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Ultragenyx Announces Initiation of Phase 2 Study for Patients with Long-Chain Fatty Acid Oxidation Disorders

Open-Label Study to Assess Safety and Clinical Effects of Triheptanoin

NOVATO, Calif., Feb. 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, today announced that the first patient has been enrolled in an open-label Phase 2 study to assess safety and clinical effects of triheptanoin, also known as UX007, in patients severely affected by long-chain fatty acid oxidation disorders (LC-FAOD). LC-FAOD are a group of autosomal recessive genetic disorders characterized by metabolic deficiencies in which the body is unable to break down and convert long chain fatty acids into energy.

"We look forward to studying triheptanoin in this prospective clinical study and taking another step towards determining the potential role for triheptanoin in helping patients with LC-FAOD to better manage their debilitating disease," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "The goal for this study is to determine the optimal patient population and endpoints for evaluation in a potential future Phase 3 clinical trial."

The prospective, interventional, open-label Phase 2 study will evaluate triheptanoin treatment in approximately 30 severely affected LC-FAOD patients, ages 6 months to 35 years, exhibiting significant clinical manifestations of LC-FAOD despite current therapy. Prior to initiating treatment with triheptanoin, subjects will continue their current therapy for four weeks to establish their baseline condition. Triheptanoin will then be titrated to an expected target dose of 25-35% of total daily caloric intake via oral administration, while ensuring tolerability. The patients will be followed to evaluate the effects of triheptanoin treatment over 24 weeks, then may continue treatment for an additional 54 weeks. The study will assess the impact of triheptanoin on several endpoints, including cycle ergometer performance, 12-minute walk test, muscle strength, creatine kinase levels, hypoglycemia, liver size, and cardiac disease. The study will be conducted at approximately eight clinical sites in the United States and Europe.

During the treatment period, the primary objective of the study is to evaluate the impact of triheptanoin on acute clinical pathophysiology associated with LC-FAOD, while the secondary objectives of the study are to evaluate the safety of triheptanoin treatment and its effects on energy metabolism in LC-FAOD patients. The objective of the extension period of the study is to evaluate the impact of triheptanoin on major clinical events, including hospitalizations, emergency room visits, and emergency interventions associated with LC-FAOD.

About LC-FAOD and Triheptanoin

LC-FAOD are a group of autosomal recessive genetic disorders characterized by metabolic deficiencies in which the body is unable to convert long-chain fatty acids into energy. It is estimated that 2,000 to 3,500 patients are afflicted with LC-FAOD in the US, where fatty acid oxidation disorders are now detected by newborn screening. There are six main genetic diseases that cause LC-FAOD, and the main clinical program is focused on the most common four forms: carnitine palmitoyltransferase II (CPT-II) deficiency, long-chain 3-hydroxyacyl-CoA dehydrogenase (LCHAD) deficiency, trifunctional protein deficiency (TFP), and very long-chain acyl-CoA dehydrogenase (VLCAD) deficiency.

LC-FAOD patients are currently treated by the avoidance of fasting, low-fat/high carbohydrate diets, carnitine supplementation and medium chain triglyceride (MCT) oil. Despite current therapy, many patients still have significant metabolic events including hospitalizations and significantly increased mortality due to LC-FAOD; a mortality rate of more than 50% has been observed in spite of treatment with current therapy.

Triheptanoin, also known as UX007, is a purified form of a specially designed synthetic triglyceride compound. Triheptanoin is intended to provide patients with medium-length, odd-chain fatty acids that are metabolized to replace intermediate substrates in fatty acid oxidation downstream of their genetic block in fatty acid metabolism. Triheptanoin is also metabolized to a substrate that replaces deficient intermediates in the tricarboxylic acid (TCA cycle), a key energy-generating process. Triheptanoin can also support production of glucose and glycogen (gluconeogenesis). Together, the substrates produced by triheptanoin during metabolism are intended to improve energy production in FAOD patients.

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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