

**Emflaza (deflazacort)**  
**Effective 10/01/2021**

<b>Plan</b>	<input type="checkbox"/> MassHealth UPPL <input checked="" type="checkbox"/> Commercial/Exchange	<b>Program Type</b>	<input checked="" type="checkbox"/> Prior Authorization <input checked="" type="checkbox"/> Quantity Limit <input type="checkbox"/> Step Therapy
<b>Benefit</b>	<input type="checkbox"/> Pharmacy Benefit <input type="checkbox"/> Medical Benefit (NLX)		
<b>Specialty Limitations</b>	This medication has been designated specialty and must be filled at a contracted specialty pharmacy.		
<b>Contact Information</b>	<b>Specialty Medications</b>		
	All Plans	Phone: 866-814-5506	Fax: 866-249-6155
	<b>Non-Specialty Medications</b>		
	MassHealth	Phone: 877-433-7643	Fax: 866-255-7569
	Commercial	Phone: 800-294-5979	Fax: 888-836-0730
	Exchange	Phone: 855-582-2022	Fax: 855-245-2134
	<b>Medical Specialty Medications (NLX)</b>		
	All Plans	Phone: 844-345-2803	Fax: 844-851-0882
<b>Exceptions</b>	N/A		

**Overview**

Deflazacort is a corticosteroid used to treat Duchenne muscular dystrophy (DMD). DMD is a rare, inherited muscle disease that occurs usually in children and young adults and is caused by an absence of a protein called dystrophin, which helps keep muscle cells intact.

**Coverage Guidelines**

Authorization may be granted for members new to the plan who are currently receiving treatment with Emflaza excluding when the product is obtained as samples or via manufacturer's patient assistance programs.

**OR**

Authorization may be granted for members when ALL the following criteria are met, and documentation is provided:

1. The member is 2 years of age or older
2. The diagnosis of DMD has been confirmed by genetic testing demonstrating a mutation in the DMD gene. Laboratory confirmation of genetic testing is required.
3. The member has tried prednisone and has experienced one of the following:
  - a. Unmanageable and clinically significant weight gain while receiving prednisone. Body mass index is in the overweight or obese category with prednisone treatment. Chart documentation of weight gain is required. Refer to Appendix A for weight status categories for children and adult.
  - b. Psychiatric/behavioral issues (e.g. abnormal behavior, aggression, irritability) have persisted beyond the first 6 weeks of treatment with prednisone. Chart documentation of persistent psychiatric/behavioral issues with prednisone treatment is required.

**Continuation of Therapy**

- 1. The member meets all initial authorization criteria.
- 2. The member is receiving a clinical benefit from Emflaza therapy, such as improvement or stabilization of muscle strength or pulmonary function.

**Limitations**

- 1. Initial approvals will be for 6 months.
- 2. Reauthorizations will be for 12 months

**Appendix A**

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

<b>Body Mass Index Percentile Range</b>	<b>Weight Status</b>
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)**

<b>Body Mass Index</b>	<b>Weight Status</b>
Below 18.5	Underweight
18.5 – 24.9	Normal or Healthy Weight
25.0 – 29.9	Overweight
30.0 and Above	Obese

**References**

- 1. Emflaza (deflazacort) [prescribing information]. South Plainfield, NJ: PTC Therapeutics, Inc; February 2021
- 2. Birnkrant DJ, Bushby K, Bann CM, et al; DMD Care Considerations Working Group. 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. doi:10.1016/S1474-4422(18)30024-3

**Review History**

07/21/2021- Reviewed July P&T; Changed from CVS standard criteria to custom template. Effective 10/01/2021.

