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Ultragenyx Announces Positive Data From Phase 2 Study of Sialic Acid Extended-Release at Emerging Sciences Session of American Academy of Neurology Annual Meeting

Upper Extremity Muscle Strength Preserved Over 48 Weeks in HIBM Patients on 6 Grams

NOVATO, Calif., April 30, 2014 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (Nasdaq:RARE), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, today announced the presentation of detailed results from a 48-week Phase 2 clinical study of sialic acid extended-release (SA-ER, UX001) tablets in 47 patients with hereditary inclusion body myopathy (HIBM; also known by its new name as GNE myopathy), a rare, progressive muscle-wasting disease. SA-ER is designed to replace the deficient sialic acid substrate in patients with HIBM.

Topline results were previously reported in December 2013, and the following abstract was presented at the Emerging Sciences session of the 66th American Academy of Neurology (AAN) Annual Meeting in Philadelphia.

Z. Argov, Y. Caraco, H. Lau, A. Pestronk, P. Shieh, A. Skrinar, J. Mayhew, J. Martinisi, E. Kakkis

Oral sialic acid extended release (SA-ER) stabilizes upper extremity muscle strength in human GNE myopathy: A Phase 2 study

Patients in the study were initially randomized to 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. Assessments included pharmacokinetics, composites of upper extremity and lower extremity muscle strength as measured by dynamometry, other clinical endpoints, patient reported outcomes, and safety.

At 24 weeks, assessments of upper extremity composite of muscle strength showed a statistically significant difference in the 6 gram group compared to placebo (+2.33 kg; 5.5% relative difference from baseline; p=0.040). At 48 weeks, a statistically significant difference between the combined 6 gram group and the combined 3 gram group was observed (+3.44 kg; 8.5% relative difference from baseline; p=0.0033). Patients with less advanced disease (able to walk more than 200 meters at baseline), a predefined subset that comprised approximately 70% of total enrollment, showed a more pronounced difference (+4.69 kg; 9.7% relative difference from baseline; p=0.00055).

"We are encouraged that the preservation of upper extremity muscle strength on 6 grams was sustained compared to a decline on placebo or 3 grams," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer of Ultragenyx. "We plan to discuss the Phase 2 and higher dose results with the regulatory authorities to establish the next steps for the SA-ER program."

The lower extremity composite showed a similar pattern of response but did not show a statistically significant difference between the dose groups. None of the groups showed a significant decline during the treatment period. Clinical endpoints related to walking, including the six-minute walk test (6MWT), did not reveal significant differences; there was no significant increase or decline.

The GNE Myopathy Functional Activity Scale (GNEM-FAS), a novel specific patient-reported outcome measure developed to assess the clinical meaningfulness of changes in function, did not show differences at 24 weeks but at 48 weeks showed a positive trend in total (p=0.086), mobility (p=0.087), and upper extremity scores (p=0.096) in the combined 6 gram versus the combined 3 gram groups. An alternative post-hoc statistical analysis using a statistical model incorporating all of the repeated data recorded over time did show statistical significance in these GNEM-FAS outcomes.

SA-ER appeared to be well tolerated with no serious adverse events observed to date in either dose group, and no dose-dependent treatment-emergent adverse events were identified. Most adverse events were mild to moderate and most commonly gastrointestinal.

The company continues to treat patients in an extension study evaluating an increased 12 gram daily dosage of sialic acid based on the dose dependence observed at weeks 24 and 48 of the Phase 2 study. 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 rare, 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 and losing most major muscle function 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 development-stage biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with an initial focus on serious, debilitating 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.

Forward-Looking Statements

Except for the historical information contained herein, the matters set forth in this press release, including statements regarding timing of release of additional data, parameters of the extension study, Ultragenyx's plans, expectations, goals, objectives, milestones, clinical studies, and product development are forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development programs, future results, performance, or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the uncertainties inherent in the clinical drug development process, including the regulatory approval process, the timing of our regulatory filings, and other matters that could affect the availability or commercial potential of our drug candidate. Ultragenyx undertakes no obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of the Company in general, see Ultragenyx's Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 24, 2014, and its future periodic reports to be filed with the Securities and Exchange Commission.

CONTACT: Ultragenyx Pharmaceutical Inc.

844-758-7273

For Media, Bee Nguyen

For Investors, Robert Anstey