

Policy: MBP 226.0

Section: Medical Benefit Pharmaceutical Policy

Subject: Viltepso (viltolarsen)

I. Policy:

Viltepso (viltolarsen)

II. Purpose/Objective:

To provide a policy of coverage regarding Viltepso (viltolarsen)

III. Responsibility:

- A. Medical Directors
- B. Medical Management
- C. Pharmacy Department

IV. Required Definitions

1. Attachment – a supporting document that is developed and maintained by the policy writer or department requiring/authoring the policy.
2. Exhibit – a supporting document developed and maintained in a department other than
3. the department requiring/authoring the policy.
4. Devised – the date the policy was implemented.
5. Revised – the date of every revision to the policy, including typographical and grammatical changes.
6. Reviewed – the date documenting the annual review if the policy has no revisions necessary.

V. Additional Definitions

Medical Necessity or Medically Necessary means Covered Services rendered by a Health Care Provider that the Plan determines are:

- a. appropriate for the symptoms and diagnosis or treatment of the Member's condition, illness, disease or injury;
- b. provided for the diagnosis and the direct care and treatment of the Member's condition, illness disease or injury;
- c. in accordance with current standards good medical treatment practiced by the general medical community;
- d. not primarily for the convenience of the Member, or the Member's Health Care Provider; and
- e. the most appropriate source or level of service that can safely be provided to the Member. When applied to hospitalization, this further means that the Member requires acute care as an inpatient due to the nature of the services rendered or the Member's condition, and the Member cannot receive safe or adequate care as an outpatient

Medicaid Business Segment

Medically Necessary — A service, item, procedure, or level of care compensable under the Medical Assistance program that is necessary for the proper treatment or management of an illness, injury, or disability is one that:

- i. Will, or is reasonably expected to, prevent the onset of an illness, condition, injury or disability.
- ii. Will, or is reasonably expected to, reduce or ameliorate the physical, mental or developmental effects of an illness, condition, injury or disability.
- iii. Will assist the Member to achieve or maintain maximum functional capacity in performing daily activities, taking into account both the functional capacity of the Member and those functional capacities that are appropriate for Members of the same age.

DESCRIPTION:

Viltepso (viltolarsen) is an antisense oligonucleotide that binds to exon 53 of dystrophin premessenger RNA (mRNA), resulting in exclusion of this exon during mRNA processing. Exon 53 skipping allows for production of an internally truncated dystrophin protein in patients with genetic mutations that are amenable to exon 53 skipping.

CRITERIA FOR USE: Requires Prior Authorization by Medical Director or Designee

Viltepso (viltolarsen) will be considered medically necessary for all lines of business when ALL of the following criteria are met:

- Medical record documentation of interdisciplinary team involvement including, at a minimum, neurology, cardiology, pulmonology, and a genetic specialist (e.g. geneticist, genetic counselor, etc.) **AND**
- Medical record documentation of Duchenne's Muscular Dystrophy (DMD) confirmed by genetic testing **AND**
- Medical record documentation that the member has a confirmed mutation of the DMD gene that is amenable to exon 53 skipping confirmed by a genetic counselor **AND**
- Medical record documentation of a baseline evaluation, including a standardized assessment of motor function by a neurologist with experience treating Duchenne muscular dystrophy **AND**
- Medical record documentation that Viltepso is being given concurrently with oral corticosteroids unless intolerant or contraindicated **AND**
- Medical record documentation that patient will receive a dose consistent with the Food and Drug Administration (FDA) approved labeling (maximum dose of 80 mg/kg infused once weekly) **AND**
- Medical record documentation that the patient has not received prior treatment with gene therapy (e.g. Elevidys)* **AND**
- **For Commercial Lines of Business only:**
 - Medical record documentation that the patient is ambulatory (e.g. able to walk with assistance, not wheelchair bound, does not have full-time dependence on motorized wheelchairs or scooters for mobility) as proven by documentation of a 6-Minute Walk Test Distance (6MWT) within the past 3 months of initiation of Viltepso **AND**
 - Medical record documentation that patient does not have a symptomatic cardiac abnormality

AUTHORIZATION DURATION Initial approval will be for 6 months or less if the reviewing provider feels it is medically appropriate. Subsequent approvals will be for an additional 6 months or less if the reviewing provider feels it is medically appropriate and will require medical record documentation of the following:

- Medical record documentation that the member continues to benefit from treatment with viltolarsen **AND**
- Medical record documentation of an annual evaluation, including an assessment of motor function ability, by a neurologist with experience treating Duchenne muscular dystrophy **AND**
- Medical record documentation that Viltepso continues to be given concurrently with oral corticosteroids unless intolerant or contraindicated **AND**
- Medical record documentation that the patient will continue to receive a dose consistent with the Food and Drug Administration (FDA) approved labeling (maximum dose of 80 mg/kg infused once weekly) **AND**
- Medical record documentation that the patient has not received prior treatment with gene therapy (e.g. Elevidys)* **AND**
- **For Commercial Lines of Business only:**
 - Medical record documentation that the patient remains ambulatory (e.g. able to walk with assistance, not wheelchair bound, does not have full-time dependence on motorized wheelchairs or scooters for mobility) as proven by documentation of a follow-up 6-Minute Walk Test Distance (6MWT) within the past 6 months **AND**
 - Medical record documentation that patient does not have a symptomatic cardiac abnormality

*Note: Requests for members that show decline in clinical status following treatment with Elevidys will be reviewed on a case by case basis.

Note: Exon Deletions* on the Duchenne Muscular Dystrophy Gene Theoretically Amenable to Exon 53 Skipping

3-52	4-52	5-52	6-52	9-52					
10-52	11-52	13-52	14-52	15-52	16-52	17-52	19-52		
21-52	23-52	24-52	25-52	26-52	27-52	28-52	29-52		
30-52	31-52	32-52	33-52	34-52	35-52	36-52	37-52	38-52	39-52
40-52	41-52	42-52	43-52	45-52	47-52	48-52	49-52		
50-52	52	54-58	54-61	54-63	54-64	54-66	54-76	54-77	

*The first number represents the first exon deleted. The last number is the last exon deleted. The dash (-) represents all exons in between the first and last exon deleted.

Note: For Medicaid (GHP Family), any requests for services that do not meet criteria set in the PARP will be evaluated on a case-by-case basis.

LINE OF BUSINESS:

Eligibility and contract specific benefit limitations and/or exclusions will apply. Coverage statements found in the line of business specific benefit document will supersede this policy.

REFERENCES:

1. Viltepso [prescribing information]. Paramus, NJ: NS Pharma Inc; January 2023.

This policy will be revised as necessary and reviewed no less than annually.

Devised: 1/19/21

Revised: 4/30/21 (per DHS), 1/17/23 (LOB carve out, Medicaid PARP statement, Medicaid business segment), 12/28/23 (references added), 1/2/24 (gene therapy edit from 12/2023)

Reviewed: 1/18/22

MA UM Committee approval: 12/31/23