



Discussing Vamorolone (Agamree) with your Health Care Provider: What Every Person with DMD and their Family Must Know (US Version)



What is vamorolone?

Vamorolone (Agamree) is a new steroid for the treatment of Duchenne muscular dystrophy (DMD). It was first approved by the Food and Drug Administration (FDA) in October 2023 for people with DMD that are 2 years and older living in the USA. Check with your health care provider to see if vamorolone is available where you live.

What are the benefits and side effects of vamorolone?

Vamorolone is a new type of steroid designed to have the beneficial anti-inflammatory and muscle-stabilizing effects of classic steroids (prednisolone/prednisone and deflazacort), while possibly reducing some of the side effects. Clinical research studies for up to 2.5 years showed that children treated with daily vamorolone at 6 mg/kg/day had similar benefits on muscle function tests and grew taller compared to children treated with prednisone and deflazacort. Similar to classic steroids, vamorolone still caused important side effects including adrenal suppression (explained below) and, in some cases, excessive weight gain. Preliminary information suggested that spine fractures may be less common on vamorolone. However, both spine and limb fractures may still occur. More time is needed to understand its effects on bone health. Early reports suggest that behavior side effects such as aggression and mood swings may also be less common in children on vamorolone compared with those on prednisone. It is not yet known how vamorolone affects the heart, lungs, and puberty. See the table below for a comparison of the side effects of vamorolone, prednisone/prednisolone, and deflazacort.

What are important considerations before starting vamorolone?

The decision to start or change to vamorolone will be different for every person with DMD. Vamorolone may not be right for everyone. Below are some things you need to know when talking to your health care provider about vamorolone.

Neuromuscular Considerations

- The recommended starting dose for vamorolone is 6 mg/kg/day (up to a maximum of 300 mg/day) for most people. Your doctor will review your medical history including your current steroid dose (if on steroids) and recommend the right dose of vamorolone for you. The vamorolone dose may need to be adjusted based upon your response.
- People with liver disease need a lower dose. You should ask your provider if additional testing for liver disease is needed before starting vamorolone.
- Switching to vamorolone may impact your eligibility for some research studies (including clinical drug trials). Discuss this with your provider and with your trial doctor before starting vamorolone.
- Vamorolone has not been studied in teens or adults. If you are a teen or an adult you should ask your provider to discuss the potential risks and benefits of taking vamorolone.
- Vamorolone has not been studied after gene therapy.

Adrenal Considerations

- Vamorolone, like other steroids, causes adrenal suppression. When a person takes high doses of steroids regularly, their adrenal glands temporarily stop making a hormone called cortisol. This is called adrenal insufficiency.
- People on high doses of steroids cannot make extra steroids to fight illness or bodily stress (such as a car accident or surgery). They need to take extra or “stress” doses of steroids in addition to their regular steroid during these times to avoid a potentially life-threatening adrenal crisis.
- All people on vamorolone must have a “stress steroid plan”. This plan will provide instructions on how to give “stress steroids” (hydrocortisone, prednisone, or prednisolone) in times of illness, injury, and surgery, or for side effects of IV bisphosphonate infusions (in people who receive them). This plan will include information about how to give stress steroids by mouth or injection (if vomiting or very sick). Additional information about adrenal insufficiency due to steroid use can be found in the PJ Nicholoff Steroid Protocol.^{1,2}
- *Vamorolone cannot be used as a stress steroid* because it blocks a hormone called aldosterone and may cause low sodium, high potassium, and low blood pressure.

Heart Considerations

- Vamorolone may increase the chance of developing electrolyte abnormalities such as low sodium and high potassium when used with some heart medications including ACE inhibitors (lisinopril, enalapril, others), angiotensin receptor blockers (losartan, others), or mineralocorticoid receptor antagonists (eplerenone, spironolactone, others).
- If you / your child is taking heart medications, be sure to ask if, when, and how frequently electrolytes should be monitored after starting vamorolone.

Side effects of vamorolone compared to classic steroids (Prednisone/Prednisolone and Deflazacort)*			
	Better	Similar	Not Known
Adrenal insufficiency		✓	
Slow growth / short stature	✓		
Weight gain / obesity**		✓	
Fractures / osteoporosis***	Possibly?		
Delayed or absent puberty			✓
Mood / behavior disturbance****	Possibly?		
Cataracts			✓

* Daily use with standard dosing. Taking classic steroids less frequently than seven days per week typically causes fewer side effects. It is not recommended to take vamorolone less frequently than daily. Severity of side effects may vary by individual and dosing regimen.

** Weight gain was similar with vamorolone versus prednisone. Because prednisone causes more weight gain than deflazacort,⁹ individuals switching to vamorolone from deflazacort may experience more weight gain compared to those switching from prednisone.

*** Preliminary data suggest that spine fractures may be less frequent than prednisone & deflazacort; limb fractures have been reported.

**** Preliminary data suggest that behavioral side effects may be less common with vamorolone compared with prednisone.

References:

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