



MVP Health Care Medical Policy

Duchenne Muscular Dystrophy

Type of Policy: Drug Therapy
Prior Approval Date: 11/01/2023
Approval Date: 04/01/2024
Effective Date: 04/01/2024

Drug Requiring Prior Authorization (covered under the pharmacy benefit)

Emflaza
Deflazacort

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.

Indications/Criteria

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

- Diagnosis of Duchenne muscular dystrophy (DMD) confirmed by genetic testing
- Patient 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 granted if the member shows all the following:

- Clinical benefit 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 Viltepsso 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 aged 4 through 5 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 indicated liver function, platelet counts and troponin-I prior to starting therapy **AND**
- Patient is not concurrently being treated with another exon skipping therapy for DMD

Exclusions

- Dosing, age, and/or frequency outside of the 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 treat all diagnoses including Duchenne muscular dystrophy, as the clinical benefit has not been confirmed.

References

1. Nayak S, Acharjya. Deflazacort versus other glucocorticoids: A comparison. *Indian J Dermatology*. 2008;53(4):167-170
2. 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

3. Gloss DG, Moxley RT, Ashwal S, Oskoui M. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy. *Neurology* 2016; 86:465-472
4. Emflaza (deflazacort tablets/suspension). Prescribing Information. South Plainfield, NJ. PTC Therapeutics
5. Exondys 51 (eteplirsen) injection. Prescribing Information. Cambridge, MA: Sarepta Therapeutics, Inc. September 2016.
6. Viltepso (viltolarsen) injection, for intravenous use. Prescribing Information. Paramus, NJ: NS Pharma. August 2020.
7. Amondys 45 (casimersen) injection. Prescribing Information. Cambridge, MA: Sarepta Therapeutics, Inc. February 2021.
8. 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 \(ny.gov\)](#)
9. Elevidys. [Package Insert - ELEVIDYS \(fda.gov\)](#). Revised 10/2023.

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
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 Medicare Gold Giveback	Refer to the MVP website for the Medicare Part B and Part D policies.
MVP Medicare Preferred Gold HMO POS	Refer to the MVP website for the Medicare Part B and Part D policies.
MVP Medicare Secure HMO POS	Refer to the MVP website for the Medicare Part B and Part D policies.
MVP Medicare Secure Plus HMO POS	Refer to the MVP website for the Medicare Part B and Part D policies.
MVP Medicare WellSelect PPO	Refer to the MVP website for the Medicare Part B and Part D policies.
MVP Medicare WellSelect Plus PPO	Refer to the MVP website for the Medicare Part B and Part D policies.
MVP Medicare Patriot Plan PPO	Refer to the MVP website for the Medicare Part B and Part D policies.
MVP DualAccess D-SNP HMO	Refer to the MVP website for the Medicare Part B and Part D policies.
MVP DualAccess Complete D-SNP HMO	Refer to the MVP website for the Medicare Part B and Part D policies.
MVP DualAccess Plus D-SNP HMO	Refer to the MVP website for the Medicare Part B and Part D policies.
UVM Health Advantage Select PPO	Refer to the MVP website for the Medicare Part B and Part D policies.
UVM Health Advantage Secure PPO	Refer to the MVP website for the Medicare Part B and Part D policies.

UVM Health Advantage Preferred PPO	Refer to the MVP website for the Medicare Part B and Part D policies.
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
UVM Health Advantage Select PPO	Refer to the MVP website for the Medicare Part B and Part D
UVM Health Advantage Secure PPO	Refer to the MVP website for the Medicare Part B and Part D
UVM Health Advantage Preferred PPO	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
<p>♦ 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).</p> <p>© 2024 MVP Health Plan, Inc. All rights reserved. Descriptions contained within MVP's Medical Policies are not a guarantee of coverage. Each MVP Group or Subscriber Contract contains specific limitations, exclusions and requirements that may affect a Policy. If there is any discrepancy between your Group or Subscriber Contract and a Policy, your Group or Subscriber Contract shall in all cases govern.</p>	

***Medical Management Requirements**

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