Agamree (vamorolone), Emflaza (deflazacort) Prior Authorization with Quantity Limit Program Summary

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POLICY REVIEW CYCLE

Effective Date **Date of Origin**
01-01-2026

FDA LABELED INDICATIONS AND DOSAGE

Agent(s)	FDA Indication(s)	Notes	Ref#
Agamree® (vamorolone) Oral suspension	Treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older		6
Emflaza® (deflazacort) Tablet* Oral suspension*	Treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older	* generic available	1

See package insert for FDA prescribing information: https://dailymed.nlm.nih.gov/dailymed/index.cfm

CLINICAL RATIONALE

Duchenne Muscular Dystrophy	Duchenne muscular dystrophy (DMD) is a genetic condition characterized by progressive muscle degeneration and weakness due to alterations in a protein called dystrophin that helps keep muscle cells intact. DMD is the most common childhood form of muscular dystrophy as well as the most prevalent of the muscular dystrophies. DMD is an X-linked recessive inherited genetic condition primarily affecting males, although females who carry the defective gene may show some symptoms. Prevalence is 15.9 per 100,000 live male births in the US and 19.5 per 100,000 live male births in the UK. Dystrophin is the protein associated with this affected gene and provides structural stability to skeletal muscles. Mutations in this gene, and subsequent lack of dystrophin in muscle fiber, result in a rapidly progressing disease involving muscle degeneration and weakness. Symptom onset is in early childhood and many children lose the ability to walk by early adolescence. Beyond muscle weakness, other symptoms include enlargement of the calf muscles, lumbar lordosis, and eventually cardiomyopathy and poor respiratory function. Until relatively recently, boys with DMD usually did not survive much beyond their teen years. Due to advances in cardiac and respiratory care, life expectancy is increasing and many young adults with DMD are surviving into their early 30s. Currently, there is no cure for DMD, and therapies are supportive in nature. Physical therapy, occupational therapy, respiratory care, speech therapy, braces/wheelchairs/contractures and glucocorticoid therapy are among the most common therapies.(2-4) Dystrophin gene deletion and duplication testing is usually the first confirmatory test.(8) Corticosteroid

	(glucocorticoids) are the standard of care for DMD, although they remain non-curative. Their use improves muscle strength, improves timed motor function, delays loss of ambulation, improves pulmonary function, reduces the need for scoliosis surgery, delays onset of cardiomyopathy, increases survival, and maintains quality of life. The choice of which glucocorticoid to use depends on cost, formulation, and perceived side-effect profiles.(3) The American Academy of Neurology (AAN) practice guidelines concluded that prednisone and deflazacort are possibly equally effective for improving motor function in patients with DMD (2 Class III studies). There is insufficient evidence to directly compare the effectiveness of prednisone versus deflazacort in cardiac function in patients with DMD (1 Class III study of a combined cohort). The AAN states that deflazacort could be offered as an intervention for patients with DMD to improve strength and timed motor function and delay the age at loss of ambulation by 1.4–2.5 years (Level C), improve pulmonary function (Level C), reduce the need for scoliosis surgery (Level C), delay the onset of cardiomyopathy by 18 years of age (Level C), increase survival at 5 and 15 years of follow-up (Level C). Prednisone is possibly associated with greater weight gain in the first 12 months of treatment, with no significant difference in weight gain with longer-term use compared with deflazacort (2 Class III studies). Deflazacort is possibly associated with an increased risk of cataracts compared with prednisone, although most are not vision-impairing (2 Class III studies).(5) Vamorolone is a first-in-class anti-inflammatory steroidal drug that has shown to have dissociative properties. The structure of vamorolone is similar to other glucocorticoids: it binds to the glucocorticoid receptor and retains the anti-inflammatory effects characteristic of traditional steroids, preferentially inducing transrepression with little-to-no transactivation or cis-repression. Transrepression is the suppression of the pro-inflammatory nuclear factor kappa B (NF-κB) signaling pathway, to exert the well-known potent anti-inflammatory effects of steroids. By not inducing transactivation or cis-repression, vamorolone is purported to elicit fewer adverse effects. Vamorolone is also a mineralocorticoid receptor antagonist, and thus may have the potential to treat DMD-associated cardiomyopathy through modulation of blood pressure.(7)
Efficacy	**Emflaza** The effectiveness of Emflaza for the treatment of DMD was established in a multicenter, randomized, double-blind, placebo-controlled, 52-week study. The study enrolled 196 male patients between the ages of 5 and 15 years old with documented mutation of the dystrophin gene, onset of weakness before 5 years of age, and serum creatinine kinase activity at least 10 times the upper limit of normal at some stage in their illness. Patients were randomized to receive Emflaza (0.9 or 1.2 mg/kg/day), an active comparator, or placebo. After 12 weeks, placebo patients were re-randomized to receive either Emflaza or the active comparator. All patients continued treatment for an additional 40 weeks. Efficacy was evaluated by assessing the change between Baseline and Week 12 in average strength of 18 muscle groups. The change in average muscle strength score between Baseline and Week 12 was significantly greater for the deflazacort 0.9 mg/kg/day dose group than for the placebo group. (p-value 0.017). Although not a pre-specified statistical analysis, compared with placebo, the deflazacort 0.9 mg/kg/day dose group demonstrated at Week 52 the persistence of the treatment effect observed at Week 12.(1) A second randomized, double-blind, placebo-controlled, 104-week clinical trial evaluated deflazacort in comparison to placebo. The study population consisted of 29 male children 6 to 12 years of age with a DMD diagnosis confirmed by the documented presence of abnormal dystrophin or a confirmed mutation of the dystrophin gene. The results of the analysis of the primary endpoint of average muscle strength scores in this 2nd study (graded on a 0-5 scale) at 2 years were not statistically significant, possibly because of a limited number of patients remaining in the placebo arm

