

Emflaza (deflazacort)

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

Medications
Emflaza (deflazacort)

APPROVAL CRITERIA

Initial therapy requests for Emflaza (deflazacort) may be approved when the following criteria are met:

- I. Individual is 2 years of age or older; **AND**
- II. Documentation is provided that 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; **AND**
- V. Requested medication has been prescribed by, or in consultation with, a board-certified neurologist who specializes in Duchenne Muscular Dystrophy.

Continuation of therapy requests for Emflaza (deflazacort) may be approved when one of the following criteria are met:

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

* 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.

Key References:

1. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. <http://dailymed.nlm.nih.gov/dailymed/about.cfm>.
2. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
3. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc. Updated periodically.
4. 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.
5. 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.
6. 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>.
7. 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.
8. 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]
9. 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.
10. National Center for Health Statistics, Z-score Data Files. Available from: <https://www.cdc.gov/growthcharts/zscore.htm>.

Federal and state laws or requirements, contract language, and Plan utilization management programs or policies may take precedence over the application of this clinical criteria.

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