On 19 May 2022, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Xenpozyme\(^2\), intended for the treatment of non-central nervous system (CNS) manifestations of acid sphingomyelinase deficiency (ASMD) type A/B or type B.

Xenpozyme was reviewed under the accelerated assessment programme of the European Medicines Agency (EMA).

The applicant for this medicinal product is Genzyme Europe BV.

Xenpozyme will be available as a 20 mg powder to be reconstituted into a concentrate for solution for infusion. The active substance of Xenpozyme is olipudase alfa, a recombinant human acid sphingomyelinase (ATC code: A16AB25), which is an enzyme replacement therapy that provides an exogenous source of acid sphingomyelinase.

The benefits of Xenpozyme are its ability to improve the percentage of the predicted DLco (diffusing capacity of the lung for carbon monoxide) and reduce spleen volume, as observed in a multicentre, randomised, double-blinded, placebo-controlled, repeat-dose phase II/III study in adult patients with ASMD types A/B and B. The most common side effects are headache, pyrexia, urticaria, nausea, vomiting, abdominal pain, myalgia, pruritus and increases in C-reactive protein.

The full indication is:

Xenpozyme is indicated as an enzyme replacement therapy for the treatment of non-Central Nervous System (CNS) manifestations of Acid Sphingomyelinase Deficiency (ASMD) in paediatric and adult patients with type A/B or type B.

Xenpozyme should be prescribed by physicians experienced in the treatment of ASMD or other inherited metabolic disorders. Xenpozyme infusion should be administered by a healthcare professional with access...
to appropriate medical support to manage potential severe reactions such as serious systemic hypersensitivity reactions.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.