Medical Policy:

Viltepso (viltolarsen)

POLICY NUMBER	LAST REVIEW	ORIGIN DATE
MG.MM.PH.321	April 2, 2025	November 11, 2020

Definitions

Viltepso, an antisense oligonucleotide, binds to exon 53 of dystrophin pre-mRNA resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amendable to exon 53 skipping. Exon 53 skipping is intended to allow for production of internally truncated dystrophin protein in patients with genetic mutations amendable to exon 53 skipping.

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 clinical benefit in a confirmatory trial.

Length of Authorization

Coverage will be provided for 6 months and may be renewed.

Dosing Limits [Medical Benefit]

Max Units (per dose and over time) [Medical Benefit]: 80 mg/kg IV once weekly; 925 billable units (9200 mg) every 7 days

Guideline

I. INITIAL APPROVAL CRITERIA

1. **Duchenne Muscular Dystrophy**

Viltepso may be considered medically necessary when all of the following conditions are met:

A. Medication is being prescribed by or in consultation with a neurologist who specializes in treatment of Duchenne muscular dystrophy (DMD); **AND**
B. Patient has been diagnosed with Duchenne muscular dystrophy (DMD); **AND**
C. Documentation has been provided confirming mutation of the DMD gene amenable to exon 53 skipping; **AND**
D. Member must have been on a stable dose of corticosteroids for at least 3 months (unless contraindicated); **AND**
E. Patient retains meaningful voluntary motor function (e.g., patient is able to speak, manipulate objects using upper extremities, ambulate, etc.); **AND**
F. Patient is receiving physical and/or occupational therapy; **AND**
G. Baseline documentation of **ONE** or more of the following:
    i. Dystrophin level
    ii. Timed function tests (e.g., 6-minute walk test [6MWT], time to stand [TTSTAND], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB], etc.)
    iii. Upper limb function (ULM) test
    iv. North Star Ambulatory Assessment (NSAA) score
    v. Forced Vital Capacity (FVC) percent predicted
H. Viltepso is not used concomitantly with other exon skipping therapies for DMD.

Limitations/Exclusions

1. Disease progression while on DRUG.
2. Indications not supported by CMS recognized compendia or acceptable peer reviewed literature may be deemed as not approvable and therefore not reimbursable.
3. Renal function should be monitored; creatinine may not be a reliable measure of renal function in DMD patients.
4. There are no clinical studies demonstrating use in the geriatric population (≥ 65 years of age) as DMD is largely a disease of children and young adults
5. Patient is not on concomitant therapy with other DMD-directed antisense oligonucleotides (e.g., eteplirsen, casimersen, golodirsen, etc.)
6. Patient is not on concomitant therapy with delandistrogene moxeparvovec-rokl; AND
7. Patient does not have symptomatic cardiomyopathy; **AND**
8. Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio (UPCR) are measured prior to starting therapy and periodically during treatment.

2. **RENEWAL CRITERIA**

Coverage can be renewed in up to 6-month intervals based upon the following:

A. Patient continues to meet INITIAL APPROVAL CRITERIA; **AND**
B. Patient has responded to therapy compared to pretreatment baseline in one or more of the following (not all-inclusive):
    i. Increase in dystrophin level
    ii. Improvement in quality of life
    iii. Stability, improvement, or slowed rate of decline in one or more of the following:
        a. Timed function tests (e.g., 6-minute walk test [6MWT], time to stand [TTSTAND], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB], etc.)
        b. Upper limb function (ULM) test

c. North Star Ambulatory Assessment (NSAA) score
d. Forced Vital Capacity (FVC) percent predicted

C. Absence of unacceptable toxicity from the drug has been documented. Examples of unacceptable toxicity include reactions (glomerulonephritis, persistent increase in serum cystatin C, proteinuria, etc.)**AND**

D. Viltepso is not used concomitantly with other exon skipping therapies for DMD.

Applicable Procedure Codes

Code	Description
J1427	njection, viltolarsen, 10 mg; 1 billable unit = 10 mg

Applicable NDCs

Code	Description
73292-0011-01	Viltepso single-dose vials containing 250 mg/5 mL (50 mg/mL)

