Prior Authorization Review Panel

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CHC-MCO Policy Submission

A separate copy of this form must accompany each policy submitted for review.
Policies submitted without this form will not be considered for review.

Plan: PA Health & Wellness	**Submission Date: 02/01/2023**
Policy Number: PA.CP.PHAR.484	**Effective Date: 08/2020** **Revision Date: 01/2023**
Policy Name: Viltolarsen (Viltepso)	

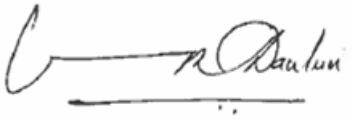
Type of Submission – Check all that apply:

- ☐ **New Policy**
- ✓ **Revised Policy***
- ☐ **Annual Review - No Revisions**
- ☐ **Statewide PDL** - *Select this box when submitting policies for Statewide PDL implementation and when submitting policies for drug classes included on the Statewide PDL.*

***All revisions to the policy must be highlighted using track changes throughout the document.**

Please provide any changes or clarifying information for the policy below:

1Q 2023 annual review: no significant changes; references reviewed and updated.

Name of Authorized Individual (Please type or print): **Venkateswara R. Davuluri, MD**	**Signature of Authorized Individual:** 

Clinical Policy: Viltolarsen (Viltepso)

Reference Number: PA.CP.PHAR.484
Effective Date: 08/2020
Last Review Date: 01/2023

Revision Log

Description
Viltolarsen (Viltepso™) is an antisense oligonucleotide.

FDA Approved Indication(s)
Viltepso is 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.

Limitation(s) of use: 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 of a clinical benefit in a confirmatory trial.

Policy/Criteria
Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of PA Health & Wellness® that Viltepso may be **medically necessary*** when the following criteria are met:

**** Viltepso was FDA-approved based on an observed increase in dystrophin in skeletal muscle, but it is unknown if that increase is clinically significant. Continued FDA-approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.***

I. Requirements for Prior Authorization of Viltepso (viltolarsen)

A. Prescriptions That Require Prior Authorization

All prescriptions for Viltepso (viltolarsen) must be prior authorized.

B. Review of Documentation for Medical Necessity

In evaluating a request for prior authorization of a prescription for Viltepso (viltolarsen), the determination of whether the requested prescription is medically necessary will take into account whether:

1. The beneficiary has a diagnosis that is:
   a. Indicated in the FDA-approved package insert, **OR**

   b. Listed in nationally recognized compendia for the determination of medically-accepted indications for off-label uses for Viltepso (viltolarsen)

AND

2. Viltepso (viltolarsen)is prescribed by or in consultation with a neurologist with experience treating Duchenne muscular dystrophy

AND

3. The beneficiary has documentation of a baseline evaluation, including a standardized assessment of motor function, by a neurologist with experience treating Duchenne muscular dystrophy

AND

4. The beneficiary will receive concurrent corticosteroids unless contraindicated or intolerant

AND

5. Viltepso (viltolarsen) is not prescribed concurrently with other exon-skipping therapies (e.g., Exondys 51™, Vyondys 53™)

OR

6. The beneficiary does not meet the clinical review guidelines listed above, but, in the professional judgment of the physician reviewer, the services are medically necessary to meet the medical needs of the beneficiary.

FOR RENEWALS OF PRESCRIPTIONS FOR Viltepso (viltolarsen) - The determination of medical necessity of requests for prior authorization of renewals of prescriptions for Viltepso (viltolarsen), that were previously approved, will take into account whether:

1. Viltepso (viltolarsen) is prescribed by or in consultation with a neurologist with experience treating Duchenne muscular dystrophy

AND

2. The beneficiary has documentation of an annual evaluation, including an assessment of motor function ability, by a neurologist with experience treating Duchenne muscular dystrophy

AND

3. Based on the prescriber's assessment, the beneficiary continues to benefit from Viltepso (viltolarsen)

AND

4. The beneficiary will receive concurrent corticosteroids unless contraindicated or intolerant

AND

5. Viltepso (viltolarsen) is not prescribed concurrently with other exon-skipping therapies (e.g., Exondys 51™, Vyondys 53™)

OR

6. The beneficiary does not meet the clinical review guidelines listed above, but, in the professional judgment of the physician reviewer, the services are medically necessary to meet the medical needs of the beneficiary.

II. Clinical Review Process

Prior authorization personnel will review the request for prior authorization and apply the clinical guidelines in Section I.B. above, to assess the medical necessity of the request for a prescription for Viltepso (viltolarsen). If the guidelines in Section I.B are met, the reviewer will prior authorize the prescription. If the guidelines are not met, the prior authorization request will be referred to a physician reviewer for a medical necessity determination. Such a request for prior authorization will be approved when, in the professional judgment of the physician reviewer, the services are medically necessary to meet the medical needs of the beneficiary.

