Treatments for Duchenne Muscular Dystrophy

EOCCO POLICY

Policy Type: PA/ SP Pharmacy Coverage Policy: EOCCO018

Description

Deflazacort (Emflaza) is a corticosteroid prodrug and vamorolone (Agamree) is a dissociative corticosteroid that suppresses the inflammatory pathway. Ataluren (Translarna) is a nonsense mutation targeting small molecule that bypasses inappropriate stop codons during dystrophin transcription.

Length of Authorization

- Initial: Six months
- Renewal: 12 months

Quantity limits

Product Name	Indication	Dosage Form	Quantity Limit
generic deflazacort	Duchenne Muscular Dystrophy (DMD)	6 mg tablets	0.9 mg/kg/day (round to nearest tablet size)
		18 mg tablets	
		30 mg tablets	
		36 mg tablets	
		22.75 mg/mL oral suspension	0.9 mg/kg/day *(see appendix)*
deflazacort (Brand Emflaza)		6 mg tablets	0.9 mg/kg/day (round to nearest tablet size)
		18 mg tablets	
		30 mg tablets	
		36 mg tablets	
		22.75 mg/mL oral suspension	0.9 mg/kg/day *(see appendix)*
vamorolone (Agamree)		40 mg/mL oral suspension	225 mL/30 days
ataluren (Translarna)	Nonsense Mutation Duchenne Muscular Dystrophy (DMD)	TBD	40 mg/kg/day

Initial Evaluation

I. **Generic deflazacort, deflazacort (Brand Emflaza) and vamorolone (Agamree)** may be considered medically necessary when the following criteria below are met:
   A. Medication is prescribed by, or in consultation with, a neuromuscular specialist or neurologist; **AND**
   B. Must not be used in combination with each other for treatment of Duchenne Muscular Dystrophy (DMD); **AND**
   C. A diagnosis of **Duchenne Muscular Dystrophy (DMD)** when the following are met**:**
      1. Documentation of Duchenne Muscular Dystrophy (DMD) gene mutation; **OR**

i. Documentation of total absence of dystrophin confirmed by muscle biopsy; **AND**

2. Member is two years or older; **AND**
3. Member displays delayed motor milestones (e.g., child not walking by 18 months, toe walking, poor head control, not running by three years old, struggling to hop, abnormal gait, difficulty ambulating without assistance, etc.); **AND**

D. Member's current weight is documented; **AND**
E. Treatment with oral prednisone for six months or greater has been ineffective, not tolerated, or contraindicated; **AND**
F. Treatment with generic deflazacort has been ineffective, not tolerated, or contraindicated

II. **Ataluren (Translarna)** is considered medically necessary when the following criteria below are met:
A. Member is five years or older; **AND**
B. Medication is prescribed by, or in consultation with, a neuromuscular specialist or neurologist; **AND**
C. Medication will not be used in combination with givinostat (Duvyzat) or a dystrophin restoration treatment for Duchenne Muscular Dystrophy [e.g., Eteplirsen (Exondys 51), Golodirsen (Vyondys 53), Viltolarsen (Viltepso), Casimersen (Amondys 45), delandistrogene moxeparvovec-rokl (Elevidys)]; **AND**
D. A diagnosis of **nonsense mutation Duchenne Muscular Dystrophy (DMD)** when the following are met**:**
1. Documentation of Duchenne Muscular Dystrophy (DMD) gene mutation; **AND**
2. Documentation of nonsense mutation; **AND**
3. Member displays delayed motor milestones (e.g., child not walking by 18 months, toe walking, poor head control, not running by three years old, struggling to hop, abnormal gait, difficulty ambulating without assistance, etc.); **AND**

E. Member must be ambulatory (e.g., able to walk); **AND**
F. Member is using a corticosteroid concomitantly with ataluren (Translarna); **OR**
1. Treatment with a corticosteroid for six months or greater has been ineffective, not tolerated, or contraindicated; **AND**

G. Member's current weight is documented

III. Deflazacort (Emflaza), vamorolone (Agamree), and ataluren (Translarna) are considered investigational when used for all other conditions, including, but not limited to:
A. Dysferlinopathies: including Miyoshi Myopathy (MM) and limb girdle muscular dystrophy type 2B (LGMD2B)
B. Ulcerative Colitis
C. Myelodysplastic Syndromes: Polycythemia vera
D. Becker muscular dystrophy (BMD)
E. Cystic fibrosis

