RXADVANCE
2 Park Central Drive
Southborough, MA 01772

Clinical Policy Title:	deflazacort
Policy Number:	RxA.364
Drug(s) Applied:	Emflaza®
Original Policy Date:	03/06/2020
Last Review Date:	09/14/2020
Line of Business Policy Applies to:	All lines of business

Background

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

Dosing Information

Drug Name	Indication	Dosing Regimen	Maximum Dose
deflazacort (Emflaza®)	DMD	0.9 mg/kg/dose orally daily	0.9 mg/kg/dose

Dosage Forms

- Tablets: 6 mg, 18 mg, 30 mg, 36 mg
- Oral suspension: 22.75 mg/mL

Clinical Policy

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

I. Initial Approval Criteria

A. Duchenne Muscular Dystrophy (must meet all)**:**

1. Diagnosis of DMD confirmed by one of the following (a or b):
   a. Genetic testing (e.g. dystrophin deletion or duplication mutation found); or
   b. If genetic studies are negative (i.e. no mutation identified), positive muscle biopsy (e.g. absence of dystrophin protein);
2. Prescribed by or in consultation with a neurologist;
3. Age 2 years of age or older;
4. Failure of a 6 month or more trial of prednisone, unless contraindicated or clinically significant adverse effects are experienced;
5. Dose does not exceed 0.9 mg/kg per day.

Approval duration
Commercial: 6 months
Medicaid: 6 months

II. Continued Therapy Approval

A. Duchenne Muscular Dystrophy (must meet all)**:**

1. Currently receiving medication that has been authorized by RxAdvance or member has previously met initial approval criteria listed in this policy;
2. Member is responding positively to therapy;
3. If request is for a dose increase, new dose does not exceed 0.9 mg/kg per day.

Approval duration
Commercial: 12 months
Medicaid: 12 months

III. Appendices

APPENDIX A: Abbreviation/Acronym Key
Duchenne muscular dystrophy FDA:
Food and Drug Administration

APPENDIX B: Therapeutic Alternatives
This table provides a listing of preferred alternative therapy recommended in the approval criteria. The drugs listed here may not be a formulary agent for all relevant lines of business and may require prior authorization.

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
prednisone	0.75 mg/kg/day orally (preferred) Alternative dosing regimens • 0.3 mg/kg/day orally *(lesser efficacy and fewer adverse events)* • 10 mg/kg/weekend orally	Varies based on weight

Therapeutic alternatives are listed as Brand name (generic) when the drug is available by brand name only and generic (Brand name) when the drug is available by both brand and generic.

APPENDIX C: Contraindications/Boxed Warnings

- Contraindication(s):
  - Hypersensitivity to deflazacort or any of the inactive ingredients
- Boxed warning(s):
  - None reported.

References

1. Emflaza Prescribing Information. South Plainfield, NJ: PTC Therapuetics, Inc.; June 2019; Available at: https://emflaza.com/. Accessed June 18, 2019; July 01, 2020.
2. 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. doi:10.1212/WNL.0000000000002337.
3. 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(1): 77-93.
4. Clinical Pharmacology [database online]. Tampa, FL: Gold Standard, Inc.; 2017. Available at: http://www.clinicalpharmacology-ip.com/.
5. 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: 251-267.
6. Deflazacort. In: Lexicomp Online Drug Database [database on the Internet]. Hudson, Ohio: Lexicomp, Inc.; 2020 [updated June 18, 2020]. Available at: http://online.lexi.com. Subscription required to view. Accessed June 07, 2020
7. Micromedex® Healthcare Series [Internet database]. Greenwood Village, Colo: Thomson Healthcare. Updated periodically. Accessed June 12, 2020. https://www.micromedexsolutions.com/micromedex2/librarian/CS/545B1E/ND_PR/evidencexpert/. Accessed July 01, 2020.

Review/Revision History	Review/Revised Date	P&T Approval Date
Policy established.	01/2020	03/06/2020
Policy updated. 1. Formatting updated. 2. Continued therapy criteria updated. 3. References updated.	07/01/2020	09/14/2020