**UNITED STATES**
**SECURITIES AND EXCHANGE COMMISSION**
**WASHINGTON, DC 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): August 22, 2017**

# ULTRAGENYX PHARMACEUTICAL INC.

(Exact name of registrant as specified in 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**

**Not Applicable**
**(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))

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 August 22, 2017, Ultragenyx Pharmaceutical Inc. (the "***Company***") issued a press release announcing top-line results from the Phase 3 study of Ace-ER in GNE Myopathy (the "***Ace-ER Release***").

On August 24, 2017, the Company issued a press release announcing the submission of a Biologics License Application with the Food and Drug Administration for Burosumab (the "***BLA Release***").

A copy of the Ace-ER Release is filed herewith as Exhibit 99.1 and a copy of the BLA Release is filed herewith as Exhibit 99.2.

**Item 9.01 Financial Statements and Exhibits**

(d) Exhibits

| **Exhibit No.** | **Description** |
|---|---|
| 99.1 | Press Release, dated August 22, 2017 |
| 99.2 | Press Release, dated August 24, 2017 |

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**SIGNATURE**

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.

Date: August 24, 2017

Ultragenyx Pharmaceutical Inc.

By: /s/ Shalini Sharp
Name: Shalini Sharp
Title: Executive Vice President, Chief Financial Officer

**EXHIBIT INDEX**

| Exhibit No. | Description |
| --- | --- |
| 99.1 | Press Release, dated August 22, 2017 |
| 99.2 | Press Release, dated August 24, 2017 |

**Exhibit 99.1**

Contact Ultragenyx Pharmaceutical Inc.
Investors & Media
Ryan Martins
415-483-8257

**Ultragenyx Announces Top-Line Results from Phase 3 Study of Ace-ER in GNE Myopathy**

*Study did not meet its primary endpoint*

**Novato, CA — August 22, 2017 —** Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, today announced that a Phase 3 study evaluating aceneuramic acid extended release (Ace-ER) in patients with GNE Myopathy (GNEM) did not achieve its primary endpoint of demonstrating a statistically significant difference in the upper extremity muscle strength composite score compared to placebo. The study also did not meet its key secondary endpoints. Adverse events were generally balanced between Ace-ER and placebo and safety was consistent with previously released Ace-ER data. Ultragenyx plans to discontinue further clinical development of Ace-ER.

“We are disappointed by these results, as we had hoped that Ace-ER would offer a new option for GNEM patients. We would like to thank the patients, caregivers, and investigators involved in the Ace-ER development program” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “This outcome does not affect our overall strategy, as the company moves forward with multiple preclinical and clinical programs and regulatory filings.”

The Phase 3 Ace-ER study enrolled 89 adults with GNEM able to walk ≥ 200 meters in the six minute walk test. Patients were randomized 1:1 to Ace-ER at a dose of 6g/day or placebo for 48 weeks. The study did not meet the primary endpoint of demonstrating a statistically significant improvement in UEC score (+0.74 kg, p=0.5387) for Ace-ER treated patients (n=45, -2.25 kg) compared to placebo (n=43, -2.99 kg) patients for the change from baseline to 48 weeks. There were three pre-specified key secondary endpoints, including the lower extremity muscle strength composite score as measured by hand-held dynamometry (HHD), physical functioning using the Mobility domain of the GNE Myopathy-functional activity scale (GNEM-FAS), and a measure of muscle strength in knee extensors. The study did not meet any of these key secondary endpoints.

Overall, Ace-ER was well tolerated, with slightly more patients experiencing treatment emergent adverse events and related treatment emergent adverse events. There were three serious adverse events including two on Ace-ER and one on placebo, none of which were considered treatment related. There were no discontinuations due to treatment emergent adverse events, and there have been no deaths in the study.

The company plans to terminate the development program based on these results and will work with investigators and patient groups to make available the valuable natural history data and development tools for the development of other therapies. The company will be

working with investigators and patients on a reasonable transition plan for patients still on Ace-ER.

**About Ultragenyx**

Ultragenyx is a clinical-stage biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with a focus on serious, debilitating genetic diseases. Founded in 2010, the company has rapidly built a diverse portfolio of product candidates with the potential to address diseases for which the unmet medical need is high, the biology for treatment is clear, and for which there are no approved therapies.

