Pharmacy Policy Bulletin: J-1362 Agamree (vamorolone) – Commercial and Healthcare Reform	
Number: J-1362	**Category:** Prior Authorization
Line(s) of Business: ☒ Commercial ☒ Healthcare Reform ☐ Medicare	**Benefit(s):** **Commercial:** **Prior Authorization (1.):** **1.** Miscellaneous Specialty Drugs Oral = Yes w/ Prior Authorization **Healthcare Reform:** Not Applicable
Region(s): ☒ All ☐ Delaware ☐ New York ☐ Pennsylvania ☐ West Virginia	**Additional Restriction(s):** None
Version: J-1362-001	**Original Date:** 12/06/2023
Effective Date: 02/16/2024	**Review Date:** 12/06/2023

Drugs Product(s):	• Agamree (vamorolone)
FDA-Approved Indication(s):	• Treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older

Background:	• Agamree is a corticosteroid that acts through the glucocorticoid receptor to exert anti-inflammatory and immunosuppressive effects. The precise mechanism of action in DMD is unknown. • DMD is a genetic disorder characterized by progressive muscle degeneration and weakness. It is caused by a mutation in the *DMD* gene resulting in a lack of functional dystrophin protein. DMD primarily affects boys and symptom onset is usually between the ages of 2 and 3. Symptoms progress from difficulty walking to impaired cardiac and respiratory function. Life expectancy may range from teens to early 30s. The prevalence is approximately 6 per 100,000 individuals. • In the clinical trial for Agamree, height percentile declined and linear growth delay occurred in prednisone-treated patients but not Agamree treated patients. All patients in the clinical trial for Agamree were ambulatory. • Prescribing Considerations: o Agamree has warnings or precautions for alterations in endocrine function, immunosuppression and increased risk of infection, alterations in cardiovascular/renal function, gastrointestinal perforation, behavioral and mood disturbances, effects on bones, ophthalmic effects, administration of live or live attenuated vaccines, effects on growth and development, myopathy, Kaposi's sarcoma, thromboembolic events, and anaphylaxis.

Approval Criteria

I. Initial Authorization

When a benefit, coverage of Agamree may be approved when all of the following criteria are met **(A. through E.)**:

A. The member has a diagnosis of DMD (ICD-10: G71.01).
B. The diagnosis of DMD has been confirmed by a mutation of the dystrophin gene.
C. The member is ambulatory.
D. The medication is being prescribed by or in consultation with a physician who specializes in treating neuromuscular disorders (e.g., neurologist).
E. The member has experienced a delay or decline in growth while on prednisone that is not expected to occur with Agamree.

II. Reauthorization

When a benefit, reauthorization of Agamree may be approved when the following criterion is met **(A.)**:

A. The prescriber attests that the member has experienced positive clinical response to therapy.

III. An exception to some or all of the criteria above may be granted for select members and/or circumstances based on state and/or federal regulations.

Limitations of Coverage

I. Coverage of drug(s) addressed in this policy for disease states outside of the FDA-approved indications should be denied based on the lack of clinical data to support effectiveness and safety in other conditions unless otherwise noted in the approval criteria.
II. For Commercial or HCR members with a closed formulary, a non-formulary product will only be approved if the member meets the criteria for a formulary exception in addition to the criteria outlined within this policy.

Authorization Duration

- Commercial and HCR Plans: If approved, up to a 12 month authorization may be granted.

Automatic Approval Criteria

None

References:

1. Agamree [package insert]. Burlington, MA: Santhera Pharmaceuticals, Inc.; October 2023.
2. Muscular Dystrophy Association. Duchenne Muscular Dystrophy. Available at: https://www.mda.org/disease/duchenne-muscular-dystrophy. Accessed November 6, 2023.
3. Gloss D, Moxley R, Ashwal S, et al. Practice guideline update summary: Corticosteroid treatment of Duchenne muscular dystrophy. Report of the Guideline Development Subcommittee of the American Academy of Neurology. Neurology. Feb 2016, 86 (5) 465-472.
4. 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 Oct 1;79(10):1005-1014.