Reference number(s)
4835-A

STANDARD MEDICARE PART B MANAGEMENT

VILTEPSO (viltolarsen)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication
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.

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.

All other indications will be assessed on an individual basis. Submissions for indications other than those enumerated in this policy should be accompanied by supporting evidence from Medicare approved compendia.

II. DOCUMENTATION

The following documentation must be available, upon request, for all submissions:
A. Initial requests: laboratory confirmation of Duchenne muscular dystrophy (DMD) diagnosis with a DMD gene mutation that is amenable to exon 53 skipping (refer to examples in Appendix).
B. Continuation of therapy requests: documentation (e.g., chart notes) of response to therapy.

III. PRESCRIBER SPECIALTIES

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of Duchenne muscular dystrophy (DMD).

IV. CRITERIA FOR INITIAL APPROVAL

Duchenne Muscular Dystrophy
Authorization of 6 months may be granted for treatment of DMD when all of the following criteria are met:
A. Genetic testing was conducted to confirm the diagnosis of DMD and to identify the specific type of DMD gene mutation.
B. The DMD gene mutation is amenable to exon 53 skipping (refer to examples in Appendix).
C. Treatment with Viltepso is initiated before the age of 10.
D. Member is able to walk independently without assistive devices.
E. Member will not exceed a dose of 80 mg/kg once weekly.

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F. The requested medication will not be used concomitantly with golodirsen.

V. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must be currently receiving therapy with the requested agent.

Authorization for 12 months may be granted when all of the following criteria are met:
A. The member is currently receiving therapy with Viltepso.
B. Viltepso is being used to treat an indication enumerated in Section IV.
C. The member is receiving benefit from therapy as evidenced by remaining ambulatory (e.g., not wheelchair dependent).
D. The member will not exceed a dose of 80 mg/kg once weekly.
E. The requested medication will not be used concomitantly with golodirsen.

VI. APPENDIX

Examples of DMD gene mutations (exon deletions) amenable to exon 53 skipping (not an all-inclusive list):

1. Deletion of exon 52
2. Deletion of exon 45-52
3. Deletion of exon 47-52
4. Deletion of exon 48-52
5. Deletion of exon 49-52
6. Deletion of exon 50-52

VII. SUMMARY OF EVIDENCE

The contents of this policy were created after examining the following resources:
1. The prescribing information for Viltepso.
2. The available compendium
    a. National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
    b. Micromedex DrugDex
    c. American Hospital Formulary Service- Drug Information (AHFS-DI)
    d. Lexi-Drugs
    e. Clinical Pharmacology

After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for Viltepso are covered.

VIII. EXPLANATION OF RATIONALE

Support for FDA-approved indications can be found in the manufacturer's prescribing information.

IX. REFERENCES

1. Viltepso [package insert]. Paramus, NJ: NS Pharma, Inc.; March 2021.

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2. Watanabe N, Nagata T, Satou Y, et al. NS-065/NCNP-01: An Antisense Oligonucleotide for Potential Treatment of Exon 53 Skipping in Duchenne Muscular Dystrophy. *Mol Ther Nucleic Acids*. 2018;13:442–449. doi:10.1016/j.omtn.2018.09.017.

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