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Ultragenyx Announces Update to UX007 Development Program in Glucose Transporter Type-1 Deficiency Syndrome

Pivotal Phase 3 Movement Disorder Study to Initiate Mid-2016

NOVATO, Calif., Nov. 06, 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 an update to its development plan for UX007 in Glut1 DS patients. Following an End-of-Phase 2 meeting with the FDA, the company now plans to initiate a Phase 3 study in Glut1 DS patients with the movement disorder phenotype in mid-2016. The ongoing Phase 2 study in patients with the seizure phenotype will continue to enroll up to 40 patients as the movement disorder study progresses. If positive, the two studies are intended to support an NDA filing for the treatment of Glut1 DS.

"The initiation of a pivotal study in the movement disorder phenotype is an exciting step forward for the Glut1 DS program," said Sunil Agarwal, M.D, Chief Medical Officer of Ultragenyx. "The combination of this study with the ongoing seizure study allows us to better assess the potential benefits of UX007 across both of the key disease manifestations of Glut1 DS."

The Phase 3 movement disorder study is designed to enroll approximately 40 patients. It is intended to be a randomized, double-blind, placebo-controlled, double cross-over study. The primary endpoint will be an assessment of the impact of UX007 on movement disorder events as recorded by a patient diary that will be further refined in discussions with the FDA. The company will continue enrollment of up to 40 patients in the randomized placebo-controlled Phase 2 seizure study. As the pivotal Phase 3 movement disorder study will be ongoing in 2016 and given the value of the seizure study in potentially supporting the filing, we will no longer conduct an interim analysis of the current Phase 2 study in the seizure phenotype. This will allow us to preserve the integrity of the Phase 2 study and maximize its utility from a regulatory perspective.

About Glut1 DS and UX007

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.

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 randomized, placebo-controlled 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. An investigator-initiated pilot study of UX007 in six Glut1 DS patients with movement disorder was recently presented at the American Association of Neurology 2015 meeting showing a substantial reduction in the frequency of movement disorders during treatment that rebounded after withdrawal and suggested a reasonable safety profile.

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 the anticipated timing of initiation of a Phase 3 study in Glut1 DS patients with the movement disorder phenotype, 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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on August 14, 2015, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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