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

Data Expected to be Released in Mid-2016

NOVATO, Calif., June 8, 2015 (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 it has completed patient enrollment 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). The study was initiated in December 2014.

"Enrolling the last patient in our Phase 3 study of rhGUS in MPS 7 takes us one step closer to potentially providing the first specific treatment for patients affected by this severe disease," commented Sunil Agarwal, M.D., Chief Medical Officer of Ultragenyx. "With FDA and EMA alignment on the pivotal study design, we look forward to completing the study and announcing results in 2016."

The Phase 3 randomized, placebo-controlled, blind-start clinical study being conducted at five sites in the U.S. is designed to assess the efficacy and safety of rhGUS in 12 patients between 5 and 35 years of age. Patients are 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 and improves the statistical power relative to a traditional parallel-group design. Patients are 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. Secondary efficacy objectives include a multi-domain responder index and an individualized clinical response measure, as well as other clinical outcomes including pulmonary function, walking, shoulder flexion, fine and gross motor function, visual acuity, and fatigue. The safety and tolerability of rhGUS will also be assessed.

Agreement has been reached with both the US Food and Drug Administration (FDA) and European Medicines Agency (EMA) on the Phase 3 study design. The FDA stated 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 difficulty of evaluating a single clinical endpoint given the heterogeneity and rarity of the disease. The EMA has agreed that approval under exceptional circumstances could be possible based on the Phase 3 study with urinary GAG levels as a surrogate primary endpoint, provided the data are strongly supportive of a favorable benefit/risk ratio and that some evidence or trend in improvement in clinical endpoints is observed.

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 birth with hydrops fetalis. 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 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 Ultragenyx's 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, the 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 candidate. 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 May 12, 2015, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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