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Ultragenyx Granted Additional Orphan Drug Designations for Triheptanoin

NOVATO, Calif., April 23, 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 that it has received two orphan drug designations for triheptanoin (UX007). The U.S. Food and Drug Administration (FDA) Office of Orphan Products Development has granted orphan drug designation for triheptanoin for the treatment of fatty acid oxidation disorders (FAOD), and the European Commission has granted orphan medicinal product designation for the treatment of glucose transporter type-1 deficiency syndrome (Glut1 DS). The FDA granted orphan drug designation for triheptanoin in Glut1 DS in October 2014.

FAOD is 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. Glut1 DS, also known as De Vivo disease, is a rare and potentially severely debilitating disease characterized by seizures, developmental delay, and movement disorder. Ultragenyx is conducting two separate Phase 2 studies of triheptanoin in patients with FAOD and Glut1 DS.

The FDA Orphan Drug Designation program provides orphan status to drugs and biologics that are intended for the safe and effective treatment, diagnosis, or prevention of rare diseases/disorders that affect fewer than 200,000 people in the U.S. Among the benefits of orphan designation in the U.S. are seven years of market exclusivity following FDA approval, waiver or partial payment of application fees, and tax credits for clinical testing expenses conducted after orphan designation is received.

The European Commission grants orphan drug status for medicinal products intended to treat diseases or conditions that affect fewer than five in 10,000 people in the European Union. The designation provides certain benefits and incentives in the EU, including protocol assistance, fee reductions, and ten years of market exclusivity once the medicine is on the market.

About Triheptanoin

Triheptanoin, also known as UX007, is a purified, pharmaceutical-grade, specially designed synthetic triglyceride compound created via a multi-step chemical process. Ultragenyx is currently evaluating triheptanoin in two clinical programs.

The first program is studying the genetic neurological disorder Glut1 DS. This disease is caused by a genetic defect in the transport of glucose into the brain and affects an estimated 3,000 to 7,000 patients in the U.S. Glut1 DS is characterized by seizures, developmental delay, and movement disorder. Triheptanoin is metabolized to heptanoate, which can diffuse across the blood-brain barrier and be converted into glucose. Heptanoate can also be further metabolized in the liver to four- and five-carbon ketone bodies that also cross the blood-brain-barrier. Both are intended to provide patients with 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 conducting a Phase 2 study in the U.S. and Europe to evaluate the potential of triheptanoin to treat Glut1 DS patients who are not on or who have failed the ketogenic diet and continue to have seizures. Ultragenyx intends to initiate a second clinical study focused on movement disorders associated with Glut1 DS.

The second program is studying LC-FAOD, a set of rare metabolic diseases caused by the inability to convert fat into energy, leading to low blood sugar, muscle rupture, and heart and liver disease. Triheptanoin is 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, triheptanoin can be converted to new glucose through the Krebs cycle, potentially providing an important added therapeutic effect, particularly when glucose levels are too low. Ultragenyx is conducting a Phase 2 study to evaluate the potential of triheptanoin to treat FAOD.

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 intention to initiate a clinical study of triheptanoin in the Glut1 DS 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 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.

CONTACT: Ultragenyx Pharmaceutical Inc.

Investors & Media

Robert Anstey

844-758-7273