FDA approves first treatment for acid sphingomyelinase deficiency

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The U.S. Food and Drug Administration has approved Xenpozyme

(olipudase alfa) for pediatric and adult patients with acid sphingomyelinase deficiency (ASMD), a rare genetic disease.

ASMD is caused by the lack of an enzyme needed to break down the complex lipid sphingomyelin, which can accumulate in the liver, spleen, lung, and brain. Xenpozyme infusions provide enzyme replacement therapy.

The approval, which received fast-track, breakthrough therapy, orphan drug, and priority review designations, was based on a randomized controlled study of 31 patients in which treatment with Xenpozyme was found to improve lung function and reduce liver and spleen size. In the clinical trial, 75 percent of pediatric patients and half of adult patients experienced adverse reactions, including headaches, nausea, and vomiting, while receiving the Xenpozyme intravenous infusion.

"ASMD has a debilitating effect on people's lives and there is a critical need to increase treatment options for patients who suffer from this rare disease," Christine Nguyen, M.D., from the FDA Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine, said in a statement. "The challenges involved with developing treatments for rare diseases are significant and unique. We believe patients who suffer from ASMD, their families, and their physicians will welcome this long-awaited advancement."

Approval of Xenpozyme was granted to Genzyme.

More information: FDA Approval

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