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Ultragenyx Announces Completion of Patient Dosing in First Cohort of Phase 1/2 Clinical Study of DTX301 Gene Therapy in Ornithine Transcarbamylase (OTC) Deficiency

First Cohort Data Expected in Early 2018

NOVATO, Calif., Nov. 16, 2017 (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 it has completed patient dosing in the first cohort of three patients enrolled in the Phase 1/2 study of DTX301, an adeno-associated virus (AAV) gene therapy for the treatment of ornithine transcarbamylase (OTC) deficiency. Initial data from this first cohort of three patients is expected in early 2018.

The phase 1/2 clinical study is an open-label, dose-finding and safety study of single ascending doses of DTX301 in adults with late-onset OTC Deficiency who are clinically stable and under good metabolic control at time of dosing. Patients in the first cohort received a single dose of 2.0 × 10^12 GC/kg. To evaluate therapeutic response of DTX301, the study measures the change in the rate of ureagenesis, the pathway for the metabolism of ammonia which is deficient in OTC patients. This is determined using a well-established stable ^{13}C-acetate labeling approach. Ammonia levels, neurocognitive assessment, biomarkers, and safety will also be evaluated. 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 OTC Deficiency

OTC deficiency, the most common urea cycle disorder, is caused by a genetic defect in a liver enzyme responsible for detoxification of ammonia. Individuals with OTC deficiency can build up excessive levels of ammonia in their blood, potentially resulting in acute and chronic neurological deficits and other toxicities. It is estimated that more than 10,000 patients are affected by OTC deficiency worldwide, of which approximately 80% are classified as late-onset. In the late onset form of the disease, elevated ammonia can lead to significant medical issues for patients who are in need of new disease-modifying therapies. The greatest percentage of patients, including males and females, experience late-onset disease, representing a clinical spectrum of disease severity. Neonatal onset disease occurs in males, presents as severe disease, and can be fatal at an early age. Approved therapies, which must be taken multiple times a day for the patient's entire life, do not eliminate the risk of future metabolic crises. Currently, the only curative approach is liver transplantation.

About DTX301

DTX301 is an investigational AAV type 8 gene therapy designed to deliver stable expression and activity of OTC following a single intravenous infusion and has been shown in preclinical studies to normalize levels of urinary orotic acid, a marker of ammonia metabolism. DTX301 was granted Orphan Drug Designation in both the United States and Europe.

About Ultragenyx Pharmaceutical Inc.

Ultragenyx is a biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with a focus on serious, debilitating genetic diseases. The Company has rapidly built and advanced 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, plans for its clinical programs, and the potential market size and the size of the patient populations for its product candidates, if approved for commercial use, 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 November 3, 2017, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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