

Clinical Policy: Golodirsen (Vyondys 53)

Reference Number: PA.CP.PHAR.453

Effective Date: 01/2020

Last Review Date: 01/2026

Description

Golodirsen (Vyondys 53[®]) is an antisense oligonucleotide.

FDA Approved Indication(s)

Vyondys 53 is 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.

Limitation(s) of use: This indication is approved under accelerated approval based on an increase in dystrophin production 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.

All requests reviewed under this policy may **require medical director review**.

Policy/Criteria

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

I. Initial Approval Criteria

A. Duchenne Muscular Dystrophy (must meet all):

1. Diagnosis of DMD with mutation amenable to exon 53 skipping (*see Appendix D*) confirmed by genetic testing;
2. Prescribed by or in consultation with a neurologist;
3. Member has documentation of a baseline evaluation, including a standardized assessment of motor function, by a neurologist with experience treating Duchenne muscular dystrophy;
4. Vyondys 53 is prescribed concurrently with an oral corticosteroid, unless contraindicated or clinically significant adverse effects are experienced;
5. Vyondys 53 is not prescribed concurrently with other exon-skipping therapies (e.g., Amondys 45[®], Exondys 51[®], Vilepso[®]);
6. Dose does not exceed 30 mg/kg per week.

Approval duration: 6 months

NOTE: The member does not meet the clinical review guidelines listed above, but, in the professional judgment of the physician reviewer, the services are medically necessary to meet the medical needs of the member.

II. Continued Therapy

A. Duchenne Muscular Dystrophy (must meet all):

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.PHARM.01) applies;
2. Member has been assessed by a neurologist within the last 12 months;
3. Member has documentation of an annual evaluation, including an assessment of motor function ability;
4. Member continues to benefit based on prescriber's assessment;
5. Vyondys 53 is prescribed concurrently with an oral corticosteroid, unless contraindicated or clinically significant adverse effects are experienced;
6. Vyondys 53 is not prescribed concurrently with other exon-skipping therapies (e.g., Amondys 45, Exondys 51, Viltepso);
7. If request is for a dose increase, new dose does not exceed 30 mg/kg per week.

Approval duration: 6 months

NOTE: The member does not meet the clinical review guidelines listed above, but, in the professional judgment of the physician reviewer, the services are medically necessary to meet the medical needs of the member.

III. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

6MWT: 6-minute walk test

DMD: Duchenne muscular dystrophy

FDA: Food and Drug Administration

FVC: forced vital capacity

ICER: Institute for Clinical and Economic Review

LVEF: left ventricular ejection fraction

Appendix B: Therapeutic Alternatives

This table provides a listing of preferred alternative therapy recommended in the approval criteria. The drugs listed here may not be a formulary agent and may require prior authorization.

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
prednisone*	0.3-0.75 mg/kg/day or 10 mg/kg/weekend PO	Based on weight
Emflaza™ (deflazacort)	0.9 mg/kg/day orally once daily	Based on weight
Agamree® (vamorolone)	6 mg/kg/day PO QD (up to a maximum of 300 mg/day) <ul style="list-style-type: none"> • If member has mild (Child-Pugh A) to moderate (Child-Pugh B) hepatic impairment: 2 mg/kg/day PO QD (up to a maximum of 100 mg/day) If co-administered with strong CYP3A4 inhibitors (e.g., itraconazole): 4 mg/kg/day PO QD (up to a maximum of 200 mg/day)	See regimen

Therapeutic alternatives are listed as Brand name® (generic) when the drug is available by brand name only and generic (Brand name®) when the drug is available by both brand and generic.

*Off-label

Appendix C: Contraindications/Boxed Warnings

- Contraindication(s): serious hypersensitivity to golodirsen or to any of the inactive ingredients in Vyondys 53
- Boxed warning(s): none reported

