**MVP Health Care Medical Policy**

**Duchenne Muscular Dystrophy**

**Type of Policy:** **Drug Therapy**
**Prior Approval Date:** **04/01/2024**
**Approval Date:** **11/01/2024**
**Effective Date:** **01/01/2025**

**Drug Requiring Prior Authorization (covered under the pharmacy benefit)**

Emflaza
Deflazacort
Agamree

**Drugs Requiring Prior Authorization (covered under the medical benefit)**

J1428 Exondys 51(eteplirsen)

J1429 Vyondys 53 (golodirsen)

J1427 Viltepso (viltolarsen)

J1426 Amondys 45 (casimersen)

J1413 Elevidys (delandistrogene moxeparvovec-rokl)

Refer to the MVP website for the Medicare Part D formulary for drugs that may be covered under the Part D benefit.

**Overview**

Duchenne muscular dystrophy is caused by a defective gene located on the X chromosome that is responsible for the production of dystrophin. The clinical onset usually occurs between two and three years of age and may include muscle weakness, cardiomyopathy and conduction abnormalities, bone fractures, and scoliosis. Treatment with glucocorticoids such as prednisone and deflazacort is beneficial in the treatment motor function, strength, pulmonary function and reducing the risk of scoliosis.

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. A clinical benefit of EXONDYS 51 has not been established. Continued FDA

approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials. If clinical trials fail to verify clinical benefit, the FDA may initiate proceedings to withdraw approval of the drug.

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. Approximately 8% of the DMD population have this mutation. Continued FDA approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

Viltepso is indicated for the treatment of Duchenne Muscular Dystrophy (DMD) in patients with a confirmed mutation in the DMD gene amenable to exon 53 skipping. Continued FDA approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

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

Elevidys is indicated for the treatment of Duchenne muscular dystrophy (DMD) in ambulatory patients with a confirmed mutation of the DMD gene. This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in trials. Continued FDA approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

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## Indications/Criteria

**A. ALL the following criteria must be met for coverage for Emflaza and Agamree:**

- Diagnosis of Duchenne muscular dystrophy (DMD) confirmed by genetic testing
- Member is 2 years of age or older
- Prescribed by or in consultation with a provider who specialized in the treatment of DMD or neuromuscular disorders

- After a minimum of a 6-month trial of prednisone the member has had at least one of the following intolerable adverse effects (chart notes supporting one of the below must be submitted):
    - Weight gain defined as at least a 10% increase in weight from baseline after 6 months of prednisone therapy
    - Cushingoid appearance
    - Severe psychiatric adverse effects such as aggression, abnormal behavior or mood swings that would necessitate a prednisone dose reduction

**Initial approval** will be for 6 months.

**Extension requests** up to 12 months will be apprved if the member shows a continued benefit to therapy such as:

- increase in muscle strength, pulmonary function tests or timed function tests
- Decrease in adverse effects experienced while receiving prednisone.

**B. Medicaid Variation**

- Medications that are a pharmacy benefit are covered and billed to New York State Fee-For-Service (FFS) program. They are defined as medications that go through a retail or specialty pharmacy, including self administered injectable products. Pharmacy medications are subject to FFS's clinical criteria including (but not limited to) coverage, quantity limit, step therapy, and prior authorization. Pharmacy benefit information can be found here: https://www.emedny.org/info/fullform.pdf
- Requests for Exondys 51, Vyondys 53, Amondys 45 and Viltepso will be reviewed when **ALL** the following criteria are met (based on New York State Department of Health Fee-For-Service criteria):
    - Member must have a diagnosis of Duchenne Muscular Disease (DMD) **AND**
    - Documentation of genetic testing must confirm the DMD gene mutation of the member is amenable to exon 45, 51, or 53 skipping **AND**
    - Documentation must confirm a stable dose of corticosteroids prior to starting therapy or a documented reason not to be on corticosteroids **AND**
    - Documentation indicates kidney function testing prior to starting therapy (except eteplirsen) **AND**
    - Member is not concurrently being treated with another exon skipping therapy for DMD
- Requests for Elevidys will be reviewed

- Member must have a diagnosis of Duchenne Muscular Disease (DMD) **AND**
- Documentation of genetic testing must confirm the DMD gene mutation **AND**
- Confirmation that member is ambulatory **AND**
- Member is at least 4 years old **AND**
- Documentation that member does not have a deletion in exon 8 and/or exon 9 in the DMD gene **AND**
- Member has anti-AAVrh74 total binding antibody titers <1:400 **AND**
- Documentation of liver function, platelet counts and troponin-I assessment prior to starting therapy **AND**
- Member is not concurrently being treated with another exon skipping therapy for DMD

C. Elevidys

- Elevidys may be considered for coverage when all the following criteria are met:
  - Member is at least 4 years old
  - Member has a confirmed diagnosis of Duchenne Muscular Disease (DMD) **AND**
  - Chart notes documenting genetic testing must confirm the DMD gene mutation
  - Confirmation that the member is ambulatory
  - Documentation that the member does not have a deletion in exon 8 and/or exon 9 in the DMD gene **AND**
  - Member has anti-AAVrh74 total binding antibody titers <1:400 **AND**
  - Documentation of liver function, platelet counts and troponin-I assessment prior to starting therapy **AND**
  - Member is not concurrently being treated with another exon skipping therapy for DMD

## Exclusions

- Age, dose, frequency of dosing, and/or duration of therapy outside of FDA approved package labeling Combination therapy with other corticosteroids

- EXONDYS 51 to treat all diagnoses including Duchenne muscular dystrophy, as the clinical benefit, including improved motor function, has not been demonstrated.
- Vyondys 53 to treat all diagnoses including Duchenne muscular dystrophy, as the clinical benefit has not been confirmed.
- Viltepso to treat all diagnoses including Duchenne muscular dystrophy, as the clinical benefit has not been confirmed.
- Amondys 45 to treat all diagnoses including Duchenne muscular dystrophy, as the clinical benefit has not been confirmed.
- Elevidys to treatnon-ambulatory members with Duchenne Muscular Dystrophy, as the clinical benefit has not been confirmed.

