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Ultragenyx Announces Initiation of Phase 1/2 Study of Recombinant Human Beta-Glucuronidase (rhGUS) in Mucopolysaccharidosis Type 7 (MPS 7)

NOVATO, CA – December 4, 2013 – Ultragenyx Pharmaceutical Inc., a biotechnology company, today announced the dosing of the first patient in a Phase 1/2 study of recombinant human beta-glucuronidase (rhGUS, UX003) for MPS 7, in Manchester, UK. MPS 7 is an ultra-rare autosomal recessive lysosomal storage disorder characterized by a deficiency of the enzyme beta-glucuronidase that results in a severe multi-system disease.

William Sly, MD, Chairman Emeritus of the Department of Biochemistry at Saint Louis University commented, "Patients, families, and researchers have been waiting many years for this advancement in the treatment of MPS 7. The initiation of clinical testing of rhGUS is the culmination of decades of work."

The Phase 1/2 open-label clinical trial will assess the safety and efficacy of rhGUS in a 12-week primary analysis phase, followed by dose-exploration and long-term extension. Five patients between 5 and 30 years of age inclusive will be enrolled and administered rhGUS every other week via intravenous infusion. Interim data from the Phase 1/2 study is expected in 2014.

"MPS 7 is a devastating condition that has been neglected by the drug development community despite 20 years of extensive nonclinical research led by Dr. Sly and his colleagues," said Emil D. Kakkis, MD, PhD, Chief Executive Officer of Ultragenyx. "The initiation of this Phase 1/2 study is an important milestone for Ultragenyx and for patients with MPS 7."

About MPS 7

Mucopolysaccharidosis type 7 (MPS 7), originally described in 1973 by William Sly, MD, 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 three approved enzyme replacement therapies for other MPS disorders: MPS 1 (Aldurazyme®, laronidase), MPS 2 (Elaprase®, idursulfase), and MPS 6 (Naglazyme®, galsulfase).

About Ultragenyx

Ultragenyx is a privately held, development-stage biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with an initial 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.