	(subjects were discontinued from the trial when they lost ambulation). Although not statistically controlled for multiple comparisons, average muscle strength scores at Months 6 and 12, as well as the average time to loss of ambulation, numerically favored deflazacort in comparison with placebo.(1) **Agamree** The effectiveness of Agamree for the treatment of DMD was evaluated in a multicenter, randomized, double-blind, parallel-group, placebo- and active-controlled, multinational 24-week study (Study 1; NCT03439670). The study randomized 121 male patients with DMD to one of the following treatment groups: AGAMREE 6 mg/kg/day (n=30), AGAMREE 2 mg/kg/day (n=30), prednisone 0.75 mg/kg/day (n=31), or placebo (n=30) for 24 weeks. After 24 weeks, patients on prednisone and placebo received either AGAMREE 6 mg/kg/day (n=29) or AGAMREE 2 mg/kg/day (n=29) for an additional 20 weeks. The study included patients 4 to less than 7 years of age at time of enrollment in the study who were corticosteroid naïve and ambulatory, with a confirmed diagnosis of DMD.(6) The primary endpoint was the change from baseline to Week 24 in Time to Stand Test (TTSTAND) velocity for AGAMREE 6 mg/kg/day compared to placebo. TTSTAND velocity is a measure of muscle function that measures the time required for the patient to stand to an erect position from a supine position (floor). The key secondary endpoints consisted of change from baseline to Week 24 in TTSTAND velocity (AGAMREE 2 mg/kg/day vs placebo), 6 Minute Walk Test (6MWT) distance (AGAMREE 6 mg/kg/day vs placebo and 2 mg/kg/day vs placebo) and Time to Run/Walk 10 meters (TTRW) velocity (AGAMREE 6 mg/kg/day vs placebo and 2 mg/kg/day vs placebo). The 6MWT measures the distance that a patient can walk on a flat, hard surface in a period of 6 minutes and TTRW measures the time that it takes a patient to run or walk 10 meters. The fixed sequential testing process was applied to the key secondary endpoints in the order listed above.(6) The primary endpoint and key secondary endpoints were met for the AGAMREE 6 mg/kg/day treatment group. The AGAMREE 2 mg/kg/day treatment group was statistically significant vs. placebo for TTSTAND and 6MWT, but was not statistically significant vs. placebo for TTRW.(6)
Safety	Emflaza is contraindicated in patients with known hypersensitivity to deflazacort or to any of the inactive ingredients. Instances of hypersensitivity, including anaphylaxis, have occurred in patients receiving corticosteroid therapy.(1) Agamree is contraindicated in patients with known hypersensitivity to vamorolone or to any of the inactive ingredients. Instances of hypersensitivity, including anaphylaxis, have occurred in patients receiving corticosteroid therapy.(6)

REFERENCES

Number	Reference
1	Emflaza prescribing information. PTC Pharmaceuticals. June 2024.
2	Duchenne muscular dystrophy (DMD). Muscular Dystrophy Association. Accessed June 2025. https://www.mda.org/disease/duchenne-muscular-dystrophy
3	Biggar, W. D., Skalsky, A., & McDonald, C. M. Comparing deflazacort and prednisone in Duchenne Muscular Dystrophy. Journal of Neuromuscular Diseases. 2022;9(4):463–476. https://doi.org/10.3233/jnd-210776
4	U.S. Department of Health and Human Services. Muscular dystrophy. National Institute of Neurological Disorders and Stroke. Accessed June 2025. https://www.ninds.nih.gov/health-information/disorders/muscular-dystrophy

