

Corticosteroids in Duchenne Muscular Dystrophy (DMD)

Guidance for patients and carers on the benefits and side effects of corticosteroid use in DMD



“This guide is for children and adults living with DMD and for parents and carers too. It refers to ‘you’ throughout, but this may apply to ‘your child’.

Please note: Throughout this document, the term corticosteroids (CS) **refers to all corticosteroids, including vamorolone**, unless otherwise specified.

Recommendations for use of corticosteroids in DMD

Treatment with CS is recommended as standard of care in DMD. Research and studies have shown that they maintain or slow down the decline of muscle strength and function over a period of time, allowing people with DMD to walk and maintain arm function for longer. Over the long term CS may also help with upper body and spine strength, as well as working to delay breathing and heart complications.

CS are anti-inflammatory drugs. Although the way they work in DMD is not fully understood, we know they help to slow down disease progression by reducing inflammation and maintaining muscle strength for longer. However, CS also have significant side effects on your body, which need to be carefully monitored and appropriately managed. It is important to remember that although there are lots of side effects listed, you are unlikely to experience all of them. In this leaflet we will summarise benefits and side effects of CS in DMD.

What types of corticosteroids are there?

There are three main CS that may be used to treat DMD: prednisolone, deflazacort or vamorolone.

Prednisolone and **deflazacort** have been used in the treatment of DMD since the late 1990s and are often referred to as “classic corticosteroids”. Both have been shown to be effective in the treatment of DMD and based on the current evidence, they do not significantly differ in terms of their effect on muscle strength and function. They also have similar side effects. However, some side effects can be seen more frequently with one type of CS compared to another.

Vamorolone (also referred to by its brand name Agamree®) is a newer CS that has been approved in the UK since January 2025 for treatment of people aged 4 years and over with DMD. Vamorolone is still a CS but it was designed to keep the benefits of classic CS, whilst reducing some of the side effects. It has been available in the UK on prescription for patients with DMD where appropriate, from neuromuscular teams since April 2025.



What do we know about CS use in DMD?

Because classic CS (prednisolone and deflazacort) have been used for treating DMD since the late 1990s, there is evidence of their short- and long-term effect on muscle strength and function in both younger and older people with DMD. We also know about the longer-term side effects of classic CS.

Vamorolone has only been used so far in clinical trials and therefore we currently have limited evidence on the long-term benefits and side effects of vamorolone and how these compare to classic CS. Moreover, vamorolone so far has only been tested in children with DMD who can walk, and it has only been used as a daily (not intermittent) treatment.

There are also no data currently on the effect of switching to vamorolone after receiving long-term prednisolone or deflazacort, or on the use of vamorolone in older, non-ambulant patients.

Further evidence on the long-term efficacy and side effects of vamorolone in DMD and at different ages and different stages of DMD, is expected to emerge in the coming years.

For more information specifically about evidence on the use of vamorolone in DMD, please see the 'Summary of evidence so far' document for patients and families here <https://tinyurl.com/ssbpj9v6>.



Benefits of corticosteroids

CS can be beneficial for people with DMD. Our clinical guidance recommends that CS should be given **daily** as this gives the greatest benefits on muscle strength and function. When given daily, CS have been shown to:

- Maintain or slow down the decline of muscle strength over a period of time.
- Keep you walking for longer. CS delay loss of walking by approximately three years (on average).
- Delay loss of upper limb function. This means you can still use your arms, shoulders, and hands for longer as you get older.
- Slow down the decline of respiratory function. CS can help keep your breathing muscles working for longer, meaning you are less likely to need support as early compared with not taking CS.
- Delay the start and slow the progression of heart disease. The heart is a muscle affected by DMD and taking CS will help keep your heart working better for longer.
- Delay or prevent the development of severe scoliosis requiring an operation. Scoliosis is the twisting and curving of the spine, like an 's' shape instead of being straight. CS have been shown to delay or prevent severe scoliosis from happening and significantly reduce the chances of needing an operation to fix it.
- Increase life expectancy. CS treatment, as part of a full multi-disciplinary standards of care approach, can help people with DMD live longer.

Studies have shown that daily CS are more beneficial in maintaining muscle strength and the ability to walk, than CS administered intermittently (e.g., 10 days on and 10 days off). Intermittent use might reduce the risks of some side effects, for example it may have less impact on growth, but it can still cause others, including weight gain and it is less effective on muscle function.

Side effects of corticosteroids

CS have several side effects. These vary in when they occur, their severity and not every person develops all side effects. Some can be seen as early as within the first six months of starting treatment, whilst others might take longer to develop.

