**Viltepso® (viltolarsen)**

| Effective Date: January 1, 2021 | Number: MG.MM.PH.321 |

**Definition**

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**

Max Units (per dose and over time) [Medical Benefit]: 80 mg/kg IV once weekly.
I. **INITIAL APPROVAL CRITERIA**

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

- Medication is being prescribed by or in consultation with a neurologist who specializes in treatment of Duchenne muscular dystrophy (DMD); **AND**
- Patient has been diagnosed with Duchenne muscular dystrophy (DMD); **AND**
- Documentation has been provided confirming mutation of the DMD gene amenable to exon 53 skipping; **AND**
- Member must have been on a stable dose of corticosteroids for at least 3 months (unless contraindicated); **AND**
- 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**
- Viltepso is not used concomitantly with other exon skipping therapies for DMD.

**Limitations/Exclusions**

n/a

II. **RENEWAL CRITERIA**

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

- 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**
- 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**
- Absence of unacceptable toxicity from the drug has been documented. Examples of unacceptable toxicity include reactions (including anaphylaxis), kidney toxicity, etc.; **AND**
- Viltepso is not used concomitantly with other exon skipping therapies for DMD.

**Applicable Procedure Codes**

| C9071 | Injection, viltolarsen; 1 billable unit = 10 mg |

**Applicable NDCs**
Viltepso® (viltolarsen) injection
Effective: January 1, 2021
Page 3 of 3

73292-011-01 Viltepso single-dose vials containing 250 mg/5 mL (50 mg/mL)

Applicable Diagnosis Codes

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<th>Code</th>
<th>Diagnosis</th>
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<tr>
<td>G71.01</td>
<td>Duchenne or Becker muscular dystrophy</td>
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Revision History

<table>
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<tr>
<th>Date</th>
<th>Description</th>
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<tr>
<td>1/1/2021</td>
<td>Updated C-code 9071</td>
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<tr>
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<td>New Medical Policy</td>
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References