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Ultragenyx Granted Orphan Drug Designation for Triheptanoin for the Treatment of Glucose Transporter Type-1 Deficiency Syndrome

Novato, CA, Oct. 27, 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 FDA Office of Orphan Products Development has granted orphan drug designation for triheptanoin (UX007) for the treatment of 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 disorder. Ultragenyx is conducting a Phase 2 study of triheptanoin in patients with Glut1 DS.

"Glut1 deficiency syndrome is a rare condition that can severely affect patients and their families. Ultragenyx is committed to addressing this high unmet medical need population and has initiated a rigorous development plan to do so. The FDA's decision to grant triheptanoin orphan drug designation for Glut1 deficiency syndrome is another important step in our plan to help treat patients with this disease," said Sunil Agarwal, M.D., Chief Medical Officer of Ultragenyx.

The [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.

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.

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 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 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.

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