PRIOR AUTHORIZATION POLICY

POLICY: Muscular Dystrophy – Agamree Prior Authorization Policy
- Agamree™ (vamorolone oral suspension – Santhera/Catalyst)

REVIEW DATE: 01/10/2024; selected revision 07/03/2024

OVERVIEW

Agamree, a corticosteroid, is indicated for the treatment of **Duchenne muscular dystrophy** (DMD) in patients ≥ 2 years of age.[1]

Disease Overview

DMD is a rare, progressive X-linked disease resulting from mutation(s) of the DMD gene, also known as the Dystrophin gene.[2,3] Due to the mutation(s), the dystrophin protein, which is key for maintaining the structural integrity of muscle cells, is not produced or very minimally produced. Since this is an X-linked mutation, DMD almost exclusively impacts young boys. DMD is a progressive muscle-weakening disease that affects skeletal, respiratory, and cardiac muscles. It is usually diagnosed in the second or third year of life. Due to progressive decline, most patients die of cardiac or respiratory complications in the third or fourth decade of life. The incidence of DMD in the US is approximately 1 in 5,000 live male births.

Guidelines

Agamree is not addressed in guidelines. Guidelines from the DMD Care Considerations Working Group (2018) state that glucocorticoids and physical therapy are the mainstays of treatment for DMD.[2-6] Both therapies should be continued after the patient loses ambulation. Guidelines for the use of corticosteroids in DMD are available from the American Academy of Neurology (AAN) [2016, reaffirmed January 2022].[4] The AAN notes that in patients with DMD, prednisone should be used to improve strength and pulmonary function (moderate evidence). Emflaza™ (deflazacort tablets and oral suspension) and prednisone may be used to improve timed motor function, reduce the need for scoliosis surgery, and to delay the onset of cardiomyopathy by 18 years of age (weak evidence). Emflaza may also be used to improve pulmonary function and to delay the age at loss of ambulation by 1.4 to 2.5 years (weak evidence). There is insufficient evidence to support or refute the benefit of prednisone on survival (insufficient evidence). Emflaza may be used to increase survival at 5 and 15 years of follow-up (weak evidence).

POLICY STATEMENT

Prior Authorization is recommended for prescription benefit coverage of Agamree. All approvals are provided for the duration noted below. 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, approval requires Agamree to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Documentation: Documentation is required for use of Agamree as noted in the criteria as **[documentation required]**. Documentation may include, but is not limited to, chart notes, prescription claims records, prescription receipts, and/or other information.

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 ALL of 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 genetic testing with a confirmed pathogenic variant in the dystrophin gene **[documentation required]**; 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 ALL of 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.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Agamree is not recommended in the following situations:

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

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.