# **AGAMREE** (vamorolone) FAQ

September 2025

#### • What is AGAMREE?

 AGAMREE (vamorolone) is a type of oral <u>corticosteroids</u> (anti-inflammatory medications used to help maintain muscle strength) for people with Duchenne muscular dystrophy.

# • How does it work?

 AGAMREE hopes to retain the beneficial anti-inflammatory and muscle strengthening aspects of <u>corticosteroids</u>, while decreasing some of the undesirable side effects (bone fragility, stunted growth, insulin resistance, mood changes, delay of puberty and others).

#### How is AGAMREE administered?

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

# • Who is eligible for AGAMREE?

- AGAMREE is <u>approved</u> for people ages 2 years and older with Duchenne muscular dystrophy.
- o AGAMREE is not dependent upon genetic variants or other factors like ambulation.

# Important things to know about AGAMREE:

- Because AGAMREE is a type of <u>steroid</u>, it's important to never stop taking or change the amount taken, without first checking with your healthcare provider.
- AGAMREE cannot be used to stress dose. If you take AGAMREE, you should have a
  prescription of prednisone on hand for situations when stress dosing is needed.
  - AGAMREE (vamorolone) Information Sheet for Families
  - <u>Discussing Vamorolone (AGAMREE) with your Health Care Provider: What Every Person with Duchenne and their Family Must Know</u>
- Be sure to talk with your neuromuscular team about risks, benefits, and precautions before starting any medications. Learn more about steroids and precautions here.

## What are the possible side effects of AGAMREE?

- The most common side effects include:
  - Cushingoid features (round face, increased fat around base of neck) difficulties sleeping, mood swings, vomiting, increased weight, 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 (eye) effects, effects on growth, and high blood pressure.
  - For a full list of side effects and precautions, please see <u>AGAMREE Prescribing</u> <u>Label.</u>

## What precautions are needed while taking AGAMREE?

 Because AGAMREE is a type of steroid, it suppresses the adrenal glands. Because of this, suddenly stopping AGAMREE or any other steroid can be extremely dangerous and cause <u>adrenal crisis</u>, 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, stress dosing and more in PJ's Protocol.
- AGAMREE can also suppress the immune system. This could affect your child's ability to receive <u>live-attenuated vaccines</u>. Be sure to talk to your neuromuscular doctor and primary care provider about your options.

# How does my child get access to AGAMREE?

- If you want to know if your child is eligible to receive AGAMREE, call your neuromuscular team to discuss whether AGAMREE is right for your child.
- If you and your neuromuscular team decide that AGAMREE is right for your child, your neuromuscular provider will submit an enrollment form to <u>Catalyst Pathways</u>. This enrollment form serves as a prescription, as well as provides additional information about your insurance coverage so that your neuromuscular team and Catalyst Pathways can work together to obtain insurance approval.
- If approved by your insurance, Catalyst Pathways will work with a specialty pharmacy to ship the medication to your home.
- Learn more about access & coverage resources for AGAMREE here.

## • Learn more about clinical trials of AGAMREE

 Clinical studies are underway in individuals with Becker to support a broader label in the future

# • Video Library

- o Agamree (vamorolone): All Your Questions, Answered
- Webinar: AGAMREE Approval What This Means for You and Your Family
- Webinar: AGAMREE Approval with Catalyst Pharmaceuticals Inc.
- o PPMD's 2024 Annual Conference: Navigating Approved Therapies