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): May 17, 2023

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 May 17, 2023, Ultragenyx Pharmaceutical Inc. (the "Company") issued a press release announcing that the U.S. Food and Drug Administration has reviewed and agreed to a protocol amendment to the Phase 1/2 study of GTX-102 in pediatric patients with Angelman syndrome that enables the Company to harmonize dose ranges in the U.S. with those being used in ex-U.S. cohorts of the study. The Phase 1/2, open-label, dose-escalating study is evaluating the safety and tolerability of GTX-102 in pediatric patients with Angelman syndrome with a genetically confirmed diagnosis of full maternal UBE3A gene deletion. The study is also looking at clinical response as measured by a panel of efficacy assessments for the functional domains impacted in Angelman syndrome. Patients in the earlier dose-escalation cohorts of the study have moved into long-term maintenance dosing, and the study is now enrolling the new expansion cohorts to verify the GTX-102 dose range and treatment regimen that will be used in the Phase 3 program. As of May 4, 2023, thirteen patients have had more than 12 months of exposure to GTX-102, with the longest more than 18 months.

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: May 17, 2023

By: /s/ Emil D. Kakkis, M.D, Ph.D.

Emil D. Kakkis, M.D., Ph.D.
President and Chief Executive Officer