DEFLAZACORT

Generic	Brand	HICL	GCN	Exception/Other
DEFLAZACORT	EMFLAZA	11668		

GUIDELINES FOR USE

INITIAL CRITERIA (NOTE: FOR RENEWAL CRITERIA SEE BELOW)

1. Does the patient have a diagnosis of Duchenne muscular dystrophy (DMD) and meet **ALL** of the following criteria?
   - Patient is 2 years of age or older
   - Documented genetic testing confirming Duchenne muscular dystrophy (DMD) diagnosis
   - Prescribed by or given in consultation with a neurologist specializing in treatment of Duchenne muscular dystrophy (DMD) at a DMD treatment center

   If yes, continue to #2.
   If no, do not approve.
   DENIAL TEXT: See the initial denial text at the end of the guideline.

2. Has the patient tried prednisone or prednisolone for at least 6 months?

   If yes, continue to #3.
   If no, do not approve.
   DENIAL TEXT: See the initial denial text at the end of the guideline.

3. Is the request for Emflaza due to lack of efficacy with prednisone or prednisolone and **ALL** of the following criteria are met?
   - Patient is not in Stage 1: pre-symptomatic phase
   - Steroid myopathy has been ruled out
   - Documented deterioration in ambulation, functional status, or pulmonary function while on prednisone or prednisolone, using standard measures over time, consistent with advancing disease (stage 2 or higher); Acceptable standard measures: [such as 6-minute walk distance (6MWD), time to ascend/descend 4 stairs, rise from floor time (Gower's maneuver), 10-meter run/walk time, or North Star Ambulatory Assessment (NSAA), Physician global assessments (PGA), pulmonary function (FVC, PFTs), upper limb strength (propelling a wheelchair 30 feet)]

   If yes, **approve for 6 months by GPID for all the following strengths with the following quantity limits:**
   (Initial approval directions continued on next page)

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DEFLAZACORT

INITIAL CRITERIA (CONTINUED)

If yes, **approve for 6 months by GPID for all the following strengths with the following quantity limits:**
- **6mg tablet (GPID 23761): #60 per 30 days**
- **18mg tablet (GPID 43012): #30 per 30 days**
- **30mg tablet (GPID 23762): #60 per 30 days**
- **36mg tablet (GPID 43015): #60 per 30 days**
- **22.75mg/mL oral suspension (GPID 43016): #39mL (3 bottles) per 30 days**

If no, continue to #4.

4. Is the patient experiencing an adverse consequence of prednisone or prednisolone and is the adverse consequence named or listed in the prescribing information adverse event profile of Emflaza?

   If yes, do not approve.
   DENIAL TEXT: See the initial denial text at the end of the guideline.
   If no, continue to #5.

5. Has documentation of literature-based evidence been provided supporting the mitigating effect of Emflaza for the named adverse consequence?

   If yes, **approve for 6 months by GPID for all the following strengths with the following quantity limits:**
   - **6mg tablet (GPID 23761): #60 per 30 days**
   - **18mg tablet (GPID 43012): #30 per 30 days**
   - **30mg tablet (GPID 23762): #60 per 30 days**
   - **36mg tablet (GPID 43015): #60 per 30 days**
   - **22.75mg/mL oral suspension (GPID 43016): #39mL (3 bottles) per 30 days**

   If no, do not approve.
   DENIAL TEXT: See the initial denial text at the end of the guideline.

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DEFLAZACORT

INITIAL CRITERIA (CONTINUED)

INITIAL DENIAL TEXT: The guideline named **DEFLAZACORT (Emflaza)** requires a diagnosis of Duchenne muscular dystrophy (DMD) and that all of the following criteria are met:

- Patient is 2 years of age or older
- Documented genetic testing confirming Duchenne muscular dystrophy (DMD) diagnosis
- Prescribed by or given in consultation with a neurologist specializing in treatment of Duchenne muscular dystrophy (DMD) at a DMD treatment center
- Trial of prednisone or prednisolone for at least 6 months and one of the following:
  - Request due to lack of efficacy with prednisone or prednisolone and all of the following criteria are met:
    - Patient is not in Stage 1: pre-symptomatic phase
    - Steroid myopathy has been ruled out
    - Documented deterioration in ambulation, functional status, or pulmonary function while on prednisone or prednisolone, using standard measures over time, consistent with advancing disease (stage 2 or higher); Acceptable standard measures: [such as 6-minute walk distance (6MWD), time to ascend/descend 4 stairs, rise from floor time (Gower's maneuver), 10-meter run/walk time, or North Star Ambulatory Assessment (NSAA), Physician global assessments (PGA), pulmonary function (FVC, PFTs), upper limb strength (propelling a wheelchair 30 feet)]
  - Request due to adverse consequence while on prednisone or prednisolone and documentation of literature-based evidence has been provided citing and supporting the mitigating effect of Emflaza for the named adverse consequence
    - Requests due to adverse consequences while on prednisone or prednisolone that is named or listed in the prescribing information of Emflaza will not be approved

