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Ultragenyx Announces First Patient Enrolled in Global Phase 3 Study of Aceneuramic Acid (Sialic Acid) Extended Release in GNE Myopathy

NOVATO, Calif., May 28, 2015 (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 initiation of the Phase 3 study of aceneuramic acid extended release (Ace-ER), previously known as sialic acid-extended release (SA-ER; UX001), tablets for the treatment of GNE Myopathy. Ace-ER is designed to replace the deficient sialic acid substrate in patients with GNE Myopathy, also known as Hereditary Inclusion Body Myopathy (HIBM), a rare, progressive muscle-wasting disease.

"This is a significant step forward for our global program to assess treatment with Ace-ER in patients with GNE Myopathy, currently an unmet medical need," said Sunil Agarwal, M.D., Chief Medical Officer of Ultragenyx. "Our pivotal Phase 3 study is intended to confirm the positive results observed in Phase 2. In parallel, we plan to file for conditional approval in Europe with the possibility of accelerating access for patients with this debilitating disease."

The Phase 3 global, randomized, double-blind, placebo-controlled clinical study in approximately 80 patients will assess the efficacy and safety of six grams per day of Ace-ER over 48 weeks. The primary endpoint of the study is a composite of upper extremity muscle strength as measured by hand-held dynamometry (HHD). Key secondary endpoints include the GNE Myopathy-functional activity scale (GNEM-FAS), a disease-specific patient-reported outcome (PRO) that measures mobility and upper-extremity function, and other measures of lower-extremity muscle strength. A Phase 2 randomized, double-blind, placebo-controlled study evaluating the same endpoints was completed in December 2013.

The Phase 3 study design was accepted by the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA). Data from the Phase 3 study are expected in the second half of 2016.

In parallel with the conduct of the Phase 3 study, Ultragenyx intends to file a Marketing Authorization Application (MAA) with the EMA in the second half of 2015. The company seeks to obtain conditional approval from the EMA for Ace-ER for the stabilization or slowing of decline in upper extremity muscle strength in patients with GNE Myopathy based on the Phase 2 data and Scientific Advice received from the EMA. Once filed, a decision from the EMA is anticipated by the end of 2016.

About GNE Myopathy

GNE Myopathy is also known as Hereditary Inclusion Body Myopathy (HIBM). GNE Myopathy is a rare, severe, progressive, genetic neuromuscular disease caused by a defect in the biosynthetic pathway for sialic acid, with onset usually occurring in the 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 2,000 GNE Myopathy patients in the developed world, and currently there is no approved therapy.

About Aceneuramic Acid Extended Release (Ace-ER) Treatment in GNE Myopathy

Aceneuramic acid (pronounced a-se-ner-AM-ic), previously known as sialic acid, is the United States Adopted Name (USAN) and International Nonproprietary Name (INN) for sialic acid, which is being developed for the treatment of GNE Myopathy. A Phase 2 randomized, double-blind, placebo-controlled study with Ace-ER has been completed. The data showed a statistically significant difference in the upper extremity composite of muscle strength at 48 weeks with a higher-dose group compared to a lower-dose group. Ace-ER appeared to be generally safe and well tolerated with no serious adverse events observed to date. Over an approximate two-year treatment period in the Phase 2 study and long-term extension, Ace-ER appeared to stabilize or slow the progression of upper extremity disease when compared to the 24-week placebo group extrapolated out to two years.

About Ultragenyx

Ultragenyx is a clinical-stage biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with a 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 the intent to file an MAA and the anticipated timing of such filing and the expected timing of receipt of a decision on the application from the EMA, as well as the timing of release of data from the pivotal study of aceneuramic acid, 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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on May 11, 2015, and its subsequent periodic reports filed with the Securities and Exchange Commission.

CONTACT: Ultragenyx Pharmaceutical Inc.

Investors & Media

Robert Anstey

844-758-7273