Ultragenyx Announces First Patient Dosed in Phase 1/2 study of DTX401, a Gene Therapy for the Treatment of Glycogen Storage Disease Type Ia (GSDIa); FDA Grants Fast Track Designation to Gene Therapy Program

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NOVATO, Calif., July 26, 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 first patient has been dosed in the Phase 1/2 study of DTX401, an adeno-associated virus vector based gene therapy for the treatment of patients with glycogen storage disease type Ia (GSDIa). Data from the three-patient first dose cohort are expected in the second half of 2018.

“For over 20 years, our team and the team at the National Institutes of Health (NIH) have worked for this day to occur. We are thrilled to see the gene therapy trial for GSD type Ia beginning,” said David Weinstein, M.D., M.M.Sc., Professor and Director, Glycogen Storage Disease Program at Connecticut Children’s Medical Center and UConn Health. “I am confident that patients with this condition around the world could benefit from this trial along with children and adults with other genetic liver diseases. This is truly an exciting day for the GSD community.”

The U.S. Food and Drug Administration (FDA) has also granted Fast Track designation to DTX401 for the treatment of GSDIa. The FDA Fast Track program is designed to facilitate the development and expedite the review of drugs that are intended to treat serious conditions and fill an unmet medical need. Fast Track designation allows for more frequent interaction with the FDA review team. It also enables eligibility for priority review if relevant criteria are met and the potential for a rolling review of the Investigational New Drug application (IND) as data become available.

“Advancing DTX401 into the clinic through our collaboration with Dr. Weinstein, the NIH and other GSD1a treatment centers marks an important milestone in developing a much-needed new potential treatment for patients with GSDIa, a highly debilitating disease with no approved treatment options,” said Eric Crombez, M.D., Chief Medical Officer of the Ultragenyx Gene Therapy development unit. “As our second inborn error of metabolism gene therapy program enters the clinic, we share the excitement of the GSDIa community. We believe that gene therapy offers the best treatment approach for the development of a new therapy that could profoundly improve the lives of patients living with GSDIa.”

About the Phase 1/2 Study
The open-label, dose-finding Phase 1/2 study evaluates 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. Patients in the first cohort receive a single dose of DTX401 of 2.0 × 10^12 GC/kg. The decision to proceed to the next, higher dose cohort will be made after the data monitoring committee evaluates the safety data for all patients in the previous dosing 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 patients with GSDIa can result in seizures and become 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

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 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 relating to Ultragenyx's expectations regarding the timing of release of additional data for product candidates, and plans for clinical programs and 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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on May 8, 2018, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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