Medical Policy

Utilization Management and Clinical Medical Policy

Policy Name:	Policy Number:	Scope:	Origination Date:	Effective Date:
Vitolarsen (Viltepso®)	**MP-RX-FP-184-26**	☒ **MMM MA** ☒ **MMM MultiHealth**	**5/6/2026** **Last Review Date:** **5/6/2026**	**5/6/2026** **Frequently Revision:** **Annual**

Service Category:

- ☐ Anesthesia
- ☐ Surgery
- ☐ Radiology Procedures
- ☐ Pathology and Laboratory Procedures
- ☐ Medicine Services and Procedures
- ☐ Evaluation and Management Services
- ☐ DME/Prosthetics or Supplies
- ☒ Other: Part B Drugs

Service Description:

This document addresses the use of Viltepso® (viltolarsen) (NS-065/NCNP-01), a phosphorodiamidate morpholino oligomer antisense oligonucleotide, in the treatment of Duchenne muscular dystrophy (DMD) with a mutation amenable to exon 53 skipping. Viltepso was approved under accelerated approval based on an increase in dystrophin production in skeletal muscle. Continued approval for DMD may be contingent upon verification and description of clinical benefit in a confirmatory trial (Viltepso label).

Background Information:

DMD is a genetic disorder characterized by decrease in muscle mass over time, including progressive damage and weakness of facial, limb, respiratory and heart muscles. In DMD patients, dystrophin, a protein that is present in skeletal and heart muscles allowing the muscles to function properly, is either absent or found in very small amounts. In theory, exon 53 skipping allows for the creation of a shorter-than-normal, but partially functional, dystrophin protein in patients with a specific type of DMD mutation. Exon 53 skipping is applicable in those with deletions in exons 45-52, 47-52, 48-52, 49-52, 50-52 and 52.

Viltepso (viltolarsen) was studied in a phase II, multi center, 2-period, randomized, placebo-controlled, dose finding study in ambulant boys ages 4-9 years of age with DMD (NCT02740972). Inclusion criteria required patients to be ambulatory and have the ability to complete the following assessments: time to stand from supine, time to run/walk 10 m, and time to climb 4 stairs. While primary outcome measures were centered around adverse events, dystrophin protein in muscle and drug concentration in plasma, secondary outcomes included 6-minute walk test (6MWT), change in time to climb 4 stairs (TTCLIMB), change in time to run/walk 10 meters (TTRW), change in time to stand (TTSTAND) and North Star Ambulatory Assessment results (NSAA) were also accounted for. The secondary outcomes were measured against matched controls in an external comparator group provided by the Cooperative International Neuromuscular Research Group (CINRG) Duchenne Natural History Study (DNHS) (Clemens 2020).

An extension trial of NCT02740972 (NCT03167255) was completed in boys ≥ 4 years and < 8 years); confirmed DMD amenable to exon 53 skipping; able to walk independently without assistive devices; TTSTAND < 10 seconds; stable dose of glucocorticoid for at least 3 months prior to study inclusion; other inclusion criteria may apply. Primary endpoint is the change in TTSTAND at 48 weeks of treatment. Secondary outcome measures include change in TTRW, 6MWT, NSAA, TTCLIMB and hand-held dynamometer. Study results have not been published yet.

Viltepso (viltolarsen) is administered at a dose of 80 mg/kg via a weekly intravenous infusion. Although kidney toxicity was not observed in clinical studies with Viltepso, it was observed in animals who had received viltolarsen. Therefore, kidney function should be monitored in patients taking Viltepso. Because serum creatinine may not be a reliable measure of kidney function in DMD patients, other measures should be monitored. Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio should be measured before starting Viltepso. Urine

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dipstick should be monitored every month; serum cystatin C and urine protein-to-creatinine ratio should be monitored every 3 months. In the event of persistent elevation in serum cystatin C or proteinuria, the patient should be referred to a pediatric nephrologist for further evaluation.

Approved Indications

A. 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.

Other Uses

A. None.

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Medical Necessity Guidelines:

When a drug is being reviewed for coverage under a member's medical benefit plan or is otherwise subject to clinical review (including prior authorization), the following criteria will be used to determine whether the drug meets any applicable medical necessity requirements for the intended/prescribed purpose.

