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Japan grants marketing authorisation to Sanofi's rare disease drug

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Xenpozyme (olipudase alfa) approved in Japan, first and only approved therapy indicated to treat acid sphingomyelinase deficiency

The Japanese Ministry of Health, Labor, and Welfare (MHLW) has granted marketing authorisation for Xenpozyme (olipudase alfa) for the treatment of adult and pediatric patients with non-central nervous system (non-CNS) manifestations of acid sphingomyelinase deficiency (ASMD), a rare, progressive, and potentially life-threatening genetic disease.

Xenpozyme is currently the only approved treatment for ASMD and represents Sanofi's first therapy to be approved under the SAKIGAKE designation, which is the Japanese government's regulatory fast-track pathway to promote research and development of innovative new medical products addressing urgent unmet medical needs.

Xenpozyme is a recombinant human acid sphingomyelinase enzyme developed to replace deficient or defective acid sphingomyelinase (ASM), an enzyme that allows for the breakdown of the lipid sphingomyelin. Accumulation of sphingomyelin in cells can cause harm to the lungs, spleen, and liver, as well as other organs, potentially leading to early death.

The approval of Xenpozyme in Japan is based on positive results from the ASCEND and ASCEND-Peds clinical trials.

Outside of Japan, olipudase alfa is being evaluated by regulatory authorities around the world. A Biologics License Application (BLA) for olipudase alfa was accepted for Priority Review by the U.S. Food and Drug Administration (FDA), with a decision expected early Q3 2022. The European Medicines Agency (EMA) has awarded olipudase alfa the PRIority MEdicines (PRIME) designation, and a decision is anticipated in the second half of 2022.