Effective Date: 04/1/2022
Last P&T Approval/Version: NA
Next Review Due By: 04/2023
Policy Number: C22327-A

Duchenne Muscular Dystrophy Drugs- NY ONLY

PRODUCTS AFFECTED

Exondys 51 (eteplirsen), Amondys 45 (casimersen), Vyondys 53 (golodirsen), Viltepso (vitolarsen)

COVERAGE POLICY

Coverage for services, procedures, medical devices and drugs are dependent upon benefit eligibility as outlined in the member's specific benefit plan. This Coverage Guideline must be read in its entirety to determine coverage eligibility, if any.
This Coverage Guideline provides information related to coverage determinations only and does not imply that a service or treatment is clinically appropriate or inappropriate. The provider and the member are responsible for all decisions regarding the appropriateness of care. Providers should provide Molina Healthcare complete medical rationale when requesting any exceptions to these guidelines

Documentation Requirements:
Molina Healthcare reserves the right to require that additional documentation be made available as part of its coverage determination; quality improvement; and fraud; waste and abuse prevention processes. Documentation required may include, but is not limited to, patient records, test results and credentials of the provider ordering or performing a drug or service. Molina Healthcare may deny reimbursement or take additional appropriate action if the documentation provided does not support the initial determination that the drugs or services were medically necessary, not investigational or experimental, and otherwise within the scope of benefits afforded to the member, and/or the documentation demonstrates a pattern of billing or other practice that is inappropriate or excessive

DIAGNOSIS:
Duchenne Muscular Dystrophy (DMD)

REQUIRED MEDICAL INFORMATION:
This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. If a drug within this policy receives an updated FDA label within the last 180 days, medical necessity for the member will be reviewed using the updated FDA label information along with state and federal requirements, benefit being administered and formulary preferencing. Coverage will be determined on a case-by case basis until the criteria can be updated through Molina Healthcare, Inc. clinical governance. Additional information may be required on a case-by-case basis to allow for adequate review
A. DUCHENNE MUSCULAR DYSTROPHY
1. Documented diagnosis of Duchenne muscular dystrophy (DMD)
   AND
2. Documentation of genetic testing that confirms DMD gene mutation of the member is amenable to exon 45, 51 or 53 skipping

AND

3. Documentation member is currently stable on an oral corticosteroid regimen for at least 6 months, unless contraindicated or member has experienced clinically significant adverse effects
AND
4. Documentation of Member's weight (in kilograms) and date weight was recorded (weight must be dated no more than 30 days before the request date) AND Recent clinical progress and medical records or chart notes relevant to member's condition

CONTINUATION OF THERAPY: NA

DURATION OF APPROVAL:
Authorization: Indefinite

PRESCRIBER REQUIREMENTS:

None

AGE RESTRICTIONS:
None

QUANTITY:
Exondys 51 (eteplirsen), Amondys 45 (casimersen), Vyondys 53 (golodirsen)- 30 mg/kg administered once weekly as a 35 to 60-minute intravenous (IV) infusion
Viltepso (vitolarsen)- 80 mg/kg by IV infusion once weekly

PLACE OF ADMINISTRATION:
The recommendation is that infused medications in this policy will be for pharmacy or medical benefit coverage administered in a place of service that is a non-hospital facility-based location as per the Molina Health Care Site of Care program.

DRUG INFORMATION

ROUTE OF ADMINISTRATION:
Intravenous

DRUG CLASS:
Muscular Dystrophy Agents

FDA-APPROVED USES:
Exondys 51 (eteplirsen - Indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmedmutation of the DMD gene that is amenable to exon 51 skipping.

COMPENDIAL APPROVED OFF-LABELED USES:
None

APPENDIX

APPENDIX:
None

BACKGROUND AND OTHER CONSIDERATIONS

BACKGROUND:

Muscular dystrophy includes a group of genetic disorders that cause muscle weakness and progressive disability. Duchenne muscular dystrophy (DMD) is the most common and progresses most rapidly.

Duchenne muscular dystrophy (DMD)

- A rare genetic disorder characterized by progressive muscle deterioration and weakness, the most common type of muscular dystrophy. DMD is caused by an absence of dystrophin, a protein that helps keep muscle cells intact.
- An X-chromosome-linked disease recessive disorder caused by mutations (mainly deletions) in the dystrophin gene that lead to an absence or defect in the dystrophin protein, dystrophin is essential for maintenance of myocyte integrity and helps keep muscle cells intact. As males have only one X chromosome, and therefore one single copy of the dystrophin gene, they have a much higher probability of developing DMD. A small number of females are also affected but remain asymptomatic and only rarely present with a mild form of the disease.
- In U.S. estimated prevalence of DMD is 1.51-2.05 per 10,000 boys aged 5-9 years; affected children with DMD typically develop symptoms in early childhood around 3-5 years old, experiencing progressive muscle weakness and deterioration.
- Patients with DMD progressively lose the ability to perform activities independently and usually become non-ambulatory by their early teenage years and require the use of a wheelchair. As the disease progresses, life-threatening heart and respiratory conditions can occur. Patients typically succumb to the disease in their 20s or 30s; however, disease severity and life expectancy vary.
- In absence of treatment, the patient experiences: wheelchair dependence before age 13 years; death around age 20 usually due to cardiac or respiratory failure
- Disease progression in patients with DMD: Scoliosis is frequent after loss of ambulation; risk for cardiomyopathy increases with age in absence of ventilatory intervention

CONTRAINDICATIONS/EXCLUSIONS/DISCONTINUATION:

All other uses of Exondys 51 (eteplirsen), Vyondys 53 (golodirsen), Amondys 45 (casimersen) and Viltepso (viltolarsen) are considered experimental/investigational and therefore, willfollow Molina's Off-Label policy.

OTHER SPECIAL CONSIDERATIONS:

None

CODING/BILLING INFORMATION

Note: 1) This list of codes may not be all-inclusive. 2) Deleted codes and codes which are not effective at the time the service is rendered may not be eligible for reimbursement

HCPCS CODE	DESCRIPTION
J1428	Injection, eteplirsen,10mg
J1427	Injection, viltolarsen, 10 mg
J1426	Injection, casimersen, 10 mg
J1429	Injection, golodirsen, 10 mg

AVAILABLE DOSAGE FORMS:

Exondys 51 SOLN 500MG/10ML (50mg/ml), Exondys 51 SOLN 100MG/2ML (50mg/ml), Viltepso SOLN 250MG/5ML, Amondys (casimersen) IV Solution: Single-dose vials containing 100 mg/2 mL (50 mg/mL), Vyondys 53 (golodirsen) IV Soln 100MG/2ML (50 MG/ML)

REFERENCES

1. Exondys 51 (eteplirsen) [prescribing information]. Cambridge, MA: Sarepta Therapeutics Inc; October 2018.
2. Vyondys 53 (golodirsen) [prescribing information]. Cambridge, MA: Sarepta Therapeutics Inc; February 2021.
3. Amondys 45 (casimersen) [prescribing information]. Cambridge, MA: Sarepta Therapeutics Inc; February 2021.
4. Viltepso (viltolarsen) [prescribing information]. Paramus, NJ: NS Pharma Inc; March 2021.