Clinical Policy: Vamorolone (Agamree)

Reference Number: CP.PHAR.659
Effective Date: 03.01.24
Last Review Date: 02.26
Line of Business: Commercial, HIM, Medicaid Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

Description

Vamorolone (Agamree®) is a corticosteroid.

FDA Approved Indication(s)

Agamree is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.

Policy/Criteria

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of health plans affiliated with Centene Corporation® that Agamree is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Duchenne Muscular Dystrophy (must meet all)**:**

1. Diagnosis of DMD confirmed by one of the following (a or b):
   a. Genetic testing (e.g., dystrophin deletion or duplication mutation found);
   b. If genetic studies are negative (i.e., no mutation identified), positive muscle biopsy (e.g., absence of dystrophin protein);
2. Prescribed by or in consultation with a neurologist;
3. Age ≥ 2 years;
4. Failure of a ≥ 6 month trial of prednisone, unless contraindicated or clinically significant adverse effects are experienced;*
   **For Illinois HIM requests, the step therapy requirement above does not apply as of 1/1/2026 per IL HB 5395*
5. Documentation of member's current weight in kg;
6. Dose does not exceed one of the following (a, b, or c):
   a. 6 mg/kg (up to a maximum of 300 mg) per day;
   b. If member has mild (Child-Pugh A) to moderate (Child-Pugh B) hepatic impairment: 2 mg/kg (up to a maximum of 100 mg) per day;
   c. If co-administered with strong CYP3A4 inhibitors (e.g., itraconazole): 4 mg/kg (up to a maximum of 200 mg) per day.

Approval duration:
Medicaid/HIM – 12 months
Commercial – 12 months or duration of request, whichever is less

B. Other diagnoses/indications (must meet 1 or 2)**:**
1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
   a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or
   b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

II. Continued Therapy

A. Duchenne Muscular Dystrophy (must meet all)**:**
1. Member meets one of the following (a or b):
   a. Currently receiving medication via Centene benefit or member has previously met initial approval criteria;
   b. Member is currently receiving medication and is enrolled in a state and product with continuity of care regulations *(refer to state specific addendums for CC.PHARM.03A and CC.PHARM.03B)*;
2. Member is responding positively to therapy;
3. Documentation of member's current weight in kg;
4. If request is for a dose increase, new dose does not exceed one of the following (a, b, or c):
   a. 6 mg/kg (up to a maximum of 300 mg) per day;
   b. If member has mild (Child-Pugh A) to moderate (Child-Pugh B) hepatic impairment: 2 mg/kg (up to a maximum of 100 mg) per day;
   c. If co-administered with strong CYP3A4 inhibitors (e.g., itraconazole): 4 mg/kg (up to a maximum of 200 mg) per day.

Approval duration:
Medicaid/HIM – 12 months
Commercial – 12 months or duration of request, whichever is less

B. Other diagnoses/indications (must meet 1 or 2)**:**
1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
   a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business:

CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or

b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or

2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key
DMD: Duchenne muscular dystrophy
FDA: Food and Drug Administration

Appendix B: Therapeutic Alternatives
This table provides a listing of preferred alternative therapy recommended in the approval criteria. The drugs listed here may not be a formulary agent for all relevant lines of business and may require prior authorization.

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
prednisone*	0.75 mg/kg/day PO (preferred) <u>Alternative dosing regimens</u> • 0.3 mg/kg/day PO *(lesser efficacy and fewer adverse events)* • 10 mg/kg/weekend PO	Varies based on weight

Therapeutic alternatives are listed as Brand name® (generic) when the drug is available by brand name only and generic (Brand name®) when the drug is available by both brand and generic.
**Off-label*

Appendix C: Contraindications/Boxed Warnings

- Contraindication(s): hypersensitivity to vamorolone or any of the inactive ingredients in Agamree
- Boxed warning(s): none reported

Appendix D: General Information

- Examples of positive response to corticosteroid therapy (e.g., Agamree, Emflaza®, prednisone) include improvement in muscle strength tests (e.g., Medical Research

Council [MRC] scale for muscle strength with 0 being no movement and 5 being normal strength), pulmonary function tests (e.g., forced vital capacity [FVC] and maximal expiratory pressure), walk tests (e.g., 6 minute walk test [6MWT] distance), and timed functional tests (e.g., standing from lying position; climbing 4 stairs; running/walking 30 feet; propelling a wheelchair 30 feet).

- In clinical trials, Agamree demonstrated similar efficacy to prednisone with regard to motor outcomes (e.g., time to stand, time to climb, time to run/walk, 6MWT distance, NorthStar Ambulatory Assessment). Agamree may have an improved safety profile compared to prednisone with regard to bone health; however, current data are insufficient to draw a definitive conclusion due to use of surrogate markers as well as a short study period of 24 weeks.

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
DMD	One of the following: • 6 mg/kg/day (up to a maximum of 300 mg/day) ○ Some patients may respond to a dose of 2 mg/kg/day. Doses may be titrated down as needed, based on individual tolerability • If member has mild (Child-Pugh A) to moderate (Child-Pugh B) hepatic impairment: 2 mg/kg/day (up to a maximum of 100 mg/day) • If co-administered with strong CYP3A4 inhibitors (e.g., itraconazole): 4 mg/kg/day (up to a maximum of 200 mg/day) Patients can be switched from oral corticosteroid treatment (such as prednisone or deflazacort) to Agamree without treatment interruption or period of prior corticosteroid dosage reduction to minimize the risk for adrenal insufficiency	See regimen

VI. Product Availability

Oral suspension: 40 mg/mL

VII. References

1. Agamree Prescribing Information. Coral Gables, FL: Catalyst Pharmaceuticals, Inc.; June 2024. Available at: https://www.agamree.com. Accessed October 21, 2025.
2. Gloss D, Moxley RT, Ashwal S, Oskoui M. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy: Report of the Guideline Development Subcommittee of the American Academy of Neurology. Neurology. 2016;86(5):465-472. doi:10.1212/WNL.0000000000002337. Reaffirmed on January 22, 2022.
3. Bushby K, Finkel R, Birnkrant DJ, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. Lancet Neurol. 2010; 9(1): 77-93.

4. Clinical Pharmacology [database online]. Tampa, FL: Gold Standard, Inc.; 2025. Available at: http://www.clinicalpharmacology-ip.com/.
5. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. Lancet Neurol. 2018; 17: 251-267.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created	11.01.23	02.24
1Q 2025 annual review: no significant changes; references reviewed and updated.	10.31.24	02.25
1Q 2026 annual review: no significant changes; added step therapy bypass for IL HIM per IL HB 5395; extended initial approval duration from 6 to 12 months for this maintenance medication for a chronic condition; references reviewed and updated.	11.03.25	02.26

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. "Health Plan" means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan's affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions, and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment, or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.

This clinical policy is the property of the Health Plan. Unauthorized copying, use, and distribution of this clinical policy or any information contained herein are strictly prohibited. Providers, members, and their representatives are bound to the terms and conditions expressed herein through the terms of their contracts. Where no such contract exists, providers, members and their representatives agree to be bound by such terms and conditions by providing services to members and/or submitting claims for payment for such services.

Note:
For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.