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FOR IMMEDIATE RELEASE:

Ultragenyx Announces a Positive Signal in Interim Data from Phase 2 Study of UX001 in Hereditary Inclusion Body Myopathy

Study to continue to 48 weeks, followed by extension study testing higher dosage

Novato, CA—July 3, 2013—Ultragenyx Pharmaceutical Inc., a biotechnology company focused on developing treatments for rare and ultra-rare genetic disorders, announced interim 24-week data from a 48-week Phase 2 clinical study of UX001 in 47 patients with hereditary inclusion body myopathy (HIBM), a progressive muscle-wasting disease. The study compared treatment with a total daily dose of 6 grams or 3 grams of UX001 with placebo. UX001, an oral sialic acid extended-release (SA-ER) tablet, is designed to replace the deficient sialic acid substrate in patients with HIBM.

The data showed dose-dependent improvement in muscle strength relative to placebo in some muscle groups, particularly in the upper extremities at the 6-gram dose. These changes were statistically significant or trended towards significance, and were more pronounced in those patients that had greater walking ability at baseline, a predefined subset. Other clinical endpoints did not reveal changes at this interim assessment. Creatine kinase levels showed a trend to improvement in the 6-gram dose group compared with placebo. UX001 appeared to be well tolerated with no serious adverse events observed to date in either dose group.

“These early data suggest a modest dose-dependent improvement in muscle strength in HIBM patients treated with UX001 compared to a decline in placebo-treated patients,” said Emil Kakkis, MD, PhD, Chief Executive Officer of Ultragenyx. “We need to evaluate whether the observed treatment effect is sustained or increased over a longer 48-week period, and if higher dosing might further enhance the efficacy signal observed.”

The primary objective of the Phase 2 study is to evaluate safety, dose and potential pharmacodynamic effect of restoring sialylation of muscle in patients with a confirmed genetic mutation for HIBM. The study is also evaluating clinical measures of muscle strength, mobility, function, self-reported disability, and changes in quality of life. The study is taking place at four sites in the United States and Israel. Patients were randomized to receive placebo, 3 grams, or 6 grams per day of UX001, in three divided

doses. At 24 weeks, placebo patients were randomized and crossed over into either of the two dose groups on a blinded basis. Patients will be evaluated again at 48 weeks, with final data anticipated around year-end. Following the 48-week analysis, the company plans to continue to treat these patients in an extension study with an increased dosage of UX001 based on the dose-dependence observed at week 24.

About Hereditary Inclusion Body Myopathy
Hereditary inclusion body myopathy (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 defect in the biosynthetic pathway for sialic acid (SA). Patients with HIBM typically begin to have weakness and abnormal walking at 18 to 30 years of age. The body's failure to produce enough sialic acid causes muscles to slowly waste away and can lead to very severe disability within 10 to 20 years of diagnosis, with patients often ending up wheelchair-bound within that time. There is currently no approved therapy.

About Ultragenyx
Ultragenyx is a privately held, clinical-stage biotechnology company committed to bringing to market life-transforming therapeutics for patients with rare and ultra-rare metabolic genetic diseases. The company, founded in 2010, is rapidly building a diverse portfolio of products addressing diseases for which the unmet medical need is high, the biology for treatment is clear, and for which there are no effective treatments. Ultragenyx has two products in Phase 2 clinical trials, UX001 for the treatment of hereditary inclusion body myopathy (HIBM), and UX007 for the treatment of fatty acid oxidation disorders (FAOD). A third compound, UX003 for MPS 7 (Sly Syndrome), will begin Phase 1/2 clinical trials later this year. 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.