BOSTON MEDICAL CENTER HEALTHNet PLAN

bmchp.org | 888-566-0008

Pharmacy Policy

Duchenne Muscular Dystrophy Agents

Policy Number: 9.302
Revision Number: 2.0
Version Effective Date: 9/1/2021

Product Applicability	☐ **All Plan+ Products**
Well Sense Health Plan ☐ New Hampshire Medicaid ☐ _____	**Boston Medical Center HealthNet Plan** ☒ MassHealth - MCO ☒ MassHealth - ACO ☐ Qualified Health Plans/ConnectorCare/Employer Choice Direct ☐ Senior Care Options ☐ _____

Note: Disclaimer and audit information is located at the end of this document.

Prior Authorization Policy

Products Affected:

- Emflaza (deflazacort)
- Exondys 51 (eteplirsen)
- Vyondys 53 (golodirsen)
- Viltepso (viltolarsen)
- Amondys 45 (casimersen)

The Plan may authorize coverage of the above products for members meeting the following criteria:

Covered Use	All FDA approved indications not otherwise excluded

+ *Plan* refers to Boston Medical Center Health Plan, Inc. and its affiliates and subsidiaries offering health coverage plans to enrolled members. The Plan operates in Massachusetts under the trade name Boston Medical Center HealthNet Plan and in other states under the trade name Well Sense Health Plan.

Required Medical Information	**<u>Emflaza:</u>** 1. Member has a diagnosis of Duchenne Muscular Dystrophy (DMD) confirmed by genetic testing (medical records required); ***AND*** 2. Member has tried prednisone for ≥ 6 months and has had a significant intolerable adverse effects (Cushingoid appearance, Central (truncal) obesity, Undesirable weight gain defined as a ≥ 10% of body weight gain increase over a 6-month period , Diabetes and/or hypertension that is difficult to manage or a severe behavioral AE while on prednisone therapy that has or would require a prednisone dose reduction); ***AND*** 3. Dosing is appropriate (about 0.9 mg/kg/day once daily); ***AND*** 4. Serum creatinine kinase activity at least 10 times the upper limit of normal (ULN) prior to initiating treatment **<u>Exondys 51:</u>** 1. Member has a diagnosis of Duchenne Muscular Dystrophy (DMD) with confirmed mutation of the DMD gene amenable to exon 51 skipping (medical records required); ***AND*** 2. Member is ambulatory (not wheelchair dependent); ***AND*** 3. Member meets ALL of the following: • Documentation of a pre-treatment baseline 6-Minute Walk Test greater than or equal to 300 meters while walking independently • Stable pulmonary function with predicted forced vital capacity (FVC) equal to or greater than 50% • Stable cardiac function with left ventricular ejection fraction (LVEF) of greater than 50%; ***AND*** 4. Member has been receiving a stable dose of corticosteroids for at least 6 months prior to therapy; ***AND*** 4. Dosing is appropriate (30mg/kg intravenously weekly) **<u>Vyondys 53:</u>** 1. Member has a diagnosis of Duchenne Muscular Dystrophy (DMD) with confirmed mutation of the DMD gene amenable to exon 53 skipping (medical records required); ***AND*** 2. Member is ambulatory (not wheelchair dependent) ***AND*** 3. Members meets ALL of the following: • Documentation of a pre-treatment baseline 6-Minute Walk Test greater than or equal to 250 meters while walking independently

- Stable pulmonary function with predicted forced vital capacity (FVC) equal to or greater than 50%
- Stable cardiac function with left ventricular ejection fraction (LVEF) of greater than 50%; ***AND***

4. Member has been receiving a stable dose of corticosteroids for at least 6 months prior to therapy ***AND***
5. Dosing is appropriate (30mg/kg intravenously weekly)

Amondys 45

1. Member has a diagnosis of Duchenne Muscular Dystrophy (DMD) with confirmed mutation of the DMD gene amenable to exon 45 skipping (medical records required); ***AND***

2. Member is ambulatory (not wheelchair dependent); ***AND***

3. Member meets ALL of the following (medical records required) :

- Documentation of a pre-treatment baseline 6-Minute Walk Test greater than or equal to 300 meters while walking independently
- Stable pulmonary function with predicted forced vital capacity (FVC) equal to or greater than 50%
- Stable cardiac function with left ventricular ejection fraction (LVEF) of greater than 50%; ***AND***

5. Member has been receiving a stable dose of corticosteroids for at least 6 months prior to therapy; ***AND***

6. Dosing is appropriate (30mg/kg intravenously weekly)

Viltepso:

