Effective Date: 02/01/2021
Reviewed: 11/2020, 5/2021, 4/2022, 4/2023, 4/2024, 8/2024, 4/2025, 4/2026 Scope: Medicaid

Deflazacort

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication
Emflaza (deflazacort) is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.

All other indications are considered experimental/investigational and not medically necessary.

II. CRITERIA FOR INITIAL APPROVAL

An authorization of 6 months may be granted for the treatment of Duchenne muscular dystrophy when all of the following criteria are met:

A. Member is at least 2 years of age
B. Emflaza is prescribed by or in consultation with a neurologist who specializes in the treatment of DMD
C. Documentation that the diagnosis of DMD was confirmed by either of the following criteria:
    1. Genetic testing demonstrating a mutation in the *DMD* gene.
    2. Muscle biopsy demonstrating absent dystrophin.
D. Documentation that the member meets one of the following criteria:
    1. Member has experienced unmanageable and/or clinically significant weight gain/obesity as evidenced by body mass index in the overweight or obese category while receiving treatment with prednisone or prednisolone for ≥6 months (refer to Appendix for weight status categories for children and adults).
    2. Member has experienced unmanageable and/or clinically significant psychiatric/behavioral issues (e.g., abnormal behavior, aggression, irritability)-with prednisone or prednisolone treatment.
    3. Member has experienced clinically significant growth stunting while receiving treatment with prednisone or prednisolone as evidenced by any of the following:
        i. Decline in mean height percentile for age from baseline
        ii. Decrease in growth trajectory and/or growth velocity
        iii. Reduction in serum biomarkers of bone formation (e.g., osteocalcin, procollagen 1 intact N-terminal propeptide [P1NP]) and/or bone turnover (e.g., type 1 collage cross-linked C-telopeptide [CTX1]).
E. Baseline documentation of one or more of the following:
    1. Dystrophin level

2. Timed function tests (e.g., time to stand [TTSTAND], 6-minute walk test [6MWT], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB] or 4-stair climb [4SC], etc.)
3. Upper limb function (ULM) test
4. North Star Ambulatory Assessment (NSAA) score
5. Forced Vital Capacity (FVC) percent predicted

III. CONTINUATION OF THERAPY

Authorization of 6 months may be granted for members requesting continuation of therapy when all of the following criteria are met:

A. The member meets all initial authorization criteria.
B. Documentation that the member is receiving a clinical benefit from deflazacort therapy compared to pretreatment baseline in one or more of the following (not all-inclusive):
    1. Increase in dystrophin level
    2. Stability, improvement, or slowed rate of decline in timed function tests (e.g., time to stand [TTSTAND], 6-minute walk test [6MWT], time to run/walk 10 meters [TTRW], time to climb 4 stairs [TTCLIMB] or 4-stair climb [4SC])
    3. Stability, improvement, or slowed rate of decline in upper limb function (ULM) test
    4. Stability, improvement, or slowed rate of decline in North Star Ambulatory Assessment (NSAA) score
    5. Stability, improvement, or slowed rate of decline in FVC% predicted
    6. Improvement in quality of life

IV. QUANTITY LIMIT

- Deflazacort 6mg tablet: 2 tablets per day
- Deflazacort 18mg, 30mg, and 36mg tablet: 1 tablet per day
- Deflazacort suspension 22.75mg/ml: 1.8mL per day (or four 13mL bottles per month)

V. APPENDIX

Body Mass Index Percentile and Weight Status Category for Children 2 Through 19 Years of Age

Body Mass Index Percentile Range	Weight Status
Less than the 5th percentile	Underweight
5th percentile to less than the 85th percentile	Healthy Weight
85th to less than the 95th percentile	Overweight
Equal to or greater than the 95th percentile	Obese

Body Mass Index and Weight Status Category for Adults (20 Years of Age and Older)

Body Mass Index	Weight Status
Below 18.5	Underweight
18.5 – 24.9	Healthy Weight
25.0 – 29.9	Overweight
30.0 and above	Obese

VI. REFERENCES

1. Emflaza [package insert]. South Plainfield, NJ: PTC Therapeutics, Inc.; June 2024.
2. Centers for Disease Control and Prevention. Assessing Your Weight. https://www.cdc.gov/healthyweight/assessing/bmi/ Accessed July 1, 2024.