CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Marketplace

DRUG NAME	Viltepso (viltolarsen)
BILLING CODE	J3490
BENEFIT TYPE	Medical
SITE OF SERVICE ALLOWED	Home/Office/Outpatient
STATUS	Prior Authorization Required

Viltepso (viltolarsen) is an antisense oligonucleotide 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. This is the second FDA-approved treatment for patients with this specific type of mutation, following the approval of Vyondys (golodirsen). Similar to the other exon-skipping agents, Viltepso has been shown to increase dystrophin production in skeletal muscle. However, the increased levels of dystrophin may or may not result in an improvement of functional outcomes. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.

Viltepso (viltolarsen) will be considered for coverage when the following criteria are met:

DUCHENNE MUSCULAR DYSTROPHY (DMD)

For **initial** authorization:

1. Member has a diagnosis of DMD with confirmed mutation of DMD gene that is amenable to exon 53 skipping (genetic testing results required); AND
2. Medication is being prescribed by or in consultation with a DMD specialist (i.e., neurologist); AND
3. Member is currently stable on corticosteroid for at least 6 months prior to starting therapy with Viltepso, unless not tolerated or contraindicated; AND
4. Chart notes have been provided to show that the member is able to walk independently without assistive devices.
5. **Dosage allowed/Quantity limit:** 80 mg/kg IV weekly.

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:

1. Chart notes must show stability or slowed rate of decline of the member's motor function compared to baseline; AND
2. Chart notes confirm that member remains able to walk independently without assistive devices.

If all the above requirements are met, the medication will be approved for an additional 6 months.

CareSource considers Viltepso (viltolarsen) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
01/06/2021	New policy for Viltepso created.

04/06/2021	Increased duration of steroid trial to 6 months.

References:

1. Viltepso [package insert]. Paramus, NJ; NS Pharma, Inc. August 2020.
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 [published correction appears in doi: 10.1001/jamaneurol.2020.2025]. *JAMA Neurol*. 2020;77(8):982-991.
3. 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 [published correction appears in Lancet Neurol. 2018 Apr 4;:]. *Lancet Neurol*. 2018;17(3):251-267.
4. 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;86(5):465-472.

Effective date: 10/1/2021
Revised date: 04/06/2021