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Ultragenyx Initiates Global Phase 3 Study of UX007 in Glut1 DS Patients with Disabling Movement Disorders

NOVATO, Calif., April 27, 2017 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ:RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, today announced the initiation of the Phase 3 study of UX007 for the treatment of Glucose Transporter Type 1 Deficiency Syndrome (Glut1 DS) patients experiencing disabling paroxysmal movement disorders.

"The initiation of this Phase 3 study in patients with Glut1 DS is a significant step forward in our development of UX007," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "Data from the previous investigator-sponsored study showed promise in substantially reducing paroxysmal motor events."

About the Phase 3 Study
This Phase 3 study is a global, randomized, double-blind, placebo-controlled, cross-over study designed to assess the efficacy and safety of UX007 in approximately 40 patients who are experiencing disabling paroxysmal movement disorders associated with Glut1 DS. Movement disorder events are defined as disabling if they affect or limit a patient's ability to perform activities of daily living. Eligible patients are randomized in a 1:1 ratio to one of two treatment sequences. Patients in the first group will begin a two-week titration period followed by an eight-week treatment period on UX007. Patients will then begin a 2-week washout period, followed by a 2-week titration period and 8-week period on placebo. Patients in the second group will follow the same schedule but will start with placebo and then cross over to UX007.

The primary endpoint compares the frequency of disabling paroxysmal movement disorder events during the 8-week treatment period with UX007, to the frequency of disabling movement disorder events during the 8-week placebo treatment period as recorded by a daily electronic diary. Secondary endpoints include the duration of disabling paroxysmal movement disorder events; walking capacity and endurance measured by the 12-minute walk test; patient-reported health-related quality of life assessments of physical function, mobility, upper extremity function, fatigue and pain; cognitive function and safety. Following the 22-week blinded crossover study period, patients may roll into the open-label extension period to continue on UX007 treatment.

About Glut1 DS and UX007

Glut1 DS is a severely debilitating disease characterized by seizures, developmental delay, and movement disorders. Glut1 DS is caused by a genetic defect in the transport of glucose into the brain. Because glucose is the primary source of energy for the brain, this disorder results in a chronic state of energy deficiency in the brain. Studies suggest a range of 3,000 to 7,000 Glut1 DS patients in the United States. There are currently no FDA approved treatments specific to Glut1 DS, though patients with the seizure phenotype are typically on the ketogenic diet.

UX007 is a highly purified, pharmaceutical-grade synthetic seven carbon fatty acid triglyceride created via a multi-step chemical process. It is an investigational medicine intended to provide patients with medium-length, odd-chain fatty acids that can be metabolized to increase intermediate substrates in the Krebs cycle, a key energy-generating process. Unlike typical even-chain fatty acids, UX007 can be converted to new glucose through the Krebs cycle, potentially providing an important added therapeutic effect, particularly when glucose levels are too low.

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 the design and timing of clinical 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 Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 17, 2017, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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