

SPECIALTY GUIDELINE MANAGEMENT

AGAMREE (vamorolone)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

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.

II. 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, prednisolone, or deflazacort treatment (where applicable).

III. PRESCRIBER SPECIALTIES

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

IV. CRITERIA FOR INITIAL APPROVAL

Duchenne Muscular Dystrophy

Authorization of 6 months may be granted for the treatment of DMD when all of 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. Member is 2 years of age or older.
- C. 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) 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 collagen cross-linked C-telopeptide [CTX1]).

V. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for members requesting continuation of therapy when all of the following criteria are met:

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

VI. APPENDIX

Body Mass Index Percentile and Weight Status Category for Children 2 Through 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 and 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

VII. REFERENCES

- Agamree [package insert]. Burlington, MA: Santhera Pharmaceuticals (USA) Inc.; October 2023.
- 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.
- 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.
- Centers for Disease Control and Prevention. Assessing Your Weight. <https://www.cdc.gov/healthyweight/assessing/bmi/> Accessed March 1, 2024.