Appendix D: General Information

- Common mutations amenable to exon 53 skipping include: 3-52, 4-52, 5-52, 6-52, 9-52, 10-52, 11-52, 13-52, 14-52, 15-52, 16-52, 17-52, 19-52, 21-52, 23-52, 24-52, 25-52, 26-52, 27-52, 28-52, 29-52, 30-52, 31-52, 32-52, 33-52, 34-52, 35-52, 36-52, 37-52, 38-52, 39-52, 40-52, 41-52, 42-52, 43-52, 45-52, 47-52, 48-52, 49-52, 50-52, 52, 54-58, 54-61, 54-64, 54-66, 54-76, 54-77.
- Corticosteroids are routinely used in DMD management with established efficacy in slowing decline of muscle strength and function (including motor, respiratory, and cardiac). They are recommended for all DMD patients per the American Academy of Neurology (AAN) and DMD Care Considerations Working Group; in addition, the AAN guidelines have been endorsed by the American Academy of Pediatrics, the American Association of Neuromuscular & Electrodiagnostic Medicine, and the Child Neurology Society.
 - The DMD Care Considerations Working Group guidelines, which were updated in 2018, continue to recommend corticosteroids as the mainstay of therapy.
 - In an evidence report published August 2019, the Institute for Clinical and Economic Review (ICER) states that current evidence is insufficient to conclude that Vyondys 53 has net clinical benefit when added to corticosteroids and supportive care versus corticosteroids and supportive care alone.
- Prednisone is the corticosteroid with the most available evidence. A second corticosteroid commonly used is deflazacort, which was FDA approved for DMD in February 2017. On October 2023, a third corticosteroid, vamorolone, was approved by the FDA for DMD.
- The inclusion criteria for Study 4053-US-101 (NCT02310906) used to support the FDA approval of Vyondys 53 enrolled male patients age 6-15 years old with a mean 6MWT distance of 250 m or more at screening and baseline visits, LVEF \geq 50% based on screening echocardiogram (ECHO), and stable pulmonary function with FVC \geq 50%.

IV. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
DMD	30 mg/kg IV once weekly	30 mg/kg

V. Product Availability

Single-dose vial for injection: 100 mg/2 mL (50 mg/mL)

VI. References

1. Vyondys 53 Prescribing Information. Cambridge, MA: Sarepta Therapeutics, Inc.; June 2024. Available at: <https://www.vyondys53.com>. Accessed October 23, 2025.

2. Bushby K, Finkel R, Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. *Lancet Neurol.* 2010; 9(1): 77-93.
3. Gloss D, Moxley RT, Ashwal S, Oskoui M. Practice guideline update summary: corticosteroid treatment of Duchenne muscular dystrophy. *Neurology.* 2016; 86: 465-472. Reaffirmed on January 22, 2022.
4. Frank D, Mercuri E, Servais L, et al. Golodirsen induces exon skipping leading to sarcolemmal dystrophin expression in patients with genetic mutations amenable to exon 53 skipping. Paper presented at: Annual Clinical Genetics Meeting of the American College of Medical Genetics and Genomics; April 2-6, 2019; Seattle, WA.
5. Chamberlain JS. Dystrophin levels required for genetic correction of Duchenne muscular dystrophy. *Basic Appl. Myol.* 1997; 7(3&4): 251-255.
6. Neri M, Torelli S, Brown S, et al. Dystrophin levels as low as 30% are sufficient to avoid muscular dystrophy in the human. *Neuromuscul Disord.* 2007; doi:10.1016/j.nmd.2007.07.005.
7. Institute for Clinical and Economic Review. Deflazacort, eteplirsen, and golodirsen for Duchenne muscular dystrophy: Effectiveness and value. Published August 15, 2019. Available at: https://icer.org/wp-content/uploads/2020/10/Corrected_ICER_DMD-Final-Report_042222.pdf. Accessed November 3, 2025.
8. Vyondys 53 Formulary Submission Dossier V1.0. Sarepta Therapeutics, Inc, December 2019.
9. Birnkrant DJ, Bushby K, Bann CM, 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: 251-267.

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J1429	Injection, golodirsen, 10 mg

Reviews, Revisions, and Approvals	Date
Policy created	01/2020
1Q 2021 annual review: added requirement that member not received concurrent Viltepso; references reviewed and updated.	01/2021
1Q 2022 annual review: added Amondys 45 to examples of exon-skipping therapies; references reviewed and updated.	01/2022
1Q 2023 annual review: no significant changes; updated Appendix D; references reviewed and updated.	01/2023
1Q 2024 annual review: updated format to match standard PAHW structure; added Agamree to list of corticosteroids in Appendix B; references reviewed and updated	01/2024

Reviews, Revisions, and Approvals	Date
1Q 2025 annual review: no significant changes; updated Appendix C with new contraindication per PI; references reviewed and updated.	01/2025
1Q 2026 annual review: no significant changes; references reviewed and updated.	01/2026