



November 15, 2017

## **Ultragenyx Announces FDA Approval of MEPSEVII™ (vestronidase alfa), the First Therapy for Progressive and Debilitating Rare Genetic Disease Mucopolysaccharidosis VII**

### **Ultragenyx to Host Conference Call Today at 5:00pm ET**

NOVATO, Calif., Nov. 15, 2017 (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 the U.S. Food and Drug Administration (FDA) has approved MEPSEVII™ (vestronidase alfa), the first medicine approved for the treatment of children and adults with Mucopolysaccharidosis VII (MPS VII, Sly syndrome). MEPSEVII is an enzyme replacement therapy designed to replace the deficient lysosomal enzyme beta-glucuronidase in MPS VII patients.

"The approval of MEPSEVII is a pivotal moment for Ultragenyx and for patients suffering from ultra-rare genetic diseases for which the investment and development of treatments has not happened yet," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "Our development program sought to create a new paradigm in study design and endpoint evaluations to help accommodate the difficulties of studying extremely heterogeneous ultra-rare diseases to fulfill the promise that the science we have all invested in over many years actually becomes something available for patients. I would like to thank the patients and their families for their participation in the clinical program."

MPS VII is a rare genetic, metabolic lysosomal storage disorder (LSD) caused by the deficiency of beta-glucuronidase, an enzyme required for the breakdown of the glycosaminoglycans (GAGs) dermatan sulfate, chondroitin sulfate and heparan sulfate. These complex GAG carbohydrates are a critical component of many tissues. The inability to properly break down GAGs leads to a progressive accumulation in many tissues and results in a multi-system tissue and organ damage. MPS VII is one of the rarest MPS disorders, with an estimated 200 patients in the developed world.

"I am thrilled beyond belief to see this treatment advance after more than 40 years of work and anticipation. Thanks to Ultragenyx for making it happen," said William S. Sly, Chairman Emeritus, Department of Biochemistry at St. Louis University. "I hope that this treatment will follow the other successful examples of enzyme therapy for LSDs and help improve the lives of patients with this rare disease."

MEPSEVII™ (vestronidase alfa-vjbc) was evaluated by the FDA with Priority Review, which is reserved for drugs that offer major advances in treatment or provide a treatment where no adequate therapy exists.

With this approval, the FDA issued a Rare Pediatric Disease Priority Review Voucher, which confers priority review to a subsequent drug application that would not otherwise qualify for priority review. The rare pediatric disease review voucher program is designed to encourage development of new drugs and biologics for the prevention or treatment of rare pediatric diseases.

MEPSEVII will be available to patients in the U.S. later this month. In order to support patients, Ultragenyx has launched UltraCare™, a comprehensive support service that will provide ongoing support to patients and caregivers. UltraCare will help patients obtain coverage and assist with financial support for both medication and administration of medication. Dedicated in-house UltraCare Guides are available Monday through Friday from 9 a.m. to 8 p.m. Eastern Standard Time at 888-756-8657 to assist patients their families.

In Europe, the European Medicines Agency (EMA) is currently reviewing the Marketing Authorization Application (MAA) for vestronidase alfa, and an opinion from the Committee for Medicinal Products for Human Use (CHMP) is expected in the first half of 2018.

### **Investor Conference Call**

Ultragenyx will host a conference call today at 5:00 pm Eastern Time to discuss the approval. The live and replayed webcast of the call will be available through the company's website at [www.ultragenyx.com](http://www.ultragenyx.com). To participate in the live call by phone, dial (855) 797-6910 (USA) or (262) 912-6260 (international) and enter the passcode 3274408. The replay of the call will be

available for one year.

## INDICATION

MEPSEVII is indicated in pediatric and adult patients for the treatment of Mucopolysaccharidosis VII (MPS VII, Sly syndrome).

### Limitations of Use

The effect of MEPSEVII on the central nervous system manifestations of MPS VII has not been determined.

## IMPORTANT SAFETY INFORMATION

### What is the most important information I should know about MEPSEVII?

- | **A severe allergic reaction called anaphylaxis has occurred with MEPSEVII treatment, as early as the first dose.**
- | **Your doctor will monitor you closely for symptoms of an allergic reaction while you are receiving MEPSEVII and for 60 minutes after your injection.**
- | **Your doctor will immediately discontinue the MEPSEVII infusion if you experience anaphylaxis.**
- | Your doctor should talk to you about the signs and symptoms of anaphylaxis and about getting medical treatment if you have symptoms after leaving the doctor's office or treatment center.

### What are the possible side effects of MEPSEVII?

- | The most common side effects of MEPSEVII are:
  - Leakage of MEPSEVII into the surrounding tissue during infusion
  - Diarrhea
  - Rash
  - Severe allergic reaction (anaphylaxis)
  - Infusion site swelling
  - Swelling around the infusion site
  - Severe itching of the skin
- | One patient experienced a seizure during a fever while taking MEPSEVII.

### Before receiving MEPSEVII, tell your doctor about all of your medical conditions, including if you:

- | are pregnant, think you may be pregnant, or plan to become pregnant. There is not enough experience to know if MEPSEVII may harm your unborn baby.
- | are breastfeeding or plan to breastfeed. There is not enough experience to know if MEPSEVII passes into your breast milk. Talk with your doctor about the best way to feed your baby while you receive MEPSEVII.

These are not all the possible side effects of MEPSEVII. Call your doctor for medical advice about side effects.

You may report side effects to the FDA at (800) FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch). You may also report side effects to Ultragenyx at 1-888-756-8657.

Please see full [Prescribing Information](#) for additional Important Safety Information including serious side effects.

## About Ultragenyx

Ultragenyx is a biopharmaceutical company committed to bringing to patients novel therapies 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 approved and investigational therapies 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 plans or expectations regarding the availability of MEPSEVII, future regulatory interactions and the potential timing and success of filings for regulatory approvals, 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, 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 SEC on November 3, 2017, and its subsequent periodic reports filed with the SEC.*

Contact Ultragenyx Pharmaceutical Inc.  
Investors & Media  
Danielle Keatley  
415-475-6876