New Hampshire Medicaid Fee-for-Service Program

Duchenne Muscular Dystrophy (DMD) Agents Criteria

Approval Date: June 10, 2024

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 mutation on the DMD gene that is amenable to exon 45 skipping (for Amondys 45™); **OR**
   - A mutation on the DMD gene that is amenable to exon 51 skipping (for Exondys 51®); **OR**
   - 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,
   - for ≥ 6 months (Amondys 45™, Exondys 51® or Vyondys 53®); **OR**
   - 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 or more of the following:
   - Dystrophin level
   - 6-minute walk test (6WMT) or other timed function tests
   - Upper limb function (ULM) test
   - North Star Ambulatory Assessment (NSAA)
   - Forced Vital Capacity (FVC) % predicted; **AND**
6. For Amondys 45™, Vyondys 53®, and Viltepso®:
   - Patient serum cystatin C, urine dipstick, and urine protein-to-creatinine ratio has been measured prior to the start of therapy; **AND**
   - 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®:
   - 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 or more of the following (not all-inclusive):
   - Increase in dystrophin level
   - Stability, improvement, or slowed rate of decline in 6MWT or other timed function tests
   - Stability, improvement, or slowed rate of decline in ULM test
   - Stability, improvement, or slowed rate of decline in NSAA
   - Stability, improvement, or slowed rate of decline in FVC% predicted
   - 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
DUR Board	Revision	05/07/2024
Commissioner Designee	Approval	06/10/2024