

What is vamorolone?

- Vamorolone (Agamree) is a new steroid used to treat Duchenne muscular dystrophy (DMD).
- It was designed to have the same benefits on muscles as classic steroids (prednisone, prednisolone, deflazacort) while possibly causing fewer side effects.
- It is currently available in the USA and parts of Europe. Ask your health care provider if it is available where you live.

What are the possible benefits and side effects of vamorolone?

- Studies for up to 2.5 years have shown that children with DMD treated with vamorolone had similar benefits on muscle function and grew taller compared to those treated with classic steroids.
- The side effects of vamorolone compared to classic steroids are summarized in the table below:

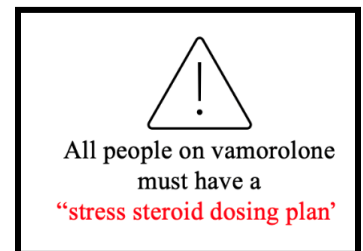
Side effects of Vamorolone compared to classic steroids (Prednisone/Prednisolone and Deflazacort)*			
	Better	Similar	Not Known
Adrenal insufficiency		✓	
Slow growth / short stature	✓		
Weight gain / obesity		✓	
Fractures / osteoporosis	Possibly?		
Delayed or absent puberty			✓
Mood / behavior disturbance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

* Daily steroid use with standard dosing. Severity of side effects may vary by individual and dosing regimen. Never change your steroid dose without discussing with your health care provider first.

Here are some things you should consider when talking to your health care provider about vamorolone:

Neuromuscular Considerations:

- The starting dose of vamorolone is 6 mg/kg/day for most people.
- People with liver disease need a lower dose.
- Switching to vamorolone may impact your ability to participate in clinical trials.
- There is limited information about vamorolone in teens & adults and after gene therapy.



Adrenal Considerations:

- Vamorolone causes adrenal suppression.
- All people on vamorolone must have a "stress steroid dosing plan".
- This plan will include information on how and when to give extra steroids for illness, trauma, or procedures.
- You will need a separate prescription for hydrocortisone, prednisone, or prednisolone for stress steroid dosing.
- Vamorolone cannot be used for stress dosing.
- More information about adrenal insufficiency due to steroid use can be found in the **PJ Nicholoff Steroid Protocol**.¹

Heart Considerations:

- It is not yet known if vamorolone will help the heart.
- Vamorolone may cause electrolyte abnormalities such as low sodium and high potassium if used with some types of heart medications.
- If you or your child is taking heart medications, be sure to ask if, when, and how frequently electrolytes should be monitored after starting vamorolone.

¹PJ Nicholoff Steroid Protocol: Available online: <https://www.parentprojectmd.org/wp-content/uploads/2018/03/PJ-Nicholoff-Steroid-Protocol.pdf>