Ultragenyx Announces Intent to Submit New Drug Application to U.S. FDA for UX007 for the Treatment of Long-chain Fatty Acid Oxidation Disorders in Mid-2019

November 14, 2018

NOVATO, Calif., Nov. 14, 2018 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for serious rare and ultra-rare genetic diseases, today announced that it has completed a pre-NDA meeting with the U.S. Food and Drug Administration (FDA) and plans to submit a New Drug Application (NDA) to the FDA for UX007 for the treatment of patients with long-chain fatty acid oxidation disorders (LC-FAOD) in mid-2019.

“In our recent pre-NDA meeting with the FDA, we were pleased to reach an agreement on the details of our regulatory submission,” said Camille L. Bedrosian, M.D., Chief Medical Officer of Ultragenyx. “We look forward to working with the FDA throughout the review process, and it is our commitment to bring this potential treatment to patients with this serious disease as quickly as possible.”

The NDA package will include data from the Phase 2 company-sponsored study of UX007 in 29 patients, a long-term efficacy and safety extension study in 75 patients, a retrospective medical record review of 20 patients, data from 56 patients treated through expanded access, and a randomized controlled investigator-sponsored 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. The pre-NDA meeting clarified what information from these studies are considered most important by the FDA, and what analyses and data presentations they will look for in the NDA filing. An advisory committee is under consideration for this product candidate, but no confirmation that one would be convened was received during the pre-NDA meeting.

About LC-FAOD
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.

About UX007
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 patients novel products for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved therapies and product candidates aimed at addressing diseases with high unmet medical need and clear biology for treatment, 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, future regulatory interactions, and the sufficiency for, and timing of, regulatory submissions and approvals, 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 November 6, 2018, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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