Clinical Policy Title:	deflazacort
Policy Number:	RxA.364
Drug(s) Applied:	Emflaza®
Original Policy Date:	03/06/2020
Last Review Date:	09/14/2021
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. The provision of provider samples does not guarantee coverage under the terms of the pharmacy benefit administered by RxAdvance. All criteria for initial approval must be met in order to obtain coverage.

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;
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. Member is currently receiving medication that has been authorized by RxAdvance or the member has 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
DMD: Duchenne muscular dystrophy
FDA: Food and Drug Administration

APPENDIX B: Therapeutic Alternatives
Below are suggested therapeutic alternatives based on clinical guidance. Please check drug formulary for preferred agents and utilization management requirements.

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
Prednisone	0.75 mg/kg/day orally (preferred) <u>Alternative dosing regimens</u> • 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 generic (Brand name®) when the drug is available by both generic and brand; Brand name® when the drug is available by brand only and generic name when the drug is available by generic only.

APPENDIX C: Contraindications/Boxed Warnings

- Contraindication(s):
  - Hypersensitivity to deflazacort or any of the inactive ingredients in Emflaza®.

- Boxed warning(s):
  - None reported.

APPENDIX D: General Information

- Vaccination: Do not administer live or live attenuated vaccines to patients receiving immunosuppressive doses of corticosteroids. Administer live attenuated or live vaccines at least 4 to 6 weeks prior to starting Emflaza®.

References

1. Emflaza® Prescribing Information. South Plainfield, NJ: PTC Therapuetics, Inc.; June 2019; Available at: https://Emflaza.com/. Accessed May 29, 2021.
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: https://www.clinicalkey.com/pharmacology/login . Accessed May 29, 2021.
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 May 15, 2021]. Available at: http://online.lexi.com. Subscription required to view. Accessed May 29, 2021.
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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
Policy was reviewed: 1. Statement about provider sample ‘‘The provision of provider samples does not guarantee coverage…” was added to Clinical Policy. 2. Continued Therapy Criteria II.A.1 was rephrased to "Member is currently receiving medication that has been authorized by RxAdvance...". 3. Appendix A was updated to include abbreviation DMD and “Food and Drug Administration.” 4. .Therapeutic Alternatives verbiage was rephrased to "Below are suggested therapeutic alternatives based on clinical guidance..". 5. Statement about drug listing format in Appendix B is rephrased to "Therapeutic alternatives are listed as generic (Brand name®) when the drug is available by both generic and brand; Brand name® when the drug is available by brand only and generic name when the drug is available by generic only". 6. Appendix C was updated to include “in Emflaza®.” 7. Appendix D was updated to include “Vaccination: Do not administer live or live attenuated vaccines…” 8. References were reviewed and updated.	05/29/2021	09/14/2021