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): November 03, 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 1.01. Entry into a Material Definitive Agreement.

On November 3, 2025, Ultragenyx Pharmaceutical Inc. (the "Company") and its wholly owned subsidiary Rare Delaware Inc. ("Seller") entered into a Royalty Purchase Agreement (the "Agreement") with OCM LS23 Holdings LP, an investment vehicle of OMERS, pursuant to which OMERS paid $400 million in cash to Seller in consideration for the right (the "Purchased Interest") to receive (i) an additional 25% of the future royalty payments due to the Company from Kyowa Kirin Co., Ltd. ("KKC") based on net sales of Crysvita® in the United States and Canada under the terms of the Company's Collaboration and License Agreement with KKC dated as of August 29, 2013, as amended (the "License Agreement") from and after January 1, 2028 and (ii) 30% of the future royalty payments due to the Company from KKC based on net sales of Crysvita® in the United States and Canada under the terms of the Company's License Agreement, from and after the date on which the Royalty Cap (as defined in the Royalty Purchase Agreement, dated as of July 14, 2022, by and among the Company, Seller and OCM LS23 Holdings LP) is met (collectively, the "Royalties"). The Agreement will automatically expire, and the payment of the Purchased Interest to OMERS will cease, on the earlier of (1) the date on which aggregate payments actually received by OMERS equals 1.55 times the purchase price ($620 million), or (2) the date of payment of the Purchased Interest with respect to the last Royalties due to the Company under the License Agreement. In connection with the foregoing, OMERS granted the Company an option, exercisable at any time for a two-year period, to repurchase in whole the Purchased Interest for an amount equal to 1.35 times the purchase price ($540 million).

The Agreement contains other customary terms and conditions, including representations and warranties, covenants, and indemnification obligations in favor of each party. The above description of the Agreement is a summary of the material terms, does not purport to be complete and is qualified in its entirety by reference to the Agreement, which will be filed as an exhibit to the Company's Annual Report on Form 10-K.

Item 2.02 Results of Operations and Financial Condition.

On November 4, 2025, Ultragenyx Pharmaceutical Inc. issued a press release announcing its financial results for the three months ended September 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 November 4, 2025.
104	The cover page from the Company's Current Report on Form 8-K dated November 3, 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: November 4, 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 Third Quarter 2025 Financial Results and Corporate Update

Third quarter total revenue of $160 million,
Crysvita® revenue of $112 million and Dojolvi® revenue of $24 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

Bolstered balance sheet with $400 million proceeds from sale of portion of Crysvita royalty interest

NOVATO, Calif. – November 04, 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 September 30, 2025.

“We are a global commercial company with multiple products generating meaningful growth that is expected to accelerate from anticipated launches from our late-stage clinical pipeline,” said Emil D. Kakkis, M.D., Ph.D., chief executive officer and president of Ultragenyx. “We announced today that we bolstered our balance sheet with a royalty financing ahead of pivotal milestones expected over the next year to support multiple late-stage data readouts, multiple regulatory submissions, and multiple launches. This includes the highly anticipated phase 3 study readouts for UX143 in osteogenesis imperfecta around the end of the year.”

Third Quarter 2025 Selected Financial Data Tables and Financial Results

Revenues (dollars in thousands), (unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Crysvita				
Product sales - Latin America and Türkiye	$ 47,003	$ 35,604	$ 136,810	$ 112,294
Royalty revenue - U.S. and Canada	57,186	55,985	177,122	163,432
Royalty revenue - Europe	7,754	6,258	21,282	18,376
Total Crysvita Revenue	111,943	97,847	335,214	294,102
Dojolvi	24,275	21,374	64,491	57,091
Evkeeza	16,717	10,657	42,321	21,788
Mepsevii	6,998	9,616	23,695	22,372
Total revenues	$ 159,933	$ 139,494	$ 465,721	$ 395,353

Total Revenues

Ultragenyx reported $160 million in total revenue for the third quarter of 2025, which represents 15% growth compared to the same period in 2024. Crysvita revenue in the third quarter 2025 was $112 million, which includes product sales of $47 million from Latin America and Türkiye. Dojolvi revenue in the third quarter 2025 was $24 million. Evkeeza revenue in the third quarter 2025 was $17 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 September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Total revenues	$ 159,933	$ 139,494	$ 465,721	$ 395,353
Operating expenses:				
Cost of sales	27,991	21,021	79,655	59,834
Research and development	216,212	170,109	546,720	510,099
Selling, general and administrative	86,620	80,351	261,063	239,115
Total operating expenses	330,823	271,481	887,438	809,048
Net loss	$ (180,413)	$ (133,516)	$ (446,444)	$ (435,798)
Net loss per share, basic and diluted	$ (1.81)	$ (1.40)	$ (4.55)	$ (4.91)

Operating Expenses

Total operating expenses for the third quarter of 2025 were $331 million, including non-cash stock-based compensation of $37 million.

Net Loss
For the third quarter of 2025, Ultragenyx reported net loss of $180 million, or $1.81 per share basic and diluted, compared with a net loss for the third quarter of 2024 of $134 million, or $1.40 per share basic and diluted.

