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): January 30, 2026

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 8.01 Other Events.

On January 30, 2026, Ultragenyx Pharmaceutical Inc. (the “Company”) issued a press release announcing that that it has resubmitted its Biologics License Application (“BLA”) seeking accelerated approval for UX111 (rebisufligene etisparvovec) AAV9 gene therapy as a treatment for patients with Sanfilippo syndrome type A (MPS IIIA) to the U.S. Food and Drug Administration (the “FDA” or the “Agency”). The submission contains substantial longer-term data on multiple measures of neurologic benefit to support an intermediate clinical endpoint for accelerated approval supported further by CSF heparan sulfate and other biomarker data, as agreed with the FDA during the last clinical review.

The resubmitted BLA includes comprehensive responses to chemistry, manufacturing, and controls (CMC)-related observations outlined in a Complete Response Letter (the “CRL”) issued in July 2025, as well as additional long-term clinical data from current patients as requested by the Agency in the CRL.

During its prior review, the FDA acknowledged that the neurodevelopmental outcome data are robust and that the biomarker data provide additional supportive evidence; updated clinical data included in the BLA representing an additional year of follow-up continue to show a durable treatment effect across multiple biomarkers and further clinical separation from natural history, while maintaining an acceptable safety profile. Detailed updates will be presented next week at the WORLDSymposium™ 2026 in San Diego.

In February 2025, the FDA granted the UX111 BLA Priority Review. A Prescription Drug User Fee Act (PDUFA) action date is expected to be assigned within a month of resubmission. The Company anticipates up to a six-month review period from the date of resubmission per FDA regulations, with a PDUFA date expected in the third quarter of 2026. If approved, UX111 will be the first approved therapy for Sanfilippo syndrome type A.

Cautionary Note Regarding Forward-Looking Statements

This Current Report on Form 8-K contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. These statements may be identified by the use of words such as, but not limited to, “anticipates,” “continue,” “will,” or other similar terms or expressions that concern the Company’s expectations, plans and intentions. Forward-looking statements including, without limitation, statements related to the Company’s ability to provide the requested documentation and address the comments in the CRL to the satisfaction of the FDA, the development, timing and progress of UX111, including the timing of FDA acceptance of the BLA resubmission 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, including the outcome of the BLA resubmission, business plans and objectives for UX111, expectations regarding the tolerability and safety of UX111, and future clinical and regulatory developments for 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 our clinical development programs, 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 ability of the Company to successfully develop UX111, the Company’s ability to achieve its projected development goals in its expected timeframes, risks related to adverse side effects, risks related to reliance on third party partners to conduct certain activities on the Company’s behalf, smaller than anticipated market opportunities for the Company’s products and product candidates, manufacturing risks, the Company’s limited experience in operating its own manufacturing facility, the ability of the Company and its third party manufacturers to comply with regulatory requirements, competition from other therapies or products, 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 candidates. The Company 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 the Company in general, see the Company's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on November 5, 2025, and its subsequent periodic reports filed with the SEC.

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: January 30, 2026

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