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FOR IMMEDIATE RELEASE:

Ultragenyx Advances Clinical Development of UX003 for the Treatment of Mucopolysaccharidosis Type 7 (MPS 7)

NOVATO, CA – May 15, 2013 - Ultragenyx Pharmaceutical Inc., a biotechnology company focused on developing treatments for rare and ultra-rare genetic disorders, today announced a Phase 1/2 study of UX003 for mucopolysaccharidosis type 7 (MPS 7, or Sly Syndrome). UX003 is a recombinant human β-glucuronidase intended as an enzyme replacement therapy (ERT) for the treatment of MPS 7, an extremely rare autosomal recessive lysosomal storage disorder characterized by a deficiency of the lysosomal enzyme β-glucuronidase and a severe multi-system disease. MPS 7 has no approved therapies.

The open-label Phase 1/2 clinical study is planned to start this year at a single center in the UK. The study will evaluate the safety, efficacy, and dose of UX003 in approximately five MPS 7 patients. The company anticipates that if the data from the Phase 1/2 trial is positive, it would proceed with a pivotal trial with a novel design.

"Extensive research has been done on MPS 7 for 40 years, and unfortunately it is one MPS left behind," said Emil D. Kakkis, MD, PhD, Chief Executive Officer of Ultragenyx. "We are pleased to announce the first clinical study in MPS 7 on International MPS Awareness Day. Our collaboration with Dr. William Sly and his colleagues at St. Louis University continues to advance development of this urgently needed therapy, using innovative and creative approaches."

"I am pleased with the progress that Ultragenyx has made with the UX003 program," commented Dr. Sly. "After many years of research, we are getting one step closer to finding a potentially life-changing treatment for patients with MPS 7."

About MPS 7

Mucopolysaccharidosis type 7 (MPS 7), originally described in 1973 by William Sly, MD, (also known as Sly syndrome), is a rare genetic, metabolic disorder and is one of 40 different lysosomal storage disorders. MPS 7 is caused by the deficiency of β-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 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 and is likely underdiagnosed as are most rare diseases. Ultragenyx currently estimates that up to 200 patients are afflicted with MPS 7 worldwide. MPS 7 has a wide spectrum of clinical manifestations and can present as early as at birth or in older patients with less rapidly progressive disease. There are no approved therapies for MPS 7 today.

About Ultragenyx

Ultragenyx is a privately held, development-stage biotechnology company committed to bringing to market life-transforming therapeutics for patients with rare and ultra-rare metabolic genetic diseases. The company focuses on diseases for which the unmet medical need is high, the biology for treatment is clear, and for which there are no effective treatments.

The company is led by a management team experienced in the development and commercialization of rare disease therapeutics. Ultragenyx' 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.