Side effects of CS that can be seen within the first six months of starting treatment include:

- **Adrenal insufficiency.** CS can cause the adrenal glands (these are small glands that sit on top of each kidney) to stop producing their own steroids and become “sleepy”, known as adrenal insufficiency. For more information, view our guide on adrenal insufficiency [here](#)
- **Behavioural changes.** Patients taking CS may be more likely to have temper-tantrums and demonstrate aggressive behaviour. They may also have difficulties sleeping well.
- **Increased appetite and weight gain.** CS use can be associated with feeling hungrier and can cause weight gain.
- **Gastrointestinal symptoms.** CS have been shown to cause a heartburn and indigestion type feeling and pain in the middle of your chest/upper tummy. You should always take steroids on a full stomach.
- **Immunosuppression.** CS can make your immune system less effective, meaning that there is an increased risk of infections. This also means that your body will be less able to respond to certain types of vaccines (“live vaccines”).
- **Hypertension.** This is also known as high blood pressure. Taking CS can be associated with increasing blood pressure levels.

Please remember it is unlikely you will experience every listed side effect.

Some side effects are more related to longer term CS use and may be noticed six months or more after starting treatment. These side effects include:

- **Cushingoid features.** Someone taking CS might develop a rounder face (also described as “puffy or moon-face”), fat deposition on the back of the neck (“buffalo hump”) and easy bruising.
- **Reduced growth.** Classic CS are known to slow growth and therefore, people taking them long-term tend to be significantly shorter than their peers.
- **Fragile bones and increased fracture risk.** This means that bones become weaker through something called osteoporosis. Fractures are more likely and people on long-term CS are more likely to break bones even after a minor stumble or fall. Fractures of the vertebrae (bones in the spine) are also more common, and these may be painful, or cause no symptoms.
- **Delayed puberty.** Boys taking long-term CS often experience puberty later or not at all. The testicles and penis grow more slowly than normal, the voice might not be as low, and facial and pubic hair will not grow at the same speed as for other boys the same age. This means that you might look younger than your peers. Boys who experience delayed puberty can be offered testosterone treatment to start the process of puberty. You can read more about delayed puberty and testosterone treatment [here](https://tinyurl.com/33dhu2dy)
- **Other gastrointestinal symptoms** e.g., peptic ulcers. These are sores in the stomach or intestine lining. They are very rare and can cause a burning or gnawing pain in the tummy.
- **Cataracts.** These form in your eye lens and are not painful but cause your vision to be blurry.
- **Excessive hair growth (hypertrichosis).** This means your body can become hairier.
- **Skin infections.** People on CS are more likely to develop skin infections, such as verrucas, warts, and fungal nail infections.
- **Other skin changes (e.g., skin fragility).** Long-term CS use can cause the skin to break easily and difficulties in skin wounds healing.
- **High sugar levels and diabetes.** CS can cause the body to struggle to convert glucose into energy like it normally does and this can result in higher blood sugar levels.

Side effects vary between different CS. Vamorolone appears to have fewer side effects, particularly on growth and on bone. You can see a summary of side effects across vamorolone, deflazacort and prednisolone in Table 1.

The risk of adrenal insufficiency occurs with all CS types and regimens in a dose dependent manner.

Side Effect	Daily prednisolone	Daily deflazacort	Daily vamorolone ¹
Adrenal Insufficiency	Common, across all three CS. Risk increases with longer treatment duration and higher doses		
Weight Gain	Common, more weight gain associated with prednisolone than deflazacort	Common, less weight gain associated with deflazacort than prednisolone	Common, similar associated weight gain to prednisolone
Growth Failure	Very common		
	Less growth failure associated with prednisolone than deflazacort	More growth failure associated with deflazacort than prednisolone	Not associated with growth failure
Bone Health (long bone fractures and vertebral fractures [VF])	Long bone & VF risk.		No data available on long bone fractures so far.
	Increased risk with daily treatment and longer treatment duration	Increased risk with daily treatment and longer treatment duration. VF risk may be slightly higher on daily deflazacort than daily prednisolone	Lower risk of VF compared with daily prednisolone and daily deflazacort ² (unpublished data)
Behavioural Issues	Can cause mood changes and aggression.		
	More likely with prednisolone when compared to deflazacort	May cause fewer behaviour problems when compared to prednisolone	May cause fewer behavioural problems compared to prednisolone (limited, unpublished data)

