

SPECIALTY GUIDELINE 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

Viltepsa 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 Viltepsa. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.

All other indications are considered experimental/investigational and not medically necessary.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:

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

V. CONTINUATION OF THERAPY

Reference number(s)
4088-A

Authorization of 12 months may be granted for members requesting continuation of therapy when all of the following criteria are met:

- A. The member has demonstrated a response to therapy as evidenced by remaining ambulatory (e.g., not wheelchair dependent).
- B. The member will not exceed a dose of 80 mg/kg once weekly.
- C. 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. REFERENCES

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