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): February 03, 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 February 3, 2026, Ultragenyx Pharmaceutical Inc. (the "Company") issued a press release announcing new long-term data from clinical studies evaluating UX111 (rebisufligene etisparvovec), an investigational AAV9 gene therapy for Sanfilippo syndrome type A (MPS IIIA), a fatal neurodegenerative lysosomal storage disorder. The results demonstrate substantial and durable biomarker improvements and meaningful functional benefits compared with natural history, with consistent and highly statistically significant results across age and disease severity. UX111 was well-tolerated and the safety profile remains favorable.

The data will be delivered in an oral presentation, *Treatment with UX111 Reduced Cerebrospinal Fluid ("CSF") Heparan Sulfate ("HS") Exposure and Stabilized or Improved Functioning across Dose, Age, and Stage of MPS IIIA*, at the WORLDSymposium™ 2026 on Friday, February 6 at 8 a.m. PST.

Clinical Improvements in Functional Abilities Compared to Natural History

Cognitive function, expressive and receptive communication, and fine and gross motor skills were measured using Bayley-III and compared to natural history data from untreated patients with reported rapid progressor phenotypes. Children under two years of age or with earlier stage disease at the time of treatment (n=17) demonstrated a +23.2 point ($p<0.0001$) treatment effect in the mean Bayley-III cognitive raw score compared to natural history data during 24-60 months of age.

In addition to cognitive function, clinical improvements were also observed across the other four subtests compared to natural history:

- Receptive communication (8.1-point improvement; p=0.0076)
- Expressive communication (11.1-point improvement; p=0.0008)
- Fine motor (9.0-point improvement; p=0.0026)
- Gross motor (3.9-point improvement; p=0.070)

On separate caregiver-reported outcome utilizing Vineland 3, there were comparable improvements in the communication, motor, and personal subdomains.

Eight children reached a 36-month cognitive developmental age, enabling higher-level testing—none of the natural-history patients reached this milestone.

Functional Skill Retention in Later-Stage Children

Patients with older age or having more advanced disease at the time of treatment (n=10), showed retention of functional abilities in at least one of three areas at the time of last assessment that exceed typical decline patterns in untreated children with Sanfilippo syndrome type A. Specifically:

- All retained communication (verbal or non-verbal) at last assessment, with a median age of 9.70 years (5.7, 15.8); median age of loss in untreated patients is approximately 7.6 years of age.
- 9/10 retained independent ambulation at last assessment, with a median age of 9.05 years (5.7, 15.8); median age of loss in natural history is approximately 11.3 years.
- 9/10 maintained the ability to eat by mouth and/or self-feed, with a median age of 9.05 years (5.7, 15.8 max).

These findings are clinically relevant as these functions progressively worsen and are eventually lost in late childhood and early adolescence.

Significant and Durable Reduction in CSF Heparan Sulfate

Levels of CSF-HS decreased within the first month following treatment with UX111 ($3x10^{13}$ vg/kg) in the overall efficacy set (N=27), regardless of age or stage of disease progression at the time of treatment. As of the September 2025 cutoff date, the median

reduction in CSF-HS exposure was 63.98% ($p<0.001$). The majority of children treated (88.2% of younger patients and 81.5% of the overall efficacy set) achieved a 50% or greater reduction.

Safety profile remains favorable

UX111 was generally well tolerated across all doses (N=33), including the highest dose of $3x10^{13}$ vg/kg, with a median follow-up 4.8 years (range 0.6–8.5). The most frequently reported treatment-emergent adverse events were elevations in liver enzymes. Treatment-related adverse events were mostly mild or moderate and resolved spontaneously. No participants experienced infusion-related hypersensitivity or anaphylaxis, and no incidences of thrombotic microangiopathy, myocarditis, dorsal root ganglion toxicity, or malignancy were associated with treatment.

BLA resubmitted to FDA with PDUFA date expected in third quarter 2026

These longer-term data were included in the resubmitted Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) seeking accelerated approval for UX111. 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.

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: February 3, 2026

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