ICD-10 Diagnoses

Code	Description
G71.01	Duchenne or Becker muscular dystrophy

Revision History

Company(ies)	DATE	REVISION
EmblemHealth & ConnectiCare	4/2/2025	Annual Review: Updated dosing limits Updated limitations/exclusions to include: Patient is not on concomitant therapy with delandistrogene moxeparvovec-rokl; AND Patient does not have symptomatic cardiomyopathy; **AND** Serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio (UPCR) are measured prior to starting therapy and periodically during treatment;
EmblemHealth & ConnectiCare	1/2/2024	Annual Review: Updated dosing limits Initial Criteria: Added: Patient retains meaningful voluntary motor function (e.g., patient is able to speak, manipulate objects using upper extremities, ambulate, etc.); AND Patient is receiving physical and/or occupational therapy; AND Baseline documentation of one or more of the following:Dystrophin level, Timed function tests (e.g., 6-minute walk test [6MWT], time to stand [TTSTAND], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB], etc.), Upper limb function (ULM) test, North Star Ambulatory Assessment (NSAA) score, Forced Vital Capacity (FVC) percent predicted" Removed: "Medical records have been provided documenting member's baseline muscle strength score while walking independently (e.g., without assist, cane, walker, wheelchair, etc.) prior to beginning Viltepso therapy by ONE of the following ambulatory functional tests: 6-minute walk test (6MWT), North Star ambulatory assessment (NSAA), Gower's test, Other appropriate test for DMD assessment; AND" Updated Limitations/Exclusions Renewal Criteria

		Added: “Patient continues to meet INITIAL APPROVAL CRITERIA; AND” Removed:”Updated documentation (recent progress notes documenting overall disease status and ambulatory status) has been provided showing that member has demonstrated a response to therapy as evidenced by remaining ambulatory (e.g. able to walk with or without assistance, not wheelchair dependent); AND” Added: ”Patient has responded to therapy compared to pretreatment baseline in one or more of the following (not all-inclusive): Increase in dystrophin level, Improvement in quality of life, Stability, improvement, or slowed rate of decline in one or more of the following: Timed function tests (e.g., 6-minute walk test [6MWT], time to stand [TTSTAND], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB], etc.) , Upper limb function (ULM) test, North Star Ambulatory Assessment (NSAA) score, Forced Vital Capacity (FVC) percent predicted” Removed: “Member has had an improvement from baseline in ONE of the following: 6-minute walk test (6MWT), North Star ambulatory assessment (NSAA), Gower’s test, Other appropriate test for DMD assessment; AND” Updated Jcode, removed C9071, replaced with J1427
EmblemHealth & ConnectiCare	4/11/2023	Annual Revision: no criteria changes
EmblemHealth & ConnectiCare	1/18/20203	Transfer to New Template
EmblemHealth & ConnectiCare	1/1/2021	Updated C-code 9071
EmblemHealth & ConnectiCare	11/11/20	New Medical Policy

References

1. Product Information: VILTEPSO™ intravenous injection, viltolarsen intravenous injection. NS Pharma Inc (per manufacturer), Paramus, NJ, 2020.
2. CureDuchenne. “ Duchenne Population Potentially Amenable to Exon Skipping.” https://www.cureduchenne.org/wp-content/uploads/2016/11/Duchenne-Population-Potentially-Amenable-to-Exon-Skipping-11.10.16.pdf
3. Kole R, Krieg AM. Exon skipping therapy for Duchenne muscular dystrophy. Adv Drug Deliv Rev. 2015; 87:104-107.
4. Landfeldt E, Lindgren P, Bell C, et al. The burden of Duchenne muscular dystrophy. Neurology. 2014; 83:529-536.
5. National Organization for Rare Disorders—Rare Disease Database. Danbury (CT): NORD. Duchenne Muscular Dystrophy. 2018 [cited 2019 Jan 25]. Available from: https://rarediseases.org/rare-diseases/duchenne-muscular-dystrophy/