Number	Reference
5	Gloss, D., Moxley, R. T., Ashwal, S., & Oskoui, M. (2016). Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy. Neurology. 2016;86(5):465–472. Reaffirmed January 2024. https://doi.org/10.1212/wnl.0000000000002337
6	Agamree prescribing information. Catalyst Pharmaceuticals Inc. June 2024.
7	Kourakis, Stephanie, Timpani, Cara A. Standard of Care Versus New-Wave Corticosteroids in the Treatment of Duchenne Muscular Dystrophy: Can we Do Better? Orphanet Journal of Rare Diseases. 2021;16(1):117. doi: 10.1186/s13023-021-01758-9
8	Birnkrant, D. J., Bushby, K., Bann, C. M., Apkon, S. D., Blackwell, A., Brumbaugh, D., Case, L. E., Clemens, P. R., Hadjiyannakis, S., Pandya, S., Street, N., Tomezsko, J., Wagner, K. R., Ward, L. M., & Weber, D. R. Diagnosis and management of Duchenne muscular dystrophy, part 1: Diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. The Lancet Neurology.2018;17(3):251–267. https://doi.org/10.1016/s1474-4422(18)30024-3

POLICY AGENT SUMMARY PRIOR AUTHORIZATION

Target Brand Agent(s)	Target Generic Agent(s)	Strength	Targeted MSC	Available MSC	Final Age Limit	Preferred Status
Emflaza	deflazacort susp	22.75 MG/ML	M ; N ; O ; Y	O ; Y		
Emflaza	deflazacort tab	18 MG ; 30 MG ; 36 MG ; 6 MG	M ; N ; O ; Y	O ; Y		
Agamree	vamorolone oral susp	40 MG/ML	M ; N ; O ; Y	N		

POLICY AGENT SUMMARY QUANTITY LIMIT

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	QL Amount	Dose Form	Day Supply	Duration	Addtl QL Info	Allowed Exceptions	Targeted NDCs When Exclusions Exist
Agamree	vamorolone oral susp	40 MG/ML	3	Bottles	30	DAYS			
Emflaza	Deflazacort Tab 18 MG	18 MG	30	Tablets	30	DAYS			
Emflaza	Deflazacort Tab 6 MG	6 MG	60	Tablets	30	DAYS			

CLIENT SUMMARY – PRIOR AUTHORIZATION

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	Client Formulary
Agamree	vamorolone oral susp	40 MG/ML	Accord Enhanced ; Accord Standard ; Choice NetR - A Select ; Choice NetR - F Performance ; Choice NetR-HIM
Emflaza	deflazacort susp	22.75 MG/ML	Accord Enhanced ; Accord Standard ; Choice NetR - A Select ; Choice NetR - F Performance ; Choice NetR-HIM
Emflaza	deflazacort tab	18 MG ; 30 MG ; 36 MG ; 6 MG	Accord Enhanced ; Accord Standard ; Choice NetR - A Select ; Choice NetR - F

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	Client Formulary
			Performance ; Choice NetR-HIM

CLIENT SUMMARY – QUANTITY LIMITS

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	Client Formulary
Agamree	vamorolone oral susp	40 MG/ML	Accord Enhanced ; Accord Standard ; Choice NetR - A Select ; Choice NetR - F Performance ; Choice NetR-HIM
Emflaza	Deflazacort Tab 18 MG	18 MG	Accord Enhanced ; Accord Standard ; Choice NetR - A Select ; Choice NetR - F Performance ; Choice NetR-HIM
Emflaza	Deflazacort Tab 6 MG	6 MG	Accord Enhanced ; Accord Standard ; Choice NetR - A Select ; Choice NetR - F Performance ; Choice NetR-HIM

PRIOR AUTHORIZATION CLINICAL CRITERIA FOR APPROVAL

Module	Clinical Criteria for Approval

Initial Evaluation

Target Agent(s) will be approved when ALL of the following are met:

1. ONE of the following:
   A. The requested agent is eligible for continuation of therapy AND ONE of the following:

Agents Eligible for Continuation of Therapy
All target agents are eligible for continuation of therapy

