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

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**FORM 8-K**

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**CURRENT REPORT**

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

**Date of Report (Date of earliest event reported): September 08, 2025**

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**Ultragenyx Pharmaceutical Inc.**

(Exact name of Registrant as Specified in Its Charter)

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**Delaware**  
(State or Other Jurisdiction  
of Incorporation)

**001-36276**  
(Commission File Number)

**27-2546083**  
(IRS Employer  
Identification No.)

**60 Leveroni Court**  
**Novato, California**  
(Address of Principal Executive Offices)

**94949**  
(Zip Code)

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

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

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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.

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## **Item 8.01 Other Events.**

On September 8, 2025, Ultragenyx Pharmaceutical Inc. (the “Company”) issued a press release announcing positive longer-term results from its Phase 3 study of DTX401 AAV gene therapy for the treatment of glycogen storage disease type Ia (“GSDIa”). Previously reported 48 Week data show that patients treated with DTX401 (n=20) experienced statistically significant and clinically meaningful reductions in daily cornstarch intake compared to placebo, while maintaining glycemic control. At Week 96, even greater reductions in cornstarch were observed with maintained low levels of hypoglycemia, improved levels of euglycemia and improved fasting tolerance. The data were presented at the International Congress of Inborn Errors of Metabolism 2025 in Kyoto, Japan.

*Participants achieved statistically significant and clinically meaningful reductions in cornstarch while maintaining glycemic control*

As previously reported, the study met its primary endpoint with patients treated with DTX401 (n=20) experiencing a mean reduction in cornstarch of 41% at Week 48 compared to 10% reduction in the placebo group (n=24) ( $p < 0.0001$ ). Data at Week 96 showed even greater improvements, with both the DTX401 group (n=20) and the crossover group (n=19) achieving a mean reduction in daily cornstarch intake of 61% from baseline. Participants in both groups also experienced statistically significant improvements in other cornstarch-related endpoints.

At Week 96, the DTX401 group saw an increased mean reduction in nighttime cornstarch of 70% and the crossover group saw a mean reduction of 75%. Two-thirds of participants across both groups eliminated at least one nighttime cornstarch dose following treatment with DTX401. Importantly, participants maintained low levels of hypoglycemia and improved levels in euglycemic range (70-120 mg/dL) throughout the second year of the study despite substantial reductions in daily cornstarch intake. Participants dosed with DTX401 also experienced improved fasting tolerance in a controlled fasting challenge through year 2 of the study, demonstrating protection from severe hypoglycemia ( $< 54$  mg/dL).

*Clinical benefits translated to improvements in patient-reported quality of life*

At Week 96, 83% of the DTX401 group and 95% of the crossover group reported improvements in disease burden (+1 to +3 change) as measured by the Patient Global Impression of Change, a single item questionnaire that asked participants how their condition changed since the start of the study.

Improvements in quality of life were further demonstrated through interviews with Phase 3 study participants. During Week 48 and Week 96 interviews, DTX401-treated participants most frequently reported reductions in cornstarch intake that, at the group level, exceeded the baseline-identified threshold for meaningful change; as well as less hypoglycemia and less tiredness—all of which were reported as treatment priorities in baseline interviews. The most frequently reported functional improvements at Week 48 and Week 96 included improvements in physical, social and diet/daily regimen impacts.

*DTX401 well tolerated with an acceptable safety profile*

The study demonstrated an acceptable and expected safety profile for DTX401 consistent with Phase 1/2 study results. Anticipated hepatic reactions were manageable with a prophylactic corticosteroid regimen. No AAV8 class effects of dorsal root ganglion toxicity, malignancy or thrombotic microangiopathy were observed in the study through Week 96. Hypertriglyceridemia was observed in all study groups but more frequently following DTX401.

*Results further supported by data from three participants treated in open-label arm in Japan*

The Phase 3 data is further supported by early results from all participants (n=3, all were pediatric ages 8-17 years old) treated with DTX401 in an open-label cohort that demonstrated a mean reduction in daily cornstarch intake of 95% at Week 24 compared to baseline. Ultimately, all three participants were able to discontinue daily cornstarch entirely as of Week 24 (n=2) and Week 36 (n=1). Glycemic control was maintained or improved with lower levels of hypoglycemia and improved euglycemia across the group with no serious adverse events reported.

### **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,”

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“continue,” “will,” or other similar terms or expressions that concern the Company’s expectations, plans and intentions. Forward-looking statements include, without limitation, statements regarding the future operating results and financial performance, business plans and objectives for DTX401, expectations regarding the tolerability and safety of DTX401, expectations regarding the adequacy of clinical data to support the marketing application and approval of DTX401, the Company’s intent to file, and potential timing and success of, the marketing application and other regulatory approvals for DTX401, expectations regarding timing of BLA submission and receiving potential approval of DTX401, expectations regarding the prevalence of patients of DTX401, future regulatory interactions, expectations regarding the Company’s ability to resolve with the FDA the observations in the complete response letter, and the value to be generated by DTX401, and future clinical and regulatory developments for DTX401. 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 DTX401, the Company’s ability to achieve its projected development goals in its expected timeframes, the risk that results from earlier studies may not be predictive of future study results, risks related to adverse side effects, risks related to reliance on third party partners to conduct certain activities on the Company’s behalf, 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, smaller than anticipated market opportunities for the Company’s products and product candidates, manufacturing risks, 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 the Company’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 August 6, 2025, and its subsequent periodic reports filed with the SEC.

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**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: September 8, 2025

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

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