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**FOR IMMEDIATE RELEASE:**

# Ultragenyx Initiates Novel Disease Monitoring Program for Hereditary Inclusion Body Myopathy

*First patient enrolled in integrated program designed to improve knowledge of rare disease*

**NOVATO, CA – April 8, 2013** - Ultragenyx Pharmaceutical Inc. today announced the launch of a unique Disease Monitoring Program (DMP) for Hereditary Inclusion Body Myopathy (HIBM), also known as GNE Myopathy. The goal of the HIBM-DMP is to improve the body of knowledge about this rare disease and its typical course. This novel program is being conducted in partnership with the University of Newcastle's TREAT-NMD organization, a global neuromuscular physician network in Newcastle, England.

The main objectives of the HIBM-DMP are to expand knowledge of the clinical presentation, progression and variation of HIBM patients; identify and qualify biomarkers and other efficacy measures; inform the design and interpretation of clinical studies of investigational products for HIBM and eventually to optimize patient management.

The HIBM-DMP is designed to assist the HIBM communities in several ways, including:
- integration of an online registry capturing patient-reported information, a fully monitored physician-driven natural history study, and potentially any post-approval patient follow-up into a single cohesive program.
- knowledge-sharing gained from the program about HIBM with investigators, physicians and patients in a manner compliant with patient data protection regulations.

The HIBM-DMP will involve up to 10 centers in North America, the European Union and Israel. The first patient in the natural history component of the HIBM-DMP was enrolled by Dr. Mark Tarnopolsky at McMaster University in Hamilton, Ontario, Canada. Data collection in the registry component of the study is expected to begin in May.

“We recognize there is a need to better understand the disease-specific features of HIBM, and as a company, we recognize the need to provide access to data that is of critical value to the scientific, medical and patient communities,” noted Emil D. Kakkis, MD, PhD, Chief Executive Officer of Ultragenyx. “We are pleased to have the support

and partnership of the TREAT-NMD organization and the HIBM community in these efforts."

Ultragenyx is currently studying UX001, extended-release sialic acid for the treatment of HIBM, in a Phase 2 clinical trial. Data from the HIBM-DMP may be supportive of the UX001 program. Ultragenyx plans to utilize this unique DMP model across its other pipeline programs as well.

**About HIBM**

HIBM has recently been renamed GNE myopathy, but historically has also been called Quadriceps Sparing Myopathy (QSM), Inclusion Body Myopathy type 2, Distal Myopathy with Rimmed Vacuoles (DMRV) and Nonaka myopathy. HIBM is a severe, adult-onset, progressive, genetic neuromuscular disease caused by a deficiency of an enzyme in the first step of sialic acid biosynthesis needed for the modification of proteins and fats. Patients with HIBM typically begin to have weakness and abnormal walking at 18 to 30 years of age. Over the ensuing 10 to 20 years, many patients progressively lose significant functional ability and become wheelchair-bound. There are no approved treatments for this disease.

**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'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.