The company is led by a management team experienced in the development and commercialization of rare disease therapeutics. Ultragenyx's strategy is predicated upon time and cost-efficient drug development, with the goal of delivering safe and effective therapies to patients with the utmost urgency.

For more information on Ultragenyx, please visit the company's website at www.ultragenyx.com.

**Forward-Looking Statements**

*Except for the historical information contained herein, the matters set forth in this press release, including statements regarding Ultragenyx's expectations regarding plans for its clinical programs and ongoing or additional studies for its product candidates, are forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development programs, 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 uncertainties inherent in the clinical drug development process, such as the regulatory approval process, the timing of our regulatory filings, and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations and the availability or commercial potential of our drug candidates. Ultragenyx 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 Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on July 28, 2017, and its subsequent periodic reports filed with the Securities and Exchange Commission.*

**Exhibit 99.2**

Contact Ultragenyx Pharmaceutical Inc.

Investors & Media

Ryan Martins

415-483-8257

Contact Kyowa Hakko Kirin Co. Ltd.

Media

Hiroki Nakamura

+81-3-5205-7205

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**Ultragenyx and Kyowa Hakko Kirin Announce Submission of Burosumab's Biologics License Application in the US**

**Novato, CA and Tokyo, Japan— August 24, 2017 —** Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, and Kyowa Hakko Kirin Co., Ltd. (Kyowa Hakko Kirin) today announced that Ultragenyx has submitted a Biologics License Application (BLA) with the Food and Drug Administration (FDA) for burosumab to treat X-Linked Hypophosphatemia (XLH) in the US. Burosumab has previously received Breakthrough Therapy Designation from the FDA for the treatment of XLH in pediatric patients one year of age and older.

The FDA will evaluate the submission and will decide on whether to accept it within 60 days, at which time the Prescription Drug User Fee Act (PDUFA) action date will be set.

Ultragenyx, Kyowa Hakko Kirin and Kyowa Kirin International PLC., a wholly owned subsidiary of Kyowa Hakko Kirin, have been collaborating in development and commercialization of burosumab globally based on the collaboration and license agreement between Kyowa Hakko Kirin and Ultragenyx.

**About Burosumab**

Burosumab is an investigational recombinant fully human monoclonal IgG1 antibody, discovered by Kyowa Hakko Kirin, against the phosphaturic hormone fibroblast growth factor 23 (FGF23). FGF23 is a hormone that reduces serum levels of phosphorus and active vitamin D by regulating phosphate excretion and active vitamin D production by the kidney. Burosumab is being developed by Ultragenyx and Kyowa Hakko Kirin to treat XLH and tumor-induced osteomalacia (TIO), diseases characterized by excess levels of FGF23.

Phosphate wasting in XLH and TIO is caused by excessive levels and activity of FGF23. Burosumab is designed to bind to and thereby inhibit the biological activity of FGF23. By blocking excess FGF23 in patients with XLH and TIO, burosumab is intended to increase phosphate reabsorption from the kidney and increase the production of vitamin D, which enhances intestinal absorption of phosphate and calcium.

A clinical program studying burosumab in adults and pediatric patients with XLH is ongoing. Burosumab is also being developed for TIO, a disease characterized by typically benign tumors that produce excess levels of FGF23, which can lead to severe osteomalacia, fractures, bone and muscle pain, and muscle weakness.

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For more information on Ultragenyx, please visit the company's website at www.ultragenyx.com.

**About Kyowa Kirin**

Kyowa Hakko Kirin Co., Ltd. is a research-based life sciences company, with special strengths in biotechnologies. In the core therapeutic areas of oncology, nephrology and immunology/allergy, Kyowa Hakko Kirin leverages leading-edge biotechnologies centered on antibody technologies, to continually discover innovative new drugs and to develop and market those drugs world-wide. In this way, the company is working to realize its vision of becoming a Japan-based global specialty pharmaceutical company that contributes to the health and wellbeing of people around the world.

Kyowa Kirin International PLC is a wholly owned subsidiary of Kyowa Hakko Kirin and is a rapidly growing specialty pharmaceutical company engaged in the development and commercialization of prescription medicines for the treatment of unmet therapeutic needs in Europe and the United States. Kyowa Kirin International is headquartered in Scotland.

You can learn more about the business at: www.kyowa-kirin.com.

**Forward-Looking Statements**

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