

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): December 19, 2024

Ultragenyx Pharmaceutical Inc.

(Exact name of Registrant as Specified in Its Charter)

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)

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 December 19, 2024, Ultragenyx Pharmaceutical Inc. (the “Company”) issued a press release announcing that the first patient has been dosed in the Company’s pivotal Phase 3 *Aspire* study (NCT06617429) evaluating the efficacy and safety of GTX-102, its investigational antisense oligonucleotide (“ASO”) for Angelman syndrome.

The global Phase 3 *Aspire* study will enroll approximately 120 children ages four to 17 with Angelman syndrome with a genetically confirmed diagnosis of full maternal UBE3A gene deletion. Participants will be 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. Participants in the active treatment group will receive three, monthly 8 mg loading doses of GTX-102 followed by dosing in a maintenance period that will increase to a maximum dose of 14 mg of GTX-102 quarterly. Patients in the sham comparator group will be eligible to crossover onto treatment after Week 48 is complete. The primary endpoint will be improvement in cognition assessed by Bayley-4 cognitive raw score, and the key secondary endpoint will be the Multi-domain Responder Index (MDRI) across the five domains of cognition, receptive communication, behavior, gross motor function, and sleep.

**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 include, without limitation, statements regarding the clinical benefit, tolerability and safety of GTX-102 and the corresponding impact on patients and timing for clinical development or regulatory review of GTX-102. Such forward-looking statements involve substantial risks and uncertainties that could cause the Company’s 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 GTX-102, 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, 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 the 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 November 6, 2024, 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: December 19, 2024

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

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