UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): August 05, 2025

Ultragenyx Pharmaceutical Inc.

(Exact name of Registrant as Specified in Its Charter)

Delaware	**001-36276**	**27-2546083**
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**
60 Leveroni Court Novato, California		**94949**
(Address of Principal Executive Offices)		**(Zip Code)**

Registrant's Telephone Number, Including Area Code: 415 483-8800

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.001 par value	RARE	Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 2.02 Results of Operations and Financial Condition.

On August 5, 2025, Ultragenyx Pharmaceutical Inc. issued a press release announcing its financial results for the three months ended June 30, 2025 (the "***Press Release***"). A copy of the Press Release is furnished herewith as Exhibit 99.1

The information set forth under Item 2.02 and in Exhibit 99.1 shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, except as shall be expressly set forth by specific reference in such filing.

Item 9.01 Financial Statements and Exhibits.

(d) **Exhibits**

Exhibit No.	**Description**
99.1	Press Release, dated August 5, 2025.
104	The cover page from the Company's Current Report on Form 8-K dated August 5, 2025 formatted in Inline XBRL.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Ultragenyx Pharmaceutical Inc.

Date: August 5, 2025

By: /s/ Howard Horn
Howard Horn
Executive Vice President, Chief Financial Officer, Corporate Strategy

Exhibit 99.1

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

Ultragenyx Reports Second Quarter 2025 Financial Results and Corporate Update

Second quarter total revenue of $166 million,
Crysvita® revenue of $120 million and Dojolvi® revenue of $23 million

Reaffirm 2025 Revenue Guidance: Total revenue between $640 million to $670 million, Crysvita revenue of $460 million to $480 million, and Dojolvi revenue of $90 million to $100 million

UX143 for osteogenesis imperfecta Phase 3 data from Orbit and Cosmic studies expected around the end of the year

GTX-102 for Angelman syndrome received Breakthrough Therapy Designation from FDA;
Phase 3 Aspire study fully enrolled

NOVATO, Calif. – August 5, 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 ended June 30, 2025.

“In the first half of the year, we delivered 20% revenue growth from our commercial therapies versus the prior year. We are continuing along our path to profitability in 2027, as we drive our top line growth and maintain our fiscal discipline,” said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. “We are excited for the potential of UX143 in osteogenesis imperfecta to reduce fractures and meaningfully improve patients’ bone health and for GTX-102 in Angelman syndrome to transform the lives of patients and their families affected by this neurodevelopment disease.”

Second Quarter 2025 Selected Financial Data Tables and Financial Results

Revenues (dollars in thousands), (unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Crysvita				
Product sales - Latin America and Türkiye	$ 34,727	$ 40,449	$ 89,807	$ 76,690
Royalty revenue - U.S. and Canada	79,083	67,045	119,936	107,447
Royalty revenue - Europe	6,596	6,176	13,528	12,118
Total Crysvita Revenue	120,406	113,670	223,271	196,255
Dojolvi	23,207	19,355	40,216	35,717
Evkeeza	14,573	7,856	25,604	11,131
Mepsevii	8,310	6,145	16,697	12,756
Total revenues	$ 166,496	$ 147,026	$ 305,788	$ 255,859

Total Revenues

Ultragenyx reported $166 million in total revenue for the second quarter of 2025, which represents 13% growth compared to the same period in 2024. Second quarter 2025 Crysvita revenue was $120 million, which includes product sales of $35 million from Latin America and Türkiye. Dojolvi revenue in the second quarter 2025 was $23 million. Evkeeza revenue in the second quarter 2025 was $15 million as we continue to launch in the Ultragenyx territories outside of the United States.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Total revenues	$ 166,496	$ 147,026	$ 305,788	$ 255,859
Operating expenses:				
Cost of sales	23,002	21,280	51,664	38,813
Research and development	164,736	161,503	330,508	339,990
Selling, general and administrative	86,646	80,604	174,443	158,764
Total operating expenses	274,384	263,387	556,615	537,567
Net loss	$ (114,951)	$ (131,598)	$ (266,031)	$ (302,282)
Net loss per share, basic and diluted	$ (1.17)	$ (1.52)	$ (2.73)	$ (3.54)

Operating Expenses

Total operating expenses for the second quarter of 2025 were $274 million, including non-cash stock-based compensation of $39 million.

