CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Marketplace

DRUG NAME	Agamree (vamorolone)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Agamree, approved by the FDA in 2023, is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.
DMD is an X-linked, progressive disease characterized by muscle wasting, weakness, loss of walking ability, and reduced life expectancy. It is caused by mutations in the dystrophin gene resulting in reduced or near absence of dystrophin, a protein that helps keep muscle cells intact.
Delaying loss of ambulation is a major goal of treatment. Corticosteroids are standard of care to improve muscle strength and function in DMD and may prolong walking ability. In the phase 2b VISION-DMD clinical trial, Agamree showed similar efficacy as prednisone, but less decline in height percentile and bone turnover markers.

Agamree (vamorolone) will be considered for coverage when the following criteria are met:

Duchenne Muscular Dystrophy (DMD)

For **initial** authorization:
1. Member is at least 2 years of age; AND
2. Medication must be prescribed by or in consultation with a neurologist or neuromuscular specialist; AND
3. Member has a diagnosis of DMD confirmed by genetic testing that shows *DMD* gene loss-of-function variation, or absence of muscle dystrophin on muscle biopsy; AND
4. Member has documentation of trial and failure of prednisone for at least 6 months; AND
5. Member's weight is documented in chart notes.
6. **Dosage allowed/Quantity limit:** Oral suspension; 6 mg/kg once daily. Max daily dose of 300 mg if >50 kg.

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:
1. Chart notes must show stability or slowed rate of decline of the member's motor function and muscle strength.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Agamree (vamorolone) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
10/31/2023	New policy for Agamree created.

References:

1. Agamree [prescribing information]. Santhera Pharmaceuticals (USA), Inc.; 2023.
2. Guglieri M, Clemens PR, Perlman SJ, et al. Efficacy and Safety of Vamorolone vs Placebo and Prednisone Among Boys With Duchenne Muscular Dystrophy: A Randomized Clinical Trial. *JAMA Neurol*. 2022;79(10):1005-1014. doi:10.1001/jamaneurol.2022.2480
3. Mah JK, Clemens PR, Guglieri M, et al. Efficacy and Safety of Vamorolone in Duchenne Muscular Dystrophy: A 30-Month Nonrandomized Controlled Open-Label Extension Trial. *JAMA Netw Open*. 2022;5(1):e2144178. Published 2022 Jan 4. doi:10.1001/jamanetworkopen.2021.44178
4. Matthews E, Brassington R, Kuntzer T, Jichi F, Manzur AY. Corticosteroids for the treatment of Duchenne muscular dystrophy. *Cochrane Database Syst Rev*. 2016;2016(5):CD003725. Published 2016 May 5. doi:10.1002/14651858.CD003725.pub4
5. Gloss D, Moxley RT 3rd, 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
6. 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 [published correction appears in Lancet Neurol. 2018 Apr 4;:]. *Lancet Neurol*. 2018;17(3):251-267. doi:10.1016/S1474-4422(18)30024-3

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