CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Marketplace

DRUG NAME	Emflaza (deflazacort)
BILLING CODE	Must use valid NDC code
BENEFIT TYPE	Pharmacy
SITE OF SERVICE ALLOWED	Home
STATUS	Prior Authorization Required

Emflaza is a corticosteroid initially approved by the FDA in 2017. It is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older. Deflazacort is an oxazoline derivative of prednisone. The effectiveness of Emflaza for the treatment of DMD was established in Study 1. In Study 1, efficacy was evaluated by assessing the change between Baseline and Week 12 in average strength of 18 muscle groups. The change in average muscle strength score between Baseline and Week 12 was significantly greater for the deflazacort 0.9 mg/kg/day dose group than for the placebo group.

Emflaza (deflazacort) will be considered for coverage when the following criteria are met:

DUCHENNE MUSCULAR DYSTROPHY (DMD)

For **initial** authorization:

1. Member must be 2 years of age or older; AND
2. Medication is being prescribed by or in consultation with a DMD specialist (i.e., neurologist); AND
3. Member has a confirmed diagnosis of Duchenne Muscular Dystrophy (DMD) with evidence of dystrophin gene mutation (genetic testing result required); AND
4. Member has documented trial and failure of prednisone for at least 6 months.
5. Member's weight from the last 30 days provided.
6. **Dosage allowed/Quantity limit:** 0.9 mg/kg/day once daily (Quantity Limit: 6mg tablets - 60 tablets per 30 days, 18 mg tablets - 30 tablets per 30 days, 30mg tablets - 90 tablets per 30 days, 36 mg tablets - 90 tablets per 30 days, 22.75mg/mL suspension - 9 bottles (117 mL) per 30 days)

If all the above requirements are met, the medication will be approved for 3 months.

For **reauthorization**:

1. Chart notes must show stability or slowed rate of decline of the member's motor function and muscle strength.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Emflaza (deflazacort) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION

05/15/2017	New policy for Emflaza created.
07/25/2019	Age coverage expanded from 5 years of age and older to 2 years of age and older.
01/15/2021	Added quantity limit for oral suspension. Removed serum CK requirement. Removed onset of weakness before 5 years of age, added must have genetic testing to confirm dystrophin gene mutation. Removed MRC score requirement in initial and reauth. Added that member must show stability or slowed rate of decline of motor function/muscle strength for reauth.
03/02/2022	Added weight requirement to ensure appropriate dosing.

References:

1. Emflaza [package insert]. Northbrook, IL; Marathon Pharmaceuticals, LLC: June, 2019.
2. Griggs RC, Miller JP, Greenberg CR, et al. Efficacy and safety of deflazacort vs prednisone and placebo for Duchenne muscular dystrophy. *Neurology*. 2016;87(20):2123-2131.
3. McDonald CM, Henricson EK, Abresch RT, et al. Long-term effects of glucocorticoids on function, quality of life, and survival in patients with Duchenne muscular dystrophy: a prospective cohort study. *Lancet*. 2018;391(10119):451-461.
4. Bello L, Gordish-Dressman H, Morgenroth LP, et al. Prednisone/prednisolone and deflazacort regimens in the CINRG Duchenne Natural History Study. *Neurology*. 2015;85(12):1048-1055.
5. 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;86(5):465-472.
6. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management [published correction appears in Lancet Neurol. 2018 Apr 4;:]. Lancet Neurol. 2018;17(3):251-267.
7. Ciafaloni E, Kumar A, Liu K, et al. Age at onset of first signs or symptoms predicts age at loss of ambulation in Duchenne and Becker Muscular Dystrophy: Data from the MD STARnet. *J Pediatr Rehabil Med*. 2016;9(1):5-11.

Effective date: 07/01/2022
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