Net Loss
For the second quarter of 2025, Ultragenyx reported net loss of $115 million, or $1.17 per share basic and diluted, compared with a net loss for the second quarter of 2024 of $132 million, or $1.52 per share basic and diluted.

Cash Balance and Net Cash Used in Operations
Cash, cash equivalents, and marketable debt securities were $539 million as of June 30, 2025, which includes $80 million of net proceeds raised through the At-The-Market (ATM) facility. For the three months ended June 30, 2025, net cash used in operations was $108 million and for the six months ended June 30, 2025 was $275 million.

2025 Financial Guidance
Ultragenyx reaffirmed its revenue guidance for 2025. Total revenues are expected to grow approximately 14-20% compared to 2024. Net cash used in operations is now expected to modestly increase compared to 2024, related to timing delays and changes for UX111, DTX401, and UX143 impacting receipts and payments. The company reaffirms its path to GAAP profitability in 2027 and plans to continue to focus on growing revenues and prioritizing its spend, including stopping and delaying certain expenses prior to upcoming potential commercial launches.

Reaffirm for the full year 2025:
- Total revenue to be in the range of $640 million to $670 million
- Crysvita revenue to be in the range of $460 million to $480 million
- Dojolvi revenue to be in the range of $90 million to $100 million

Recent Updates and Clinical Milestones

UX143 (setrusumab) monoclonal antibody for osteogenesis imperfecta (OI): Final analysis for Phase 3 Orbit and Cosmic studies around the end of 2025

The Phase 3 *Orbit* and *Cosmic* studies, which evaluate setrusumab in pediatric and young adult patients with OI, are progressing towards their final analyses around the end of 2025. The randomized, placebo-controlled Phase 3 portion of the *Orbit* study was evaluated by the Data Monitoring Committee at an interim analysis in July 2025 and they informed the company that UX143 demonstrated an acceptable safety profile and that the study should continue to the final analysis. Conduct of the study is going well and patient safety in the Phase 3 is consistent with the Phase 2.

Data from the *Cosmic* study were not analyzed at the interim timepoint, consistent with the statistical analysis plan. Study conduct is going well and safety in this younger patient population is consistent with the safety profile in the other studies.

Patients will continue dosing in the ongoing Phase 3 *Orbit* and *Cosmic* clinical studies with the final analyses to be conducted after patients have been on therapy for at least 18-months. The threshold for the Phase 3 *Orbit* final analysis is $p<0.04$ and for the Phase 3 *Cosmic* final analysis is $p<0.05$.

GTX-102 an antisense oligonucleotide for Angelman syndrome: Phase 3 study fully enrolled; Phase 3 data expected in the second half of 2026

In June 2025, Breakthrough Therapy Designation (BTD) was granted by the FDA for GTX-102 as a treatment for Angelman syndrome. The FDA's decision was based on preliminary clinical evidence including positive data from the Phase 1/2 study in 74 patients (4-17 years of age) with a full maternal UBE3A gene deletion, that showed participants have made consistent developmental gains with rapid, sustained and continuing improvements across multiple symptom domains when treated for up to 3 years. BTD aims to expedite the development and review of drugs that are intended to treat serious or life-threatening diseases and whose preliminary clinical evidence indicates that the drug may demonstrate substantial improvement on one or more clinically significant endpoints over existing therapies.

In July 2025, enrollment of the global Phase 3 *Aspire* study was completed, ahead of plan due to patient and investigator interest, with 129 patients screened and randomized across 28 global sites. Participants are randomized 1:1 to receive GTX-102 by intrathecal injection via lumbar puncture or to the sham comparator group during the 48-week primary efficacy analysis period. The primary endpoint is improvement in cognition assessed by Bayley-4 cognitive raw score, and the key secondary endpoint (with a 10% allocation of alpha) is the Multi-domain Responder Index (MDRI) across the five domains of cognition, receptive communication, behavior, gross motor function, and sleep. Data from this study are expected in the second half of 2026.

The Phase 2/3 *Aurora* study, which will evaluate GTX-102 in other Angelman syndrome genotypes and ages, is expected to initiate in the second half of 2025.

