Drugs

PEER-REVIEWED FEATURE

Olipudase Alfa: Adis Evaluation

Key Points

- A recombinant acid sphingomyelinase enzyme that has been developed by Sanofi, for the treatment of ASMD
- Received its first approval on 28 March 2022 in Japan
- Approved for use in adult and paediatric patients with non-CNS manifestations of ASMD

Summary

Olipudase alfa (XENPOZYME®) is a recombinant human acid sphingomyelinase that has been developed by Sanofi, for the treatment of acid sphingomyelinase deficiency (ASMD).

Olipudase alfa catalyzes the hydrolysis of sphingomyelin accumulated in hepatocytes and in mononuclear-macrophage cells, such as the lungs, liver, spleen, kidneys and bone marrow.

Olipudase alfa was approved in Japan under the SAKIGAKE designation on 28 March 2022 for use in adult and paediatric patients with non-CNS manifestations of ASMD and has received a positive Committee for Medicinal Products for Human Use opinion in the EU. Regulatory review in the USA is underway.

Keam, S. Drugs. 2022. https://doi.org/10.1007/s40265-022-01727-x