Harvard Pilgrim Health Care | TUFTS Health Plan

Medical Necessity Guideline
Medical Benefit Drug
Xenpozyme™ (olipudase alfa-rpcp)

Effective: April 1, 2023

Prior Authorization Required If REQUIRED, submit supporting clinical documentation pertinent to service request.	Yes ☒ No ☐

Applies to:

Commercial Products

☐ Harvard Pilgrim Health Care Commercial products; Fax 617-673-0988
☐ Tufts Health Plan Commercial products; Fax 617-673-0988
CareLink℠ – Refer to CareLink Procedures, Services and Items Requiring Prior Authorization

Public Plans Products

☐ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product); Fax 617-673-0988
☐ Tufts Health Together – MassHealth MCO Plan and Accountable Care Partnership Plans; Fax 617-673-0939
☐ Tufts Health RITogether – A Rhode Island Medicaid Plan; Fax 617-673-0939
☒ Tufts Health Unify* – OneCare Plan (a dual-eligible product); Fax 617-673-0956
*The MNG applies to Tufts Health Unify members unless a less restrictive LCD or NCD exists.

Senior Products

☒ Harvard Pilgrim Health Care Stride Medicare Advantage; Fax 617-673-0956
☒ Tufts Health Plan Senior Care Options (SCO), (a dual-eligible product); Fax 617-673-0956
☒ Tufts Medicare Preferred HMO, (a Medicare Advantage product); Fax 617-673-0956
☒ Tufts Medicare Preferred PPO, (a Medicare Advantage product); Fax 617-673-0956

Note: While you may not be the provider responsible for obtaining prior authorization, as a condition of payment you will need to ensure that prior authorization has been obtained.

Overview

Acid Sphingomyelinase Disease or ASMD (historically known as Niemann-Pick Disease types A, A/B and B) is a rare, progressive and potentially life threatening autosomal recessive genetic disorder that results in a deficiency of the enzyme acid sphingomyelinase. It is caused by pathogenic variants in the sphingomyelin phosphodiesterase 1 gene. ASM degrades sphingomyelin to ceramide and phosphocholine. The deficiency of ASM causes an intra-lysosomal accumulation of sphingomyelin (as well as cholesterol and other cell membrane lipids) in various tissues. Current management of the disease includes palliative and supportive care such as nutritional support, supplemental oxygen, and blood transfusions to manage symptoms only.

XENPOZYME (olipudase alfa-rpcp) provides an exogenous source of ASM which is required to breakdown a fatty substance called sphingomyelin. Type A is the most severe and type B is the milder form. Many cases of ASMD fall between these two extremes and are referred to as type A/B disease. Fewer than 120 patients are currently diagnosed with ASMD in the United States, although this number may be an underestimation due to the rarity of the disease and low disease awareness.

Food and Drug Administration (FDA) Approved Indications:

- XENPOZYME (olipudase alfa-rpcp) is a hydrolytic lysosomal sphingomyelin-specific enzyme indicated for treatment of non–central nervous system manifestations of acid sphingomyelinase deficiency (ASMD) [types B and A/B] in adult and pediatric patients.

Clinical Guideline Coverage Criteria

The Plan may cover Xenpozyme (olipudase alfa-rpcp) when all the following clinical criteria is met:

Initial Authorization Criteria:

1. The Member has a diagnosis of acid sphingomyelinase deficiency (ASMD) confirmed by enzyme assay, and judged by the provider to be ASMD type B or type A/B.

AND

2. The Member's alanine aminotransferase (ALT) or aspartame aminotransferase (AST) is less than 250 IU/L.
Note: *Baseline transaminase (alanine aminotransferase [ALT] and aspartate aminotransferase [AST]) levels should be performed within 1 month prior to treatment initiation; within 72 hours prior to any infusion during dose escalation; or prior to the next scheduled Xenpozyme infusion upon resuming treatment following a missed dose.*

AND

3. For adult Members, baseline diffusion capacity of the lungs for carbon monoxide (DLco) is less than or equal to 70% of predicted normal.

AND

4. The Member has a spleen volume measured by MRI that is greater than or equal to 6 MN (multiples of normal) for adults or greater than or equal to 5 MN for patients less than 18 years of age, unless the Member has already undergone a splenectomy.

AND

5. Xenpozyme is being prescribed by or in consultation with a specialist familiar with the treatment of lysosomal storage disorders.

