



Nonprofit corporations and independent licensees  
of the Blue Cross and Blue Shield Association

Medical benefit drug policies are a source for BCBSM and BCN medical policy information only. These documents are not to be used to determine benefits or reimbursement. Please reference the appropriate certificate or contract for benefit information. This policy may be updated and therefore subject to change.

**Effective Date: 10/12/2023**

### **Xenpozyme™ (olipudase alfa-rpcp)**

**HCPCS: J0218**

#### **Policy:**

*Requests must be supported by submission of chart notes and patient specific documentation.*

- A. Coverage of the requested drug is provided when all the following are met:
  - a. FDA approved indication
  - b. FDA approved age
  - c. Confirmation of diagnosis by biochemical assay showing decreased acid sphingomyelinase (ASM) activity in white blood cells or skin fibroblasts less than 10% of normal AND genotyping revealing two pathogenic mutations of the SMPD1 gene
  - d. Must have type A/B or type B disease
  - e. Must have the following:
    - i. Adults
      - 1. Diffusion capacity of the lungs for carbon monoxide (DLco) of less than or equal to 70% of the predicted normal value
      - 2. A spleen volume greater than or equal to 6 times the normal (MN) measured volume by magnetic resonance imaging (MRI)
    - ii. Pediatrics
      - 1. A spleen volume greater than or equal to 5 MN measured by MRI
  - f. Trial and failure, contraindication, OR intolerance to the preferred drugs as listed in BCBSM/BCN's utilization management medical drug list.
  
- B. Quantity Limitations, Authorization Period and Renewal Criteria
  - a. Quantity Limits: Align with FDA recommended dosing
  - b. Authorization Period: One year at a time
  - c. Renewal Criteria: Clinical documentation must be provided to confirm that current criteria are met and that the medication is providing clinical benefit

\*\*\*Note: Coverage and approval duration may differ for Medicare Part B members based on any applicable criteria outlined in Local Coverage Determinations (LCD) or National Coverage Determinations (NCD) as determined by Center for Medicare and Medicaid Services (CMS). See the CMS website at <http://www.cms.hhs.gov/>. Determination of coverage of Part B drugs is based on medically accepted indications which have supported citations included or approved for inclusion determined by CMS approved compendia.

This policy and any information contained herein is the property of Blue Cross Blue Shield of Michigan and its subsidiaries, is strictly confidential, and its use is intended for the P&T committee, its members and BCBSM employees for the purpose of coverage determinations.

## Background Information:

- Acid sphingomyelinase deficiency (ASMD) is an autosomal recessive lysosomal storage disorder caused by a mutation on the sphingomyelin phosphodiesterase-1 (SMPD1) gene. It is characterized by accumulation of undegraded sphingomyelin in the spleen, liver, lungs, bone marrow, and brain due to a deficiency or insufficient activity of the enzyme ASM. Symptoms may include lack of muscle coordination, brain degeneration, learning problems, loss of muscle tone, increased sensitivity to touch, spasticity, feeding and swallowing difficulties, slurred speech, and an enlarged liver and spleen. ASMD is also known as Niemann-Pick disease (NPD) types A and B. NPD type C is now considered a separate disorder, distinct from NPD types A and B.
- ASMD is broken down into two subgroups, neuronopathic (type A) and non-neuronopathic (type B). Type A generally causes severe neurodegenerative disease during infancy, while type B is generally not considered to be a neurologic disease. There are cases that fall in between these two classifications and are referred to as type A/B. Xenozyme has only been studied in patients with type A/B or type B disease so use should be reserved for patients who fall into those two subgroups.
- The American College of Medical Genetics 2011 guidelines state ASMD is confirmed through identifying reduced ASM activity in peripheral leukocytes or skin fibroblasts of less than 10% of normal and through genetic testing that shows the patient has two pathogenic mutations of the SMPD1 gene.
- Xenozyme has only been studied in patients experiencing symptoms of non-neuronopathic disease. Adults with a DLco of less than or equal to 70% of the predicted normal value and a spleen volume greater than or equal to 6 times the normal (MN) measured volume by MRI and pediatric patients with a spleen volume greater than or equal to 5 MN measured by MRI were included in clinical trials. As these are the patients who have shown benefit with treatment and there is no data to support use in those without these symptoms, Xenozyme should be limited to those with similar clinical markers.

## References:

1. Xenozyme [prescribing information]. Cambridge, MA: Genzyme Corporation; July 2023.
2. Wasserstein M, Lachmann R, Hollak C, et al. A randomized, placebo-controlled clinical trial evaluating olipudase alfa enzyme replacement therapy for chronic acid sphingomyelinase deficiency (ASMD) in adults: one-year results. *Genet Med.* 2022; 24: 1425 – 36.
3. Diaz GA, Jones SA, Scarpa M, et al. One-year results of a clinical trial of olipudase alfa enzyme replacement therapy in pediatric patients with acid sphingomyelinase deficiency. *Genet Med.* 2021 Aug; 23 (8): 1543 - 50.
4. National Organization of Rare Disorders. Acid sphingomyelinase deficiency. 2019. Available at: <https://rarediseases.org/rare-diseases/acid-sphingomyelinase-deficiency/>. Accessed on September 2, 2022.
5. McGovern MM, Dionisi-Vici C, Giugliani R, et al. Consensus recommendation for a diagnostic guideline for acid sphingomyelinase deficiency. *Genet Med.* 2017 Sep; 19 (9): 967 - 74.

Policy History												
#	Date	Change Description										
1.2	Effective Date: 10/12/2023	Updated to remove prescriber requirement										
1.1	Effective Date: 11/01/2022	UM medical management system update for MAPPO and BCNA <table border="1" data-bbox="483 338 1365 548"> <thead> <tr> <th>Line of Business</th> <th>PA Required in Medical Management System (Yes/No)</th> </tr> </thead> <tbody> <tr> <td>BCBS</td> <td>Yes</td> </tr> <tr> <td>BCN</td> <td>Yes</td> </tr> <tr> <td>MAPPO</td> <td>Yes</td> </tr> <tr> <td>BCNA</td> <td>Yes</td> </tr> </tbody> </table>	Line of Business	PA Required in Medical Management System (Yes/No)	BCBS	Yes	BCN	Yes	MAPPO	Yes	BCNA	Yes
Line of Business	PA Required in Medical Management System (Yes/No)											
BCBS	Yes											
BCN	Yes											
MAPPO	Yes											
BCNA	Yes											
1.0	Effective Date: 10/06/2022	New policy UM medical management system update for BCBS and BCN <table border="1" data-bbox="483 663 1365 873"> <thead> <tr> <th>Line of Business</th> <th>PA Required in Medical Management System (Yes/No)</th> </tr> </thead> <tbody> <tr> <td>BCBS</td> <td>Yes</td> </tr> <tr> <td>BCN</td> <td>Yes</td> </tr> <tr> <td>MAPPO</td> <td>No</td> </tr> <tr> <td>BCNA</td> <td>No</td> </tr> </tbody> </table>	Line of Business	PA Required in Medical Management System (Yes/No)	BCBS	Yes	BCN	Yes	MAPPO	No	BCNA	No
Line of Business	PA Required in Medical Management System (Yes/No)											
BCBS	Yes											
BCN	Yes											
MAPPO	No											
BCNA	No											

\* The prescribing information for a drug is subject to change. To ensure you are reading the most current information it is advised that you reference the most updated prescribing information by visiting the drug or manufacturer website or <http://dailymed.nlm.nih.gov/dailymed/index.cfm>.