RX.PA.085.MPC Xenpozyme (olipudase alfa-rpcp)

The purpose of this policy is to define the prior authorization process for Xenpozyme® (olipudase alfa-rpcp).

Xenpozyme® (olipudase alfa-rpcp) is a hydrolytic lysosomal sphingomyelin-specific enzyme indicated for the treatment of non-central nervous system manifestations of acid sphingomyelinase deficiency (ASMD) in pediatric and adult patients.

PROCEDURE

A. Initial Authorization Criteria

1. Must meet ALL of the criteria listed below:

- Diagnosis of acid sphingomyelinase deficiency (ASMD) confirmed by one of the following:
  - Genetic testing that confirms biallelic pathogenic mutations in the SMPD1 gene
  - Enzyme assay
- Prescribed by or in consultation with a geneticist, pulmonologist, hepatologist or metabolic specialist
- Members ≤ 18 years of age must meet all of the following (a and b):
  - Spleen volume ≥ 5MN measured by an MRI
  - Height Z-score ≤ -1
- Members ≥ 18 years of age must meet all of the following (a and b):
  - Spleen volume ≥ 6MN measured by an MRI
  - Splenomegaly-related score (SRS) ≥ 5
  - Diffuse capacity of the lung for carbon monoxide (DLoc) ≤ 70% of predicted normal value
- Provider attests that female members will have a negative pregnancy test prior to initiation of therapy (within 30 days of requesting authorization)
- Female members of childbearing potential must use effective contraception during treatment and for at least 14 days after last dose of Xenpozyme
- Documentation of ALL the following baseline measurements (within 30 days of requesting authorization):
  - Diffuse capacity of the lung for carbon monoxide (DLoc)
  - Liver volume
  - Platelet count
- Dose does not exceed FDA approved indication
- Prescriber attests that Xenopozyme will be used to only manage non-CNS

manifestations of ASMD

B. Must be prescribed at a dose within the manufacturer's dosing guidelines (based on diagnosis, weight, etc) listed in the FDA approved labeling.

C. Xenpozyme will be considered investigational or experimental for any other use and will not be covered.

D. Reauthorization Criteria:

MPC Renewal:

- All prior authorization renewals are reviewed on a 1-year interval basis to determine the Medical Necessity for continuation of therapy.
- Documentation of a positive clinical response or stabilization in disease, as determined by a positive change in at least one or more baseline values:
  - Improvement in diffuse capacity of the lung for carbon monoxide (DLoc)
  - Increased platelet count
  - Reduction in spleen volume
  - Reduction in liver volume
  - Improvement in height Z-scores (members ≤ 18 years of age only)
- Prescribed by or in consultation with a geneticist, pulmonologist, hepatologist or metabolic specialist
- Female members of childbearing potential must use effective contraception during treatment and for at least 14 days after last dose of Xenpozyme

Renewal from Previous Insurer:

- Members who have received prior approval (from insurer other than MPC) and have been taking Xenpozyme have been receiving medication samples, should be considered under criterion A (Initial Authorization Criteria).Provider has a documented clinical response of the member's condition which has improved based or stabilized upon the prescriber's assessment as evidenced by the factors in the MPC renewal section
- Prescribed by or in consultation with a geneticist, pulmonologist, hepatologist or metabolic specialist

Limitations:

Length of Authorization (if above criteria met)	
Initial Authorization	6 months
Reauthorization	12 months

Codes:

Code	Description
J0218	Injection, olipudase alfa-rpcp, 1 mg

REFERENCES

1. Xenpozyme [package insert]. Cambridge, MA: Genzyme Corporation.; July 2023.

REVIEW HISTORY

DESCRIPTION OF REVIEW / REVISION	DATE APPROVED
Annual Review	*02/2026*
Annual Review	*02/2025*
Annual policy review. Update to reauthorization criteria for non-MPC renewals	*02/2024*
New Policy	*08/2023*