

Market Applicability							
Market	DC	GA	KY	MD	NJ	NY	WA
Applicable	X	X	X	X	X	X	NA

## Emflaza (deflazacort)

Override(s)	Approval Duration
Prior Authorization	Initial Approval Duration: 6 months Subsequent Approval Duration: 12 months

Medications
Emflaza (deflazacort)

### APPROVAL CRITERIA

Requests for initial therapy with Emflaza (deflazacort) may be approved when the following criteria are met:

- I. Individual is 2 years of age or older; **AND**
- II. Individual has a diagnosis of Duchenne Muscular Dystrophy (DMD); **AND**
- III. Individual has had a 6 month trial of oral prednisone (AAN 2016, DrugPoints B, IIa); **AND**
- IV. One of the following:
  - A. Documentation has been provided regarding the presence of clinically significant neuropsychiatric side effects while on prednisone (such as but not limited to aggression); **AND**
  - B. Neuropsychiatric side effects are likely to be the direct result of prednisone use;

**OR**

  - C. Documentation has been provided for excessive weight-gain with prednisone (increase of >0.5 Z score from prior growth curve expectations [American Academy of Pediatrics/CDC Weight for Age Growth Chart\*; Z-score data files, CDC, Weight-for-age charts, 2 to 20 years, selected weight z-scores in kilograms, by sex and age\*\*]); **AND**
  - D. Weight gain is likely to be a direct result of prednisone use.

Requests for continuation of therapy with Emflaza (deflazacort) may be approved when one of the following criteria are met:

- I. When approved due to excessive weight gain with prednisone, individual has experienced a return to baseline growth curve expectations or remained on the same

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New Program Date 04/03/2017

This policy does not apply to health plans or member categories that do not have pharmacy benefits, nor does it apply to Medicare. Note that market specific restrictions or transition-of-care benefit limitations may apply.

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growth curve that was in effect when Emflaza (deflazacort) was initiated (American Academy of Pediatrics/CDC Weight for Age Growth Chart\*; Z-score data files, CDC, Weight-for-age charts, 2 to 20 years, selected weight z-scores in kilograms, by sex and age\*\*); **OR**

- II. When approved due to neuropsychiatric side effects while on prednisone, individual has shown improvement in neuropsychiatric symptoms.

Requests for continuation of therapy with Emflaza (deflazacort) for individuals who had previously received the agent via the manufacturer prior to FDA approval and commercial availability may be approved if the following are met:

- I. Individual has been using the medication for at least 90 days; **AND**
- II. Individual has shown improvement compared to the predicted natural history trajectory of disease.

\* American Academy of Pediatrics/CDC Weight for Age Growth Chart:  
<https://www.cdc.gov/growthcharts/data/set1clinical/cj41c021.pdf>

\*\* Z-score data files, CDC, Weight-for-age charts, 2 to 20 years, selected weight z-scores in kilograms, by sex and age: <https://www.cdc.gov/growthcharts/data/zscore/zwtage.xls>; this file contains the z-score values for the z-scores of -2, -1.5, -1, -0.5, 0, 0.5, 1, 1.5 and 2 by sex (1 = male; 2 = female) and half month of age. For example, 1.5 months represents 1.25-1.75 months. Information needed: age in months, weight in kilograms, and gender.

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Applicable	X	X	X	X	X	X	NA

State Specific Mandates		
State name	Date effective	Mandate details (including specific bill if applicable)
N/A	N/A	N/A

#### **Key References:**

1. Clinical Pharmacology [database online]. Tampa, FL: Gold Standard, Inc.: 2019. URL: <http://www.clinicalpharmacology.com>. Updated periodically.
2. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. <http://dailymed.nlm.nih.gov/dailymed/about.cfm>. Accessed: June 18, 2019.
3. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
4. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc.; 2019 Updated periodically.
5. Balaban B, Matthews DJ, Clayton GH, Carry T. Corticosteroid treatment and functional improvement in Duchenne muscular dystrophy: Long-term effect. *Phys Med Rehabil.* 2005; 34:843-850.
6. Bushby K, Finkel R, Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. *Lancet Neurol.* 2010; 9(1): 77-93.
7. CDC Growth Charts. 2 to 20 years: Buys Stature-for-age and Weight-for-age percentiles. Available from: <https://www.cdc.gov/growthcharts/data/set1clinical/cj41c021.pdf>. Accessed June 18, 2019.
8. Gloss D, Moxley III RT, 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;86(5):465-72.
9. Griggs RC. Efficacy and safety of deflazacort vs prednisone and placebo for Duchenne muscular dystrophy. *Neurology.* 2016 Aug 26. pii: 10.1212/WNL.0000000000003217. [Epub ahead of print]
10. Matthews E, Brassington R, Kuntzer T, Jichi F, Manzur AY. Corticosteroids for the treatment of Duchenne muscular dystrophy. *Cochrane Database Syst Rev.* 2016 May 5;(5):CD003725.
11. National Center for Health Statistics, Z-score Data Files. Available from: <https://www.cdc.gov/growthcharts/zscore.htm>. Accessed June 18, 2019.

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