Agamree® (vamorolone) – New orphan drug approval

- On October 26, 2023, Catalyst Pharmaceuticals announced the FDA approval of Agamree (vamorolone), for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.

- DMD is a rare and neuromuscular disorder characterized by progressive muscle dysfunction, ultimately leading to loss of ambulation, respiratory failure, and death.

    — It is estimated that between 11,000 and 13,000 patients in the U.S. are affected by DMD, with approximately 70% of patients currently receiving concomitant corticosteroid treatment.

- Agamree is a corticosteroid that acts through the glucocorticoid receptor to exert antiinflammatory and immunosuppressive effects. The precise mechanism by which Agamree exerts its effect in patients with DMD is unknown.

- The efficacy of Agamree was established in a randomized, double-blind, placebo- and active-controlled study in 121 male patients with DMD. Patients were randomized to one of the following treatment groups: Agamree 6 mg/kg/day, Agamree 2 mg/kg/day, prednisone 0.75 mg/kg/day, or placebo for 24 weeks. The primary endpoint was the change from baseline to week 24 in Time to Stand Test (TTSTAND) velocity for Agamree 6 mg/kg/day compared to placebo. TTSTAND velocity is a measure of muscle function that measures the time required for the patient to stand to an erect position from a supine position (floor).

    — The mean change from baseline in TTSTAND velocity (rises/sec) at week 24 was 0.048 and -0.012 for Agamree 6 mg/kg/day and placebo, respectively (treatment difference 0.060, 95% CI: 0.023, 0.098; p = 0.002).
    — Key secondary endpoints were also met for the Agamree 6 mg/kg/day treatment group. Refer to the drug label for complete study results.

- Warnings and precautions for Agamree include 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; immunizations; effects on growth and development; myopathy; Kaposi's Sarcoma; thromboembolic events; and anaphylaxis.

- The most common adverse reactions (> 10% for Agamree and greater than placebo) with Agamree use were cushingoid features, psychiatric disorders, vomiting, weight increased, and vitamin D deficiency.

- The recommended dosage of Agamree is 6 mg/kg taken orally once daily preferably with a meal, up to a maximum daily dosage of 300 mg for patients weighing more than 50 kg.

    — Some patients may respond to a dose of 2 mg/kg daily. Doses may be titrated down to 2 mg/kg/day as needed, based on individual tolerability.
    — Patients can be switched from oral corticosteroid treatment (such as prednisone or deflazacort) to Agamree without treatment interruption or period of prior corticosteroid dosage reduction to minimize the risk for adrenal insufficiency. Patients switching after long term treatment with oral corticosteroids should start Agamree at a dosage of 6 mg/kg/day.

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- Catalyst plans to launch Agamree in the first quarter 2024. Agamree will be available as a 40 mg/mL oral suspension.
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