Cash Balance and Net Cash Used in Operations
Cash, cash equivalents, and marketable debt securities were $447 million as of September 30, 2025.

The company announced today that it received $400 million through the sale of an additional 25% of its royalty interest on the future sales of Crysvita in the United States and Canada, to OMERS. Payments to OMERS will begin in January of 2028. OMERS will also continue to receive 30% of Crysvita® net sales in the U.S. and Canada following the achievement of the 2022 royalty purchase agreement transaction's cap of 1.45 times the purchase price. Total payments to OMERS pursuant to the new agreement are capped at 1.55 times the 2025 purchase price.

For the three months ended September 30, 2025, net cash used in operations was $91 million and for the nine months ended September 30, 2025 was $366 million.

2025 Financial Guidance
Ultragenyx reaffirmed its revenue guidance for 2025. Total revenues are expected to grow approximately 14-20% compared to 2024.

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

Ultragenyx also reaffirmed its net cash used in operations guidance for 2025, which is expected to modestly increase compared to 2024 and its path to full year GAAP profitability in 2027.

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 final analyses at which time patients will have been on therapy for at least 18 months. Data from these studies are expected around the end of 2025.

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

In July 2025, enrollment of the global Phase 3 *Aspire* study was completed 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. Data from this study are expected in the second half of 2026.

Enrollment has begun in the supportive Phase 2/3 *Aurora* study, which is evaluating GTX-102 in other Angelman syndrome genotypes and ages.

UX111 AAV gene therapy for Sanfilippo syndrome type A (MPS IIIA): expect to resubmit Biologics License Application (BLA) early in 2026

Following receipt of a Complete Response Letter (CRL), the company has had constructive formal and informal discussions with the FDA. The additional clinical data requested by the agency, and that will be included in the BLA, continues to show a durable treatment effect across multiple biomarkers and further clinical separation from natural history, while maintaining an acceptable safety profile.

The company plans to resubmit the BLA early in 2026 and will be followed by an up to 6-month review per FDA regulations.

DTX401 AAV gene therapy for Glycogen Storage Disease Type Ia (GSDIa): BLA rolling submission underway, expect to complete in the fourth quarter of 2025

Rolling submission of a BLA for DTX401 for the treatment of GSDIa began in August 2025. The BLA will include data from the randomized, placebo-controlled Phase 3 study that demonstrated statistically significant and clinically meaningful reductions in daily cornstarch intake compared with placebo at Week 48. It will also include longer-term data that was announced in September 2025 that demonstrated patients showed an even greater reduction in mean daily cornstarch intake in the 48-week crossover period. Both the originally treated DTX401 group (n=20) and the crossover group (n=19) who received DTX401 at Week 48 had a mean reduction in daily cornstarch intake of 61% at Week 96. Quality of life improved following treatment with DTX401 for patients in both groups as measured by the Patient Global Impression of Change (PGIC). At Week 96, improvements in disease management were reported by 83% (10/12) of patients in the DTX401 group and 95% (18/19) of patients in the crossover group.

Rolling submission of the BLA is expected to complete in the fourth quarter of 2025.

UX701 AAV gene therapy for Wilson Disease: Cohort 4 enrollment complete, data expected in the first half of 2026

In September 2025, the company completed enrollment of 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. A total of five patients were enrolled in Cohort 4. These patients received 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. Data from this study are expected in the first half of 2026.

Conference Call and Webcast Information

Ultragenyx will host a conference call today, Tuesday, November 4, 2025, at 2 p.m. PT/5 p.m. ET to discuss the third 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 August 6, 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 thousands, except share and per share amounts)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Statement of Operations Data:				
Revenues:				
Product sales	$ 94,993	$ 77,251	$ 267,317	$ 213,545
Royalty revenue	64,940	62,243	198,404	181,808
Total revenues	159,933	139,494	465,721	395,353
Operating expenses:				
Cost of sales	27,991	21,021	79,655	59,834
Research and development	216,212	170,109	546,720	510,099
Selling, general and administrative	86,620	80,351	261,063	239,115
Total operating expenses	330,823	271,481	887,438	809,048
Loss from operations	(170,890)	(131,987)	(421,717)	(413,695)
Change in fair value of equity investments	678	678	512	433
Non-cash interest expense on liabilities for sales of future royalties	(14,148)	(15,712)	(42,531)	(47,519)
Other income, net	4,820	13,808	20,422	26,599
Loss before income taxes	(179,540)	(133,213)	(443,314)	(434,182)
Provision for income taxes	(873)	(303)	(3,130)	(1,616)
Net loss	$ (180,413)	$ (133,516)	$ (446,444)	$ (435,798)
Net loss per share, basic and diluted	$ (1.81)	$ (1.40)	$ (4.55)	$ (4.91)
Shares used in computing net loss per share, basic and diluted	99,771,297	95,493,996	98,186,433	88,811,157

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Non-cash stock-based compensation	$ 37,010	$ 41,569	$ 115,535	$ 117,866

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

	September 30, 2025	December 31, 2024
Balance Sheet Data:		
Cash, cash equivalents, and marketable debt securities	$ 447,315	$ 745,029
Working capital	303,184	472,970
Total assets	1,190,445	1,503,456
Total stockholders' equity	9,159	255,297