Harvard Pilgrim Health Care | TUFTS Health Plan

Medical Necessity Guidelines:
Medical Benefit Drugs
Vyondys53® (golodirsen)

Effective: April 1, 2025

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

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 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

Pharmacological approaches to treating Duchenne muscular dystrophy (DMD) slow disease progression by reducing inflammation, increasing muscle strength, improving forced vital capacity, delaying scoliosis, and reducing the need for surgery. Corticosteroids are considered the standard of care, delaying loss of ambulation and respiratory decline by several years. Exon-skipping antisense oligonucleotide therapies slow the progression of DMD in about 30% of patients but have not been proven to improve survival or functional outcomes.

Approval of Vyondys53 was based on an increase in a surrogate marker, dystrophin production in skeletal muscle. No functional outcome improvement has been shown in the clinical trials for Vyondys53.

Food and Drug Administration (FDA) - Approved Indications

Vyondys53 (golodirsen) is an antisense oligonucleotide indicated for the treatment of 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 Vyondys 53 when **ALL** the following criteria is 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

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 Vyondys53, 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

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: HCPCS Codes

HCPCS Codes	Description
J1429	Injection, golodirsen, 10 mg

References:

1. Vyondys 53 (golodirsen) [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; February 2021.
2. Gloss D, et al. Practice guideline update summary: corticosteroid treatment of Duchenne muscular dystrophy: Report of the Guideline Development Subcommittee of the American Academy of Neurology. Neurology. 2016;86(5):465-472.
3. Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. Lancet Neurol. 2018;17(3):251-267.
4. Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: respiratory, cardiac, bone health, and orthopaedic management. Lancet Neurol. 2018;17(4):347-361.
5. 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. Lancet Neurol. 2018;17(5):445-455.
6. American Academy of Neurology. Evidence-Based Guideline Summary: Evaluation, Diagnosis, and Management of Congenital Muscular Dystrophy. Published March 2015. Accessed March 4, 2021.

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.
- November 2023: Administrative Update in support of calendar year 2024 Medicare Advantage and PDP Final Rule.
- March 12, 2024: Added step through corticosteroids. Updated provider specialty requirements. Added Vyondys 53 will not be used concomitantly with other disease-modifying therapies for Duchenne muscular dystrophy (e.g., viltolarsen). Removed the Limitation Any indications other than FDA-approved indications are considered experimental or investigational and will not be approved by the Plan (eff 6/1/2024).
- February 11, 2025: No changes. Administrative update to remove Harvard Pilgrim Health Care Stride Medicare Advantage from the Medical Necessity Guideline template (eff 4/1/25).
- March 2025: Joint Medical Policy and Health Care Services UM Committee review (eff 4/1/25).

Background, Product and Disclaimer Information

Point32Health prior authorization criteria to be applied to Medicare Advantage plan members is based on guidance from Medicare laws, National Coverage Determinations (NCDs) or Local Coverage Determinations (LCDs). When no guidance is provided, Point32Health uses clinical practice guidance published by relevant medical societies, relevant medical literature, Food and Drug Administration (FDA)-approved package labeling, and drug compendia to develop prior authorization criteria to apply to Medicare Advantage plan members. Medications that require prior authorization generally meet one or more of the following criteria: Drug product has the potential to be used for cosmetic purposes; drug product is not considered as first-line treatment by medically accepted practice guidelines, evidence to support the safety and efficacy of a drug product is poor, or drug product has the potential to be used for indications outside of the indications approved by the FDA. Prior authorization and use of the coverage criteria within this Medical Necessity Guideline will ensure drug therapy is medically necessary, clinically appropriate, and aligns with evidence-based guidelines. We revise and update Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests revisions.

Treating providers are solely responsible for the medical advice and treatment of Members. The use of this guidelines 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.