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FOR IMMEDIATE RELEASE:

Ultragenyx Granted Orphan Drug Designation for UX003 for the Treatment of Mucopolysaccharidosis Type 7 (MPS 7)

NOVATO, CA – February 28, 2012 - Ultragenyx Pharmaceutical Inc., a biotechnology company focused on developing treatments for rare and ultra-rare genetic disorders, today announced that the FDA Office of Orphan Products Development has granted orphan drug designation for UX003 for the treatment of MPS 7. MPS 7 is an extremely rare autosomal recessive lysosomal storage disorder characterized by a deficiency of the lysosomal enzyme β-glucuronidase, required for the degradation of the glycosaminoglycans dermatan sulfate (DS) and heparan sulfate (HS). UX003 is a recombinant human β-glucuronidase intended as an enzyme replacement therapy for the treatment of MPS 7. MPS 7 was originally described in 1973 by William Sly, MD, St. Louis University School of Medicine, and is also known as Sly Syndrome. Ultragenyx in-licensed the MPS 7 program from St. Louis University.

"Obtaining orphan drug designation for UX003 is a significant achievement for Ultragenyx that adds value to our development pipeline and attests to the importance of this product candidate in filling an unmet medical need," said Emil D. Kakkis, MD, PhD, Chief Executive Officer of Ultragenyx. "We look forward to continuing to collaborate with Dr. Sly and colleagues at St. Louis University to advance this urgently needed therapy into clinical testing."

The Orphan Drug Designation program provides orphan status to drugs and biologics intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 people in the U.S. Among the benefits of orphan designation in the US are seven years of market exclusivity following FDA approval, waiver or partial payment of application fees, and tax credits for clinical testing expenses conducted after orphan designation is received.

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About MPS 7

Mucopolysaccharidosis type 7 (MPS 7), 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 breakdown GAGs leads to a progressive accumulation in many tissues and multi-system disease. There are a wide variety of clinical symptoms including enlarged organs, stiff joints, respiratory disease and cardiac complications.

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. 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, developmental stage biotechnology company committed to bringing life-enhancing therapeutics for patients with rare and ultra-rare genetic diseases, also known as orphan and ultra-orphan diseases, to market. The company focuses on rare metabolic diseases that affect small numbers of patients, but for which the unmet medical need is high and there are no effective treatments. Ultragenyx intends to build a sustainable pipeline of safe and effective therapies to address these underserved diseases. Ultragenyx' lead program, UX001, is being evaluated as a potential treatment for GNE myopathy, also known as hereditary inclusion body myopathy (HIBM). UX001 program has been granted orphan drug designation in the U.S.

The company is led by an experienced management team in rare disease therapeutics. Ultragenyx is striving toward an improved model for successful rare disease drug development which has the potential to increase efficiency while maintaining appropriate safety and efficacy standards. The company believes that it can deliver significant value to patients by building a diverse and high quality pipeline of rare disease therapeutics and efficiently transforming good science into great medicine.

For more information on Ultragenyx, please visit the company's website at www.ultragenyx.com.