Exhibit 99.1

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Ultragenyx Reports Second Quarter 2026 Financial Results and Corporate Update

Second quarter total revenue of $214 million,
Crysvita® revenue of $156 million and Dojolvi® revenue of $27 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

Catalysts in second half of 2026 include two PDUFA dates and pivotal data readout from GTX-102 Phase 3 Aspire study for Angelman syndrome

NOVATO, Calif. – August 4, 2026 – 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 June 30, 2026 and reaffirmed its financial guidance for 2026.

“In the second quarter we generated the highest quarterly revenue in the history of the company, supporting our full-year revenue guidance, and keeping us on track toward profitability in 2027,” said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. “As we look to the second half of the year, we are entering a transformative period with multiple important catalysts. We are ready to launch two gene therapy products and are preparing for a pivotal Phase 3 GTX-102 data readout in Angelman syndrome. This puts us in position to broaden our patient impact with sustained growth in the years to come.”

Second Quarter 2026 Revenue Highlights and 2026 Revenue Guidance

- **Total revenue** in the second quarter of 2026 was $214 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 second quarter of 2026 was $156 million, consistent with expected seasonality in the U.S. and Canada and ordering patterns in Latin America. The company reaffirms its full year 2026 Crysvita revenue guidance of $500 million to $520 million.

- **Dojolvi** revenue in the second quarter 2026 was $27 million. The company reaffirms its full year 2026 Dojolvi revenue guidance of $100 million to $110 million.
- **Evkeeza®** revenue in the second quarter 2026 was $21 million, driven by increased demand from new country launches and early access.
- **Mepsevii®** revenue in the second quarter 2026 was $10 million.

Milestones and Upcoming Catalysts

- **Dojolvi for the treatment of LC-FAOD:** In May 2026, Dojolvi was listed on the National Health Insurance (NHI) drug price list and was launched in Japan following the receipt of manufacturing and marketing approval under the Conditional Approval System for Pharmaceuticals on March 23, 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 and assigned a Prescription Drug User Fee Act (PDUFA) action date of August 23, 2026.

- **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 and assigned a PDUFA action date of September 19, 2026.

- **GTX-102 (apazunersen) antisense oligonucleotide (ASO) for the treatment of Angelman syndrome (AS):** 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 September or October timeframe.

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

- **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 the fourth quarter of 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.

- **DTX301 (avalotcagene ontaparvovec) AAV8 gene therapy for the treatment of Ornithine Transcarbamylase, or OTC, deficiency:** The Phase 3 Enh3ance study continues with patients in both treatment and cross-over groups progressing through 64 weeks of follow-up. Data from the second primary endpoint, which evaluates reduction in treatment burden, including use of ammonia scavengers and dietary management, are expected in the first half of 2027.

Summary of Second Quarter 2026 Financial Results

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Total revenues	$ 214	$ 167	$ 350	$ 306
Operating expenses:				
Cost of sales	34	23	64	52
Research and development	167	165	354	331
Selling, general and administrative	88	87	176	174
Total operating expenses	289	275	594	557
Net loss	$ (92)	$ (115)	$ (277)	$ (266)
Net loss per share, basic and diluted	$ (0.90)	$ (1.17)	$ (2.73)	$ (2.73)

Operating Expenses

Total operating expenses for the second quarter 2026 were $289 million, including $34 million of non-cash stock-based compensation. 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 second quarter 2026 was $92 million, or $0.90 per share basic and diluted, compared with a net loss for the second quarter 2025 of $115 million, or $1.17 per share basic and diluted.

Cash Balance and Net Cash Used in Operations

Cash, cash equivalents, and marketable securities were $436 million as of June 30, 2026. For the three months ended June 30, 2026, net cash used in operations was $97 million.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Tuesday, August 4, 2026, at 2 p.m. PT/5 p.m. ET to discuss the second 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 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 2026 and 2027, anticipated benefits and savings from its strategic restructuring plan, and the timing and sustainability of profitability; the timing, progress, results and plans for its clinical programs and studies, including the anticipated timing and outcome of the Phase 3 Aspire study of GTX-102, enrollment in the Aurora study, data from the UX701 and DTX301 studies, and initiation of the UX016 study; the FDA's review of the BLAs for DTX401 and UX111, including the anticipated PDUFA action dates, the potential approval of either product candidate, whether the FDA may require additional information, studies or manufacturing changes, and the timing and outcome of regulatory inspections; Ultragenyx's manufacturing and commercial readiness and the timing and success of any potential launches of DTX401 and UX111, if approved; 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, 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 May 6, 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-/).

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Crysvita				
Product sales - Latin America and Türkiye	$ 54	$ 35	$ 100	$ 90
Royalty revenue - U.S. and Canada	94	79	133	120
Royalty revenue - Europe	8	7	16	14
Total Crysvita Revenue	156	121	249	224
Dojolvi	27	23	45	40
Evkeeza	21	14	39	25
Mepsevii	10	9	17	17
Total revenues	$ 214	$ 167	$ 350	$ 306

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Statement of Operations Data:				
Revenues:				
Product sales	$ 112	$ 81	$ 201	$ 172
Royalty revenue	102	86	149	134
Total revenues	214	167	350	306
Operating expenses:				
Cost of sales	34	23	64	52
Research and development	167	165	354	331
Selling, general and administrative	88	87	176	174
Total operating expenses	289	275	594	557
Loss from operations	(75)	(108)	(244)	(251)
Non-cash interest expense on liabilities for sales of future royalties	(22)	(14)	(43)	(28)
Other income, net	6	8	12	15
Loss before income taxes	(91)	(114)	(275)	(264)
Provision for income taxes	(1)	(1)	(2)	(2)
Net loss	$ (92)	$ (115)	$ (277)	$ (266)
Net loss per share, basic and diluted	$ (0.90)	$ (1.17)	$ (2.73)	$ (2.73)
Shares used in computing net loss per share, basic and diluted	101.9	98.5	101.3	97.4

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Non-cash stock-based compensation	$ 34	$ 39	$ 64	$ 79
Restructuring expense	—	—	30	—

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

	June 30, 2026	December 31, 2025
Balance Sheet Data:		
Cash, cash equivalents, and marketable securities	$ 436	$ 737
Working capital	255	567
Total assets	1,264	1,532
Total stockholders' deficit	(291)	(80)