Reference number(s)
5560-A

SPECIALTY GUIDELINE MANAGEMENT

XENPOZYME (olipudase alfa-rpcp)

POLICY

I. 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 Indication
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.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
A. Initial requests: acid sphingomyelinase enzyme assay or genetic testing results supporting the diagnosis.
B. Continuation of therapy 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).

III. CRITERIA FOR INITIAL APPROVAL

Acid Sphingomyelinase Deficiency (ASMD)
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. A documented deficiency of acid sphingomyelinase as measured in peripheral leukocytes, cultured fibroblasts, or lymphocytes, or
B. Genetic testing results documenting a mutation in the sphingomyelin phosphodiesterase-1 (*SMPD1*) gene.

IV. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in Section III 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).

V. REFERENCES

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

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 7, 2023).