AETNA BETTER HEALTH®
Coverage Policy/Guideline

Name:	Emflaza		Page:	1 of 3
Effective Date:	11/27/2024		Last Review Date:	10/22/2024
Applies to:	☒Illinois ☒Maryland ☒Pennsylvania Kids	☐Florida ☒Florida Kids ☐Virginia	☐New Jersey ☐Michigan ☐Arizona	

Intent:

The intent of this policy/guideline is to provide information to the prescribing practitioner outlining the coverage criteria for Emflaza under the patient's prescription drug benefit.

Description:

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.

Applicable Drug List:

Emflaza (deflazacort)

Policy/Guideline:

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.

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

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)
   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).

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).

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

Approval Duration and Quantity Restrictions:

Approval:
- Initial = 6 months; renewal = 12 months

Quantity Level Limit:

- Emflaza tablets 6 mg: 60 tablets per 30 days
- Emflaza tablets 18 mg: 30 tablets per 30 days
- Emflaza tablets 30 mg: 30 tablets per 30 days
- Emflaza tablets 36 mg: 30 tablets per 30 days
- Emflaza suspension 22.75 mg/mL: 52 mL per 30 days (1.8 mL/day)

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.