Revenue	109	112	358	336
DOJOLVI	30	24	75	64
EVKEEZA	24	17	63	42
MEPSEVII	10	7	27	24
Total revenues	$ 173	$ 160	$ 523	$ 466

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	**2025**	**2026**	**2025**
Statement of Operations Data:				
Revenues:				
Product sales	$ 105	$ 95	$ 306	$ 267
Royalty revenue	68	65	217	199
Total revenues	173	160	523	466
Operating expenses:				
Cost of sales	32	28	96	80
Research and development	154	216	508	547
Selling, general and administrative	97	87	273	261
Total operating expenses	283	331	877	888
Loss from operations	(110)	(171)	(354)	(422)
Non-cash interest expense on liabilities for sales of future royalties	(20)	(14)	(63)	(42)

Other income, net	3	6	15	21
Loss before income taxes	(127)	(179)	(402)	(443)
Benefit from (provision for) income taxes	9	(1)	7	(3)
Net loss	$ (118)	$ (180)	$ (395)	$ (446)
Net loss per share, basic and diluted	$ (1.16)	$ (1.81)	$ (3.89)	$ (4.55)
Shares used in computing net loss per share, basic and diluted	102.1	99.8	101.6	98.2

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2026	**2025**	**2026**	**2025**
Non-cash stock-based compensation	$ 34	$ 37	$ 98	$ 116
Restructuring expense	—	—	30	—

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

	September 30, 2026	**December 31, 2025**
Balance Sheet Data:		
Cash, cash equivalents, and marketable securities	$ 401	$ 737
Working capital	155	567
Total assets	1,187	1,532
Total stockholders' deficit	(376)	(80)

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