Reference number(s)
4088-A

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
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 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:
   1. 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).
   2. If applicable, medical records confirming a worsening in clinical status since receiving gene replacement therapy.
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 meets one of the following criteria:
   1. Member has not previously received gene replacement therapy for DMD (e.g., Elevidys).

2. Member has previously received gene replacement therapy for DMD (e.g., Elevidys) and has experienced a worsening in clinical status since receiving gene replacement therapy (e.g., decline in ambulatory function).

F. Member will not exceed a dose of 80 mg/kg once weekly.
G. The requested medication will not be used concomitantly with golodirsen.

V. CONTINUATION OF THERAPY

Note: Members who were previously established on Viltepso and subsequently administered gene replacement therapy (e.g., Elevidys) must meet all initial criteria prior to re-starting Viltepso.

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
3. Clinical Consult: CVS Caremark Clinical Programs Review. Focus on Neuromuscular Disorders Clinical Programs. September 2020.