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): October 03, 2024

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 October 3, 2024, Ultragenyx Pharmaceutical Inc. (the "Company") reported that the Phase 1/2/3 Cyprus2+ study of its UX701 gene therapy has demonstrated meaningful clinical activity as well as improvements in copper metabolism in Stage 1. Multiple responders have completely tapered off of standard-of-care treatment with responses seen in all three dose cohorts. The Company plans to enroll an additional cohort in Stage 1 at a moderately increased dose and with an optimized immunomodulation regimen to enhance the efficiency and efficacy of the gene therapy, with the objective of having the majority of patients come off of standard-of-care treatment before selecting a dose for the randomized placebo-controlled stage of the study.

In Stage 1, 15 patients were enrolled into three sequential dosing cohorts and followed for at least 24 weeks. Six of the patients have completely tapered off of standard-of-care treatment with chelators and/or zinc therapy, and a seventh patient has begun to taper as of the data cut-off date in August. In patients who have tapered off standard-of-care, non-ceruloplasmin bound copper (NCC) has stabilized to normal, healthy levels. In some patients, there were increases in ceruloplasmin-copper activity consistent with improved ATP7b function. From a safety perspective, UX701 has been well tolerated, with no unexpected related treatment emergent adverse events and no significant immunologic safety events as of the data cut-off. The Company will be submitting a protocol amendment for the additional cohort at a moderately increased dose and with an optimized immunomodulation regimen to optimize delivery efficiency and efficacy for the AAV vector.

Phase 1/2/3 Cyprus2+ Study Design

This study evaluating UX701 for the potential treatment of Wilson disease is designed with three stages. During the first stage (Stage 1), the safety and efficacy of multiple dose levels of UX701 will be evaluated and a dose will be selected for further evaluation in Stage 2. To date, 15 patients have been enrolled into three sequential dosing cohorts to evaluate doses of 5.0 x 10^12 GC/kg, 1.0 x 10^13 GC/kg, and 2.0 x 10^13 GC/kg. A fourth dosing cohort will be added and all patients in Stage 1 will be evaluated over the course of 52 weeks.

In Stage 2, a new cohort of patients will be randomized 2:1 to receive the selected dose of UX701 or placebo. The primary safety and efficacy analyses will be conducted at Week 52 of Stage 2. The primary efficacy endpoints are change in 24-hour urinary copper concentration and percent reduction in standard-of-care medication by Week 52. After the initial 52-week study period, all patients will have long-term follow up in Stage 3.

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 UX701 and the corresponding impact on patients, timing for enrollment of additional cohorts under the study and the anticipated timing and expected dosing in future stages of the UX701 study. 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 UX701, 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 August 2, 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: October 3, 2024

By: /s/ Howard Horn

Howard Horn
Executive Vice President, Chief Financial Officer, Corporate Strategy