



Ultragenyx Announces Filing and FDA Clearance of an Investigational New Drug Application for DTX401, a Gene Therapy for the Treatment of Glycogen Storage Disease Type Ia

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NOVATO, Calif., April 23, 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 cleared the Investigational New Drug (IND) application for DTX401, an adeno-associated virus vector based gene therapy for the treatment of glycogen storage disease type Ia (GSDIa). Enrollment in the Phase 1/2 study is expected to begin in the first half of 2018, with data from the first cohort expected in the second half of 2018.

"GSDIa is a devastating disease that requires patients to adhere to a strict and burdensome cornstarch feeding protocol to maintain normal blood glucose levels and prevent hypoglycemia. Failure of dietary therapy can lead to episodes of severe hypoglycemia resulting in seizures and death," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "We look forward to initiating our clinical program for DTX401, our gene therapy designed to replace the deficient enzyme in the liver to improve glucose control and prevent the devastating short and long-term consequences of this disease."

The open-label, multicenter Phase 1/2 study will evaluate the safety, tolerability and therapeutic response of DTX401 in adults with GSDIa. Key efficacy assessments include time to hypoglycemia, impact on biomarkers such as lipids, uric acid, and measurement of glycogen in liver. There are three potential dosing cohorts in the study, and three patients will be enrolled in each cohort.

About GSDIa

GSDIa is the most common genetically inherited glycogen storage disease. It is caused by a defective gene for the enzyme glucose-6-phosphatase- α (G6Pase- α), resulting in the inability to regulate blood sugar (glucose). Hypoglycemia in GSDIa patients can be life-threatening, while the accumulation of the complex sugar glycogen in certain organs and tissues can impair the ability of these tissues to function normally. If chronically untreated, patients can develop severe lactic acidosis, progress to renal failure, and potentially die in infancy or childhood. There are no approved pharmacological therapies. An estimated 6,000 patients worldwide are affected by GSDIa.

About DTX401

DTX401 is an investigational adeno-associated virus vector (AAV) type 8 gene therapy designed to deliver stable expression and activity of G6Pase- α following a single intravenous infusion and has been shown in preclinical studies to improve G6Pase- α activity and reduce hepatic glycogen levels, a well-described biomarker of disease progression. DTX401 has been granted Orphan Drug Designation in both the United States and Europe.

About Ultragenyx Pharmaceutical Inc.

Ultragenyx is a biopharmaceutical company committed to bringing to patients novel products for the treatment of rare and ultra-rare diseases, with a focus on serious, debilitating genetic diseases. Founded in 2010, the company has rapidly built 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 relating to Ultragenyx's expectations regarding the timing of release of additional data for its product candidates, and plans for its clinical programs and its clinical studies, 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 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 21, 2018, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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