swissmedic

Public Summary SwissPAR dated 28 June 2024

Xenpozyme® (active substance: olipudase alfa)

Authorisation in Switzerland: 19 December 2023

Powder for concentrate for solution for infusion for enzyme replacement therapy to treat non-central nervous system manifestations of type A/B or type B acid sphingomyelinase deficiency (ASMD) in paediatric and adult patients.

Information on authorisation

The medicinal product Xenpozyme is used for the treatment of acid sphingomyelinase deficiency (ASMD), a very rare genetic lysosomal storage disease. The disease may occur with varying degrees of severity and is very rare, with an estimated incidence (statistical frequency of occurrence) of approximately 0.4–0.6 per 100,000 births.

Since ASMD is a rare and life-threatening disease, the medicinal product Xenpozyme has been authorised as an orphan drug. "Orphan drug" is a designation given to medicinal products for rare diseases.

In deciding whether to authorise Xenpozyme, Swissmedic took into account the assessments of the European Medicines Agency (EMA) and the US Food and Drug Administration (FDA), and the corresponding medicinal product information texts.

Since the assessment of the clinical data was based on the assessment reports of this foreign authorities, the preconditions for a full SwissPAR (Swiss Public Assessment Report – a detailed report for professionals) and a resulting Public Summary SwissPAR are not met. Swissmedic refers to the authorisations by the foreign reference authorities.

Further information on the medicinal product

Information for healthcare professionals: Information for healthcare professionals – Xenpozyme®

Healthcare professionals can answer any further questions.

The date of revision of this text corresponds to that of the SwissPAR. New information concerning the authorised medicinal product in question will not be incorporated into the Public Summary SwissPAR.

Swissmedic monitors medicinal products authorised in Switzerland. Swissmedic initiates the necessary action in the event of newly discovered adverse drug reactions or other safety-relevant signals. New findings that could impair the quality, efficacy, or safety of this medicinal product are recorded and published by Swissmedic. If necessary, the medicinal product information is adapted.