AND

6. The Provider attests that the Member **does not** have one (1) or more of the following circumstances:
    a. The Member has acute or rapidly progressive neurologic abnormalities
    b. The Member requires use of invasive ventilatory support or requires noninvasive ventilatory support while awake and for greater than 12 hours a day
    c. The Member has a platelet count less than 60 x 103/µL

Renewal Authorization Criteria:

1. The Member has a diagnosis of acid sphingomyelinase deficiency (ASMD) confirmed by enzyme assay, and judged by the provider to be ASMD type B or type A/B.

AND

2. Xenpozyme is being prescribed by or in consultation with a specialist familiar with the treatment of lysosomal storage disorders.

AND

3. Documentation from the treating provider that confirms the Member is experiencing a positive clinical response to Xenpozyme treatment (e.g. improvement in DLco, reduction in spleen volume, reduction of symptoms/manifestations of ASMD, reduction in level of supportive care).

Limitations

- The Plan may authorize initial coverage of Xenpozyme (olipudase alfa-rpcp) for 12 months if initial criteria are met
- The Plan may reauthorize coverage of Xenpozyme (olipudase alfa-rpcp) for 12 months if renewal criteria are met
- Authorization for Members new to the Plan and already established on treatment with Xenpozyme (olipudase alfa-rpcp) should be reviewed using Renewal Authorization Criteria.
- The Plan will not cover Xenpozyme (olipudase alfa-rpcp) for Members with a diagnosis of ASMD type A or any other non-FDA-approved indication(s).

Codes

The following code(s) require prior authorization:

Table 1: HCPCS Codes

HCPCS Codes	Description
J0218	Injection, olipudase alfa-rpcp, 1 mg

References:

1. XENPOZYME™ (olipudase alfa-rpcp) for injection. [Prescribing Information]. Genzyme Corporation; Cambridge, MA. August 2022.
2. Xenpozyme™ (olipudase alfa-rpcp) approved by FDA as first disease-specific treatment for ASMD (non-CNS manifestations). Sanofi Press Release. November 30, 2022.
3. McGovern MM, Avetisyan R, Sanson B-J, Lidove O. Disease manifestations and burden of illness in patients with acid sphingomyelinase deficiency (ASMD). Orphanet J Rare Dis. 2017;12(1):41.
4. Schuchman EH, Desnick RJ. Types A and B Niemann-Pick disease. Mol Genet Metab. 2017;120(1-2):27-33.
5. McGovern MM, Dionisi-Vici C, Giugliani R, et al. Consensus recommendation for a diagnostic guideline for acid sphingomyelinase deficiency. Genet Med. 2017;19(9):967-974.
6. Cox GF, Clarke LA, Giugliani R, et al. Burden of illness in acid sphingomyelinase deficiency: a retrospective chart review of 100 patients. JIMD Rep. 2018;41:119-129.
7. Wasserstein MP, Jones SA, Soran H, et al. Successful within-patient dose escalation of olipudase alfa in sphingomyelinase deficiency. Mol Genet Metab. 2015;116(1-2):88-97.

Approval And Revision History

December 13, 2022: Reviewed and approved by Pharmacy and Therapeutics Committee (P&T)

December 21, 2022: Reviewed by the Medical Policy Approval Committee (MPAC)

Subsequent endorsement date(s) and changes made:

- Administrative update: March 2023 added Medical Benefit Drugs to title and updated MATogether and RITogether fax numbers to 617-673-0939
- Coding update per HCPCS level II quarterly release. Effective date April 1, 2023, the following HCPCS codes have been added: J0218

Background, Product and Disclaimer Information

Medical Necessity Guidelines are developed to determine coverage for benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. We make coverage decisions using these guidelines, along with the Member's benefit document, and in coordination with the Member's physician(s) on a case-by-case basis considering the individual Member's health care needs.

Medical Necessity Guidelines are developed for selected therapeutic or diagnostic services found to be safe and proven effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in our service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. We revise and update Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern. For Tufts Health Together (Medicaid), coverage may be available beyond these guidelines for pediatric members under age 21 under the Early and Periodic Screening, Diagnostic and Treatment (EPSDT) benefits of the plan in accordance with 130 CMR 450.140 and 130 CMR 447.000, and with prior authorization.

Treating providers are solely responsible for the medical advice and treatment of Members. The use of this guideline is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to eligibility and benefits on the date of service, coordination of benefits, referral/authorization, utilization management guidelines when applicable, and adherence to plan policies, plan procedures, and claims editing logic.