Emflaza

Description

Emflaza (deflazacort)

Background
Emflaza (deflazacort) is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD). Specifically, deflazacort is a corticosteroid prodrug, whose active metabolite acts through the glucocorticoid receptor to exert anti-inflammatory and immunosuppressive effects. The precise mechanism by which deflazacort exerts its therapeutic effects in patients with DMD is unknown (1).

Regulatory Status
FDA approved indication: Emflaza is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older (1).

Emflaza can suppress the immune system and increase the risk of infection with any pathogen, including viral, bacterial, fungal, protozoan, or helminthic. Corticosteroids reduce resistance to new infections, exacerbate existing infections, increase the risk of disseminated infections, increase the risk of reactivation or exacerbation of latent infections, and mask some signs of infection (1).

All immunizations should be administered according to immunization guidelines prior to starting Emflaza. Live or live attenuated vaccines should be administered at least 4 to 6 weeks prior to starting Emflaza. Patients on Emflaza may receive concurrent vaccinations, except for live or live-attenuated vaccines (1).
Monitoring motor changes in patients with DMD requires functional evaluation along with measurement of muscle strength. The need for a reliable outcome measure in diseases of rapid deterioration such as DMD has led to the use of motor functional tests. In a large, multicenter, international clinical trial, the six minute walk test (6MWT) proved to be feasible and highly reliable. This study and additional longitudinal natural history support acceptance of the 6MWT as the primary outcome measure of choice for ambulatory DMD clinical trials. And it was confirmed that in the 6MWT a clinically meaningful change in 6MWD to be in the range of 20–30 meters, which can serve as the targeted treatment effect. Also used are the Motor Function Measure (MFM), North Star Ambulatory Assessment (NSAA) and Hammersmith Functional Motor Scale (HFMS) to help predict loss of ambulation 1 year before its occurrence in order to allow time to adapt rehabilitation, change the patient’s environment, and consider acquisition of assistive aids or the use of medications (2-5).

Safety and effectiveness in patients 2 years and older have been established (1).

### Related policies
Exondys 51, Spinraza

### Policy
This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Emflaza may be considered medically necessary for patients 2 years of age or older with the diagnosis of Duchenne muscular dystrophy (DMD) and if the conditions indicated below are met.

Emflaza may be considered investigational in patients less than 2 years of age and for all other indications.

### Prior-Approval Requirements

**Age**
2 years of age or older

**Diagnosis**
Patient must have the following:

1. Duchenne muscular dystrophy (DMD)

AND ALL of the following:

a. Diagnosis confirmed by the documented presence of abnormal dystrophin or a confirmed mutation of the dystrophin gene
b. Serum creatinine kinase activity at least 10 times the upper limit of normal (ULN) prior to initiating treatment
c. Inadequate treatment response, intolerance, or contraindication to a 3 month trial of prednisone
d. Obtain a baseline motor milestone score from ONE the following assessments:
   i. 6-minute walk test (6MWT)
   ii. North Star Ambulatory Assessment (NSAA)
   iii. Motor Function Measure (MFM)
   iv. Hammersmith Functional Motor Scale (HFMS)

e. NOT given concurrently with live vaccinations
f. Absence of active infection (including tuberculosis and hepatitis B virus (HBV))
g. If the patient has a history of Hepatitis B (HBV) infection
   i. Prescriber agrees to monitor for HBV reactivation

Prior – Approval **Renewal Requirements**

**Age**

2 years of age or older

**Diagnosis**

Patient must have the following:

1. Duchenne muscular dystrophy (DMD)

AND ALL of the following:

a. Improvement in motor milestone score from baseline from ONE the following assessments:
   i. 6MWT – improvement of 20 meters from baseline
   ii. NSAA – improvement of 2 points from baseline
   iii. MFM – improvement of 2 points from baseline
iv. HFMS – improvement of 2 points from baseline
b. NOT given concurrently with live vaccinations
c. Absence of active infection (including tuberculosis and hepatitis B virus (HBV))
d. If the patient has a history of Hepatitis B (HBV) infection
   i. Prescriber agrees to monitor for HBV reactivation

Policy Guidelines

Pre - PA Allowance
None

Prior - Approval Limits
Duration 6 months

Prior – Approval Renewal Limits
Duration 12 months

Rationale

Summary
Emflaza (deflazacort) is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD). Specifically, deflazacort is a corticosteroid prodrug, whose active metabolite, 21-desDFZ, acts through the glucocorticoid receptor to exert anti-inflammatory and immunosuppressive effects. The most common adverse reactions are Cushingoid appearance, increase weight, increase appetite, upper respiratory tract infection, cough, pollakiuria, hirsutism, central obesity, and nasopharyngitis (1).

Prior authorization is required to ensure the safe, clinically appropriate and cost effective use of Emflaza while maintaining optimal therapeutic outcomes.

References


Policy History

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<thead>
<tr>
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<tbody>
<tr>
<td>March 2017</td>
<td>Addition to PA</td>
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<tr>
<td>April 2017</td>
<td>Addition of NSAA, MFM and HFMS assessment tools to obtain baseline scores and improvement requirements</td>
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<tr>
<td>June 2017</td>
<td>Annual review</td>
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<tr>
<td>September 2018</td>
<td>Annual review and reference update</td>
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<tr>
<td>June 2019</td>
<td>Reduced age requirement to 2 and older from 5 and older and revised regulatory status section</td>
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<tr>
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<td>Annual review</td>
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Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on September 13, 2019 and is effective on October 1, 2019.