aGamree® (vamorolone) oral suspension 40mg/mL

DMD Patient Profile

Mateo

Age 7 years

Parents Are Concerned With Current Corticosteroid Therapy

Hypothetical patient.

Disease history

- Mateo received a diagnosis of DMD at 4 years of age
- His signs and symptoms include:
  - Mild delays in motor function[1]
  - Began walking at age 14 months and noticeably walks on his toes[1,2]
- Type of gene mutation: exon 53 deletion

Treatment history

- Mateo has tried a variety of steroid regimens since diagnosis. Currently, Mateo is on a regimen including steroids dosed on weekend days
- His current treatment regimen also includes golodirsen injection, which was initiated at age 6.5 years[3]

Current status

- Mateo walks independently but gets tired or complains of pain in his legs after he walks longer distances[1,4]
- He has maintained a normal weight
- He has experienced continued behavioral issues on steroids despite a modified dosing schedule
  - Specifically, Mateo presents with aggression, hitting other children at school. This has resulted in Mateo being suspended from school on several occasions

Primary treatment goals

- Slow the progression of weakness
- Maintain motor function
- Manage behavioral adverse effects of treatment

Other considerations

- Mateo's parents are frustrated with his behavior on weekend dosing of steroids
- Due to the behavioral adverse effects associated with corticosteroid use, Mateo's parents were uncomfortable adding a new therapy, which resulted in a delay in the initiation of golodirsen

DMD, Duchenne muscular dystrophy.

INDICATIONS AND USAGE

AGAMREE is a corticosteroid indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.

IMPORTANT SAFETY INFORMATION

Contraindications

AGAMREE is contraindicated in patients with known hypersensitivity to vamorolone or any of the inactive ingredients in AGAMREE.

Warnings & Precautions

- **Alterations in Endocrine Function:** Corticosteroids, such as AGAMREE, can cause serious and life-threatening alterations in endocrine function, especially with chronic use. Monitor patients receiving AGAMREE 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. Acute adrenal insufficiency can occur if AGAMREE is withdrawn abruptly, and could be fatal. The risk of adrenal insufficiency is reduced by gradually tapering the dose when withdrawing treatment. For patients already taking corticosteroids during times of stress, the dosage may need to be increased.

ADDITIONAL IMPORTANT SAFETY INFORMATION

Warnings & Precautions

- **Immunosuppression and Increased Risk of Infection:** Use of corticosteroids, including AGAMREE, increases the risk of new infection, exacerbation of existing infections, dissemination, and reactivation or exacerbation of latent infection and may mask some signs of infection; these infections can be severe, and at times fatal. Tell patients and/or caregivers to inform their healthcare provider if the patient has had recent or ongoing infections or has recently received a vaccine. Advise patients taking AGAMREE to avoid exposure to chickenpox or measles and to alert their healthcare provider immediately if they are exposed.
- **Alterations in Cardiovascular/Renal Function:** Monitor for elevated blood pressure and monitor sodium and potassium levels in patients chronically treated with AGAMREE.
- **Gastrointestinal Perforation:** Use of corticosteroids increases the risk of gastrointestinal perforation in patients with certain gastrointestinal disorders, such as active or latent peptic ulcers, diverticulitis, fresh intestinal anastomoses, and non-specific ulcerative colitis. Signs and symptoms of gastrointestinal perforation may be masked.
- **Behavioral and Mood Disturbances:** Potentially severe psychiatric adverse reactions may occur with systemic corticosteroids, including AGAMREE, and may include hypomanic or manic symptoms (eg, euphoria, insomnia, mood swings) during treatment and depressive episodes after discontinuation of treatment. Encourage patients to seek medical attention if psychiatric symptoms develop.
- **Effects on Bones:** Prolonged use of corticosteroids, such as AGAMREE, can lead to osteoporosis, which can predispose patients to vertebral and long bone fractures. Monitor bone mineral density in patients on long-term treatment with AGAMREE.
- **Ophthalmic Effects:** The use of corticosteroids, such as AGAMREE, may increase the risk of cataracts, ocular infections, and glaucoma. Monitor intraocular pressure if treatment with AGAMREE is continued for more than 6 weeks.
- **Vaccination:** Do not administer live-attenuated or live vaccines to patients receiving AGAMREE. Administer live-attenuated or live vaccines at least 4 to 6 weeks prior to starting AGAMREE.
- **Effects on Growth and Development:** Long-term use of corticosteroids, including AGAMREE, can have negative effects on growth and development in children.
- **Thromboembolic Events:** Observational studies have shown an increased risk of thromboembolism. Use AGAMREE with caution in patients who have or may be predisposed to thromboembolic disorders.

Adverse Reactions

The most common adverse reactions (>10% for AGAMREE and greater than placebo) are cushingoid features, psychiatric disorders, vomiting, weight increased, and vitamin D deficiency.

Use in Specific Populations

- In patients with mild to moderate hepatic impairment, the recommended daily dose of AGAMREE is 2 mg/kg, preferably with a meal, up to a maximum daily dosage of 100 mg for patients weighing more than 50 kg.
- When used concomitantly with strong CYP3A4 inhibitors, the maximum recommended daily dose of AGAMREE is 4 mg/kg, preferably with a meal, up to a maximum daily dosage of 200 mg for patients weighing more than 50 kg.
- The safety and effectiveness of AGAMREE have not been established in pediatric patients below the age of 2 years.

Please see full Prescribing Information for additional Important Safety Information.

To report SUSPECTED ADVERSE REACTIONS, contact Catalyst Pharmaceuticals, Inc. at 1-844-347-3277 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

LEARN MORE AT **AGAMREEhcp.com**

References

1. Duan D, Goemans N, Takeda S, Mercuri E, Aartsma-Rus A. Duchenne muscular dystrophy. *Nat Rev Dis Primers.* 2021;7(1):13. **2.** Ohlendieck K, Swandulla D. Complexity of skeletal muscle degeneration: multi-systems pathophysiology and organ crosstalk in dystrophinopathy. *Pflugers Arch.* 2021;473(12):1813-1839. **3.** VYONDYS 53 (golodirsen) Injection [prescribing information]. Sarepta Therapeutics, Inc.; 2021. **4.** Henricson EK, Abresch RT, Cnaan A, et al. The Cooperative International Neuromuscular Research Group Duchenne Natural History Study: glucocorticoid treatment preserves clinically meaningful functional milestones and reduces rate of disease progression as measured by manual muscle testing and other commonly used clinical trial outcome measures. *Muscle Nerve.* 2013;48(1):55-67.

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