Ultragenyx Announces Negative Topline Results from Phase 3 Study of UX007 in Patients with Glut1 DS with Disabling Movement Disorders

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Ultragenyx discontinuing development of UX007 in Glut1 DS indication

Company on track with separate UX007 program in LC-FAOD with pre-NDA meeting later this year

NOVATO, Calif., Oct. 26, 2018 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for serious rare and ultra-rare genetic diseases, today announced its Phase 3 study of UX007 in patients with glucose transporter type-1 deficiency syndrome (Glut1 DS) experiencing disabling paroxysmal movement disorders did not achieve its primary endpoint of demonstrating a statistically significant reduction in the frequency of paroxysmal movement events with UX007 treatment compared to placebo, and did not demonstrate a meaningful difference between treatment groups. The study also did not meet its key secondary endpoints. The safety profile observed in this study was consistent with what has been previously reported with UX007. Ultragenyx plans to discontinue the Glut1 DS development program, and will work with investigators and patients on a reasonable transition plan for patients with Glut1 DS who are still on UX007.

Ultragenyx has a separate program evaluating UX007 in long-chain fatty oxidation disorders (LC-FAOD), which continues on track. The U.S. Food and Drug Administration (FDA) has accepted the Company's proposal to submit a New Drug Application (NDA) for UX007 for the treatment of LC-FAOD based on existing data, and a pre-NDA meeting with the FDA will take place before the end of 2018. In the EU, Ultragenyx will discuss these data with the European Medicines Agency and expects to have an update this year.

"We had previously observed significant improvements in individual cases of Glut1 DS with UX007 and so we are particularly disappointed by the results of the Glut1 DS study in a larger group of patients that did not demonstrate this same effect. We are grateful for the commitment and dedication of patients, caregivers, and investigators as well as our employees to this program," said Emil Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "We continue to move forward with our UX007 program in long-chain fatty acid disorders which is not affected by this Glut1 DS outcome. We look forward to providing an update on UX007 regulatory discussions later this year."

The Phase 3 UX007 Glut1 DS study enrolled 44 children and adults experiencing disabling paroxysmal movement disorders associated with Glut1 DS. Eligible patients were randomized in a 1:1 ratio to one of two treatment sequences. Patients in the first group had a two-week titration period followed by an eight-week treatment period on UX007. They then began a two-week washout period, followed by a two-week titration period and an eight-week period on placebo. Patients in the second group followed the same schedule but started with placebo and then crossed over to UX007. Following the 22-week blinded crossover study period, patients could roll into the open-label extension period to continue on UX007 treatment. The primary endpoint was the difference in frequency of disabling paroxysmal movement disorders with UX007 compared to placebo. Secondary efficacy endpoints included: duration of disabling paroxysmal movement disorder events; walking capacity and endurance; patient-reported health-related quality of life assessments of physical function, mobility, upper extremity function, fatigue and pain.

About Ultragenyx Pharmaceutical Inc.
Ultragenyx is a biopharmaceutical company committed to bringing to patients novel products for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved therapies and product candidates aimed at addressing diseases with high unmet medical need and clear biology for treatment, 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 relating to Ultragenyx's plans for its clinical programs and future regulatory interactions, 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 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 products and 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 Ultragenyx in general, see Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on August 3, 2018, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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