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## References


1. Griggs RC, Miller JP, Greenber CR, et al. Efficacy and safety of deflazacort vs prednisone and placebo for Duchenne muscular dystrophy. Neurology 2016; 87:2123-2131
2. Gloss DG, Moxley RT, Ashwal S, Oskoui M. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy. Neurology 2016; 86:465-472
3. Emflaza (deflazacort tablets/suspension). Prescribing Information. South Plainfiled, NJ. PTC Therapeutics
4. Exondys 51 (eteplirsen) injection. Prescribing Information. Cambridge, MA: Sarepta Therapeutics, Inc. September 2016.
5. Viltepso (viltolarsen) injection, for intravenous use. Prescribing Information. Paramus, NJ: NS Pharma. August 2020.
6. Amondys 45 (casimersen) injection. Prescribing Information. Cambridge, MA: Sarepta Therapeutics, Inc. February 2021.
7. New York State Medicaid Update. January 2022. Volume 38: Number 1. Medicaid Fee-For-Service Guidance for Duchenne Muscular Dystrophy Drugs. New York State Medicaid Update - January 2022 Volume 38 - Number 1 (ny.gov)
8. Elevidys. Package Insert - ELEVIDYS (fda.gov). Revised August 2024.
9. Agamree. Package Insert. Santhera Pharmaceuticals. March 2024.


| Member Product | Medical Management Requirements* |
|---|---|
| **New York Products** | |
| HMO | Prior Auth |
| PPO in Plan | Prior Auth |
| PPO OOP | Prior Auth |
| POS in Plan | Prior Auth |
| POS OOP | Prior Auth |

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| Essential Plan | Prior Auth |
| MVP Medicaid Managed Care | Pharmacy benefit carved out to Medicaid FFS, Medical benefit Prior Authorization |
| MVP Child Health Plus | Prior Auth |
| MVP Harmonious Health Care Plan | Pharmacy benefit carved out to Medicaid FFS, Medical benefit Prior Authorization |
| MVP Complete Wellness | Refer to the MVP website for the Medicare Part B and Part D |
| MVP Medicare Preferred Gold HMO POS | Refer to the MVP website for the Medicare Part B and Part D |
| MVP Medicare Secure HMO POS | Refer to the MVP website for the Medicare Part B and Part D |
| MVP Medicare Secure Plus HMO POS | Refer to the MVP website for the Medicare Part B and Part D |
| MVP Medicare WellSelect PPO | Refer to the MVP website for the Medicare Part B and Part D |
| MVP Medicare WellSelect Plus PPO | Refer to the MVP website for the Medicare Part B and Part D |
| MVP Medicare Patriot Plan PPO | Refer to the MVP website for the Medicare Part B and Part D |
| MVP DualAccess D-SNP HMO | Refer to the MVP website for the Medicare Part B and Part D |
| MVP DualAccess Complete D-SNP HMO | Refer to the MVP website for the Medicare Part B and Part D |
| MVP DualAccess Plus D-SNP HMO | Refer to the MVP website for the Medicare Part B and Part D |
| UVM Health Advantage Select PPO | Refer to the MVP website for the Medicare Part B and Part D |
| USA Care PPO | Prior Auth |
| Healthy NY | Prior Auth |
| MVP Premier | Prior Auth |
| MVP Premier Plus | Prior Auth |
| MVP Premier Plus HDHP | Prior Auth |
| MVP Secure | Prior Auth |
| MVP EPO | Prior Auth |
| MVP EPO HDHP | Prior Auth |
| MVP PPO | Prior Auth |
| MVP PPO HDHP | Prior Auth |
| Student Health Plans | Prior Auth |
| ASO | See SPD |
| **Vermont Products** | |
| POS in Plan | Prior Auth |
| POS OOP | Prior Auth |
| MVP Medicare Preferred Gold HMO POS | Refer to the MVP website for the Medicare Part B and Part D |
| MVP Medicare Secure Plus HMO POS | Refer to the MVP website for the Medicare Part B and Part D |
| MVP VT HMO | Prior Auth |
| MVP VT Plus HMO | Prior Auth |
| MVP VT HDHP HMO | Prior Auth |
| MVP VT Plus HDHP HMO | Prior Auth |
| MVP Secure | Prior Auth |
| ASO | See SPD |
| **♦ Note: Prior authorization requirements for HDHP products are the same as the base product (e.g. HDHP HMO auth requirements are the same as listed for HMO).** | |
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***Medical Management Requirements**

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| Prior Auth | Prior Authorization Required |
| Potential for Retrospective Review | No Prior Authorization Required. May be subject to Retrospective Review. |
| Retro Review | Retrospective Review Required |
| Not Covered | Service is not a covered benefit. |
| See SPD | See Specific Plan Design |