Ultragenyx Announces First Patient Dosed in Phase 1/2 Clinical Study of UX053, an mRNA Therapy for the Treatment of Glycogen Storage Disease Type III

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NOVATO, Calif., Dec. 01, 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 first patient has been dosed in its Phase 1/2 study of UX053, an investigational messenger RNA (mRNA) therapy in development for the treatment of Glycogen Storage Disease Type III (GSDIII).

“Initiation of this trial paves the way for UX053 to potentially become the first medicine for people living with GSDIII, who currently are burdened with managing a complex metabolic disorder with diet alone,” said Camille L. Bedrosian, M.D., Chief Medical Officer of Ultragenyx. “We believe mRNA is a promising treatment modality for rare diseases like GSDIII because this technology can deliver large genes to targeted cells and achieve a high uniformity of protein expression. We can also individualize mRNA therapies based on the needs of each patient by adjusting the dose level and dose frequency.”

UX053 is Ultragenyx’s most advanced investigational mRNA therapy. The company is developing a preclinical pipeline of mRNA therapies through its long-term collaboration with Arcturus Therapeutics Inc., a clinical-stage messenger RNA medicines company. More information about Ultragenyx’s broad portfolio of rare disease medicines can be found here: https://www.ultragenyx.com/our-research/our-pipeline/

Phase 1/2 Clinical Trial Design

The two-part Phase 1/2 clinical trial will evaluate the safety, tolerability and efficacy of UX053 in adults age 18 and older with GSDIII. Part 1 is open label and will enroll up to 10 patients who will receive a single ascending dose of UX053 administered via intravenous infusion. Part 2 is double-blind and will evaluate five repeat doses at escalating dose levels in up to 16 patients across four cohorts randomized 3:1 to UX053 or placebo. The primary endpoints are treatment-emergent adverse events (TEAEs), serious TEAEs and related TEAEs in both parts of the study. Secondary endpoints include pharmacokinetic parameters. Exploratory endpoints include clinician- and patient-reported outcomes, muscle strength, blood sugar, and biomarkers of liver, cardiac and muscle health. Further details can be referenced at: https://clinicaltrials.gov/ct2/show/NCT04990388.

About Glycogen Storage Disease Type III

GSDIII is caused by deficiency in the glycogen debranching enzyme, which results in glycogen accumulation in the liver, heart, and muscle and the inability to release glucose as needed to provide energy and regulate blood glucose levels. Patients with GSDIII experience hepatomegaly (enlarged liver), hypoglycemia, hyperlipidemia, progressive liver fibrosis (scarring), and skeletal and cardiac muscle disease. There are no approved medicines for GSDIII and the current standard of care is a strict diet, including frequent doses of cornstarch to reduce the risk of hypoglycemia, high protein intake, avoidance of fasting, and avoidance of simple sugars. GSDIII affects more than 10,000 patients worldwide.

About UX053
UX053 is an investigational mRNA therapy encoding full-length, glycogen debranching enzyme that is encapsulated in a lipid nanoparticle. It is designed to provide the deficient protein in patients with GSDIII. Preclinical studies of UX053 demonstrated reductions in glycogen content in the liver in multiple models of the disease. The U.S. Food and Drug Administration (FDA) and the European Commission have granted Orphan Drug Designation for UX053 for the treatment of 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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on November 3, 2021, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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