

# Specialty Guideline Management

## deflazacort products

### 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
Jaythari	deflazacort
Pyquvi	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 Indications

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

Deflazacort (generic), Jaythari, and Pyquvi are 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 Dystrophy

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

Body Mass Index Percentile Range	Weight Status
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Less than the 5th percentile	Underweight
5th percentile to less than the 85th percentile	Healthy Weight
85 <sup>th</sup> percentile 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

## References

1. Emflaza [package insert]. South Plainfield, NJ: PTC Therapeutics, Inc.; June 2024.
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.; June 2024.
9. Jaythari [package insert]. Pennington, NJ: Zydus Pharmaceuticals (USA) Inc.; May 2025.
10. Pyquvi [package insert]. Piscataway, NJ: Aucta Pharmaceuticals, Inc.; February 2025.