UX111 AAV gene therapy for Sanfilippo syndrome type A (MPS IIIA): Working with FDA to resolve observations from Complete Response Letter (CRL)

In July 2025, the FDA issued a CRL for the Biologics License Application (BLA) for UX111 requesting additional information and improvements related to specific aspects of chemistry, manufacturing and controls (CMC) procedures and validation as well as observations from the recently completed manufacturing facility inspections. The company believes the observations are readily addressable and many have been addressed. The company will work with the FDA through a Type A meeting to agree on the planned resolution of the observations. Once agreement on the contents of a filing have been reached, the company expects to resubmit the BLA and anticipates up to a 6-month review period to follow the resubmission.

Clinical review had been ongoing and the FDA has acknowledged that the neurodevelopmental outcome data provided to date are robust and the biomarker data provide additional supportive evidence. The CRL did not note any review issues related to the clinical data package nor clinical inspections and specified that updated clinical data for particular endpoints from the current patients be included in the resubmission.

DTX401 AAV gene therapy for Glycogen Storage Disease Type Ia (GSDIa): BLA submission expected in the fourth quarter of 2025

A BLA for DTX401 for the treatment of GSDIa is planned to be submitted in the fourth quarter of 2025. The BLA will include data from the randomized, placebo controlled Phase 3 study and the previously disclosed 96-week data that demonstrated patients had even greater reductions in total daily cornstarch at their last visit compared to baseline in both the ongoing DTX401 group (-60%) and the Crossover Placebo to DTX401 group (-64%) when compared to the 48-week data. It will also include updates to proactively respond to related FDA observations identified in the UX111 CRL in the CMC section and at the company's gene therapy manufacturing facilities.

UX701 AAV gene therapy for Wilson Disease: Phase 1/2/3 study ongoing; Cohort 4 enrollment ongoing, completion expected in second half of 2025

Enrollment is ongoing in the fourth cohort evaluating a 4.0e13 GC/kg dose in the ongoing, dose-finding, stage of the pivotal *Cyprus2+* study of UX701 for the treatment of Wilson disease. The company is on track to enroll five patients in Cohort 4 who will receive immunomodulation therapy with rituximab and tacrolimus, in addition to the prophylactic oral corticosteroid regimen patients in Cohorts 1 through 3 received, prior to being dosed with UX701. Enrollment in Cohort 4 is expected to complete in the second half of 2025.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Tuesday, August 5, 2025, at 2 p.m. PT/5 p.m. ET to discuss the second quarter 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, 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, the company's ability to provide the requested documentation and address the comments in the CRL to the satisfaction of the FDA, the timing of resubmission of the BLA and the timing of FDA review of any such resubmission, the timing and outcome of any FDA inspections related to UX111, the timing of future regulatory interactions related to UX111 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 May 7, 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-/).

###

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Statement of Operations Data:				
Revenues:				
Product sales	$ 80,817	$ 73,805	$ 172,324	$ 136,294
Royalty revenue	85,679	73,221	133,464	119,565
Total revenues	166,496	147,026	305,788	255,859
Operating expenses:				
Cost of sales	23,002	21,280	51,664	38,813
Research and development	164,736	161,503	330,508	339,990
Selling, general and administrative	86,646	80,604	174,443	158,764
Total operating expenses	274,384	263,387	556,615	537,567
Loss from operations	(107,888)	(116,361)	(250,827)	(281,708)
Change in fair value of equity investments	(9)	(3,991)	(166)	(245)
Non-cash interest expense on liabilities for sales of future royalties	(14,041)	(15,960)	(28,383)	(31,807)
Other income, net	7,934	5,572	15,602	12,791
Loss before income taxes	(114,004)	(130,740)	(263,774)	(300,969)
Provision for income taxes	(947)	(858)	(2,257)	(1,313)
Net loss	$ (114,951)	$ (131,598)	$ (266,031)	$ (302,282)
Net loss per share, basic and diluted	$ (1.17)	$ (1.52)	$ (2.73)	$ (3.54)
Shares used in computing net loss per share, basic and diluted	98,460,445	86,580,516	97,381,745	85,433,443

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Non-cash stock-based compensation	$ 38,615	$ 39,363	$ 78,525	$ 76,297

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

	June 30, 2025	December 31, 2024
Balance Sheet Data:		
Cash, cash equivalents, and marketable debt securities	$ 539,039	$ 745,029
Working capital	426,544	472,970
Total assets	1,306,265	1,503,456
Total stockholders' equity	151,286	255,297