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FOR IMMEDIATE RELEASE:

Ultragenyx Announces Results from Phase 2 Study of Sialic Acid Extended-Release Treatment in Hereditary Inclusion Body Myopathy

Upper extremity muscle strength composite shows statistically significant difference between the 6 gram and 3 gram dose groups

Novato, CA—December 20, 2013—Ultragenyx Pharmaceutical Inc., a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, today announced topline results from a 48-week Phase 2 clinical study of sialic acid extended-release (SA-ER, UX001) tablets in 46 patients with hereditary inclusion body myopathy (HIBM), a progressive muscle-wasting disease. SA-ER is designed to replace the deficient sialic acid substrate in patients with HIBM. Patients were initially randomized to either receive placebo, 3 grams or 6 grams of SA-ER per day. After 24 weeks, placebo patients crossed over to either 3 grams or 6 grams total daily dose, on a blinded basis, for an additional 24 weeks. The final analysis compared change at week 48 from baseline for the combined groups at 6 grams versus 3 grams of SA-ER.

Similar to the results observed at the 24-week interim analysis, at 48 weeks the comparison of the upper extremity composite of muscle strength for the combined group of patients on 6 grams showed a modest increase and a statistically significant difference relative to the decline in strength observed in the combined groups on 3 grams. In the 6-gram cohort treated for 48 weeks, the modest increase in upper extremity strength observed at 24 weeks was sustained relative to a further decline in the comparable 3-gram group. These changes were more pronounced in those patients that have less advanced disease as assessed by a greater walking ability at baseline, a predefined subset. The lower extremity composite did not show a statistically significant difference between the dose groups, but neither group showed a significant decline during the treatment period. The upper extremity and lower extremity composites are predefined endpoints that aggregate change in muscle strength across multiple individually measured muscle groups.

The GNE Myopathy Functional Activity Scale, a patient-reported outcome measure developed to assess the clinical meaningfulness of changes in function, showed a trend toward a positive effect in the combined 6-gram group as compared to the combined 3-gram group. The sit-to-stand test also showed a trend toward a positive effect. Other clinical endpoints related to walking, including the 6 minute walk test did not reveal significant changes in function, upward or downward. SA-ER appeared to be well

tolerated with no serious adverse events observed to date in either dose group. Most adverse events were mild to moderate and most commonly gastrointestinal.

“These 48 week data suggest that 6 grams per day of SA-ER is mitigating the normally expected decline in upper extremity muscle strength,” said Emil Kakkis, M.D., Ph.D., Chief Executive Officer of Ultragenyx. “The maintenance of this effect from the 24-week data is encouraging. Given the difference between the dose groups and good safety profile, we plan to test an even higher dose of sialic acid in these patients who have no other approved treatment options.”

The data from the 48-week analysis are expected to be presented in full at a scientific conference in 2014. All 46 patients from the original study have elected to continue into the extension study. The company plans to treat patients with an increased dosage of sialic acid based on the dose-dependence observed at week 48. The first patient has already been treated with a higher dose of sialic acid, and data from the increased dose is expected in late 2014.

About Hereditary Inclusion Body Myopathy
Hereditary inclusion body myopathy (HIBM) is also known as GNE myopathy. HIBM is a severe, progressive, genetic neuromuscular disease caused by a defect in the biosynthetic pathway for sialic acid, with onset in the late teens or twenties. The body’s failure to produce enough sialic acid causes muscles to slowly waste away and can lead to very severe disability, with patients typically becoming wheelchair bound within ten to 20 years from onset. There are approximately 1,200 to 2,000 HIBM patients in the developed world, and there is currently no approved therapy.

About Ultragenyx
Ultragenyx is a privately held, clinical-stage biotechnology company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with an initial focus on serious, debilitating metabolic genetic diseases. Founded in 2010, the company has rapidly built a diverse portfolio of product candidates with the potential to address diseases for which the unmet medical need is high, the biology for treatment is clear, and for which there are no approved therapies.

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.