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Ultragenyx Announces Positive Data From Investigator-Sponsored Trial of Triheptanoin in Glucose Transporter Type-1 Deficiency Syndrome

*Data Demonstrate a Substantial Reduction in Movement Disorder Events
Company-Sponsored Study in Glut1 DS Movement Disorder Phenotype Planned*

NOVATO, Calif., April 22, 2015 (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 presentation of positive data from an investigator-sponsored trial of triheptanoin (UX007) for the treatment of movement disorders associated with glucose transporter type-1 deficiency syndrome (Glut1 DS). Glut1 DS, also known as De Vivo disease, is a rare and potentially severely debilitating disease characterized by seizures, developmental delay, and movement disorders. The data were presented at the American Academy of Neurology (AAN) Annual Meeting in Washington D.C. Based on the study results, Ultragenyx intends to initiate a confirmatory randomized double-blind placebo-controlled study of triheptanoin in the Glut1 DS movement disorder phenotype and anticipates discussions with the U.S. Food and Drug Administration (FDA) on final study design details in the second half of 2015.

"The movement disorders associated with Glut1 deficiency are a significant unmet medical need that can persist into adulthood and there are no approved treatment options," commented Sunil Agarwal, M.D., Chief Medical Officer of Ultragenyx. "The positive data presented at AAN have prompted us to expand our development efforts for triheptanoin in Glut1 deficiency to include a new study focused on movement disorders, in addition to the ongoing study in seizures."

The open-label investigator-sponsored trial enrolled eight Glut1 DS patients between 7 and 47 years old with non-epileptic paroxysmal manifestations, a neurological symptom characterized by sudden, transient, involuntary movements. Patients completed comprehensive diaries to record all motor and non-motor events as well as a clinical global impression scale during a baseline, treatment, and withdrawal phase, each lasting two months. Six patients completed the study. Ultragenyx provided financial support and triheptanoin for the study.

During the baseline phase without triheptanoin, patients experienced on average 31 paroxysmal manifestations, including 16 dystonic events. During the triheptanoin treatment phase, patients reported a statistically significant 90% reduction in these events to an average of three paroxysmal manifestations, including two dystonic events ($p=0.028$). In the withdrawal phase, when triheptanoin treatment was discontinued, the rate of paroxysmal manifestations increased substantially to an average of 24 events per patient, including 12 dystonic events ($p=0.043$).

In addition, patients reported an improvement in the clinical global impression scale during the treatment phase with triheptanoin and a worsening after withdrawal. This improvement was also associated with a normalization of induction of brain energy metabolism during visual stimulation in patients receiving triheptanoin as measured by ^{31}P -NMR spectroscopy ($p=0.021$).

With respect to safety, triheptanoin was well tolerated in all patients. Two patients were considered not compliant for reasons unrelated to safety or tolerability.

About Glut1 DS and Triheptanoin

Glut1 DS is a severely debilitating disease characterized by seizures, developmental delay, and movement disorder. 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.

Triheptanoin, also known as UX007, is a purified, pharmaceutical-grade, specially designed synthetic triglyceride compound created via a multi-step chemical process. Triheptanoin is metabolized to and intended to provide patients with heptanoate, which can diffuse across the blood-brain barrier and be converted into glucose. Heptanoate can also be further metabolized to four- and five-carbon ketone bodies in the liver that also cross the blood-brain-barrier and provide an additional energy source to the brain. Heptanoate and five-carbon ketone bodies can also regenerate new glucose in the brain, which is deficient in

these patients. Ultragenyx is currently conducting a Phase 2 study in the U.S. and Europe to evaluate the potential of triheptanoin to treat Glut1 DS patients who have failed the ketogenic diet and who continue to have breakthrough seizures.

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 timing of initiation of a clinical study of triheptanoin in the Glut1 DS movement disorder phenotype and the timing of expected discussions with the FDA regarding study design, 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, including 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 March 27, 2015, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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