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Ultragenyx Announces Aceneuramic Acid Prolonged Release Marketing Authorization Application Filed and Accepted for Review by European Medicines Agency

NOVATO, Calif., Oct. 2, 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 it has successfully filed a Marketing Authorization Application (MAA) with the European Medicines Agency (EMA) for aceneuramic acid prolonged release (Ace-ER; UX001) tablets intended for patients with 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.

"We have reached an important milestone for both Ultragenyx and patients living with GNE Myopathy," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer of Ultragenyx. "It is the company's first filing for marketing approval, just five years after its founding, and the first marketing application of a potential treatment for patients affected by this progressive debilitating muscle disease."

Ultragenyx is seeking to obtain conditional approval from the EMA for Ace-ER for the treatment of adult patients with GNE Myopathy. The MAA submission is based on positive data from a Phase 2 randomized, double-blind, placebo-controlled clinical study. If a positive opinion is received from the Committee for Medicinal Products for Human Use (CHMP), a decision from the European Commission would be expected in the second half of 2016.

A Phase 3 global, randomized, double-blind, placebo-controlled clinical study of Ace-ER in approximately 80 patients was initiated in May 2015 and continues to enroll patients. The primary endpoint of the study is upper extremity muscle strength. The study was designed based on feedback from the U.S. Food and Drug Administration (FDA) and the EMA, and data are expected in early 2017. This study is intended to satisfy the EMA's requirement for confirmatory data and serve as the basis for a filing for FDA approval.

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 within 10 to 20 years and ultimately having to rely on others for daily care due to the loss of upper extremity and other muscle function. There are approximately 2,000 GNE Myopathy patients in the developed world, and currently there is no approved therapy.

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

Aceneuramic acid prolonged release, also known as aceneuramic acid extended release (Ace-ER) outside of Europe, is being developed as a substrate replacement therapy for the treatment of adult patients with GNE Myopathy who have a genetic defect in sialic acid production. A Phase 1 single and multiple dose study and a Phase 2 randomized, double-blind, placebo-controlled study with Ace-ER have been completed. The Phase 2 study data were presented previously at the American Academy of Neurology Emerging Science section in April 2014. Data on the use of a higher dose were presented at the World Muscle Society Meeting in October 2014. These data plus data from a non-interventional natural history study are contained in the MAA filing to support a 6 gram per day dose.

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 expected timing of receipt of a decision on the MAA 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 August 14, 2015, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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