AETNA BETTER HEALTH®
Coverage Policy/Guideline

Name:	Agamree (vamorolone)	Page:	1 of 3
Effective Date:	10/21/2025	Last Review Date:	9/18/2025
Applies to:	☒Maryland ☒Florida Kids ☒Pennsylvania Kids ☐Virginia		

Intent:

The intent of this policy/guideline is to provide information to the prescribing practitioner outlining the coverage criteria for Agamree under the patient's prescription drug benefit.

Description:

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

All other indications are considered experimental/investigational and not medically necessary.

Applicable Drug List:

Agamree

Policy/Guideline:

Documentation
Submission of the following information is necessary to initiate the prior authorization review:

A. Laboratory confirmation of the DMD diagnosis by genetic testing or muscle biopsy.
B. Chart documentation of weight gain/obesity, persistent psychiatric/behavioral issues, and/or growth stunting with previous prednisone or prednisolone or deflazacort treatment (where applicable).

Prescriber Specialties
This medication must be prescribed by or in consultation with a physician who specializes in the treatment of Duchenne muscular dystrophy (DMD).

Criteria for Initial Approval

Duchenne Muscular Dystrophy
Authorization may be granted for the treatment of DMD when ALL the following criteria are met:

A. The diagnosis of DMD was confirmed by EITHER of the following:
    1. Genetic testing documenting a mutation in the DMD gene.
    2. Muscle biopsy documenting absent dystrophin.
B. The member is 2 years of age or older.
C. The member meets ONE of the following criteria:
    1. Member has experienced unmanageable and/or clinically significant weight gain/obesity as evidenced by body mass index in the overweight or obese category while receiving treatment with prednisone or prednisolone (refer to Appendix for weight status categories for children and adults).

2. Member has experienced unmanageable and/or clinically significant psychiatric/behavioral issues (e.g., abnormal behavior, aggression, irritability) that persisted beyond the first 6 weeks of treatment with prednisone or prednisolone.
3. Member has experienced clinically significant growth stunting while receiving treatment with prednisone, prednisolone, or deflazacort as evidenced by ANY of the following:
    i. Decline in mean height percentile for age from baseline
    ii. Decrease in growth trajectory and/or growth velocity
    iii. Reduction in serum biomarkers of bone formation (e.g., osteocalcin, procollagen 1 intact N-terminal propeptide [P1NP]) and/or bone turnover (e.g., type 1 collage cross-linked C-telopeptide [CTX1]).

Criteria for Continuation of Therapy

Authorization may be granted for members requested continuation of therapy when ALL the following criteria are met:

A. The member meets all initial authorization criteria.
B. The member is receiving a clinical benefit from Agamree therapy (e.g., improvement or stabilization in muscle strength and/or motor function)

Appendix

Body Mass Index Percentile & Weight Status Category for Children 2 - 19 Years of Age

Body Mass Index Percentile Range	**Weight Status**
Less than the 5th percentile	Underweight
5th percentile to less than the 85th percentile	Healthy Weight
85th to less than the 95th percentile	Overweight
Equal to or greater than the 95th percentile	Obese

Body Mass Index & Weight Status Category for Adults (20 Years of Age and Older)

Body Mass Index	**Weight Status**
Below 18.5	Underweight
18.5 – 24.9	Healthy Weight
25.0 – 29.9	Overweight
30.0 and Above	Obese

Approval Duration and Quantity Restrictions:

Initial Approval: 6 months

Renewal Approval: 12 months

Quantity Level Limit:

Medication	Standard Limit	FDA-recommended dosing
Agamree (vamorolone) 40 mg/mL oral suspension (100 mL per bottle)	3 bottles (300 mL) per 30 days	The recommended dosage is 6 mg/kg taken orally once daily, up to a maximum daily dosage of 300 mg for patients weighing more than 50 kg.

References:

1. Agamree [package insert]. Burlington, MA: Santhera Pharmaceuticals (USA) Inc.; June 2024.
2. Smith EC, Conklin LS, Hoffman EP, et al. Efficacy and safety of vamorolone in Duchenne muscular dystrophy: An 18-month interim analysis of a non-randomized open-label extension study. PLoS Med. 2020;17(9):e1003222. Published 2020 Sep 21.
3. 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.
4. Centers for Disease Control and Prevention. Assessing Your Weight. https://www.cdc.gov/healthyweight/assessing/bmi/ Accessed March 11, 2025.
5. CVS Caremark National P&T Committee. Duchenne Muscular Dystrophy (DMD) Agents. May 29, 2024.