Ultragenyx Announces Approval of Mepsevii™ (vestronidase alfa) in Brazil for the Treatment of Mucopolysaccharidosis VII

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Mepsevii, an enzyme replacement therapy, is the first treatment approved in Latin America for this rare genetic disease

NOVATO, Calif., Oct. 18, 2018 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for serious rare and ultra-rare genetic diseases, today announced that Brazil's National Health Surveillance Agency (ANVISA) has approved Mepsevii™ (vestronidase alfa) for the treatment of Mucopolysaccharidosis VII (MPS VII; Sly syndrome) for patients of all ages.

"The approval of Mepsevii in Brazil is an important milestone, particularly for children with this progressive and debilitating disorder, and also for Ultragenyx because it marks the first regulatory clearance for this important medicine outside of the U.S. and Europe," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "Occurring less than one year after Mepsevii was approved in the U.S., this approval validates our strategic plan to rapidly expand into other regions of the world, including Latin America."

Mepsevii was approved by the U.S. Food and Drug Administration (FDA) in November 2017 for the treatment of pediatric and adult patients with MPS VII. In August 2018, the European Commission (EC) approved the Marketing Authorization Application (MAA) for Mepsevii under exceptional circumstances for the treatment of non-neurological manifestations of Mucopolysaccharidosis VII. With this decision, Mepsevii is approved in all 28 EU countries, Iceland, Liechtenstein and Norway.

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 sulphate, chondroitin sulphate and heparan sulphate. 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 multisystem 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). 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 to know about Mepsevii?

- **A severe allergic reaction called anaphylaxis has occurred with Mepsevii treatment, as early as the first dose.**
- **Symptoms of an allergic reaction will be monitored closely while receiving Mepsevii and for 60 minutes after the infusion.**
- **If anaphylaxis is experienced, the Mepsevii infusion will be immediately discontinued.**

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, doctors should be informed about all of medical conditions, including if:

- One is pregnant, thinks she may be pregnant, or plans to become pregnant. There is not enough experience to know if

Mepsevii may harm an unborn baby.
- One is breastfeeding or plans to breastfeed. There is not enough experience to know if Mepsevii passes into breast milk. Women should talk with their doctor about the best way to feed their babies while receiving Mepsevii.

These are not all the possible side effects of Mepsevii. Doctors should be contacted for medical advice about side effects.

Side effects may be reported to the FDA at (800) FDA-1088 or www.fda.gov/medwatch.

Side effects may also be reported to Ultragenyx at 1-888-756-8657.

Please see full United StatesPrescribing Information for additional Important Safety Information including serious side effects.

About Ultragenyx Pharmaceutical Inc.
Ultragenyx is a biopharmaceutical company committed to bringing to patients novel products for the treatment of serious rare and ultra-rare genetic diseases. The company has 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.

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