MEDICAL MUTUAL®

Drug Policy

Policy:	Agamree (vamorolone ora suspension)	Annual Review Date: New policy Last Revised Date: 03/20/2024

OVERVIEW

Agamree, a corticosteroid, is indicated for the treatment of **Duchenne muscular dystrophy** (DMD) in patients ≥ 2 years of age.

POLICY STATEMENT

This policy involves the use of Agamree. Prior authorization is recommended for pharmacy benefit coverage of Agamree. Approval is recommended for those who meet the conditions of coverage in the **Criteria and Initial/Extended Approval** for the diagnosis provided. **Conditions Not Recommended for Approval** are listed following the recommended authorization criteria. Requests for uses not listed in this policy will be reviewed for evidence of efficacy and for medical necessity on a case-by-case basis.

Because of the specialized skills required for evaluation and diagnosis of patients treated with Agamree as well as the monitoring required for adverse events and long-term efficacy, initial approval requires Agamree be prescribed by or in consultation with a physician who specializes in the condition being treated. All approvals for initial therapy are provided for the initial approval duration noted below; if reauthorization is allowed, a response to therapy is required for continuation of therapy unless otherwise noted below.

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Agamree is recommended in those who meet the following criteria:

FDA-Approved Indication

1. **Duchenne Muscular Dystrophy.** Approve for 1 year if the patient meets one of the following (A or B):
   - **A)** Initial Therapy. Approve if the patient meets the following (i, ii, iii, and iv):
     - **i.** Patient is ≥ 2 years of age; AND
     - **ii.** Patient's diagnosis of Duchenne Muscular Dystrophy is confirmed by one of the following (a or b) **[documentation required]**:
       - **a)** Genetic testing with a confirmed pathogenic variant in the dystrophin gene; OR
       - **b)** Muscle biopsy showing the absence of, or marked decrease in, dystrophin protein; AND
     - **iii.** Patient meets ONE of the following (a or b):

a) Patient has tried prednisone or prednisolone for ≥ 6 months **[documentation required]** AND according to the prescriber, the patient has had at least one of the following significant intolerable adverse effects [1, 2, 3, or 4]:
  1) Cushingoid appearance **[documentation required]**; OR
  2) Central (truncal) obesity **[documentation required]**; OR
  3) Undesirable weight gain defined as ≥ 10% body weight increase over a 6-month period **[documentation required]**; OR
  4) Diabetes and/or hypertension that is difficult to manage according to the prescriber **[documentation required]**; OR

b) According to the prescriber, the patient has experienced a severe behavioral adverse event while on prednisone or prednisolone therapy that has or would require a prednisone or prednisolone dose reduction **[documentation required]**.

iv. The medication is prescribed by or in consultation with a physician who specializes in the treatment of Duchenne muscular dystrophy and/or neuromuscular disorders.

B) Patient is Currently Receiving Agamree. Approve if the patient meets the following (i, ii, iii, and iv):

i. Patient is ≥ 2 years of age; AND

ii. Patient has tried prednisone or prednisolone **[documentation required]**; AND

iii. According to the prescriber, the patient has responded to or continues to have improvement or benefit from Agamree therapy **[documentation required]**; AND
Note: Examples of improvement or benefit from Agamree therapy would include improvements in motor function (e.g., time from supine to standing, time to climb four stairs, time to run or walk 10 meters, 6-minute walk test), improvement in muscle strength, and improved pulmonary function.

iv. The medication is prescribed by or in consultation with a physician who specializes in the treatment of Duchenne muscular dystrophy and/or neuromuscular disorders.

Initial Approval/ Extended Approval.
A) *Initial Approval:* 1 year
B) *Extended Approval:* 1 year

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Agamree has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions. (Note: This is not an exhaustive list of Conditions Not Recommended for Approval).

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

Documentation Requirements:

The Company reserves the right to request additional documentation as part of its coverage determination process. The Company may deny reimbursement when it has determined that the drug provided or services performed were not medically necessary, investigational, or experimental, not within the scope of benefits afforded to the member and/or a pattern of billing or other practice has been found to be either inappropriate or excessive. Additional documentation supporting medical necessity for the services provided must be made available upon request to the Company. Documentation requested may include patient records, test results and/or credentials of the provider ordering or performing a service. The Company also reserves the right to modify, revise, change, apply and interpret this policy at its sole discretion, and the exercise of this discretion shall be final and binding.

REFERENCES

1. Agamree® oral suspension [prescribing information]. Burlington, MA: Santhera/Catalyst; October 2023.
2. 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(3):251-267.
3. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 2: respiratory, cardiac, bone health, and orthopaedic management. *Lancet Neurol*. 2018;17(4):347-361.
4. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 3: primary care, emergency medicine, psychological care, and transitions of care across the lifespan. *Lancet Neurol*. 2018;17(5):445-455.
5. Gloss D, Moxley RT III, 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.
6. Summary of Practice Guidelines for Clinicians. Practice Guideline Update: Corticosteroid Treatment of Duchenne Muscular Dystrophy. Available at: https://www.aan.com/Guidelines/Home/GuidelineDetail/731. Accessed on November 7, 2023.