RENEWAL CRITERIA

1. Does the patient have a diagnosis of Duchenne muscular dystrophy (DMD) and is currently ambulatory?

   If yes, continue to #2.
   If no, continue to #3.

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RENEWAL CRITERIA (CONTINUED)

2. Has the patient shown function, stabilization or improvement in a standard set of ambulatory or functional status measures since being on Emflaza that are being monitored, tracked, and documented consistently; Acceptable standard measures: [such as 6-minute walk distance (6MWD), time to ascend/descend 4 stairs, rise from floor time (Gower's maneuver), 10-meter run/walk time, or North Star Ambulatory Assessment (NSAA), Physician global assessments (PGA)]?

   If yes, **approve for 12 months by GPID for all the following strengths with the following quantity limits:**
   - **6mg tablet (GPID 23761): #60 per 30 days**
   - **18mg tablet (GPID 43012): #30 per 30 days**
   - **30mg tablet (GPID 23762): #60 per 30 days**
   - **36mg tablet (GPID 43015): #60 per 30 days**
   - **22.75mg/mL oral suspension (GPID 43016): #39mL (3 bottles) per 30 days**

   If no, do not approve.
   DENIAL TEXT: See the denial text at the end of the guideline.

3. Is the patient non-ambulatory and has the patient maintained or demonstrated a less than expected decline in pulmonary function and/or upper limb strength assessed by standard measures since being on Emflaza, that are being monitored, tracked and documented consistently; Acceptable standard measures: pulmonary function (FVC, PFTs), upper limb strength measures (propelling a wheelchair 30 feet), Physician Global assessments (PGA)?

   If yes, **approve for 12 months by GPID for all the following strengths with the following quantity limits:**
   - **6mg tablet (GPID 23761): #60 per 30 days**
   - **18mg tablet (GPID 43012): #30 per 30 days**
   - **30mg tablet (GPID 23762): #60 per 30 days**
   - **36mg tablet (GPID 43015): #60 per 30 days**
   - **22.75mg/mL oral suspension (GPID 43016): #39mL (3 bottles) per 30 days**

   If no, do not approve.
   DENIAL TEXT: See the denial text at the end of the guideline.

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RENEWAL CRITERIA (CONTINUED)

RENEWAL DENIAL TEXT: The guideline named **DEFLAZACORT (Emflaza)** requires a diagnosis of Duchenne muscular dystrophy (DMD) and one of the following criteria are met:

- **For patient who are currently ambulatory, approval requires:**
  - Patient has shown function, stabilization or improvement in a standard set of ambulatory or functional status measures since being on Emflaza, that are being monitored, tracked, and documented consistently; Acceptable standard measures: [such as 6-minute walk distance (6MWD), time to ascend/descend 4 stairs, rise from floor time (Gower's maneuver), 10-meter run/walk time, or North Star Ambulatory Assessment (NSAA), Physician global assessments (PGA)]
- **For patient who are currently non-ambulatory, approval requires:**
  - Patient has maintained or demonstrated a less than expected decline in pulmonary function and/or upper limb strength assessed by standard measures since being on Emflaza that are being monitored, tracked, and documented consistently; Acceptable standard measures: pulmonary function (FVC, PFTs), upper limb strength measures (propelling a wheelchair 30 feet), Physician Global assessments (PGA)

RATIONALE

For further information, please refer to the Prescribing Information and/or Drug Monograph for Emflaza.

REFERENCES

- Emflaza [Prescribing Information]. Northbrook, IL: Marathon Pharmaceuticals. June 2019.

Library	Commercial	NSA
Yes	Yes	No

Part D Effective: N/A
Commercial Effective: 07/22/19
Created: 02/17
Client Approval: 07/19
P&T Approval: 07/19