



November 11, 2016

## **Ultragenyx Announces Withdrawal of Marketing Authorization Application for Aceneuramic Acid Prolonged Release (Ace-ER) in the European Union**

NOVATO, Calif., Nov. 11, 2016 (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company focused on the development of novel products for rare and ultra-rare diseases, today announced that it has withdrawn its conditional Marketing Authorization Application (MAA) from the European Medicines Agency (EMA) for Aceneuramic Acid Prolonged Release (Ace-ER) for the treatment of adult patients with GNE Myopathy.

The conditional MAA was based on data from the company's Phase 2 study. During the Committee for Medicinal Products for Human Use (CHMP) meeting, the CHMP indicated that the Phase 2 study was encouraging but did not provide a sufficient amount of evidence to support an approval at this time. Ultragenyx intends to obtain additional efficacy data from its fully-enrolled global phase 3 study to confirm the effects of Ace-ER, and plans to submit an MAA for full approval after data from this study are available in the second half of 2017.

"Our Phase 3 study is on track and is designed to confirm the encouraging results seen in the Phase 2 study through a larger trial with a primary endpoint and a placebo period that lasts for the full 48-week duration of the study," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "We are committed to bringing this potential therapy to patients affected by this progressive and debilitating muscle disease."

### **About the Phase 3 Ace-ER study**

The Phase 3 global, randomized, double-blind, placebo-controlled clinical study is designed to assess the efficacy and safety of six grams per day of Ace-ER over 48 weeks in 89 patients. The primary endpoint of the study is a comparison between active and placebo treated patients for the change from baseline to 48 weeks in the 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 includes measures of mobility and upper-extremity function, a composite of lower extremity muscle strength, and other measures of lower-extremity muscle strength and function. The Phase 3 study design and primary endpoint were based on feedback from the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA).

### **About GNE Myopathy**

GNE Myopathy, also known as Hereditary Inclusion Body Myopathy (HIBM), 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 or thirties. The body's failure to produce enough sialic acid causes muscles to slowly waste away, leading to severe disability. Patients are typically wheelchair-bound and ultimately having to rely on others for daily care due to the loss of upper extremity 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 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. A Phase 3 study has completed enrollment and data are expected in the second half of 2017.

### **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](http://www.ultragenyx.com).

### **Forward-Looking Statements**

*Except for the historical information contained herein, the matters set forth in this press release, including statements regarding Ultragenyx's expectations regarding the timing of release of additional data for its product candidates, plans regarding ongoing studies and timing regarding these studies, expectations regarding the adequacy of clinical data to support acceptance of filings or approval of product candidates, and its expectations regarding timing of receiving potential approval of its product candidates, 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, such as the regulatory approval process, whether the Phase 3 results for Ace-ER will in fact confirm or mirror the results from the prior Phase 2 study, the timing of our regulatory filings and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations and the availability or commercial potential of our drug candidates. 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 November 8, 2016, and its subsequent periodic reports filed with the Securities and Exchange Commission.*

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