1. Member has a diagnosis of Duchenne Muscular Dystrophy (DMD) with confirmed mutation of the DMD gene amenable to exon 53 skipping (medical records required); ***AND***
2. Member is ambulatory (not wheelchair dependent); ***AND***
3. Following baseline tests have been completed (medical records required): (medical records required):

- Time to stand (TTSTAND) less than 10 seconds
- Time to run/walk (TTRW)
- 6 minute walk test (6MWT)
- Time to climb (TTCLIMB); ***AND***

4. Member has been receiving a stable dose of corticosteroids for at least 3 months prior to therapy; ***AND***

	5. Dosing is appropriate (80mg/kg intravenously weekly)
Age Restriction	Emflaza: 2 years of age and older
Prescriber Restriction	Prescribed by or in consultation with a physician who specializes in treatment of Duchenne Muscular Dystrophy (e.g. neuromuscular neurologist)
Coverage Duration	Initial : 3 months Reauthorization: 12 months
Other criteria	**Emflaza:** 1. Member has a diagnosis of Duchenne Muscular Dystrophy (DMD) confirmed with genetic testing (medical records required); ***AND*** 2. The clinical condition has improved or stabilized since start of therapy without treatment related side effects that were present with prednisone use; ***AND*** 3. Member remains ambulatory (e.g., able to walk with or without assistance, not wheelchair dependent) **Exondys:** ***1.*** Member has a diagnosis of Duchenne Muscular Dystrophy (DMD) with confirmed mutation of the DMD gene amenable to exon 51 skipping (medical records required); ***AND*** 2. Member is responding positively to therapy as shown by ALL of the following (medical records required): • 6-Minute Walk Test greater than or equal to 300 meters • Cardiac function with left ventricular ejection fraction (LVEF) of greater than 50% • Pulmonary function with predicted forced vital capacity (FVC) equal to or greater than 50%; ***AND*** 3. Member continues to use oral corticosteroid unless contraindicated; ***AND*** 4. Dosing is appropriate (30mg/kg intravenously weekly) **Vyondys:** 1. Member has a diagnosis of Duchenne Muscular Dystrophy (DMD) with confirmed mutation of the DMD gene amenable to exon 53 skipping (medical records required); ***AND*** 2. Member is responding positively to therapy as shown by ALL of the following (medical records required): • 6-Minute Walk Test greater than or equal to 250 meters

- Cardiac function with left ventricular ejection fraction (LVEF) of greater than 50%
- Pulmonary function with predicted forced vital capacity (FVC) equal to or greater than 50%; ***AND***

3. Member continues to use oral corticosteroid unless contraindicated; ***AND***
4. Dosing is appropriate (30mg/kg intravenously weekly)

Amondys 45:

1. Member has a diagnosis of Duchenne Muscular Dystrophy (DMD) with confirmed mutation of the DMD gene amenable to exon 45 skipping (medical records required); ***AND***
2. Member is responding positively to therapy as shown by ALL of the following (medical records required):
    - 6-Minute Walk Test greater than or equal to 300 meters
    - Cardiac function with left ventricular ejection fraction (LVEF) of greater than 50%
    - Pulmonary function with predicted forced vital capacity (FVC) equal to or greater than 50%; ***AND***
3. Member continues to use oral corticosteroid unless contraindicated; ***AND***
4. Dosing is appropriate (30mg/kg intravenously weekly)

Viltepso:

1. Member has a diagnosis of Duchenne Muscular Dystrophy (DMD) with confirmed mutation of the DMD gene amenable to exon 53 skipping (medical records required); ***AND***
2. Member's clinical condition has improved or stabilized based on performance on at least two of the following function tests (medical records required):
    - time to stand (TTSTAND)
    - Time to run/walk (TTRW)
    - 6 minute walk test (6MWT)
    - Time to climb (TTCLIMB); ***AND***

3. Member continues to use oral corticosteroid unless contraindicated; ***AND***

4. Dosing is appropriate (80mg/kg intravenously weekly)

Applicable coding:

Code	Medication
J1428	Injection eteplirsen, 10 mg
J1429	Injection golodirsen (Vyondys 53)