Renewal Evaluation

I. Member has received a previous prior authorization approval for this agent through this health plan or has been established on therapy from a previous health plan; **AND**
II. Member is not continuing therapy based off being established on therapy through samples, manufacturer coupons, or otherwise. If they have, initial policy criteria must be met for the member to qualify for renewal evaluation through this health plan; **AND**
III. Treatment provides clinical benefit to the member, defined as slow or stabilize disease progression in net motor function, compared to pretreatment baseline [e.g., stability or improvement in gait, muscle strength, slowed rate of decline in timed function tests (e.g., 6MWT, 4SC, NSAA, etc.)]; **AND**
IV. The request is for **generic deflazacort, deflazacort (Brand Emflaza), or vamorolone (Agamree)**; **AND**
    - Medication requested will not be used in combination with another corticosteroid (e.g., prednisone, deflazacort, etc.); **OR**
V. The request is for **ataluren (Translarna); AND**
    - Member must be ambulatory (e.g., able to walk); **AND**
    - Medication will not be used in combination with givinostat (Duvyzat) or a dystrophin restoration treatment for Duchenne Muscular Dystrophy (e.g., Eteplirsen (Exondys 51), Golodirsen (Vyondys 53), Viltolarsen (Viltepso), Casimersen (Amondys 45), delandistrogene moxeparvovec-rokl (Elevidys)); **AND**
    - The medication will be used in combination with a steroid unless contraindicated or not tolerated

Supporting Evidence

I. DMD (Duchenne Muscular Dystrophy) is a rare X-linked genetic disorder characterized by progressive muscle degeneration and weakness due to alterations in DMD genes required to synthesize a protein called dystrophin. Dystrophin is a major component of the cytoskeleton structure that prevents contraction-induced damage. Muscles with low levels of dystrophin are more sensitive to damage, resulting in progressive muscle loss and function. Duchenne Muscular Dystrophy initially presents as developmental delay and weakness in proximal limb muscle in young males ages three to five years old. Although rare, DMD may affect girls. Due to its gradual progression, if left untreated, most patients with DMD will lose ambulation before the age of 12 years and require non-invasive ventilation. Treatments in DMD target improving motor function and delaying the onset of cardiac and respiratory complications. Given the rarity and complexity of diagnosis and management of DMD, the treatment of DMD must be initiated by, or in consultation with, a neurologist or neuromuscular specialist.
II. Suspected cases of DMD should be referred to a neuromuscular specialist to evaluate creatinine kinase levels. If these are elevated, the diagnosis of DMD should be confirmed by dystrophin genetic testing. In rare cases genetic testing may be negative, but a diagnosis can still be confirmed by a muscle biopsy and dystrophin analysis.

III. There are no curative therapies for DMD. Supportive care is crucial for optimizing health and quality of life for patients with DMD. The 2018 DMD Care Considerations Working Group Diagnosis and Management of Duchenne Muscular Dystrophy guidelines note that glucocorticoids may be used to improve physical functioning and should be started prior to substantial physical decline. Prednisone and deflazacort have been shown to improve motor and pulmonary function and improve survival. Guidelines have not been updated to include vamorolone (Agamree).

IV. There are five gene-based therapies FDA-approved to treat patients with DMD: eteplirsen (Exondys 51), golodirsen (Vyondys 53), viltolarsen (Viltepso), casimersen (Amondys 45), and delandistrogene moxeparvovec-rokl (Elevidys). The accelerated approval of all five drugs was based on the surrogate endpoint of an increase in dystrophin production in the skeletal muscle observed in some patients treated with the drugs. However, the amount of dystrophin expression to be translated into a clinical benefit has yet to be established in patients with DMD.

deflazacort (Emflaza)

V. Deflazacort (Emflaza) was evaluated in two multicenter, randomized, double-blind, placebo-controlled trials in 225 patients. Study 1 consisted of 196 male pediatric patients, five to 15 years of age with documented mutation of the dystrophin gene, and onset of weakness before five years of age. The primary endpoint was the average change in muscle strength score between baseline and week 12. The average change was 0.15 (95% CI 0.01, 0.28) and -0.10 (95% CI -0.23, 0.03) for the deflazacort (Emflaza) and placebo groups, respectively. Study 2 consisted of 29 male pediatric patients, six to 12 years of age with documented mutation of the dystrophin gene. The primary endpoint was the average muscle strength score at two years. The results were not statistically significant.

vamorolone (Agamree)

VI. Vamorolone (Agamree) is an FDA approved dissociative steroid indicated for treatment of DMD. Vamorolone (Agamree) is available as a 40mg/mL suspension and dosing is weight-based. Per label, the recommended dose of vamorolone (Agamree) is 6mg/kg, with a max of 300mg daily for those >50kg. A dissociative steroid retains a selective anti-inflammatory profile and assumed to have a favorable side effect profile compared to traditional corticosteroids with reduced bone fragility, metabolic disturbance, and immune suppression.

