Ultragenyx Announces FDA Accepts Proposal to Submit an NDA for UX007 for the Treatment of Long-Chain Fatty Acid Oxidation Disorders

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NOVATO, Calif., Aug. 29, 2018 (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 U.S. Food and Drug Administration (FDA) has accepted Ultragenyx's most recent proposal to submit a New Drug Application (NDA) for UX007 for the treatment of long-chain fatty acid oxidation disorders (LC-FAOD) based on existing data. Further details regarding timing will be forthcoming following a pre-NDA meeting, which is being scheduled for the second half of 2018.

"We appreciate FDA's review of multiple data submissions and collaboration with us to develop a path for an early filing, and it is our commitment to get this important potential treatment to patients with this serious disease as quickly as possible," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "We will meet with the FDA to discuss the details of the NDA submission and, if approved, appropriate post-approval commitments to further evaluate long-term outcomes of UX007 in patients with LC-FAOD."

The data submitted to the FDA for evaluation included the recently published 78-week Phase 2 study results in 29 patients, a now published retrospective medical record review of 20 patients, and 56 emergency IND cardiomyopathy and other patients. In addition, these data were supported by results of a published randomized controlled investigator study of 32 patients showing an effect of triheptanoin on cardiac function. In the 78-week sponsored Phase 2 study, the data showed a 48.1 percent reduction in the mean annualized rate of major clinical events (MCEs) and a 50.6 percent reduction in the median annualized rate of MCEs after 78 weeks of treatment with UX007 compared to an annualized rate of MCEs in the 18 months prior to treatment with UX007. There was also a 50.3 percent reduction in the mean annualized duration of MCEs and a 76.7 percent reduction in the median annualized duration of MCEs following 78 weeks of UX007 treatment. The safety profile was consistent with what has been previously observed with UX007.

In the EU, Ultragenyx will discuss these data with the European Medicines Agency (EMA) and expects to have an update in the second half of 2018.

About LC-FAOD and UX007

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. The inability to produce energy from fat can lead to severe depletion of glucose in the body, and serious liver, muscle and heart disease, which can lead to hospitalizations or early death. LC-FAOD are included in newborn screening panels across the U.S. and in certain European countries. Patients with LC-FAOD are currently treated with the avoidance of fasting, low-fat/high carbohydrate diets, carnitine, and medium-chain triglyceride (MCT) oil, a medical food product. Despite current therapy, many patients have significant metabolic events including hospitalizations and mortality due to LC-FAOD.

UX007 is a highly purified, pharmaceutical-grade, synthetic, seven-carbon fatty acid triglyceride created via a multi-step chemical process. It is an investigational medicine 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, UX007 can be converted to new glucose through the Krebs cycle, potentially providing an important added therapeutic effect, particularly when glucose levels are too low.

About Ultragenyx Pharmaceutical Inc.

Ultragenyx is a 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. The Company has rapidly built and advanced 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 related to Ultragenyx's expectations regarding plans for its clinical programs and future regulatory interactions, 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, such as the regulatory approval process, the timing of 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 products and 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 Ultragenyx in general, see

Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on August 3, 2018, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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