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**FOR IMMEDIATE RELEASE:**

**Ultragenyx Announces Phase 1 Results of UX001 in Hereditary Inclusion Body Myopathy (HIBM), a Rare Neuromuscular Disease**

**NOVATO, CA – May 1, 2012** - Ultragenyx Pharmaceutical Inc., a biotechnology company focused on developing treatments for rare and ultra-rare genetic disorders, today announced results from a first-in-human, multi-center, Phase 1 study of UX001 in patients with hereditary inclusion body myopathy (HIBM) showing that UX001 was well-tolerated with an expected extended release profile on absorption after oral administration. UX001 is an extended release formulation of sialic acid intended as a substrate replacement therapy for HIBM, a severe, neuromuscular disease caused by sialic acid deficiency. Based on the Phase 1 results, Ultragenyx plans to initiate an international, multi-center, randomized, double-blind, placebo-controlled, parallel group Phase 2 study of UX001 in HIBM patients in the second quarter of this year.

The Phase 1 clinical trial was a multi-center, sequential dose-escalation study designed to characterize the safety, tolerability and pharmacokinetics of UX001 Sialic Acid-Extended Release (SA-ER) tablets in patients with HIBM disease. Twenty-six subjects received SA-ER tablets orally at one of five (5) dose levels in the single-dose phase and one of four (4) dose levels in the repeat-dose phase. Preliminary data showed that SA-ER was well-tolerated at the doses evaluated, with no serious adverse events reported. The adverse event profile was unremarkable and showed no pattern or dose dependent relationship. Pharmacokinetic analysis from the study showed that single doses of SA-ER are absorbed and provide significant drug levels over a 12-16 hour period. On repeated 3 times per day dosing, serum free sialic acid concentrations reached relatively steady levels over a 24 hour cycle.

“We are encouraged by the results of this early stage clinical trial which suggest SA-ER in single and repeated oral dosing is well-tolerated and has the potential to be a treatment for patients suffering from HIBM,” said Emil D. Kakkis, MD, PhD, Chief Executive Officer of Ultragenyx. “Based on these favorable Phase 1 results, we look forward to moving quickly to initiate a Phase 2 study of UX001.”

*Transforming good science into great medicine for rare genetic diseases*

[www.ultragenyx.com](http://www.ultragenyx.com)

Ultragenyx plans to present the Phase 1 data at the 17th Annual International World Muscle Society Congress in Perth, Australia later this year.

### **About HIBM**

HIBM is also known as GNE myopathy, distal myopathy with rimmed vacuoles (DMRV) and Nonaka disease. 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 current 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 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](http://www.ultragenyx.com).