

Policy Name:	Emflaza (deflazacort)	Policy #:	2607P
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Purpose of the Policy

The purpose of this policy is to establish the criteria for coverage of Emflaza.

Statement of the Policy

Health Alliance Medical Plans will approve the use of Emflaza under the Specialty Pharmacy benefit when the following criteria have been met.

Criteria

1. Coverage Criteria

- 1.1 Diagnosis of Duchenne Muscular Dystrophy confirmed by one of the following:
 - Genetic testing documenting a mutation in the dystrophin (DMD) gene
 - Muscle biopsy documenting lack of muscle dystrophin
- 1.2 Age 2 years of age or older
- 1.3 Prescribed by or in consultation with a physician who specializes in the treatment of Duchenne Muscular Dystrophy (DMD) and/or neuromuscular disorders
- 1.4 Documented trial of prednisone for 6 months and documentation that the member experienced at least one of the following significant intolerable adverse effects (AE)
 - Cushingoid appearance
 - Central (truncal) obesity
 - Undesirable weight gain defined as a 10% of body weight gain increase over a 6-month period
 - Diabetes and/or hypertension that is difficult to manage
 - Severe behavioral adverse effects that would require a prednisone dose reduction
 - Clinically significant growth stunting as evidenced by decline in mean height percentile from baseline, decrease in growth velocity or decrease in serum bone formation biomarkers
- 1.5 Documentation of a baseline motor milestone score from one of the following assessments:
 - 6-Minute Walk Test (6MWT)
 - North Star Ambulatory Assessment (NSAA)
 - Motor Function Measure (MFM)
 - Hammersmith Functional Motor Scale (HFMS)
- 1.6 Coverage of brand Emflaza requires a documented allergic reaction to generic deflazacort

2. Approval Period

- 2.1 Initial: 12 months
- 2.2 Reapproval: 12 months with documentation of improvement in one of the following motor milestone scores:
 - 6-Minute Walk Test (6MWT)
 - North Star Ambulatory Assessment (NSAA)
 - Motor Function Measure (MFM)
 - Hammersmith Functional Motor Scale (HFMS)

CPT Codes

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HCPCS Codes

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References

1. Emflaza (deflazacort) [prescribing information]. Northbrook, IL: Marathon Pharmaceuticals; June 2021.
2. DJ Birnkrant, K Bushby, CM Bann, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: respiratory, cardiac, bone health, and orthopaedic management. *Lancet Neurol* 2018, 17;4:347-61.
3. DJ Birnkrant, K Bushby, CM Bann, et al. Diagnosis and management of Duchenne muscular dystrophy, part 3: primary care, emergency management, psychosocial care, and transitions of care across the lifespan. *Lancet Neurol* 2018; 17:445.
4. Gloss D, Moxley RT, Ashwal S, Oskoui M. Practice guideline update summary: corticosteroid treatment of Duchenne muscular dystrophy. *American Academy of Neurology*. 2016.
5. Kang PB, Morrison L, Iannaccone ST, et al; Guideline Development Subcommittee of the American Academy of Neurology and the Practice Issues Review Panel of the American Association of Neuromuscular & Electrodiagnostic Medicine. Evidence-based guideline summary: evaluation, diagnosis, and management of congenital muscular dystrophy. *Neurology*. 2015 Mar 31;84(13):1369-78.

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DISCLAIMER

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