Reference number(s)
1636-A

SPECIALTY GUIDELINE MANAGEMENT

EMFLAZA (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.

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. 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. The member is 2 years of age or older.
C. The 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):
   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).
   2. For psychiatric/behavioral issues: psychiatric/behavioral issues persisted beyond the first 6 weeks of treatment with prednisone or prednisolone.

IV. 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 Emflaza therapy, such as improvement or stabilization of muscle strength or pulmonary function.

V. 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	Normal or 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	Normal or Healthy Weight
25.0 – 29.9	Overweight
30.0 and Above	Obese

VI. 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 25, 2022.
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.