VII. Vamorolone (Agamree) was studied in a Phase IIb, multicenter, double-blinded, randomized, placebo- and prednisone-controlled trial (VISION-DMD phase IIb) in 121 boys ages ≥ 4 years and <7 years old with confirmed DMD via DMD gene mutation or muscle biopsy. Participants were randomized to receive low or high dose vamorolone (2mg/kg [N=30] or 6mg/kg [N=30]), prednisone (0.75mg/kg [N=30]) or placebo (N=31) daily. Patients that received prior treatment with oral glucocorticoids or other immunosuppressants or clinically significant cardiac disease were excluded. Baseline characteristics were similar between all groups with a mean age of 5.4 years, weight of 20kg, 82.9% Caucasian, and baseline time to stand (TTSTAND) velocity of 1.7m/s.

VIII. The primary endpoint was TTSTAND from supine velocity in the vamorolone 6 mg/kg per day group vs placebo. Treatment with vamorolone 6 mg/kg/day resulted in statistically significant lower TTSTAND scores relative to placebo at week 24, least square mean (LSM) 0.05 m/s in high

dose vamorolone compared to -0.01 m/s in the placebo group, LSM difference 0.06m/s, p=0.002 (95% CI, 0.02-0.10). Secondary endpoints included TTSAND velocity in the vamorolone 2mg/kg per day group vs placebo, 6-minute walk test (6MWT) and time to run/walk (TTRW) between high dose vamorolone compared to placebo and low dose vamorolone compared to placebo.

- TTSTAND velocity in the vamorolone 2mg/kg per day 0.03 m/s vs placebo -0.01 m/s, LSM difference 0.05 m/s, p=0.02 (95% CI, 0.01-0.08)
    i. TTSTAND is a validated outcome measure for DMD. A difference of 0.05 m/s in TTSTAND velocity is indicative of clinically meaningful changes. Strength testing is reliable and reflects differences between steroid-treated and naive populations between the ages of 4 and 9 years and for stronger and more mobile subpopulations aged 10 and older. However, strength testing has limited continuity across the entire age range of affected individuals from young children to adults.
- 6MWT in the vamorolone 6mg/kg per day 28.3m vs placebo -13.3m LSM difference 41.6m, p=0.003 (95% CI, 14.2-68.9); vamorolone 2mg/kg per day 23.9 m vs placebo -13.3m, LSM difference 37.1, p=0.009 (95% CI, 9.6-64.7)
- TTRW velocity in the vamorolone 6mg/kg per day 0.26m/s vs placebo 0.01m/s LSM difference 0.024 m/s, p=0.024 (95% CI, 0.09-0.39); vamorolone 2mg/kg per day 0.16 m/s vs placebo 0.01 m/s, LSM difference 0.02 m/s, p>0.05 (95% CI, -0.03-0.28)

IX. The number of participants reporting at least one adverse event (AE) was similar between all groups. Participants in the prednisone group experienced linear growth delay, which was not present in the vamorolone group. There were two treatment-emergent vertebral fractures at week 24; one participant in the prednisone group had a total of four incident vertebral fractures, and one participant in the placebo group had a single incident vertebral fracture.

X. The FDA-label for vamorolone (Agamree) has similar warnings and precautions as prednisone and deflazacort (Emflaza). These glucocorticoids have similar safety and efficacy profiles and requiring step through prednisone and generic deflazacort is both clinically appropriate and cost-effective.

XI. Additionally, 41 participants enrolled in a 30-month open label extension trial. Participants were matched and compared with participants from the DNHS. Participants in DNHS were first eligible for inclusion in the control group after they had experienced 6 months of continuous glucocorticoid exposure. There was a decrease in mean TTSTAND velocity from baseline to 30 months (0.206 rises/s vs 0.189 rises/s), which was not a statistically significant change (−0.011 rises/s; CI, −0.068 to 0.046 rises/s). There were no statistically significant differences between participants receiving high dose vamorolone and matched participants in the historical control groups receiving glucocorticoid treatment.

