New Hampshire Medicaid Fee-for-Service Program

Duchenne Muscular Dystrophy (DMD) Agents Criteria

Approval Date: January 26, 2023

Indications

Eteplirsen (Exondys 51®), an antisense oligonucleotide, is FDA-approved for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. Viltolarsen (Viltepso®) and golodirsen (Vyondys 53®) are also antisense oligonucleotides indicated for the treatment of DMD; in contrast to eteplirsen, these agents are indicated in DMD patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping. Casimersen (Amondys 45™) is an antisense oligonucleotide indicated for the treatment of DMD in patients with a confirmed DMD gene mutation amenable to exon 45 skipping.

Medications

Brand Names	Generic Names	Dosage
Amondys 45™	casimersen	100 mg/2 mL vial
Exondys 51®	eteplirsen	100 mg/2 mL vial; 500 mg/10 mL vial
Viltepso®	viltolarsen	250 mg/5 mL vial
Vyondys 53®	golodirsen	100 mg/2 mL vial

Criteria for Approval

1. Patient must have documentation of a confirmed diagnosis of DMD with genetic testing demonstrating one of the following:
   a. A mutation on the DMD gene that is amenable to exon 45 skipping (for Amondys 45™); **OR**
   b. A mutation on the DMD gene that is amenable to exon 51 skipping (for Exondys 51®); **OR**
   c. A mutation on the DMD gene that is amenable to exon 53 skipping (for Viltepso® or Vyondys 53®); **AND**
2. Patient has been on a stable dose of corticosteroids, unless contraindicated or intolerable,
   a. for ≥ 6 months (Amondys 45™, Exondys 51® or Vyondys 53®); **OR**
   b. for ≥ 3 months (Viltepso®); **AND**

3. Patient retains meaningful voluntary motor function (patient can speak, manipulate objects using upper extremities, ambulate, etc.); **AND**
4. Patient should be receiving physical therapy and/or occupational therapy; **AND**
5. Baseline documentation of ≥ 1 of the following:
    a. Dystrophin level
    b. 6-minute walk test (6WMT) or other timed function tests
    c. Upper limb function (ULM) test
    d. North Star Ambulatory Assessment (NSAA)
    e. Forced Vital Capacity (FVC) % predicted; **AND**
6. For Amondys 45™, Vyondys 53®, and Viltepso®:
    a. Patient serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio has been measured prior to the start of therapy; **AND**
    b. Prescriber attestation that serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio will be measured and during treatment (monthly urine dipstick with serum cystatin C and urine protein-to creatinine ratio every 3 months).
7. For Viltepso®:
    a. Patient does not have symptomatic cardiomyopathy.

Length of Authorization

Initial 6 months, extended approval for 6 months if additional criteria are met.

Criteria for 6-Month Renewal

1. Patient must continue to meet the above criteria; **AND**
2. Patient has demonstrated a response to therapy compared to pretreatment baseline in ≥ 1 of the following (not all-inclusive):
    a. Increase in dystrophin level
    b. Stability, improvement, or slowed rate of decline in 6MWT or other timed function tests
    c. Stability, improvement, or slowed rate of decline in ULM test
    d. Stability, improvement, or slowed rate of decline in NSAA
    e. Stability, improvement, or slowed rate of decline in FVC% predicted
    f. Improvement in quality of life; **AND**
3. Patient has not experienced any treatment-restricting adverse effects (severe hypersensitivity reactions, renal toxicity/proteinuria, etc.).

Criteria for Denial

1. Above criteria are not met; **OR**
2. Patient has unacceptable toxicity from therapy.

References

Available upon request.

Revision History

Reviewed by	Reason for Review	Date Approved
DUR Board	New	12/15/2020
Commissioner Designee	Approval	02/24/2021
DUR Board	Revision	06/08/2021
Commissioner Designee	Approval	08/13/2021
DUR Board	Revision	12/13/2022
Commissioner Designee	Approval	01/26/2023