Harvard Pilgrim Health Care | TUFTS Health Plan

Medical Necessity Guidelines
Medical Benefit Drugs
Xenpozyme (olipudase alfa-rpcp)

Effective: October 13, 2026

Guideline Type	☒ Prior Authorization ☐ Non-Formulary ☐ Step-Therapy ☐ Administrative

Applies to:

Commercial Products

☒ Harvard Pilgrim Health Care Commercial products; Fax 617-673-0988

☒ Tufts Health Plan Commercial products; Fax 617-673-0988

Public Plans Products

☒ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product); Fax 617-673-0988

☐ Tufts Health Together – MassHealth Accountable Care Partnership Plans; Fax 617-673-0939

☒ Tufts Health RITogether – A Rhode Island Medicaid Plan; Fax 617-673-0939

☐ Tufts Health One Care* – A Medicare-Medicaid Plan (a dual eligible product); Fax 617-673-0956

*The MNG applies to Tufts Health One Care members unless a less restrictive LCD or NCD exists.

Senior Products

☐ 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

Food and Drug Administration - 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 authorize Xenpozyme (olipudase alfa-rpcp) when **ALL** the following clinical criteria is met:

Initial Authorization Criteria

1. Documented diagnosis of acid sphingomyelinase deficiency (ASMD) confirmed by **both** of the following:
   a. The diagnosis of ASMD has been established by acid sphingomyelinase (ASM) enzymatic assay testing
   b. The diagnosis of ASMD has been confirmed by mutation testing

AND

2. Documentation the patient has **one (1)** of the following:
   a. ASMD Type B
   b. ASMD Type A/B

AND

3. Documentation the patient has **two (2) or more** non-central nervous system signs of ASMD type B or type A/B according to the prescriber (examples: hepatosplenomegaly, interstitial lung disease, decreased diffusing capacity of the lungs, progressive liver disease with cirrhosis or fibrosis, dyslipidemia, osteopenia, thrombocytopenia, anemia, leukopenia)

AND

4. Prescribed by or in consultation with a specialist familiar with the treatment of lysosomal storage disorders

Renewal Authorization Criteria

1. Documented diagnosis of acid sphingomyelinase deficiency (ASMD) 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 diffusion capacity of the lungs for carbon monoxide, reduction in spleen volume, reduction of symptoms/manifestations of acid sphingomyelinase deficiency, reduction in level of supportive care)

Limitations

- Xenpozyme will be authorized in 12-month intervals.
- Members new to the plan stable on Xenpozyme should be reviewed against Reauthorization Criteria.

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. December 2024.
2. 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.
3. Schuchman EH, Desnick RJ. Types A and B Niemann-Pick disease. Mol Genet Metab. 2017;120(1-2):27-33.
4. 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.
5. 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.
6. 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)

Subsequent endorsement date(s) and changes made:

- December 21, 2022: Reviewed by the Medical Policy Approval Committee (MPAC)
- Administrative update: March 2023 added Medical Benefit Drugs to title
- Coding update per HCPCS level II quarterly release. Effective date April 1, 2023, the following HCPCS codes have been added: J0218
- November 14, 2023: Removed the Limitation 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). Updated Initial Authorization Criteria to confirmation of ASMD by established by acid sphingomyelinase (ASM) enzymatic assay testing and confirmed mutation testing, confirmation the patient has ASMD type B or A/B, presence of two or more non-central nervous system signs of ASMD type B or type A/B, and provider specialty requirements. Removed baseline ALT and AST, diffusion capacity of the lungs for carbon monoxide and spleen volume requirements. Removed requirement for provider attestation the member does not have one or more of the following circumstances: The Member has acute or rapidly progressive neurologic abnormalities, The Member requires use of invasive ventilatory support or requires noninvasive ventilatory support while awake and for greater than 12 hours a day, and The Member has a platelet count less than 60 x 103/µL. Updated Reauthorization Criteria to remove diagnosis requires confirmation by enzyme assay (effective 2/1/2024).
- November 2023: Administrative update to rebrand Tufts Health Unify to Tufts Health One Care for 2024.
- October 8, 2024: No changes
- July 2025: Administrative update to remove Harvard Pilgrim Health Care Stride Medicare Advantage from the Medical Necessity Guideline template.
- October 14, 2025: No changes.
- October 13, 2026: Administrative updates: Template updated to remove CareLink from the Medical Necessity Guideline template. Template updated from “Tufts Health Together – MassHealth MCO Plan and Accountable Care Partnership Plans” to “Tufts Health Together- MassHealth Accountable Care Partnership Plans.”

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.