

## Viltepso® (viltolarsen) (Intravenous)

Document Number: MH-0562

Last Review Date: 05/02/2024

Date of Origin: 09/01/2020

Dates Reviewed: 09/2020, 01/2021, 04/2021, 08/2021, 08/2022, 08/2023, 05/2024

### I. Length of Authorization

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

### II. Dosing Limits

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

- Viltepso 250 mg/5 mL single-dose vial: 37 vials per 7 days

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

- 925 billable units every 7 days

### III. Initial Approval Criteria

Submission of medical records (chart notes) related to the medical necessity criteria is **REQUIRED** on all requests for authorizations. Records will be reviewed at the time of submission. Please provide documentation related to diagnosis, step therapy, and clinical markers (i.e. genetic and mutational testing) supporting initiation when applicable. Medical records may be submitted via direct upload through the PA web portal or by fax.

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, casimersen, etc.); **AND**
- 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; **AND**

Duchenne Muscular Dystrophy (DMD) † Φ <sup>1-8,11,13</sup>

- Patient has 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 there is a contraindication 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 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

† FDA Approved Indication(s); ‡ Compendia Recommended Indication(s); Ⓢ Orphan Drug

#### IV. **Renewal Criteria** <sup>1,5,6,13</sup>

Coverage may be renewed based upon the following criteria:

- Patient continues to meet the 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: kidney toxicity (e.g., glomerulonephritis, persistent increase in serum cystatin C, proteinuria, etc.), 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
  - 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

## V. Dosage/Administration <sup>1</sup>

Indication	Dose
Duchenne Muscular Dystrophy	Administer 80 mg/kg intravenously once weekly

## VI. Billing Code/Availability Information

### HCPCS Code:

- J1427 – Injection, viltolarsen, 10 mg; 1 billable unit = 10 mg

### NDC:

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

## VII. References

1. Vilepso [package insert]. Paramus, NJ; NS Pharma, Inc.; January 2023. Accessed April 2024.
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
7. 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.
8. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: respiratory, cardiac, bone health, and orthopaedic management. *Lancet Neurol* 2018; 17:347.

9. Moxley RT 3rd, Ashwal S, Pandya S, et al. Practice parameter: corticosteroid treatment of Duchenne dystrophy: report of the Quality Standards Subcommittee of the American Academy of Neurology and the Practice Committee of the Child Neurology Society. *Neurology*. 2005;64:13–20.
10. Gloss D, Moxley RT 3rd, Ashwal S, Oskoui M. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy: Report of the Guideline Development Subcommittee of the American Academy of Neurology. *Neurology*. 2016 Feb 2;86(5):465-72. Doi: 10.1212/WNL.0000000000002337. Reaffirmed on January 22, 2022.
11. Darras BT, Urion DK, Ghosh PS. Dystrophinopathies. *GeneReviews*. [www.ncbi.nlm.nih.gov/books/NBK1119/](http://www.ncbi.nlm.nih.gov/books/NBK1119/). Initial Posting: September 5, 2000; Last Revision: January 20, 2022. Accessed on March 29, 2024.
12. Childs, AM, Turner, C, Astin, R, et al. (2023). Development of respiratory care guidelines for Duchenne muscular dystrophy in the UK: key recommendations for clinical practice. *thorax*.
13. Landfeldt, Erik. Measuring health-related quality of life in Duchenne muscular dystrophy: Current perspectives and recommendations. *Journal of the Neurological Sciences* 446 (2023).

## 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)

The preceding information is intended for non-Medicare coverage determinations. 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 Determinations (NCDs) and/or Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. Local Coverage Articles (LCAs) may also exist for claims payment purposes or to clarify benefit eligibility under Part B for drugs which may be self-administered. The following link may be used to search for NCD, LCD, or LCA documents: <https://www.cms.gov/medicare-coverage-database/search.aspx>. Additional indications, including any preceding information, may be applied 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)

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	Applicable State/US Territory	Contractor
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
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA
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