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Ultragenyx Announces License of Intellectual Property for the Treatment of Epilepsy and Other Seizure-Related Disorders With Triheptanoin

New Patent Also Issued With Composition Claims for Triheptanoin

NOVATO, Calif., Aug. 5, 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 a license agreement with UniQuest Pty Limited for intellectual property related to the treatment of refractory epilepsy and other seizure-related and neurologic disorders with triheptanoin (UX007). The intellectual property originated from research on epilepsy and other neurologic models conducted at The University of Queensland.

Separately, a United States (U.S.) patent has been issued with claims directed to compositions of triheptanoin above a certain level of purity. The patent term expires in October 2025. A second U.S. patent with claims directed to compositions of triheptanoin in a dosage unit was issued in August 2013 and expires in June 2020. These patent terms do not include any potential for patent term extension of up to five additional years. Similar claims are either granted or being pursued by Ultragenyx in other territories outside the U.S.

"The completion of the license agreement with UniQuest and the issuance of additional composition patent claims mark the continued strengthening of our triheptanoin portfolio," commented Emil D. Kakkis, Ph.D., M.D., Chief Executive Officer and President of Ultragenyx. "We are excited about the potential for triheptanoin to be used in certain difficult-to-treat seizure disorders and hope it will provide a new treatment option for affected patients."

About Refractory Epilepsy

Epilepsy is a family of chronic neurological disorders characterized by spontaneous recurrent seizures. There are approximately two million epilepsy patients in the U.S. of various types, some of which are genetic in origin. Refractory epilepsy occurs in patients whose seizures cannot be adequately controlled by anti-epileptic drugs, which happens in approximately one third of cases.

About Triheptanoin

Triheptanoin, also known as UX007, is a purified form of a specially designed synthetic triglyceride compound. 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 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.

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 are 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 any potential for patent term extension, the pursuit of patent claims in territories outside of the U.S., the potential for triheptanoin to be used in difficult-to-treat seizure disorders and to provide a new treatment option for seizure treatment of these patients and the potential for triheptanoin to provide a therapeutic effect to patients with LC-FAOD, 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 patent term extension, 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 May 12, 2014, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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