Clinical Background Information and References

1. Exondys 51 (eteplirsen) [prescribing information]. Cambridge, MA; Sarepta Therapeutics, Inc: September 2016.
2. Bushby K, Finkel R, Birnkrant DJ, et al for the DMD Care Considerations Working Group. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. *Lancet Neurol*. 2010a; 9(1):77-93.
3. Darras BT. Clinical features and diagnosis of Duchenne and Becker muscular dystrophy. In: Bashe JF (Ed). UpToDate [database on the internet]. Waltham (MA): UpToDate; 2016 [cited 2016 Oct 13]. Available from: http://www.utdol.com/utd/index.do
4. Gloss D, Moxley RT 3rd, Ashwal S, Oskoui M. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy: Report of the Guideline Development Subcommittee of the American Academy of Neurology. Neurology. 2016 Feb 2;86(5):465-72. doi: 10.1212/WNL.0000000000002337.
5. Sarepta Therapeutics. An Open-Label, Multi-Center Study to Evaluate the Safety and Tolerability of Eteplirsen in Early Stage Duchenne Muscular Dystrophy. NLM Identifier: NCT02420379. Last Updated on March 07, 2016. Available at: https://clinicaltrials.gov/ct2/show/NCT02420379?term=02420379&rank=1.
6. Sarepta Therapeutics. An Open-Label, Multi-Center Study to Evaluate the Safety and Tolerability of Eteplirsen in Patients With Advanced Stage Duchenne Muscular Dystrophy. NLM Identifier: NCT02286947. Last Updated on March 04, 2016. Available at: https://clinicaltrials.gov/ct2/show/NCT02286947?term=NCT02286947&rank=1.
7. Sarepta Therapeutics. Confirmatory Study of Eteplirsen in DMD Patients (PROMOVI). NLM Identifier: NCT02255552. Last Updated on August 16, 2016. Available at:https://www.clinicaltrials.gov/ct2/show/nct02255552?term=eteplirsen&rank=4.
8. Product Information: EMFLAZA(TM) oral tablets, suspension, deflazacort oral tablets, suspension. Marathon Pharmaceuticals LLC (per FDA), Northbrook, IL, 2017.
9. Marathon Pharmaceuticals, LLC. http://marathonpharma.com/news/2017/02/emflaza-deflazacort-just-fda-approved/. Published February 10, 2017. Accessed June 12, 2017.
10. Fda.gov/drugs/drug-approvals-and-databases/drug-trials-snapshots-vyondys-53
11. Vyonzys 53 (golodiseron) [prescribing information]. Cambridge, MA. Sarepta Inc. December 2019.
12. Viltepso (viltolarsen) [prescribing information]. Paramus, NJ. NS Pharma, Inc. August 2020.
13. Amondys 45 (casimersen) [prescribing information]. Cambridge, MA. Sarepta Inc. August 2020.

Original Approval Date	Original Effective Date	Policy Owner	Approved by
12/1/2020	1/1/2021	Pharmacy Services	Pharmacy & Therapeutics (P&T) Committee, NH DHHS

Policy Revisions History			
Review Date	**Summary of Revisions**	**Revision Effective Date**	**Approved by**
12/1/2020	P&T Committee: Discontinued policy 9.072 and created a separate policy for NH	1/1/2021	P&T Committee NH DHHS
5/13/2021	P&T Committee: Updated initial criteria for Vyondys, Exondys and Emflaza; added drug specific reauth criteria for Vyondys, Exondys and Emflaza; added new drugs Viltepso and Amondys to formulary with PA	9/1/2021	P&T Committee

Next Review Date

2/2022

Other Applicable Policies

Reference to Applicable Laws and Regulations, If Any

Disclaimer Information

Medical Policies are the Plan's guidelines for determining the medical necessity of certain services or supplies for purposes of determining coverage. These Policies may also describe when a service or supply is considered experimental or investigational, or cosmetic. In making coverage decisions, the Plan uses these guidelines and other Plan Policies, as well as the Member's benefit document, and when appropriate, coordinates with the Member's health care Providers to consider the individual Member's health care needs.

Plan Policies are developed in accordance with applicable state and federal laws and regulations, and accrediting organization standards (including NCQA). Medical Policies are also developed, as appropriate, with consideration of the medical necessity definitions in various Plan products, review of current literature, consultation with practicing Providers in the Plan's service area who are medical experts in the particular field, and adherence to FDA and other government agency policies. Applicable state or federal mandates, as well as the Member's benefit document, take precedence over these guidelines. Policies are reviewed and updated on an annual basis, or more frequently as needed. Treating providers are solely responsible for the medical advice and treatment of Members.

The use of this Policy is neither a guarantee of payment nor a final prediction of how a specific claim(s) will be adjudicated. Reimbursement is based on many factors, including member eligibility and benefits on the date of service; medical necessity; utilization management guidelines (when applicable); coordination of benefits; adherence with applicable Plan policies and procedures; clinical coding criteria; claim editing logic; and the applicable Plan – Provider agreement.