

## PHARMACY POLICY STATEMENT North Carolina Marketplace

<b>DRUG NAME</b>	<b>Emflaza (deflazacort)</b>
BENEFIT TYPE	Pharmacy
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.

DMD is an X-linked, progressive disease characterized by muscle wasting, weakness, loss of walking ability, and reduced life expectancy. It is caused by mutations in the dystrophin gene resulting in reduced or near absence of dystrophin, a protein that helps keep muscle cells intact. Delaying loss of ambulation is a major goal of treatment. Corticosteroids are standard of care to improve muscle strength and function in DMD and may prolong walking ability.

The effectiveness of Emflaza for the treatment of DMD was established in Study 1, in which 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 was associated with less weight gain than prednisone.

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

### Duchenne Muscular Dystrophy (DMD)

For **initial** authorization:

1. Member is at least 2 years of age; AND
2. Medication is being prescribed by or in consultation with a DMD specialist (i.e., neurologist or neuromuscular specialist); AND
3. Member has a diagnosis of DMD confirmed by genetic testing that shows *DMD* gene loss-of-function variation, or absence of muscle dystrophin on muscle biopsy; AND
4. Member has documentation of trial and failure of prednisone for at least 6 months; AND
5. Member's weight is documented in chart notes.
6. **Dosage allowed/Quantity limit:** 0.9 mg/kg/day once daily by mouth.

***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.
11/27/2023	Updated genetic test wording and allow biopsy as alternative diagnostic method. Removed requirement that weight must be within the last 30 days. Removed QL's due to complexity. Added reference.

References:

1. Emflaza [package insert]. PTC Therapeutics, Inc.; 2021.
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 3<sup>rd</sup>, 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.
8. Matthews E, Brassington R, Kuntzer T, Jichi F, Manzur AY. Corticosteroids for the treatment of Duchenne muscular dystrophy. *Cochrane Database Syst Rev*. 2016;2016(5):CD003725. Published 2016 May 5. doi:10.1002/14651858.CD003725.pub4

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