CVS Caremark®

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| Reference number(s) |
| 5257-A |

# Initial Prior Authorization Duchenne Muscular Dystrophy (DMD) Agents New York Medicaid

## Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

| Brand Name | Generic Name |
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| Amondys 45 | casimersen |
| Exondys 51 | eteplirsen |
| Viltepso | viltolarsen |
| Vyondys 53 | golodirsen |

## Indications

### FDA-approved Indications

#### Amondys 45

Amondys 45 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 45 skipping. This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with AMONDYS 45. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

#### Exondys 51

Exondys 51 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 51 skipping. This indication is approved under accelerated approval based on an increase in dystrophin in skeletal muscle observed in some patients treated with EXONDYS 51. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

#### Viltepso

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.

#### Vyondys 53

Vyondys 53 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 Vyondys 53. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

## Coverage Criteria

### Duchenne Muscular Dystrophy (DMD)

Authorization may be granted for the diagnosis of Duchenne muscular dystrophy (DMD) when ALL of the following criteria are met:

* There is documentation of genetic testing to confirm that the DMD gene mutation of the patient is amenable to exon 45, 51, or 53 skipping. [ACTION REQUIRED: Documentation is required for approval.]
* There is documentation to confirm a stable dose of corticosteroids has been established prior to starting therapy or a documented reason that the patient cannot be on corticosteroids. [ACTION REQUIRED: Documentation is required for approval.]
* There is documentation that indicates kidney function testing has been done prior to starting therapy (except for eteplirsen). [ACTION REQUIRED: Documentation is required for approval.]
* The patient is NOT concurrently being treated with another exon skipping therapy for DMD.

## Duration of Approval (DOA)

* 5257-A: DOA: 12 months

## References

1. Amondys 45 [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; July 2024.
2. Exondys 51 [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; December 2024.
3. Viltepso [package insert]. Paramus, NJ: NS Pharma, Inc.; January 2023.
4. Vyondys 53 [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; June 2024.
5. New York – Coverage of Duchenne Muscular Dystrophy (DMD) Drugs. New York State Medicaid Update. January 2022.