Ultragenyx Announces FDA Clearance of Investigational New Drug (IND) Application for UX053, an mRNA for the Treatment of Glycogen Storage Disease Type III

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NOVATO, Calif., March 08, 2021 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel products for serious rare and ultra-rare genetic diseases, today announced that the U.S. Food and Drug Administration (FDA) has cleared the Investigational New Drug (IND) application for UX053, an investigational mRNA therapy being evaluated for the treatment of Glycogen Storage Disease Type III (GSDIII). Enrollment in a Phase 1/2 study is expected to begin in the second half of 2021.

“FDA IND clearance paves the way for UX053 to enter the clinic as the first possible pharmacologic treatment option for patients with GSDIII,” said Camille L. Bedrosian, M.D., Chief Medical Officer of Ultragenyx. “UX053 is the most advanced of our investigational mRNA therapies, with multiple others in earlier preclinical development, and will be one of the first mRNA programs to enter clinical development for rare genetic diseases.”

Ultragenyx is developing UX053 and a number of other mRNA therapies in the preclinical stage for undisclosed indications. These mRNA therapies come out of a long-term collaboration with Arcturus Therapeutics, a clinical-stage messenger RNA medicines company.

Study Design

The two-part Phase 1/2 clinical trial will evaluate the safety, tolerability, and efficacy of UX053 in adults with GSDIII. Part 1 is open label and will enroll up to 12 patients who receive a single ascending dose of UX053. Part 2 is a randomized, double-blind, placebo-controlled multi-ascending study of 5 doses. It will enroll up to 16 patients across four cohorts randomized 3:1 to UX053 or placebo. In addition to safety, tolerability, and pharmacokinetics, study endpoints include clinician- and patient-reported outcomes, muscle strength, and biomarkers of liver, cardiac, and muscle health.

About Glycogen Storage Disease Type III

Our preclinical candidate UX053 is being developed for the treatment of GSDIII, a disease caused by a glycogen debranching enzyme (AGL) deficiency that results in glycogen accumulation in the liver and muscle. GSDIII can cause hepatomegaly, hypoglycemia, hyperlipidemia, some progressive liver cirrhosis, and skeletal and cardiac muscle disease. There are no approved treatment options for GSDIII and the current standard of care is a strict diet, including frequent doses of cornstarch, to reduce the risk of hypoglycemia. GSDIII affects more than 10,000 patients in the developed world.

About UX053

UX053 is an investigational mRNA-based biologic therapy encoding full-length, glycogen debranching enzyme encapsulated in a lipid nanoparticle (LNP) designed to provide the deficient protein in GSDIII.

About Ultragenyx Pharmaceutical Inc.
Ultragenyx is a biopharmaceutical company committed to bringing novel products to patients 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 and projections regarding its future operating results and financial performance, anticipated cost or expense reductions, the timing, progress and plans for its clinical programs and clinical studies, future regulatory interactions, and the components and timing of regulatory submissions 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, collaboration with third parties, 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 effects from the COVID-19 pandemic on the company's clinical activities, business and operating results, risks related to reliance on third party partners to conduct certain activities on the company's behalf, uncertainty and potential delays related to clinical drug development, smaller than anticipated market opportunities for the company's products and product candidates, manufacturing risks, competition from other therapies or products, and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations, the company's future operating results and financial performance, the timing of clinical trial activities and reporting results from same, and the availability or commercial potential of Ultragenyx's 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 12, 2021, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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