Side Effect	Daily prednisolone	Daily deflazacort	Daily vamorolone ¹
Cushingoid Features (round face, fat build-up)	Common		Data not available
	Similar frequency as deflazacort	Similar frequency as prednisolone	
Delayed Puberty	Common with long-term use, with a similar frequency to each other		Data not available
Stomach Problems (pain, reflux, ulcers)	Possible		Data not available
Cataracts	Risk present with long-term use	Risk present with long-term use. Higher risk than prednisolone	Data not available
Increased Infection Risk	Possible		Data not available
Excess Hair Growth	Can occur but does not cause any health problems		Data not available
High Blood Pressure	Can occur soon after starting treatment and after long-term use		Data not available
Blood Sugar Problems (increased levels of sugar in the blood and diabetes)	Rare can occur with long-term use		Data not available
Skin Fragility (slow wound healing and pink/red marks on the skin)	Can occur with long-term use		Data not available
Acne and Skin Infections	Can occur with long-term use		Data not available

Table 1: Summary of side effects associated with daily prednisolone, deflazacort and vamorolone in DMD

¹It is important to remember that vamorolone is a relatively new drug, so we don't yet have information about all its benefits and risks or over

a long period of time. Vamorolone has not been compared directly with deflazacort in studies so far.

²Intermittent prednisolone (10 days on/10 days off) has been shown to have a lower risk of vertebral fracture compared to daily use of any CS, including vamorolone.

What should you expect from your GP once you are on CS?

After treatment is started, CS are usually prescribed by your GP. However, the benefits and side effects will be monitored by your neuromuscular care team so that they can make any necessary adjustments to your treatment dose and schedule. Your GP still has a critical role in managing your CS treatment.



Before starting CS treatment your GP should:

- Ensure you are up to date with vaccinations according to national guidance.
- Establish varicella (chickenpox) and measles immunity, according to the national guidance.
- Assess for tuberculosis (TB) risk according to national guidance and if there are concerns refer you to a specialist for advice.

Once CS are initiated:

- Monitor your blood pressure monthly for the first three months. Give you an annual injectable influenza vaccine (you must not receive the nasal vaccine as it is a live vaccine).
- Give you pneumococcal polysaccharide conjugate vaccines (Prevenar13) as per national guidance.
- Ensure you have continuation of the prescription for CS.
- Provide you with a stress dose of steroids (hydrocortisone) at home for use during sickness or injury as recommended by the neuromuscular team. For more information view our endocrine guidance here: <https://www.duchenneuk.org/wp-content/uploads/2022/07/DMD-Care-Adrenal-insufficiency-leaflet-digital-updated-July-22.pdf>

CS recommendations:

- ➔ CS should be taken on a **full stomach** at breakfast time or after breakfast, as this helps mimic the body's own production of steroids and food provides protection for the stomach lining.
- ➔ **All CS cause adrenal insufficiency.** When oral CS are taken for more than a month, they cause the adrenal glands to stop producing their own steroids and become “sleepy” (adrenal insufficiency). This can be important when you are unwell, have had surgery, or any physical injuries. In these situations, you will require additional CS, and you will be provided with an emergency steroid plan that explains what to do. The neuromuscular care team will make sure you have a plan to deliver the additional medications. Your clinician should be aware of the DMD Care UK Endocrine guidance for management of adrenal insufficiency in DMD on long-term corticosteroids (<https://dmdcareuk.org/clinical-recommendations>).
- ➔ For the same reason, **CS doses must not be missed**, and it is important to not run out of CS. Your emergency steroid plan will include information about how to manage situations when you are unable to take oral CS (e.g., in case of vomiting).
- ➔ It is important to **avoid** non-steroidal anti-inflammatory medication (NSAIDs) such as **ibuprofen** and similar medications as they can worsen the irritation of the stomach lining. Paracetamol should be used instead.
- ➔ Children receiving long-term CS **should not receive live vaccines**. This is because your immune system might not react as well as expected. It is therefore important that all preschool vaccinations are completed before starting CS and that you receive an annual flu jab as intramuscular injection (and NOT nasal vaccination). Immunity against chicken pox and measles must be confirmed before starting CS.

