

Specialty Guideline Management

Xenpozyme

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Xenpozyme	olipudase alfa-rpcp

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-approved Indication¹

Xenpozyme is indicated for treatment of non-central nervous system manifestations of acid sphingomyelinase deficiency (ASMD) in adult and pediatric patients.

All other indications are considered experimental/investigational and not medically necessary.

Documentation

Submission of the following information is necessary to initiate the prior authorization review:

Initial Requests

- Acid sphingomyelinase enzyme assay or genetic testing results supporting the diagnosis.

Continuation Requests

- Documentation (e.g., chart notes, lab results) of a response to therapy (e.g., improvement in lung function, reduction in spleen volume, reduction in liver volume, improvement in platelet count, improvement in linear growth progression).

Prescriber Specialties

This medication must be prescribed by or in consultation with a physician knowledgeable in the management of acid sphingomyelinase deficiency (ASMD).

Coverage Criteria

Acid Sphingomyelinase Deficiency (ASMD)^{1,2}

Authorization of 12 months may be granted for treatment of non-central nervous system manifestations of acid sphingomyelinase deficiency (ASMD) when the diagnosis is confirmed by either of the following:

- A documented deficiency of acid sphingomyelinase as measured in peripheral leukocytes, cultured fibroblasts, or lymphocytes, or
- Genetic testing results documenting pathogenic variant(s) in the sphingomyelin phosphodiesterase-1 (SMPD1) gene.

Continuation of Therapy

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the coverage criteria section who are responding to therapy (e.g., improvement in lung function, reduction in spleen volume, reduction in liver volume, improvement in platelet count, improvement in linear growth progression).

References

1. Xenpozyme [package insert]. Cambridge, MA: Genzyme Corporation; December 2024.
2. Wasserstein MP, Schuchman EH. Acid sphingomyelinase deficiency. 2006 Dec 7 [Updated 2023 Apr 27]. In: Adam MP, Everman DB, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2023. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK1370/> (Accessed on November 18, 2025).