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): March 12, 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 March 12, 2026, Ultragenyx Pharmaceutical Inc. (the "Company") issued a press release announcing positive results from its Phase 3 *Enh3ance* study of DTX301, an investigational AAV8 gene therapy for the treatment of ornithine transcarbamylase ("OTC") deficiency. At Week 36 in the randomized, double-blind placebo-controlled period of the trial, DTX301-treated patients (n=18) demonstrated a statistically significant and clinically meaningful 18% (p=0.018) reduction in 24-hour plasma ammonia ("AUC0-24") compared to placebo (n=19) and maintained average ammonia AUC0-24 in the normal range through Week 36. Eight of nine patients with abnormal ammonia AUC0-24 at baseline despite optimal current drug treatment and diet restriction reached normal ammonia levels rapidly, which were generally maintained during this treatment period.

Baseline 24-hour ammonia AUC levels were normal in 50% of DTX301-treated patients and 68% of placebo patients, who were all on current care of scavenger medications and strict dietary control of protein intake. Patients treated with DTX301 (n=18) experienced reductions in 24-hour ammonia rapidly by Week 6 and were decreased by 18% (p=0.018) compared to placebo (n=19) at Week 36. Ammonia was generally maintained in normal range in treated patients despite their mean 27% reduction in ammonia scavenger medications at Week 36 (n=18) and an approximately 13% increase in protein intake relative to no change in placebo (n=19).

Assessed at Week 24, patient global impression scale ("PGIC") for OTC symptoms (total n=15 reporting) overall showed 71% of treated patients were much improved (equivalent to +3) versus 0% of placebo. For Week 24, PGIC evaluations of OTC deficiency symptoms and OTC impact on daily living (total n=30 reporting), both showed 64% were either much improved (43%) or moderately improved (21%) and on placebo only 19% were moderately improved (none were much improved).

DTX301 was well tolerated with an acceptable safety profile, consistent with prior Phase 1/2 safety data. The most common treatment-emergent adverse events were mild to moderate transient hepatic reactions managed with steroids. One serious adverse event ("SAE") of acute hepatitis was assessed as treatment-related and resolved with steroids. No SAEs or AEs related to thrombotic microangiopathy, dorsal root ganglion toxicity, malignancies, or other complex immune reactions. Hyperammonemic crises requiring hospitalization occurred five times in the placebo group with one death, and only one such event in the treated group and no deaths. Two patients in the placebo arm discontinued, including one death due to hyperammonemia crisis and one patient who became AAV8 antibody positive prior to crossover. One patient in the DTX301 arm discontinued after Week 36 for non-clinical reasons.

As planned, the study is continuing to its second primary endpoint which evaluates reduction in treatment burden, including use of ammonia scavengers and dietary management, across both the treatment and placebo-crossover groups following treatment with DTX301 through 64 weeks of follow-up. Data are expected in the first half of 2027. The conduct of the program is reflected in the Company's February 2026 guidance on 2026 spend and will be managed within the company goals of 2027 profitability.

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," "expects," "plans," "continues," "will," "may," "potential," or other similar terms or expressions that concern the Company's expectations, plans, objectives, and intentions.

Forward-looking statements include, without limitation, statements regarding the related to the development, timing and progress of DTX301, the timing, scope and outcome of future data results from the Phase 3 study of DTX301, including data expected in the first half of 2027, future regulatory interactions related to DTX301, the safety and tolerability profile of DTX301, the potential clinical benefit of DTX301 for patients with OTC deficiency, business plans and objectives for DTX301 and the management of development activities within the Company's previously issued guidance on 2026 spend and its goal of achieving profitability in 2027.

These forward-looking statements are based on current expectations and assumptions that are subject to substantial risks and uncertainties that could cause actual results to differ materially 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 risk that interim or topline clinical results may not be predictive of final study results or longer-term outcomes, the ability of the Company to successfully develop DTX301, complexities related to the development of gene therapy product candidates such as DTX301, 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 and supply risks, 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 Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on February 18, 2026, 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: March 12, 2026

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