Harvard Pilgrim Health Care | TUFTS Health Plan

Medical Necessity Guidelines

Medical Benefit Drugs

Vyondys53™ (golodirsen)

Effective: March 1, 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 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 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

Vyondys 53 (golodirsen) is an antisense oligonucleotide indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping.

This indication is approved under accelerated approval based on an increase in dystrophin in skeletal muscle observed in patients treated with Vyondys 53. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

Clinical Guideline Coverage Criteria

The Plan may authorize coverage of Vyondys 53 for Members when the following criteria are met:

Initial Authorization Criteria

1. Documented diagnosis of Duchenne muscular dystrophy with medical records confirming a mutation of the Duchenne muscular dystrophy gene that is amenable to exon 53 skipping

   Note: *Common Duchenne muscular dystrophy deletions that are theoretically amenable to exon 53 skipping include: 52, 45-52, 47-52, 48-52, 49-52, and 50-52*

AND

2. The prescribing physician is a neurologist or a provider who specializes in the treatment of Duchenne muscular dystrophy

AND

3. Documentation of **one (1)** of the following:
   a. Member has been receiving a stable dose of corticosteroids for a period of at least 6 months and will continue to utilize corticosteroids with Vyondys 53
   b. Member has a contraindication to corticosteroids

AND

4. Vyondys 53 will not be used concomitantly with other disease-modifying therapies for Duchenne muscular dystrophy (e.g., viltolarsen)

Reauthorization Criteria

1. Documented diagnosis of Duchenne muscular dystrophy with medical records confirming a mutation of the Duchenne muscular dystrophy gene that is amenable to exon 53 skipping

   Note: *Common Duchenne muscular dystrophy deletions that are theoretically amenable to exon 53 skipping include: 52, 45-52, 47-52, 48-52, 49-52, and 50-52*

AND

2. The prescribing physician is a neurologist or a provider who specializes in the treatment of Duchenne muscular dystrophy

AND

3. Documentation of **one (1)** of the following:
   a. Member continues to utilize corticosteroids in combination with Vyondys 53
   b. Member has a contraindication to corticosteroids

AND

4. Documentation that based on the prescriber's assessment, the Member continues to benefit from Vyondys 53 as documented by a standardized assessment of motor function or respiratory function

AND

5. Vyondys 53 will not be used concomitantly with other disease-modifying therapies for Duchenne muscular dystrophy (e.g., viltolarsen)

Limitations

- Initial Authorizations will be provided for 6 months. Reauthorizations will be provided for 12 months.
- Members new to the plan stable on Vyondys 53 should be reviewed against Reauthorization Criteria.
- The Plan will not authorize the use of Vyondys 53 in Members with Duchenne muscular dystrophy who do not have a confirmed mutation of the Duchenne muscular dystrophy gene that is amenable to exon 53 skipping.

Codes

The following code(s) require prior authorization:

Table 1: Codes

HCPCS Codes	Description
J1429	Injection, golodirsen, 10 mg

References

1. Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. The Lancet Neurology. 2018 March;17(3):251-267.
2. Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: respiratory, cardiac, bone health, and orthopaedic management. The Lancet Neurology. 2018 April;17(4):347-361.
3. Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 3: primary care, emergency management, psychosocial care, and transitions of care across the lifespan. The Lancet Neurology. 2018 May; 17(5):445-455.
4. Gloss D, Moxley RT, Ashwal S, et al. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy. Neurology. 2016;86:465-72.
5. Vyondys 53 (golodirsen) [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; June 2024.
6. Watanabe N, Nagata T, Satou Y, et al. NS-065/NCNP-01: An Antisense Oligonucleotide for Potential Treatment of Exon 53 Skipping in Duchenne Muscular Dystrophy. *Mol Ther Nucleic Acids*. 2018;13:442–449.

Approval And Revision History

April 19, 2023: year: Reviewed by the Medical Policy Approval Committee (MPAC)

May 9, 2023: Reviewed by Pharmacy and Therapeutics Committee (P&T)

Subsequent endorsement date(s) and changes made:

- Originally approved September 13, 2022 by P&T and September 21, 2022 by MPAC committees effective January 1, 2023

- Administrative update: April 2023 added Medical Benefit Drugs to title and updated MATogether and RITogether fax numbers to 617-673-0939
- May 17, 2023: Annual review, no change, effective July 1, 2023
- August 2023: Administrative update to rebrand Tufts Health Unify to Tufts Health One Care for 2024.
- March 12, 2024: Added “Vydonys 53 will not be used concomitantly with other disease-modifying therapies for Duchenne muscular dystrophy (e.g., viltolarsen).” Updated prerequisite language to state “Member has been receiving a stable dose of corticosteroids for a period of at least 6 months and will continue to utilize corticosteroids in combination with Vyondys 53” (effective 6/1/24).
- January 14, 2025: No changes (eff 1/14/25).
- July 2025: Administrative update to remove Harvard Pilgrim Health Care Stride Medicare Advantage from the Medical Necessity Guideline template.
- February 10, 2026: no changes. Administrative update: removed Carelink from template.

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.