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# Ultragenyx Announces Initiation of Phase 2 Study of Recombinant Human Beta-Glucuronidase in Patients Under Five Years Old With Mucopolysaccharidosis 7

NOVATO, Calif., Aug. 5, 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 the dosing of the first patient in a Phase 2 study of investigational recombinant human beta-glucuronidase (rhGUS, UX003) in patients under five years old with mucopolysaccharidosis 7 (MPS 7, Sly syndrome), potentially including patients with non-immune hydrops fetalis, a severe infantile presentation of the disease.

"This new Phase 2 study will enable us to assess the benefit of early intervention with rhGUS in very young patients as well as the potential to prevent some of the devastating consequences of this disease," said Sunil Agarwal, M.D., Chief Medical Officer of Ultragenyx. "The resulting data from patients under five years old, which can include infants with hydrops fetalis, will complement the data from the ongoing Phase 3 study in older patients with MPS 7."

The Phase 2 open-label, multi-center clinical study will assess the safety, tolerability, and efficacy of rhGUS in up to seven pediatric patients under five years old. Patients will receive 4 mg/kg of rhGUS treatment every other week for 48 weeks, followed by a long-term continuation period. The primary efficacy endpoint is the reduction in urinary GAG excretion. Additional efficacy measures include growth velocity, hepatosplenomegaly, functional development, and cardiac and pulmonary function. Interim data from the study are expected by the end of 2016.

This study is also intended to enroll patients with non-immune hydrops fetalis, a phenotype of MPS 7 in which patients are born with severe edema and often die within a few months to one year. Ultragenyx is currently treating one MPS 7 patient born with hydrops fetalis under compassionate use.

**FDA Fast Track Designation**

Ultragenyx also announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track Designation to the rhGUS program in MPS 7. Fast Track Designation is intended to facilitate the development and expedite the review of drugs for serious and life-threatening conditions that have the potential to address an unmet medical need. The designation allows for more frequent interaction with the FDA review team. It also enables eligibility for priority review and the potential for a rolling review of the Biologics License Application, when and if filed.

**About MPS 7**

Mucopolysaccharidosis 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 in a severe form called non-immune 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).

Ultragenyx is conducting a Phase 3 study of rhGUS in older MPS 7 patients. The Phase 3 study completed enrollment in June 2015 and data are expected in mid-2016.

**About Ultragenyx**

Ultragenyx is a clinical-stage biotechnology 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 expectations regarding the timing of release of data and intent to enroll patients with non-immune hydrops fetalis in the Phase 2 study, 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 May 12, 2015, and its subsequent periodic reports filed with the Securities and Exchange Commission.*

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