III. Approval Duration: 6 months

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

6MWT: 6-minute walk test
DMD: Duchenne muscular dystrophy
FDA: Food and Drug Administration
FVC: forced vital capacity
ICER: Institute for Clinical and Economic Review
LVEF: left ventricular ejection fraction
TTSTAND: time to stand

Appendix B: Therapeutic Alternatives
This table provides a listing of preferred alternative therapy recommended in the approval criteria. The drugs listed here may not be a formulary agent for all relevant lines of business and may require prior authorization.

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
prednisone*	0.3-0.75 mg/kg/day or 10 mg/kg/weekend PO	Based on weight
Emflaza™ (deflazacort)	0.9 mg/kg/day PO QD	Based on weight

Therapeutic alternatives are listed as Brand name® (generic) when the drug is available by brand name only and generic (Brand name®) when the drug is available by both brand and generic.
**Off-label*

Appendix C: Contraindications/Boxed Warnings
None reported

Appendix D: General Information

- Common mutations amenable to exon 53 skipping include: 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, 54-64, 54-66, 54-76, 54-77.
- Corticosteroids are routinely used in DMD management with established efficacy in slowing decline of muscle strength and function (including motor, respiratory, and cardiac). They are recommended for all DMD patients per the American Academy of Neurology (AAN) and DMD Care Considerations Working Group; in addition, the AAN guidelines have been endorsed by the American Academy of Pediatrics, the American Association of Neuromuscular & Electrodiagnostic Medicine, and the Child Neurology Society.
  - The DMD Care Considerations Working Group guidelines, which were updated in 2018, continue to recommend corticosteroids as the mainstay of therapy.
  - In an evidence report published August 2019, the Institute for Clinical and Economic Review (ICER) states that current evidence is insufficient to conclude that other exon-skipping therapies (Exondys 51, Vyondys 53) have net clinical benefit when added to corticosteroids and supportive care versus corticosteroids and supportive care alone.
- Prednisone is the corticosteroid with the most available evidence. A second corticosteroid commonly used is deflazacort, which was FDA approved for DMD in February 2017.
- The phase 2 dose-finding, safety study for viltolarsen (NCT02740972) enrolled male patients age 4-9 years with the lowest 6MWT distance at baseline being 201 m. In addition, inclusion criteria for the ongoing phase 3 efficacy study for viltolarsen (RACER 53; NCT04060199) enrolled male patients age 4-7 years old with a TTSTAND < 10 seconds.
- Having an LVEF below 40% may indicate presence of cardiomyopathy or heart failure, while a predicted FVC below 50% may indicate presence of severe pulmonary disease.

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
DMD	80 mg/kg IV once weekly	80 mg/kg/week

VI. Product Availability

Solution for injection in a single-dose vial: 250 mg/5 mL (50 mg/mL)

VII. References

1. Viltepso Prescribing Information. Paramus, NJ: NS Pharma, Inc.; March 2021. Available at: www.viltepso.com. Accessed November 7, 2022.
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. JAMA Neurol. 2020; 77(8) 982-991.
3. ClinicalTrials.gov. Study to assess the efficacy and safety of viltolarsen in ambulant boys with DMD (RACER53). Available at: https://clinicaltrials.gov/ct2/show/NCT04060199. Accessed November 7, 2022.
4. 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: 251-267.
5. Gloss D, Moxley RT, Ashwal S, Oskoui M. Practice guideline update summary: corticosteroid treatment of Duchenne muscular dystrophy. Neurology. 2016; 86: 465-472. Reaffirmed on January 22, 2022.
6. Institute for Clinical and Economic Review. Deflazacort, eteplirsen, and golodirsen for Duchenne muscular dystrophy: Effectiveness and value. Published August 15, 2019. Available at: https://icer-review.org/material/dmd-final-evidence-report. Accessed November 7, 2022.
7. NS Pharma. Viltepso (viltolarsen) injection: Long-term efficacy and safety data presented at the PPMD 2021 Virtual Annual Conference. Published July 1, 2021. Press release available at: https://www.nspharma.com/pdfs/NSPharma_Long-term_Data_PPMD_New.pdf. Accessed November 7, 2022.

Reviews, Revisions, and Approvals	Date
Policy created	08/2020
1Q 2021 annual review: no significant changes; references reviewed and updated.	01/2021
1Q 2022 annual review: references reviewed and updated.	01/2022
1Q 2023 annual review: no significant changes; references reviewed and updated.	01/2023