XII. The clinical program for vamorolone (Agamree) consisted of a moderate to well-designed randomized clinical trial reporting consistent improvement in TTSTAND score, which is an objective, validated measure of muscular function in DMD. Milestones of disease progression, such as loss of ability to rise from floor, ambulate 10 m and self-feed occur in a predictable order, and loss of those abilities can be predicted by timed functional evaluations. The generalizability of current clinical data may be limited due to the exclusion of patients with

severe disease. However, in the absence of substantial physical decline, vamorolone (Agamree) may provide potential clinical benefit similar to standard of care glucocorticoids.

ataluren (Translarna)

VI. Ataluren (Translarna) is a nonsense mutation targeting small molecule pending FDA-approval for the treatment of nonsense mutation Duchenne Muscular Dystrophy (DMD). Ataluren (Translarna) was studied in a Phase III, double-blinded, randomized, placebo-controlled study (study 041) and an observational propensity-matched trial. Study 041 was prespecified to analyze a modified intent to treat population. However, after completion of the trial, no statistical difference was identified in the primary and secondary endpoints. The manufacturer then completed a secondary analysis of with all participants (ITT population) enrolled in Study 041.

VII. A total of 185 boys ages 5 years and older with confirmed nonsense mutation DMD were randomized to receive ataluren (Translarna) 40mg/kg/day, split into three doses, or placebo orally daily for 72 weeks. Concomitant corticosteroid use was permitted if the participant was stable on therapy before randomization and participants were required to be ambulatory. Baseline characteristics were similar between both cohorts with a mean age of 8 years, most participants had a mean baseline six-minute walking distance (6MWD) of >400 meters (41%), and all participants had prior exposure to corticosteroids. The primary endpoint was change in 6MWD in the intent to treat population, which was -53.0m in the ataluren (Translarna) group and -67.4m in the placebo arm, difference 14.4m, p=0.0248. Ataluren (Translarna) had a lesser decrease in NSAA total score compared to placebo (-3.7 vs -4.5, difference 0.9, p=0.0235) and time to climb 4 stairs (4.98 seconds vs 6.04 seconds, difference -1.06, p=0.0078).

VIII. Additionally, 298 participants were enrolled in an observational extension trial. Participants were continued on ataluren (Translarna) plus standard of care treatment and followed for at least 5 years or until study withdrawal and matched and compared to participants on standard of care treatment from the Duchenne Natural History Study. After a mean duration of treatment of 67 months, participants receiving ataluren (Translarna) plus standard of care demonstrated a significant delay in the median age at loss of ambulation compared natural history data matches receiving standard of care alone (16.5 yrs vs 13.0 yrs, HR 0.455, p<0.0001). Interim results also suggest a trend toward a delay in worsening of pulmonary function (median age at FVC <60% was 17.7 years in those treated with ataluren (Translarna) compared to 15.6 years in the natural history comparator (HR = 0.63, p=0.0086). The observational study demonstrated that participants on ataluren plus standard of care loss ambulation at a later age compared to propensity matched participants on standard of care in a natural history database and patients that remained on therapy did not have any new safety concerns.

- The secondary analysis demonstrated that the change in 6MWD was significantly less in the ataluren group compared to placebo (difference of 14.4 m, p=0.0248). The difference between both arms did not meet the minimal clinically important difference of 28.5 m and the difference is not considered clinically significant. Additionally, changing the targeted population after completion of the trial significantly compromises the integrity of the trial.

XIII. The rate of treatment emergent adverse events was similar between ataluren (Translarna) and placebo (85.3% vs 84.7%). The most common adverse events include vomiting, upper respiratory tract infection, nasopharyngitis, headache, and fall. No participants discontinued therapy due to treatment emergent adverse events.

XIV. Exposure to treatment with dystrophin restoration agents for DMD (e.g., ataluren (Translarna), eteplirsen (Exondys 51), golodirsen (Vyondys 53), viltolarsen (Viltepso), casimersen (Amondys 45), delandistrogene moxeparvovec-rokl (Elevidys) etc.) was excluded from the EPIDYS trial. Exposure to treatment with givinostat (Duvyzat) and dystrophin restoration agents for DMD was excluded from Study 041, and the efficacy and safety of combination treatment with ataluren (Translarna) has not been evaluated.

XV. Optimal sequencing of DMD gene therapies and use of givinostat (Duvyzat) has not been evaluated. Givinostat (Duvyzat) is not limited to use in any specific DMD variant and could potentially be used in most patients with DMD, including those that previously received gene therapies.

