Ultragenyx Announces Positive CHMP Opinion for Mepsevii™ (vestronidase alfa) For the Treatment of Mucopolysaccharidosis VII

June 29, 2018

NOVATO, Calif., June 29, 2018 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ:RARE) today announced that the Committee for Medicinal Products for Human Use (CHMP), the scientific committee of the European Medicines Agency (EMA), has adopted a positive opinion recommending the marketing authorization under exceptional circumstances of Mepsevii™ (vestronidase alfa) for the treatment of non-neurological manifestations of Mucopolysaccharidosis VII (MPS VII; Sly syndrome). Mepsevii is an enzyme replacement therapy designed to replace the deficient lysosomal enzyme beta-glucuronidase in patients with MPS VII, a progressive and debilitating rare genetic disease.

The CHMP's positive opinion will now be reviewed by the European Commission (EC), which has the authority to approve medicines for the European Union (EU). The EC decision is expected in the third quarter of 2018 and will apply to all 28 countries of the European Union, Norway, Iceland and Liechtenstein.

“This positive CHMP opinion takes us closer to bringing Mepsevii to patients across Europe who are living with this ultra-rare, highly debilitating disease and currently have no approved treatment options,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “This important regulatory milestone underscores our commitment to developing innovative medicines for patients around the world with both rare and even ultra-rare diseases for which the investment and development of the science into first-ever medicines has not yet happened.”

The CHMP considered that the totality of the data provided for this debilitating orphan disease warranted a recommendation for approval under exceptional circumstances. This type of authorization can be granted for medicines that offer new or improved treatment options for patients with no or only limited alternatives, in cases where the company is not able to provide comprehensive data. In the case of Mepsevii, data on the efficacy and safety are limited due to the extreme rarity of the disease, and the marketing authorization requires collection and submission of further data on the clinical efficacy and safety, which will be reviewed by the Committee.

Mepsevii was approved by the U.S. Food and Drug Administration for the treatment of children and adults with MPS VII in November 2017.

About MPS VII

MPS VII is a progressive 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 multi-system tissue and organ damage. MPS VII symptoms can include an abnormally coarsened face, pulmonary disease, cardiovascular complications, hepatosplenomegaly (in which the liver and spleen swell beyond their normal size), joint stiffness, short stature, cognitive impairment and the skeletal disease known as dysostosis multiplex.

MPS VII is one of the rarest MPS disorders, affecting an estimated 200 patients in the developed world.

About Mepsevii™ (vestronidase alfa)

INDICATION (IN THE U.S.)

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.

U.S. 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. You may also report side effects to Ultragenyx at 1-888-756-8657.

Please see full U.S. Prescribing Information for additional Important Safety Information including serious side effects.

About Ultragenyx

Ultragenyx is a biopharmaceutical company committed to bringing to patients 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 approved therapies and product candidates aimed at addressing diseases with high unmet medical need and clear biology for treatment, 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 relating to future regulatory interactions, the potential timing and success of filings for regulatory approvals and potential indications for 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, the timing of 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 Ultragenyx in general, see Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on May 8, 2018, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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Source: Ultragenyx Pharmaceutical Inc.

CORRECTION: A previous version of this press release incorrectly stated that the EC decision will apply to Switzerland, which it does not. This has been corrected.