March 11, 2014

Ultragenyx Announces First Patient Enrolled in Phase 2 Study of Triheptanoin in Glucose Transporter Type-1 Deficiency Syndrome

Novato, CA, March 11, 2014 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, announced the first patient enrolled in the Phase 2 study of triheptanoin (UX007) for the treatment of glucose transporter type-1 deficiency syndrome (Glut1 DS), at Columbia University. Glut1 DS, also known as De Vivo disease, is a rare, severely debilitating disease characterized by seizures, developmental delay, and movement disorder.

"Glut1 deficiency was first described by us at the Columbia University Medical Center in 1991, so we are pleased to have the opportunity to enroll the first patient in the triheptanoin clinical trial sponsored by Ultragenyx. It is clear that more effective symptomatic and disease-modifying treatments are needed for this rare disease, and we hope this trial is the next step towards that ultimate goal," said Dr. Darryl De Vivo, the Sidney Carter Professor of Neurology and Professor of Pediatrics at Columbia University Medical Center in New York City.

The global, randomized, double-blind, placebo-controlled, parallel-group Phase 2 clinical trial will evaluate safety and efficacy in up to 50 Glut1 DS patients between 3 and 17 years of age inclusive, who are currently not on or not fully compliant with ketogenic diet and continue to have seizures. The primary efficacy objective is the reduction in frequency of seizures compared to placebo following a 6-week baseline period and subsequent 8-week placebo-controlled treatment period. The blinded treatment period will be followed by an open-label extension period in which all patients will be treated with triheptanoin through week 52. Targeted enrollment may be modified based on an interim analysis. We anticipate that data from this trial will be available in 2015.

"We are advancing triheptanoin for Glut1 DS based on its anticipated ability to provide an alternative source of energy to the brain that could result in seizure control and improvement in other aspects of the disease," commented Emil D. Kakkis, M.D., Ph.D., Ultragenyx's Chief Executive Officer. "The placebo-controlled Phase 2 study is a significant step in the evaluation of triheptanoin for this rare and devastating disease."

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 mutation in the gene encoding the Glut1 protein, which is responsible for transporting glucose across the blood-brain barrier. Because glucose is the primary source of energy for the brain, this disorder results in a chronic state of energy deficiency in the brain. Glut1 DS is a rare disease. Studies suggest a range of 3,000 to 7,000 Glut1 DS patients in the United States. There are currently no approved treatments specific to Glut1 DS.

Triheptanoin is a specially designed synthetic triglyceride compound intended to provide patients with the medium-length, odd-chain fatty acid, heptanoate. Heptanoate can circulate and also be further metabolized to four- and five-carbon ketone bodies in the liver. All of these metabolites are able to cross the blood-brain barrier without using the deficient Glut1 transporter, and can provide an alternative energy source for the brain when glucose is limited. Heptanoate also crosses the blood-brain barrier and can be converted to glucose.

About Ultragenyx

Ultragenyx is a development-stage biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with an initial focus on serious, debilitating metabolic 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 plans, potential opportunities, expectations, projections, goals, objectives, milestones, strategies, product pipeline, clinical studies, product development and the potential benefits of its products under development 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 the availability or commercial potential of our drug candidate. 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 prospectus filed with the Securities and Exchange Commission on January 31, 2014, and its future periodic reports to be filed with the Securities and Exchange Commission.

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