

Clinical Policy: Viltolarsen (Viltepso)

Reference Number: PA.CP.PHAR.484

Effective Date: 08/2020

Last Review Date: 01/2026

Description

Viltolarsen (Viltepso™) is an antisense oligonucleotide.

FDA Approved Indication(s)

Viltepso 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 Viltepso. Continued approval for this indication may be contingent upon verification and description of a clinical benefit in a confirmatory trial.

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.

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

It is the policy of PA Health & Wellness® that Viltepso may be **medically necessary*** when the following criteria are met:

**** Viltepso was FDA-approved based on an observed increase in dystrophin in skeletal muscle, but it is unknown if that increase is clinically significant. Continued FDA-approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.***

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. Viltepso is prescribed concurrently with an oral corticosteroid, unless contraindicated or clinically significant adverse effects are experienced;
5. Viltepso is not prescribed concurrently with other exon-skipping therapies (e.g., Amondys 45®, Vyondys 53™, Exondys 51®);
6. Dose does not exceed 80 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. Viltepso is prescribed concurrently with an oral corticosteroid, unless contraindicated or clinically significant adverse effects are experienced;
6. Viltepso is not prescribed concurrently with other exon-skipping therapies (e.g., Amondys 45, Vyondys 53, Exondys 51™);
7. If request is for a dose increase, new dose does not exceed 80 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

TTSTAND: time to stand

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 PO QD	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) 	See regimen

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
	If co-administered with strong CYP3A4 inhibitors (e.g., itraconazole): 4 mg/kg/day PO QD (up to a maximum of 200 mg/day)	

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

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 other exon-skipping therapies (Exondys 51, Vyondys 53) have 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.
- The phase 2 dose-finding, safety study for viltolarsen (NCT02740972) enrolled male patients age 4-9 years with the lowest 6MWT distance at baseline being 201 m. In addition, inclusion criteria for the ongoing phase 3 efficacy study for viltolarsen (RACER 53; NCT04060199) enrolled male patients age 4-7 years old with a TTSTAND < 10 seconds.
- Having an LVEF below 40% may indicate presence of cardiomyopathy or heart failure, while a predicted FVC below 50% may indicate presence of severe pulmonary disease.

IV. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
DMD	80 mg/kg IV once weekly	80 mg/kg/week

V. Product Availability

Solution for injection in a single-dose vial: 250 mg/5 mL (50 mg/mL)

VI. References

1. Viltepso Prescribing Information. Paramus, NJ: NS Pharma, Inc.; March 2021. Available at: www.viltepso.com. Accessed October 23, 2025.
2. Clemens PR, Rao VK, Connolly AM, et al. Safety, tolerability, and efficacy of viltolarsen in boys with Duchenne muscular dystrophy amenable to exon 53 skipping: A phase 2 randomized clinical trial. *JAMA Neurol.* 2020; 77(8) 982-991.
3. ClinicalTrials.gov. Study to assess the efficacy and safety of viltolarsen in ambulant boys with DMD (RACER53). Available at: <https://clinicaltrials.gov/ct2/show/NCT04060199>. Accessed November 3, 2025.
4. 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.
5. 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.
6. 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.
7. NS Pharma. Viltepso (viltolarsen) injection: Long-term efficacy and safety data presented at the PPMD 2021 Virtual Annual Conference. Published July 1, 2021.

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
J1427	Injection, viltolarsen

Reviews, Revisions, and Approvals	Date
Policy created	08/2020
1Q 2021 annual review: no significant changes; references reviewed and updated.	01/2021
1Q 2022 annual review: references reviewed and updated.	01/2022
1Q 2023 annual review: no significant changes; 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
1Q 2025 annual review: no significant changes; added HCPCS code [J1427]; references reviewed and updated.	01/2025
1Q 2026 annual review: no significant changes; references reviewed and updated.01/2026	01/2026