CVS Caremark®

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| Reference number(s) |
| 1636-A |

# Specialty Guideline Management deflazacort-Emflaza

## Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

| Brand Name | Generic Name |
| --- | --- |
| Emflaza | deflazacort |

## Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

### FDA-Approved Indications1,7-8

Emflaza is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.

Deflazacort (generic) is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 5 years of age and older.

All other indications are considered experimental/investigational and not medically necessary.

## Documentation

Submission of the following information is necessary to initiate the prior authorization review:

* Laboratory confirmation of DMD diagnosis by genetic testing or muscle biopsy.
* Chart documentation of weight gain/obesity or persistent psychiatric/behavioral issues with previous prednisone or prednisolone treatment.

## Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of Duchenne muscular dystrophy (DMD).

## Coverage Criteria

### Duchenne Muscular Dystrophy1-9

Authorization of 6 months may be granted for treatment of DMD when all of the following criteria are met:

* The diagnosis of DMD was confirmed by one of the following criteria:
  + Genetic testing demonstrating a mutation in the DMD gene.
  + Muscle biopsy demonstrating absent dystrophin.
* Member is 2 years of age or older.
* Member has tried prednisone or prednisolone and experienced unmanageable and clinically significant weight gain/obesity or psychiatric/behavioral issues (e.g., abnormal behavior, aggression, irritability):
  + For weight gain/obesity: body mass index is in the overweight or obese category while receiving treatment with prednisone or prednisolone (refer to Appendix for weight status categories for children and adults).

## Continuation of Therapy

Authorization of 12 months may be granted for members requesting continuation of therapy when all of the following criteria are met:

* The member meets all requirements in the coverage criteria section.
* The member is receiving a clinical benefit from therapy with the requested medication (e.g., improvement or stabilization of muscle strength or pulmonary function).

## Appendix

### Body Mass Index Percentile and Weight Status Category for Children 2 Through 19 Years of Age

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| --- | --- |
| Body Mass Index Percentile Range | Weight Status |
| Less than the 5th percentile | Underweight |
| 5th percentile to less than the 85th percentile | Healthy Weight |
| 85th to less than the 95th percentile | Overweight |
| Equal to or greater than the 95th percentile | Obese |

### Body Mass Index and Weight Status Category for Adults (20 Years of Age and Older)

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| --- | --- |
| ****Body Mass Index**** | ****Weight Status**** |
| Below 18.5 | Underweight |
| 18.5 – 24.9 | Healthy Weight |
| 25.0 – 29.9 | Overweight |
| 30.0 and Above | Obese |

## References

1. Emflaza [package insert]. South Plainfield, NJ: PTC Therapeutics, Inc.; June 2021.
2. 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;9:77-93.
3. Gloss D, Moxley 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;86(5):465-472.
4. 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.
5. Centers for Disease Control and Prevention. Assessing Your Weight. https://www.cdc.gov/healthyweight/assessing/bmi/ Accessed March 1, 2024.
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. Lancet Neurol. 2018;17(3):251-267.
7. Deflazacort [package insert]. East Windsor, NJ: Aurobindo Pharma USA, Inc.; June 2024.
8. Deflazacort oral suspension [package insert]. Monmouth Junction, NJ: Tris Pharma, Inc.; April 2024.