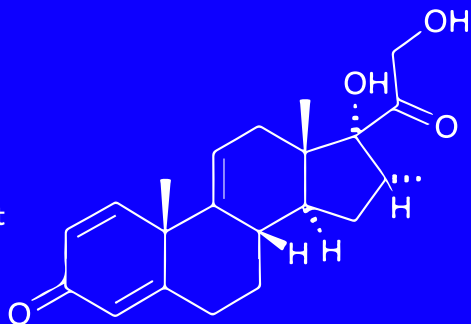
Key points to remember:

- Daily CS treatment gives greater benefits than intermittent treatment on muscle strength and function.
- CS treatment, its benefits and side effects, should be discussed with all patients and families diagnosed with DMD.
- Children can show variable response to CS both in terms of benefit on muscle function and side effects.
- CS causes side effects, which can vary in their intensity, however not everybody will experience the same side effects.
- There is significant evidence and recent studies that suggest continuing to take CS can help adult patients and people who can no longer walk to maintain the use of their arms, and slow down problems with breathing and heart health.
- CS must not be discontinued suddenly and must not be withdrawn during acute illness. This can cause serious harm or death.
- If switching or stopping CS treatment, this should only be done carefully and under supervision of your doctor.
- Always inform health care professionals that you are taking a high dose of CS during medical emergencies and hospital admissions.
- For more in-depth information please refer to the CS guidance document for clinicians here: <https://dmdcareuk.org/corticosteroids-in-dmd>

Vamorolone – key considerations:

What is vamorolone?

Vamorolone is a CS medicine that has been recently approved in the UK to help treat boys aged 4 and older with DMD. It works to support muscle function, similar to other CS treatments.



How does vamorolone compare to classic CS?

(Remember this is based on evidence over a relatively short term and only in young, ambulant boys with DMD)

- Evidence shows that vamorolone helps maintain muscle function like other CS.
- It has shown a better profile on growth and bone health compared to other daily CS after up to 30 months of treatment.
- It has similar side-effects on weight gain and adrenal function compared to other CS.
- It might cause fewer behaviour-related side effects than other CS, but more research is needed.
- Vamorolone has only been studied as a daily, not intermittent (on and off) treatment.

How is vamorolone taken?

- It comes in a liquid form and is taken orally (by mouth).
- Like other CS it should be taken in the morning on a full stomach.
- The usual dose is based on body weight, and the recommended dose is **6mg/kg/day**.
- The maximum daily dose is **240 mg/day** for individuals weighing over 40kg.

What if my child is already on another steroid?

- If switching from other CS (like prednisolone or deflazacort), this should only be done under close medical supervision.
- When switching from prednisolone or deflazacort to vamorolone, your child might develop symptoms of adrenal insufficiency. Doctors will keep a close eye out for any symptoms of this, especially in the first few weeks after the switch. If your child weighs more than 40 kg, or if the vamorolone dose is less than 6 mg per kg of body weight, the doctor will also prescribe hydrocortisone tablets. **Taking hydrocortisone alongside vamorolone** is thought to help lower the risk of adrenal insufficiency symptoms during the changeover.
- The effects of switching, especially on weight and other side effects, are still being studied.



Who might benefit from vamorolone the most?

- Younger children, as it may have fewer effects on growth and bone health.
- Those who were hesitant to start other CS because of side effects on growth and bone health.
- Older children already taking CS may also consider switching, but this should be discussed with their doctor.

Is vamorolone available for adults?

- It is possible for doctors to prescribe vamorolone for adults with DMD.
- It has not been studied in adults, but some adults in other countries may be using it.
- More research is needed to understand how it works for adults or those who have been on other CS for many years.

Vamorolone is a new treatment option for people with DMD. While it may have some advantages over other CS, there is still much to learn about its long-term effects. You should discuss the benefits and risks with your doctor to decide if it's the right choice for you.

KEY THINGS TO REMEMBER ABOUT VAMOROLONE

- The long-term effects of vamorolone compared to other CS are still being studied.
- Vamorolone is a corticosteroid and can cause adrenal insufficiency.
- Vamorolone should not be used for extra doses during illness (stress dosing).
- Patients on vamorolone must be provided with hydrocortisone for stress dosing to use during illnesses, surgeries or following a trauma.
- When switching from classic corticosteroids to vamorolone, patients might develop symptoms of adrenal insufficiency and might require oral hydrocortisone.

About DMD Care UK

DMD Care UK is a nationwide initiative to ensure every person living with Duchenne muscular dystrophy (DMD) in the UK has access to the best care.

DMD Care UK is a collaborative initiative between the John Walton Muscular Dystrophy Research Centre at Newcastle University and Duchenne UK, embedded in the UK North Star Network. It is funded by Duchenne UK, Duchenne Research Fund and Joining Jack.

Find out more at dmdcareuk.org

Do you have questions or feedback about this booklet? Get in touch with support@duchenneuk.org

Leaflet developed by the Corticosteroid Working Group of DMD Care UK (chair: Prof Michela Guglieri (Newcastle upon Tyne))
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