Viltepso® (viltolarsen)

A. Criteria For Initial Approval (*Provider must submit documentation [such as office chart notes, lab results, pathology reports, imaging studies, and any other pertinent clinical information] supporting the patient's diagnosis for the drug and confirming that the patient has met **all** approval criteria.)*

Initial requests for Viltepso (viltolarsen) may be approved if the following criteria are met:

i. Individual has a confirmed diagnosis of Duchenne muscular dystrophy (DMD); **AND**
ii. Documentation is provided that individual has a genetic mutation that is amenable to exon 53 skipping; **AND**
iii. Individual is age 4-9 years (NCT02740972) (Clemens 2020); AND IV. Individual is using a corticosteroid; **AND**
iv. Documentation is provided that individual is ambulatory; **AND**
v. Individual is able to complete the following assessments: (NCT02740972, NCT04060199; Clemens 2020)
    A. Time to stand from supine; **AND**
    B. Time to run/walk 10 meters; **AND**
    C. Time to climb 4 stairs.

B. Criteria For Continuation of Therapy

i. MMM considers continuation of Viltepso therapy medically necessary in members requesting reauthorization for an indication listed in Section A Above (Criteria for Initial Approval) if the following criterion are met:
    A. Documentation is provided that individual remains ambulatory (with or without needing an assistive device, including but not limited to a cane or walker).

C. Authorization Duration

i. Initial Approval Duration: 6 months
ii. Reauthorization Approval Duration: 6 months

D. Conditions Not Covered

Any other use is considered experimental, investigational, or unproven, including the following (this list may not be all inclusive):

i. Requests for Viltepso may not be approved when the above criteria (Section A: Criteria for Initial Approval) are not met and for all other indications:

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a. Concomitant use with another exon skipping agent for DMD (including but not limited to Exondys 51, Vyondys 53).

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Limits or Restrictions:

A. Therapeutic Alternatives

The list below includes preferred alternative therapies recommended in the approval criteria and may be subject to prior authorization.

i. N/A

B. Quantity Limitations

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines. The chart below includes dosing recommendations as per the FDA-approved prescribing information.

Dosage Form & Strengths	Recommended Dosing/Limits
Viltepso injection 250 mg/5 mL (50 mg/mL) single-dose vial	• 80 mg/kg once weekly (body weight)
Exceptions	
None	

Codes Information:

The following list(s) of procedure and/or diagnosis codes is provided for reference purposes only and may not be all inclusive. Inclusion or exclusion of a procedure, diagnosis or device code(s) does not constitute or imply member coverage or provider reimbursement policy. Benefit coverage for health services is determined by the member specific benefit plan document and applicable laws that may require coverage for a specific service. The inclusion of a code does not imply any right to reimbursement or guarantee claim payment. Other Policies and Guidelines may apply.

ICD-10 Diagnostic Codes:

Codes	Description
G71.01	Duchenne or Becker muscular dystrophy

HCPCS Codes:

Codes	Description
J1427	Injection, viltolarsen, 10 mg [Viltepso]

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Reference Information:

1. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. http://dailymed.nlm.nih.gov/dailymed/about.cfm.
2. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
3. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc. Updated periodically.
4. 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 [published correction appears in JAMA Neurol. 2020 Aug 1;77(8):1040. doi: 10.1001/jamaneurol.2020.2025]. JAMA Neurol. 2020;77(8):982-991. doi:10.1001/jamaneurol.2020.1264.
5. Clemens PR, Rao VK, Connolly AM, et al. Efficacy and Safety of Viltolarsen in Boys With Duchenne Muscular Dystrophy: Results From the Phase 2, Open-Label, 4-Year Extension Study. J Neuromuscul Dis. 2023;10(3):439-447. doi:10.3233/JND-221656.
6. Kole R, Krieg AM. Exon skipping therapy for Duchenne muscular dystrophy. Ad Drug Del Rev. 2015; 87:140-107.
7. 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. Molecular Therapy: Nucleic Acids. 2018; 13:442-449.
8. Viltepso [package insert]. Paramus, NJ; NS Pharma, Inc; 2020.

Federal and state laws or requirements, contract language, and Plan utilization management programs or polices may take precedence over the application of this clinical criteria.

Policy History:

Type of Review	Summary of Changes	P&T Approval Date	UM/CMPC Approval Date
Policy Inception	Elevance Health's Medical Policy adoption.	5/1/2026	5/6/2026