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Ultragenyx Announces Initiation of Phase 3 Study of Recombinant Human Beta-Glucuronidase in Mucopolysaccharidosis Type 7

Agreement Reached With Both FDA and EMA on Pivotal Trial Design

NOVATO, Calif., Dec. 15, 2014 (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 the dosing of the first patient in the pivotal Phase 3 study of recombinant human beta-glucuronidase (rhGUS, UX003), an investigational therapy for the treatment of Mucopolysaccharidosis 7 (MPS 7, Sly syndrome).

"We are pleased to have reached alignment with both the FDA and EMA on our single pivotal study design for this devastating disease," commented Sunil Agarwal, M.D., Chief Medical Officer of Ultragenyx. "This is an important step forward for these patients who have no approved treatment options. It is also an important milestone for Ultragenyx as it is the start of our first Phase 3 program."

The Phase 3 global, randomized, placebo-controlled, blind-start clinical study will assess the efficacy and safety of rhGUS in 12 patients between 5 and 35 years of age. Patients will be randomized to one of four groups. One cohort begins rhGUS therapy immediately, while the other three start on placebo and cross over to rhGUS at different predefined time points in a blinded manner. This novel trial design generates treatment data from all 12 patients, improving the statistical power of the study relative to a traditional parallel-group design. Based on data from the Phase 1/2 study, patients will be dosed with 4 mg/kg of rhGUS every other week for up to a total of 48 weeks, and all groups will receive a minimum of 24 weeks of treatment with rhGUS.

The primary objective of the study is to determine the efficacy of rhGUS as determined by the reduction in urinary GAG excretion after 24 weeks of treatment. The Phase 3 study will also evaluate the safety and tolerability of rhGUS, pulmonary function, walking, stair climb, shoulder flexion, fine and gross motor function, hepatosplenomegaly, cardiac size and function, visual acuity, patient and caregiver assessment of most significant clinical problems, global impressions of change, a multi-domain responder index, and other endpoints.

Agreement with the U.S. Food and Drug Administration (FDA) was reached that their evaluation of the pivotal Phase 3 study will be based on the totality of the data on a patient-by-patient basis. FDA advised against the declaration of a primary clinical endpoint in order to allow for more flexibility in the overall efficacy evaluation, appreciating the heterogeneous and rare nature of this disease.

The European Medicines Agency (EMA) has already agreed that approval under exceptional circumstances could be possible based upon a single positive Phase 3 study using urinary GAG excretion as the primary endpoint with a trend toward improvement in the most important clinical endpoints (six-minute walk test, forced expiratory volume, spleen/liver volume).

Data from the Phase 3 study is expected in the first half of 2016. Longer-term 36-week data from the Phase 1/2 study is expected to be presented at the Lysosomal Disease Network's 11th Annual World Symposium in February 2015. Two additional patients continue to be treated under emergency Investigational New Drug (eIND) applications.

About MPS 7

Mucopolysaccharidosis type 7 (MPS 7, Sly syndrome), originally described in 1973 by William Sly, M.D., is a rare genetic, metabolic disorder and is one of 11 different MPS disorders. MPS 7 is caused by the deficiency of beta-glucuronidase, an enzyme required for the breakdown of the glycosaminoglycans (GAGs) dermatan sulfate and heparan sulfate. These complex GAG carbohydrates are a critical component of many tissues. The inability to properly break down GAGs leads to a progressive accumulation in many tissues and results in a multi-system disease.

While its clinical manifestations are similar to MPS 1 and MPS 2, MPS 7 is one of the rarest among the MPS disorders. MPS 7 has a wide spectrum of clinical manifestations and can present as early as at birth. There are no approved therapies for MPS 7 today. The use of enzyme replacement therapy as a potential treatment is based on 20 years of research work in murine models of the disease. Enzyme replacement as a strategy is well established in the MPS field as there are currently four

approved enzyme replacement therapies for other MPS disorders: MPS 1 (Aldurazyme®, laronidase), MPS 2 (Elaprase®, idursulfase), MPS 4A (Vimizim™, elosulfase alfa), and MPS 6 (Naglazyme®, galsulfase).

About Ultragenyx

Ultragenyx is a clinical-stage biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with a focus on serious, debilitating metabolic 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 regarding expectations regarding the timing of release of data, 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, uncertainties inherent in the clinical drug development process, including the regulatory approval process, the timing of our regulatory filings, and other matters that could affect 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 the Company in general, see Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on November 10, 2014, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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