October 26, 2023

AGAMREE® (vamorolone) FAQ

- **What is AGAMREE®?**
  - AGAMREE® (vamorolone) is a first-in-class drug that works similarly to corticosteroids (anti-inflammatory medications used to help maintain muscle strength). AGAMREE® hopes to retain the beneficial anti-inflammatory and muscle strengthening aspects of corticosteroids, while decreasing some of the undesirable side effects (bone fragility, stunted growth, insulin resistance, mood changes, delay of puberty and others).

- **Is my child eligible for AGAMREE®?**
  - AGAMREE® is approved for patients ages 2 years and older with Duchenne muscular dystrophy.

- **How does my child get access to AGAMREE®?**
  - If you want to know if your child is eligible to receive AGAMREE® and if your neuromuscular center is able to prescribe it, call your neuromuscular team, who will be able to provide you with the most appropriate information on next steps.
  - If your child is eligible and approved for AGAMREE® your neuromuscular provider will send a prescription for AGAMREE® to a specialty pharmacy, where the prescription will be filled and shipped to your home.
  - At this time of this writing, we anticipate that AGAMREE® will become accessible to patients in the first quarter of 2024.

- **How soon will my child be able to access AGAMREE®?**
  - Now that the FDA has approved AGAMREE®, the drug company responsible for its commercialization, Catalyst Pharmaceuticals,will work to make the drug available to individuals who wish to take it. The process of commercialization can take weeks to months. PPMD will update the community as new information becomes available to facilitate the quickest access possible to this and other approved therapies.

- **How is AGAMREE® administered?**
  - AGAMREE® is administered as an oral suspension (40mg/ml) at 6 mg/kg, given once daily with a meal.

- **What is the most important information I should know about AGAMREE®?**
  - While AGAMREE® is considered a dissociative steroid, it's important to understand the potential side effects of treatment. Be sure to talk with your neuromuscular team about risks, benefits, and precautions before starting any medications.

- **What are possible side effects of AGAMREE®?**

    - The most common side effects include:
        - Cushingoid features (round face, increased fat around base of neck) insomnia, mood swings, vomiting, weight increased, and vitamin D deficiency.
        - Patients should be monitored for other common steroid side effects when taking AGAMREE® such as changes in endocrine function, immunosuppression, effects on bone health, ophthalmic effects, effects on growth, and high blood pressure. For a full list of side effects and precautions, please see AGAMREE® Prescribing Label.

- **What precautions are needed while taking AGAMREE®?**
    - Because AGAMREE® is a type of steroid, it suppresses the adrenal glands. Because of this, suddenly discontinuing AGAMREE® or any other steroid can be extremely dangerous and cause adrenal crisis, which can be life threatening. Talk to your neuromuscular doctor about what to do if you run out of medication, or are too sick to take your medication. Learn more about adrenal suppression, adrenal crisis, when stress dosing is needed, and more in PJ's Protocol.
    - **Note: while PJ's Protocol has not yet been updated to reflect the approval of AGAMREE®, these important concepts remain true. Speak with your neuromuscular physician about stress dosing, steroid conversion, and other considerations before starting AGAMREE®. PPMD looks forward to updating PJ's Protocol as soon as more information is available.
    - AGAMREE® can also suppress the immune system. This could affect your child's ability to receive live-attenuated vaccines. Be sure to talk to your neuromuscular doctor and primary care provider about your options**.**

- **How often will I need to follow up with my doctor?**
    - It's important to talk to your neuromuscular provider before starting AGAMREE® to understand if more frequent visits or labs will be needed.

- **Will insurance cover AGAMREE®?**
    - Because it has only recently been approved, insurance companies are still evaluating their coverage policies for AGAMREE®. If you have questions about whether your insurance covers AGAMREE®, you can call your insurance company directly to ask about their coverage policy. PPMD is closely tracking insurance plans and will provide updates and support as additional information becomes available.
    - PPMD has resources to support you as you navigate insurance coverage benefits and reimbursements. Schedule time with the PPMD team to understand your insurance benefits through the PPMD For You "Navigating Benefits and Resources" appointment type.

- **What if my child is not eligible for AGAMREE®?**

- AGAMREE® is currently approved for individuals with Duchenne age 2 years and older. Clinical studies are underway in individuals with Becker to support a broader label in the future.
- AGAMREE® is not dependent upon genetic variants or other factors like ambulation.
- PPMD has genetic counselors and clinical experts on staff who can help you understand your child's genetic variant and which types of treatments may be available. Email the PPMD genetic counselors at coordinator@parentprojectmd.org or the PPMD care team at careteam@parentprojectmd.org.

- **What clinical studies are ongoing or planned for AGAMREE®?**
  - If you are interested in clinical trials or want to know more about actively recruiting trials, visit clinicaltrials.gov, PPMD's Clinical Trials site, or reach out to one of PPMD's genetic counselors (coordinator@parentprojectmd.org).

- **Where can I get more answers to my questions about AGAMREE®?**
  - If you have more questions about AGAMREE®, talk to your neuromuscular provider. If you have nonmedical questions about AGAMREE®, you can visit www.yourcatalystpathways.com or call 1-833-422-8259.
  - If you have general questions about approved or investigational therapies in Duchenne and whether your child is eligible, contact the PPMD Genetic Counselors at coordinator@parentprojectmd.org.
  - If you have questions about access, navigating care, or are looking for other types of individualized support, connect with the PPMD Team directly through PPMD for You.