

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

## SPECIALTY GUIDELINE MANAGEMENT

### EMFLAZA (deflazacort) deflazacort

#### POLICY

##### I. 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 Indication

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.

##### II. DOCUMENTATION

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

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

##### III. PRESCRIBER SPECIALTIES

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

##### IV. CRITERIA FOR INITIAL APPROVAL

##### **Duchenne Muscular Dystrophy**

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

- A. The diagnosis of DMD was confirmed by one of the following criteria:
  - 1. Genetic testing demonstrating a mutation in the DMD gene.
  - 2. Muscle biopsy demonstrating absent dystrophin.
- B. Member is 2 years of age or older.
- C. 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)

|                            |
|----------------------------|
| <b>Reference number(s)</b> |
| 1636-A                     |

1. 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).

## V. CONTINUATION OF THERAPY

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

- A. The member meets all initial authorization criteria.
- B. The member is receiving a clinical benefit from therapy with the requested medication (e.g., improvement or stabilization of muscle strength or pulmonary function).

## VI. APPENDIX

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

| 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)

| 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          |

## VII. 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.