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Sialic Acid Extended-Release Phase 2 Study Data to be Presented at American Academy of Neurology Annual Meeting

Abstract Was One of Ten Chosen for Late-Breaking Emerging Sciences Session

NOVATO, Calif., April 28, 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 upcoming presentation of data from a Phase 2 clinical study of sialic acid extended-release (SA-ER, UX001) tablets in hereditary inclusion body myopathy (HIBM; also known by its new name as GNE myopathy). HIBM is a rare, progressive muscle-wasting disease, and SA-ER is designed to replace the deficient sialic acid substrate in patients with HIBM.

Detailed results from the 48-week Phase 2 study in 47 HIBM patients will be presented during the Emerging Sciences session of the 66th American Academy of Neurology (AAN) Annual Meeting in Philadelphia. The presentation will take place on April 30th at approximately 6:30pm ET in Grand Ballroom A. Topline results from the study were previously reported in December 2013. This presentation will be the first detailed release of the results at a scientific conference.

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, 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 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 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.

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