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Ultragenyx Announces License of Intellectual Property Related to the Treatment of Huntington's Disease With Triheptanoin

NOVATO, Calif., Jan. 7, 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 a license agreement with Inserm Transfert SA and Institut du Cerveau et de la Moelle Epiniere (ICM) for intellectual property related to the treatment of Huntington's disease with triheptanoin (UX007).

"Huntington's is a severe and lethal rare genetic disease," commented Emil D. Kakkis, Ph.D., M.D., Chief Executive Officer and President of Ultragenyx. "Energy deficiency is believed to play a role in the pathophysiology of Huntington's disease, and we are encouraged by data from a small pilot study suggesting improvement in brain energy metabolism after treatment with triheptanoin."

"This pilot study, as well as previous metabolic studies that we performed, indicate that brain energy deficit is a realistic therapeutic target in Huntington's disease," said Fanny Mochel, M.D., Ph.D., principal investigator of the study at ICM.

Ultragenyx is supporting a second investigator-sponsored clinical study being planned by ICM to evaluate the safety and efficacy of triheptanoin in patients with Huntington's disease.

About Huntington's Disease

Huntington's disease is an autosomal dominant neurodegenerative disease characterized by movement disorders (chorea), behavioral and psychiatric disturbances, dementia, and death. The clinical features of the disease usually emerge between 30 and 50 years of age. The only approved therapy is indicated for the treatment of chorea associated with the disease. There are an estimated 30,000 patients with Huntington's disease in the U.S.

A pilot study was completed in ten patients with early stage Huntington's disease in which triheptanoin appeared to impact brain energy metabolism and the Unified Huntington's Disease Rating Scale motor score. Results from the study were published in the January 7, 2015 online issue of *Neurology*, the medical journal of the American Academy of Neurology.

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 seizure disorder Glut1 deficiency syndrome (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.

The second program is studying long-chain fatty acid oxidation disorders (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 LC-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 a planned investigator-sponsored clinical study, 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 related to timing and results of the planned investigator-sponsored clinical study, whether or not we ever pursue our own clinical study and whether or not we ever seek to obtain approval for the use of triheptanoin to treat Huntington's disease, as well as 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 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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on November 10, 2014, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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