Blue Cross Blue Shield Blue Care Network of Michigan

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: 10/03/2024

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.

Background Information:

- Acid sphingomyelinase deficiency (ASMD) is an autosomal recessive lysosomal storage disorder caused by a mutation on the sphingomyelin phophodiesterase-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 thazt fall in between these two classifications and are referred to as type A/B. Xenpozyme 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.

- Xenpozyme 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, Xenpozyme should be limited to those with similar clinical markers.

References:

1. Xenpozyme [prescribing information]. Cambridge, MA: Genzyme Corporation; December 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, at al. Consensus recommendation for a diagnostic guideline for acid sphingomyelinase deficiency. Genet Med. 2017 Sep; 19 (9): 967 - 74.

<table>
<tr><th colspan="3">Policy History</th></tr>
<tr><th>#</th><th>Date</th><th>Change Description</th></tr>
<tr><td>1.3</td><td>Effective Date: 10/03/2024</td><td>Annual review – no changes to the criteria at this time</td></tr>
<tr><td>1.2</td><td>Effective Date: 10/12/2023</td><td>Updated to remove prescriber requirement</td></tr>
<tr><td>1.1</td><td>Effective Date: 11/01/2022</td><td>UM medical management system update for MAPPO and BCNA
<table><tr><th>Line of Business</th><th>PA Required in Medical Management System (Yes/No)</th></tr><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></table></td></tr>
<tr><td>1.0</td><td>Effective Date: 10/06/2022</td><td>New policy
UM medical management system update for BCBS and BCN
<table><tr><th>Line of Business</th><th>PA Required in Medical Management System (Yes/No)</th></tr><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></table></td></tr>
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** 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.*

Blue Cross Blue Shield/Blue Care Network of Michigan
Medication Authorization Request Form

Xenpozyme™ (olipudase alfa-rpcp): **J0218**

Blue Cross Blue Shield Blue Care Network of Michigan

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This form is to be used by participating physicians to obtain coverage for Xenpozyme. For commercial members only, please complete this form and submit via fax to 1-877-325-5979. If you have any questions regarding this process, please contact BCBSM Provider Relations and Servicing or the Medical Drug Helpdesk at 1-800-437-3803 for assistance.

PATIENT INFORMATION	PHYSICIAN INFORMATION
Name	**Name**
ID Number	**Specialty**
D.O.B. ☐Male ☐Female	**Address**
Diagnosis	**City /State/Zip**
Drug Name	**Phone/Fax: P**: ( ) - **F**: ( ) -
Dose and Quantity	**NPI**
Directions	**Contact Person**
Date of Service(s)	**Contact Person Phone / Ext.**

STEP 1: DISEASE STATE INFORMATION

1. Is this request for: ☐ Initiation ☐ Continuation of therapy *Date patient started therapy:* _____

2. Please provide the NPI number for the place of administration: _____

3. **Initiation AND Continuation of Therapy:**
   a. What is the patient's diagnosis?
      ☐ Non–central nervous system manifestations of acid sphingomyelinase deficiency (ASMD)
      ☐ Other, list diagnosis: _____
   b. How has the patient been diagnosed with ASMD? ***(Please attach any tests confirming diagnosis)***
      ☐ Biochemical assay showing decreased acid sphingomyelinase (ASM) activity in white blood cells
      ☐ Skin fibroblasts less than 10% of normal, Results: _____ Date: _____
      ☐ Genotyping revealing two pathogenic mutations of the SMPD1 gene
      ☐ Other: _____
   c. What type of disease does the patient have?
      ☐ Type A/B
      ☐ Type B
      ☐ Other; Please specify: _____
   d. What symptoms is the patient experiencing?
      i. For Adults:
         ☐ Diffusion capacity of the lungs for carbon monoxide (DLco) of less than or equal to 70% of the predicted normal value
         ☐ A spleen volume greater than or equal to 6 times the normal (MN) measured volume by magnetic resonance imaging (MRI)
      ii. For Pediatrics:
         ☐ A spleen volume greater than or equal to 5 MN measured by MRI
         ☐ Other: _____

4. **Continuation of therapy:**
   a. If the patient is continuing therapy, please give the patient's current disease status since beginning treatment:
      ☐ Improved: Please describe: _____
      ☐ Stable; Please describe: _____
      ☐ Worsened; Please describe: _____
      ☐ Other; Please describe: _____

5. ***Please add any other supporting medical information necessary for our review***

Coverage will not be provided if the prescribing physician's signature and date are not reflected on this document.

☐ Request for expedited review: I certify that applying the standard review time frame may seriously jeopardize the life or health of the member or the member's ability to regain maximum function

Physician's Name	Physician Signature	Date

Step 2: Checklist	☐ Form Completely Filled Out ☐ Attached Chart Notes	☐ Diagnostic Tests Attached
Step 3: Submit	**By Fax: BCBSM Specialty Pharmacy Mailbox 1-877-325-5979**	**By Mail: BCBSM Specialty Pharmacy Program P.O. Box 312320, Detroit, MI 48231-2320**