Appendix

I. **Table 2. Recommended dosing of vamorolone (Skyclarys)**

Weight (kg)	Dosage	Oral suspension volume	Volume per 28-day supply	# bottles per day supply
10 – 19 kg	114 mg once daily	3 mL once daily	84 mL	1 bottle/33 days
20 – 39 kg	234 mg once daily	6 mL mg once daily	168 mL	1 bottle/16 days
40 – 45 kg	270 mg once daily	6.75 mL once daily	190 mL	2 bottles/29 days
> 46 kg	300 mg once daily	7.5 mg once daily	210 mL	2 bottles/26 days

- Vamorolone (Agamree) is available as an 100mL oral solution, containing 40mg of vamorolone per mL.
- The recommended dose of vamorolone (Agamree) is 6 mg/kg taken orally once daily, up to a maximum daily dosage of 300 mg for members weighing more than 50 kg (110 lb)

II. **Table 3. Recommended dosing of deflazacort (Emflaza)**

Weight (kg)	Dosage	Oral suspension volume	Volume per 28-day supply	# bottles per day supply
10 – 19 kg	17 mg once daily	0.75 mL once daily	21 mL	2 bottle/34 days
20 – 39 kg	35 mg once daily	1.6 mL mL once daily	45 mL	4 bottles/32 days
40 – 59 kg	53 mg once daily	2.4 mL once daily	68 mL	5 bottles/27 days
> 60 kg	63 mg once daily	2.8 mg once daily	79 mL	6 bottles/27 days

- Deflazacort is available as a 13mL oral solution, containing 22.75mg of deflazacort per mL.
- The recommended dose of deflazacort is weight based, 0.9 mg/kg, and taken orally once daily. In patients who experience intolerable adverse effects, may decrease the dose by 25% to 33%.

Investigational or Not Medically Necessary Uses

I. Dysferlinopathies: including Miyoshi Myopathy (MM) and limb girdle muscular dystrophy type 2B (LGMD2B)
   A. Deflazacort as an ineffective therapy in dysferlinopathies was shown in a double-blinded, placebo-controlled trial. Further evaluation is needed to support use of deflazacort (Emflaza) in this setting.

II. Vamorolone (Agamree) has not been FDA-approved, or sufficiently studied for safety and efficacy for Ulcerative Colitis
   A. There is a withdrawn phase I/II study evaluating the use of vamorolone in pediatric ulcerative colitis.

III. Cystic fibrosis
   A. Ataluren was an investigational therapy for CF caused by nonsense mutations, which prevent the production of a normal cystic fibrosis transmembrane conductance regulator (CFTR) protein. A dysfunctional or nonexistent CFTR protein disrupts the salt and water balance in the lungs and other tissues and leads to the thick, sticky mucus associated with cystic fibrosis. Phase 3 data did not show significant improvement in lung function or enough of a decrease in exacerbations and development in this space has ceased.

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Related Policies

Currently there are no related policies.

Policy Implementation/Update

Action and Summary of Changes	Date
Removed givinostat (Duvyzat) from the policy as it is now a part of the HCDCO program and covered directly through the state for Medicaid members.	01/2026
Added criteria for new medication, ataluren (Translarna). Updated QL table, supporting evidence, removed 2016 American Academy of Neurology Recommendations on Corticosteroids in the supporting evidence as the guidelines are retired as of 2/2025.	7/2025
Added ambulatory requirement in renewal. Updated supporting evidence at QL table.	07/2024
Renamed deflazacort and vamorolone policy to “Treatments for Duchenne muscular dystrophy” policy. Added criteria for new medication, givinostat (Duvyzat). Updated QL table, supporting evidence, E/I section, references. Added appendix and related policies.	05/2024
Added step therapy requirement through generic deflazacort for brand vamorolone (Agamree) and brand deflazacort (Emflaza) and updated supporting evidence to reflect required step therapy.	02/2024
Realigned QL table and placed dosage form next to quantity limit. Added vamorolone to QL table Updated initial evaluation to include muscle biopsy and clinical features for diagnosis of DMD. Added vamorolone criteria and investigational condition. Updated supporting evidence.	11/2023
Updated initial approval duration to six months, and QLL box with weight-based dosing. Added requirement for neuromuscular specialist or neurologist. Included requirement for confirmation of diagnosis by genetic testing and addition of member weight to confirm dosing. Requires prednisone be tried and failed for six months to be deemed ineffective or have intolerance. Updated renewal criteria to include requirement for previous approval by Moda and not allowing establishing therapy with samples. Added examples of symptom improvement to renewal criteria.	05/2020

Revised to policy format, include use in pediatric patients down to two years of age.	07/2019
Update to criteria	01/2017
Criteria creation	05/2017