Ultragenyx Announces UX007 Granted Fast Track Designation and Rare Pediatric Disease Designation by U.S. FDA for Treatment of Long-Chain Fatty Acid Oxidation Disorders

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Company on Track to Submit NDA to FDA in Mid-2019

NOVATO, Calif., April 16, 2019 (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 the U.S. Food and Drug Administration (FDA) has granted Fast Track designation and Rare Pediatric Disease designation to UX007 for the treatment of long-chain fatty acid oxidation disorders (LC-FAOD), a group of genetic disorders in which the body is unable to convert long-chain fatty acids into energy.

“These designations for UX007 underscore FDA’s belief that new treatments are needed for patients with LC-FAOD, a severe and potentially life-threatening disease. In addition, they offer the potential to speed up our ability to bring UX007 to patients,” said Camille L. Bedrosian, M.D., Chief Medical Officer of Ultragenyx.

Ultragenyx is on track to submit a New Drug Application (NDA) in mid-2019. The submission will include data from a company-sponsored Phase 2 study of UX007 in 29 patients, data from a long-term safety and efficacy extension study in 75 patients, a retrospective medical record review of 20 original compassionate use patients, data from 70 patients treated through expanded access, and a randomized controlled investigator-sponsored study of 32 patients showing an effect of triheptanoin on cardiac function.

About Fast Track Designation
The FDA’s Fast Track designation is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. Fast Track designation allows for early and frequent communication with the FDA throughout the entire drug development and review process, and allows for a rolling review of a company’s New Drug Application. It also enables eligibility for Priority Review, if relevant criteria are met.[i]

About Rare Pediatric Disease Designation
The FDA defines a "rare pediatric disease" as a serious or life-threatening rare disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years. Under the FDA's Rare Pediatric Disease Priority Review Voucher program, a sponsor who receives an approval for a drug or biologic for a “rare pediatric disease” may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product.[ii]

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 management, 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 typically no approved

therapies treating the underlying disease.

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 the potential timing and success of filings for regulatory approvals, the potential issuance of a priority review voucher, and the demonstrated impact and adequacy of clinical data and other information to support approval of product candidates, 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 Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 20, 2019, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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[i] https://www.fda.gov/forpatients/approvals/fast/ucm405399.htm. Accessed March 28, 2019.

[ii] https://www.fda.gov/forindustry/developingproductsforrarediseasesconditions/rarepediatricdiseasepriorityvoucherprogram/default.htm. Accessed March 28, 2019.

Source: Ultragenyx Pharmaceutical Inc.