   1. The patient has been treated with the requested agent (starting on samples is not approvable) within the past 90 days **OR**
   2. The prescriber states the patient has been treated with the requested agent (starting on samples is not approvable) within the past 90 days AND is at risk if therapy is changed **OR**

   B. ALL of the following:
      1. ONE of the following:
         A. The patient has a diagnosis of Duchenne Muscular Dystrophy confirmed by genetic analysis (i.e., dystrophin deletion or duplication mutation) (genetic test required) **OR**
         B. The patient has another FDA labeled indication for the requested agent and route of administration **AND**
      2. If the patient has an FDA labeled indication, then ONE of the following:
         A. The patient's age is within FDA labeling for the requested indication for the requested agent **OR**
         B. There is support for the use of the requested agent for the patient's age for the requested indication **AND**
      3. The patient has ONE of the following:
         A. Tried and had an inadequate response after 6 months of therapy with ONE prerequisite agent (i.e., generic prednisone or prednisolone) **OR**
         B. An intolerance or hypersensitivity to ONE prerequisite agent that is NOT expected to occur with the requested agent **OR**
         C. An FDA labeled contraindication to ALL prerequisite agents **AND**

Module	Clinical Criteria for Approval

2. If the request is for one of the following brand agents with an available generic equivalent, then the patient has ONE of the following:

Brand	Generic Equivalent
Emflaza	deflazacort

   A. An intolerance or hypersensitivity to the generic equivalent that is not expected to occur with the requested brand agent **OR**
   B. An FDA labeled contraindication to the generic equivalent that is not expected to occur with the requested brand agent **OR**
   C. Support for the use of the requested brand agent over the generic equivalent **AND**
3. The prescriber is a specialist in the area of the patient's diagnosis (e.g., pediatric neurologist), or the prescriber has consulted with a specialist in the area of the patient's diagnosis **AND**
4. The patient does NOT have any FDA labeled contraindications to the requested agent **AND**
5. The requested quantity (dose) does NOT exceed the maximum FDA labeled dose based on the patient's weight

Length of Approval: 6 months for Agamree, 12 months for Emflaza

NOTE: If Quantity Limit applies, please refer to Quantity Limit criteria.

Renewal Evaluation

Target Agent(s) will be approved when ALL of the following are met:

1. The patient has been previously approved for the requested agent through the plan's Prior Authorization process (Note: patients not previously approved for the requested agent will require initial evaluation review) **AND**
2. The patient has had improvements or stabilization with the requested agent (e.g., slowed disease progression, improved strength, timed motor function, pulmonary function; reduced need for scoliosis surgery) **AND**
3. If the request is for one of the following brand agents with an available generic equivalent, then the patient has ONE of the following:

Brand	Generic Equivalent
Emflaza	deflazacort

   A. Has an intolerance or hypersensitivity to the generic equivalent that is not expected to occur with the rquested brand agent **OR**
   B. Has an FDA labeled contraindication to the generic equivalent that is not expected to occur with the reqeusted brand agent **OR**
   C. Support for the use of the requested brand agent over the generic equivalent **AND**
4. The prescriber is a specialist in the area of the patient's diagnosis (e.g., pediatric neurologist), or the prescriber has consulted with a specialist in the area of the patient's diagnosis **AND**
5. The patient does NOT have any FDA labeled contraindications to the requested agent **AND**
6. The requested quantity (dose) does NOT exceed the maximum FDA labeled dose based on the patient's weight

Module	Clinical Criteria for Approval
	Length of Approval: 12 months NOTE: If Quantity Limit applies, please refer to Quantity Limit criteria.

QUANTITY LIMIT CLINICAL CRITERIA FOR APPROVAL

Module	Clinical Criteria for Approval
QL	**Quantity Limit for the Target Agent(s)** will be approved when ONE of the following is met: 1. The requested quantity (dose) does NOT exceed the program quantity limit **OR** 2. The requested agent strength does not have a program quantity limit **OR** 3. The request agent is Emflaza and ONE of the following: A. The requested agent is Emflaza SUSPENSION **OR** B. BOTH of the following: 1. The requested quantity (dose) exceeds the program quantity limit **AND** 2. The requested quantity (dose) cannot be achieved with a lower quantity of any combination of the four Emflaza tablet strengths **OR** 4. ALL of the following: A. The requested quantity (dose) exceeds the program quantity limit **AND** B. The requested quantity (dose) does NOT exceed the maximum FDA labeled dose for the requested indication **AND** C. The requested quantity (dose) cannot be achieved with a lower quantity of a higher strength that does NOT exceed the program quantity limit **Approval Length:** up to 12 months