FDA News Release

FDA approves treatment for rare genetic enzyme disorder

For Immediate Release

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Summary

First FDA approved treatment for pediatric and adult patients with MPS VII

Release

The U.S. Food and Drug Administration today approved Mepsevii (vestronidase alfa-vjbk) to treat pediatric and adult patients with an inherited metabolic condition called mucopolysaccharidosis type VII (MPS VII), also known as Sly syndrome. MPS VII is an extremely rare, progressive condition that affects most tissues and organs.

“This approval underscores the agency’s commitment to making treatments available to patients with rare diseases,” said Julie Beitz, M.D., director of the Office of Drug Evaluation III in the FDA’s Center for Drug Evaluation and Research (CDER). “Prior to today’s approval, patients with this rare, inherited condition had no approved treatment options.”

MPS VII is an inherited, rare genetic condition and impacts less than 150 patients worldwide. The features of MPS VII vary widely from patient to patient, but most patients have various skeletal abnormalities that become more pronounced with age, including short stature. Affected individuals can also develop heart valve abnormalities, enlarged liver and spleen, and narrowed airways which can lead to lung infections and trouble breathing. The life expectancy of individuals with MPS VII depends on the severity of symptoms. Some affected individuals do not survive infancy, while others may live into adolescence or adulthood. Heart disease and airway obstruction are major causes of death in people with MPS VII. Affected individuals may have developmental delay and progressive intellectual disability.

MPS VII is a lysosomal storage disorder caused by deficiency of an enzyme called beta-glucuronidase, which causes an abnormal buildup of toxic materials in the body’s cells. Mepsevii is an enzyme replacement therapy that works by replacing the missing enzyme.

The safety and efficacy of Mepsevii were established in clinical trial and expanded access protocols enrolling a total of 23 patients ranging from 5 months to 25 years of age. Patients received treatment with Mepsevii at doses up to 4 mg/kg once every two weeks for up to 164 weeks. Efficacy was primarily assessed via the six-minute walk test in ten patients who could perform the test. After 24 weeks of treatment, the mean difference in distance walked relative to placebo was 18 meters. Additional follow-up for up to 120 weeks suggested continued improvement in three patients and stabilization in the others. Two patients in the Mepsevii

development program experienced marked improvement in pulmonary function. Overall, the results observed would not have been anticipated in the absence of treatment. The effect of Mepsevii on the central nervous system manifestations of MPS VII has not been determined.

The most common side effects after treatment with Mepsevii include infusion site reactions, diarrhea, rash and anaphylaxis.

The FDA granted this application Fast Track (/ForPatients/Approvals/Fast/ucm405399.htm) designation, which seeks to expedite the development and review of drugs that are intended to treat serious conditions where initial evidence showed the potential to address an unmet medical need. Mepsevii also received Orphan Drug (http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/HowtoapplyforOrphanProductDesignation/default.htm) designation, which provides incentives to assist and encourage the development of drugs for rare diseases.

The sponsor is receiving a Rare Pediatric Disease Priority Review Voucher (http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/RarePediatricDiseasePriorityVoucherProgram/default.htm) under a program intended to encourage development of new drugs and biologics for the prevention and treatment of rare pediatric diseases. A voucher can be redeemed by a sponsor at a later date to receive Priority Review (/ForPatients/Approvals/Fast/ucm405405.htm) of a subsequent marketing application for a different product. This is the twelfth rare pediatric disease priority review voucher issued by the FDA since the program began.

The FDA is requiring the manufacturer to conduct a post-marketing study to evaluate the long-term safety of the product.

The FDA granted approval of Mepsevii to Ultragenyx Pharmaceutical, Inc.

The FDA, an agency within the U.S. Department of Health and Human Services, protects the public health by assuring the safety, effectiveness, and security of human and veterinary drugs, vaccines, and other biological products for human use, and medical devices. The agency also is responsible for the safety and security of our nation’s food supply, cosmetics, dietary supplements, products that give off electronic radiation, and for regulating tobacco products.

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