Reference number(s)
5561-A

STANDARD MEDICARE PART B 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 will be assessed on an individual basis. Submissions for indications other than those enumerated in this policy should be accompanied by supporting evidence from Medicare approved compendia.

II. DOCUMENTATION

The following documentation must be available, upon request, for all submissions:
A. Initial requests: acid sphingomyelinase enzyme assay 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-CNS manifestations of acid sphingomyelinase deficiency (ASMD) when the diagnosis is confirmed by a documented deficiency of acid sphingomyelinase as measured in peripheral leukocytes, cultured fibroblasts, or lymphocytes.

IV. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must be currently receiving therapy with the requested agent.

Authorization for 12 months may be granted when all of the following criteria are met:
A. The member is currently receiving therapy with the requested medication
B. The requested medication is being used to treat an indication enumerated in Section III

C. The member is receiving benefit from 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. SUMMARY OF EVIDENCE

The contents of this policy were created after examining the following resources:

1. The prescribing information for Xenpozyme.
2. The available compendium
    a. National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
    b. Micromedex DrugDex
    c. American Hospital Formulary Service- Drug Information (AHFS-DI)
    d. Lexi-Drugs
    e. Clinical Pharmacology

After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for Xenpozyme are covered.

VI. EXPLANATION OF RATIONALE

Support for FDA-approved indications can be found in the manufacturer's prescribing information.

Support for using an enzyme assay prior to confirm the diagnosis prior to initiating treatment with Xenpozyme to treat ASMD can be found in the clinical trials cited in the prescribing information. To be included in the trial, the patient must have had a documented deficiency of acid sphingomyelinase as measured in peripheral leukocytes, cultured fibroblasts, or lymphocytes.

VII. REFERENCES

1. Xenpozyme [package insert]. Cambridge, MA: Genzyme Corporation; August 2022.