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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
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BCBS	Yes											
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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
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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



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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. <input type="checkbox"/> Male <input type="checkbox"/> 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	<input type="checkbox"/> Form Completely Filled Out <input type="checkbox"/> Attached Chart Notes	<input type="checkbox"/> 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

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