

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 the submission of a Biologics License Application (“BLA”) to the U.S. Food and Drug Administration (the “FDA” or the “Agency”) seeking accelerated approval for UX111 (ABO-102) AAV gene therapy as a treatment for patients with Sanfilippo syndrome type A (MPS IIIA).

Earlier this year, the Company reached agreement with the Agency that cerebral spinal fluid (“CSF”) heparan sulfate (“HS”) can be used as a surrogate endpoint for accelerated approval based on the body of data presented by the Company along with a consortium of academics and other industry sponsors at a workshop hosted by the Reagan-Udall Foundation for the FDA in February 2024.

The BLA submission for UX111 is supported by available data, including from the ongoing pivotal *Transpher A* study, demonstrating treatment with UX111 resulted in rapid and sustained decreased levels of HS in CSF in patients with Sanfilippo syndrome type A, and that sustained reduction in CSF HS exposure over time was correlated with improved long-term cognitive development compared to the decline observed during the same period of time in natural history data. The most frequently reported treatment-related adverse events to date were elevations in liver enzymes and the majority of these events were mild (Grade 1) or moderate (Grade 2) in severity and all resolved.

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 UX111 and the corresponding impact on patients and timing for clinical development or regulatory review of UX111. 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 UX111, 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.

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
