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FOR IMMEDIATE RELEASE:

Ultragenyx Announces Completion of Phase 2 Study Enrollment for UX001 in Hereditary Inclusion Body Myopathy

NOVATO, CA – October 30, 2012 - Ultragenyx Pharmaceutical Inc., a biotechnology company focused on developing treatments for rare and ultra-rare genetic disorders, today announced it has completed patient enrollment in a randomized, double-blind, placebo-controlled Phase 2 study of UX001 in patients with hereditary inclusion body myopathy (HIBM). The study's primary objective is to evaluate safety, dose, and improvements in sialylation biochemistry of muscle from pre-treatment to post-treatment biopsies (pharmacodynamic endpoint). Muscle strength, clinical function and patient-reported outcomes will also be evaluated, though the study is not powered for these endpoints. A total of 45 patients between 18 and 65 years of age, with a confirmed genetic mutation for HIBM, have been randomized to placebo versus two dose levels of active drug at four clinical sites in the US and Israel.

"Promptly completing enrollment of the Phase 2 study is another important milestone in the development of UX001 for the treatment of HIBM," Emil D. Kakkis, MD, PhD, Chief Executive Officer of Ultragenyx commented. "We want to express our appreciation to the investigators, clinical sites and enrolled patients for their dedication and support. We look forward to completing the study and announcing top-line results in 2013."

About HIBM

HIBM is also known as GNE myopathy, 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

Transforming good science into great medicine for rare genetic diseases

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, 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 hereditary inclusion body myopathy (HIBM), also known as GNE myopathy. The UX001 program has been granted orphan drug designation in the US and the EU.

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 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.