BlueCross BlueShield
Federal Employee Program

AGAMREE
(vamorolone)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Agamree (vamorolone) is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD). Specifically, Agamree acts through the glucocorticoid receptor to exert anti-inflammatory and immunosuppressive effects. The precise mechanism by which Agamree exerts its therapeutic effects in patients with DMD is unknown (1).

Regulatory Status

FDA-approved indication: Agamree is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older (1).

Agamree, can cause serious and life-threatening alterations in endocrine function, especially with chronic use. Monitor patients for Cushing's syndrome, hyperglycemia, and adrenal insufficiency after Agamree withdrawal. In addition, patients with hypopituitarism, primary adrenal insufficiency or congenital adrenal hyperplasia, altered thyroid function, or pheochromocytoma may be at increased risk for adverse endocrine events (1).

Agamree can suppress the immune system and increase the risk of infection with any pathogen, including viral, bacterial, fungal, protozoan, or helminthic. Corticosteroids reduce resistance to new infections, exacerbate existing infections, increase the risk of disseminated infections, increase the risk of reactivation or exacerbation of latent infections, and mask some signs of infection (1). Agamree may affect cardiovascular and renal function, gastrointestinal perforation, behavior and mood, bone mineral density, and ophthalmic health. Blood pressure, serum potassium, signs of gastrointestinal perforation, mood, bone mineral density, and intraocular pressure should be monitored (1).

Agamree had lower rates of bone turnover versus prednisone. In clinical studies, there was a significant improvement in linear growth after crossover in the prednisone to Agamree group, and rapid reversal of prednisone-induced decline in bone turnover biomarkers in both crossover groups (2).

All immunizations should be administered according to immunization guidelines prior to starting

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Agamree. Live or live-attenuated vaccines should be administered at least 4 to 6 weeks prior to starting Agamree. Patients on Agamree may receive concurrent vaccinations, except for live or live-attenuated vaccines (1).

Monitoring motor changes in patients with DMD requires functional evaluation along with measurement of muscle strength. The need for a reliable outcome measure in diseases of rapid deterioration such as DMD has led to the use of motor functional tests. In a large, multicenter, international clinical trial, the six minute walk test (6MWT) proved to be feasible and highly reliable. Also used are the Motor Function Measure (MFM) and North Star Ambulatory Assessment (NSAA) to help predict loss of ambulation 1 year before its occurrence in order to allow time to adapt rehabilitation, change the patient's environment, and consider acquisition of assistive aids or the use of medications (3-5).

Safety and effectiveness in pediatric patients below the age of 2 years have not been established (1).

Summary

Agamree (vamorolone) is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD). Agamree acts through the glucocorticoid receptor to exert anti-inflammatory and immunosuppressive effects. The most common adverse reactions are changes in endocrine function, cardiovascular and renal function, gastrointestinal perforation, behavior and mood, bone mineral density, and ophthalmic health. Safety and effectiveness in patients less than 2 years of age have not been established (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Agamree while maintaining optimal therapeutic outcomes.

References

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3. Mcdonald C, Henricson E, et al. The 6-Minute Walk test and Other Clinical Endpoints in Duchenne Muscular Dystrophy: Reliability, Concurrent Validity, and Minimal Clinically Important Differences from a Multicenter Study. Muscle Nerve. 2013 Sep; 48(3): 357–368.
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