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FOR IMMEDIATE RELEASE:

Ultragenyx In-Licenses Therapeutic Program for Rare Genetic Disease Mucopolysaccharidosis Type 7 from St. Louis University

MPS 7 Patients May at Last have an Opportunity for Enzyme Replacement Therapy

NOVATO, CA – January 5, 2012 - Ultragenyx Pharmaceutical Inc., a biotechnology company focused on developing treatments for rare and ultra-rare genetic disorders, today announced it has in-licensed an enzyme replacement therapy program from St. Louis University to treat mucopolysaccharidosis type 7 (MPS 7). The in-licensed program is a treatment for an ultra-rare genetic, metabolic disorder that results from the deficiency of the beta-glucuronidase (GUS) enzyme. Also known as Sly syndrome, the disorder was first identified in 1973 by William S. Sly, MD, a world-renowned researcher in inherited diseases, who is currently Professor and Chairman Emeritus, Department of Biochemistry and Molecular Biology, at St. Louis University School of Medicine. Dr. Sly will collaborate with Ultragenyx on the MPS 7 development program.

"We are pleased to have the opportunity to develop this treatment for MPS 7 which has been in the research stage for a long time and has yet to be made available to patients. We look forward to working in collaboration with Dr. Sly and the MPS community on this program." said Emil D. Kakkis, MD, PhD, Chief Executive Officer of Ultragenyx.

Dr. Sly noted, "After so many years of research by my laboratory and my research colleagues, I am pleased to finally have the chance to see if MPS 7 patients can be successfully treated with enzyme replacement therapy. I have confidence in Ultragenyx's ability to advance the MPS 7 program through the development process and fulfill our shared goal of bringing this potentially life-changing therapy to patients. We look forward to working closely with the Ultragenyx team on this program."

About MPS 7

Mucopolysaccharidosis type 7 (MPS 7), also known as Sly syndrome, is a rare genetic, metabolic disorder that is one of the 40 different lysosomal storage disorders. MPS 7 is caused by the deficiency of beta-glucuronidase, an enzyme required for the breakdown of the glycosaminoglycans (GAG) 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 similar to other MPS disorders.

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 manifestations and can present clinically 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 clinically underserved diseases. Ultragenyx' lead program, UX001, is being evaluated as a potential treatment for GNE myopathy, also known as hereditary inclusion body myopathy (HIBM).

The company is led by Emil Kakkis, MD, PhD, a recognized industry leader in rare disease research and development, along with an experienced management team in rare disease therapeutics. Ultragenyx is striving for an improved model for successful rare disease drug development that has the potential to increase efficiency and effectiveness by changing the way development is organized and conducted. 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.