



## Viltepso™ (viltolarsen) (Intravenous)

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### I. Length of Authorization

Authorization is valid for 6 months and may be renewed.

### II. Dosing Limits

#### A. Quantity Limit (max daily dose) [NDC unit]:

- Viltepso 250 mg vial: 36 vials per 7 days

#### B. Max Units (per dose and over time) [HCPCS Unit]:

##### Duchenne muscular dystrophy

- 9200 mg every 7 days

### III. Initial Approval Criteria<sup>1-6</sup>

Coverage is provided in the following conditions:

#### Universal Criteria<sup>1,5</sup>

- Patient is not on concomitant therapy with other DMD-directed antisense oligonucleotides (e.g., eteplirsen, golodirsen, etc.); **AND**
- Patient does not have symptomatic cardiomyopathy; **AND**
- Patient serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio will be measured prior to start of therapy and during treatment (monthly urine dipstick with serum cystatin C and urine protein-to-creatinine ratio every three months); **AND**

#### Duchenne muscular dystrophy (DMD) † Φ<sup>1-6</sup>

- Patient must have a confirmed mutation of the *DMD* gene that is amenable to exon 53 skipping; **AND**
- Patient has been on a stable dose of corticosteroids, unless contraindicated or intolerance, for at least 3 months; **AND**

- Patient retains meaningful voluntary motor function (e.g., patient is able to speak, manipulate objects using upper extremities, ambulate, etc.); **AND**
- Patient should be receiving physical and/or occupational therapy; **AND**
- Baseline documentation of one or more of the following:
  - Dystrophin level
  - 6-minute walk test (6MWT) or other timed function tests (e.g., time to stand [TTSTAND], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB])
  - Upper limb function (ULM) test
  - North Star Ambulatory Assessment (NSAA)
  - Forced Vital Capacity (FVC) percent predicted

† FDA-labeled indication(s), ‡ Compendia recommended indication(s); Ⓢ Orphan Drug

#### IV. Renewal Criteria <sup>1,5,6</sup>

- Patient continues to meet universal and other indication-specific relevant criteria such as concomitant therapy requirements (not including prerequisite therapy), etc. identified in section III; **AND**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include the following: renal toxicity/proteinuria, etc.; **AND**
- Patient has responded to therapy compared to pretreatment baseline in one or more of the following (not all-inclusive):
  - Increase in dystrophin level
  - Stability, improvement, or slowed rate of decline in 6MWT or other timed function tests (e.g., time to stand [TTSTAND], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB])
  - Stability, improvement, or slowed rate of decline in ULM test
  - Stability, improvement, or slowed rate of decline in NSAA
  - Stability, improvement, or slowed rate of decline in FVC% predicted
  - Improvement in quality of life

#### V. Dosage/Administration

Indication	Dose
Duchenne muscular dystrophy	The recommended dosage of Viltepso is 80 mg/kg administered once weekly as a 60-minute intravenous infusion.

#### VI. Billing Code/Availability Information

HCPCS Code:

- J3490 – Unclassified drugs.

NDC:

- Viltepso 250 mg/5 mL single-dose vial: 73292-0011-xx

## VII. References

1. Viltepso [package insert]. Paramus, NJ; NS Pharma, Inc.; August 2020. Accessed August 2020.
2. Topaloglu H, Gloss D, Moxley RT 3<sup>rd</sup>, 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 Jul 12;87(2):238.
3. 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 Jan; 9(1):77-93.
4. Bushby K, Finkel R, Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: implementation of multidisciplinary care. *Lancet Neurol*; 2010 Jan; 9(2):177-189.
5. Clemens PR, Rao VK, Connolly AM, et al; CINRG DNHS Investigators. 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 May 26. doi: 10.1001/jamaneurol.2020.1264. [Avail at: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC7251505/>]
6. Bushby K, Connor E. Clinical outcome measures for trials in Duchenne muscular dystrophy: report from International Working Group meetings. *Clin Investig (Lond)*. 2011;1(9):1217-1235

## Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
G71.01	Duchenne or Becker muscular dystrophy

## Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determination (NCD), Local Coverage Articles (LCAs) and Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. They can be found at: <http://www.cms.gov/medicare-coverage-database/search/advanced-search.aspx>. Additional indications may be covered at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCA/LCD): N/A

### Medicare Part B Administrative Contractor (MAC) Jurisdictions

Jurisdiction	Applicable State/US Territory	Contractor
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA, LLC
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA, LLC
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA)	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC