



University Hospitals Bristol
NHS Foundation Trust

Patient information service
Neurology

Steroid medication in Duchenne muscular dystrophy



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What are steroids?

Steroids (also called corticosteroids or glucocorticoids) are produced naturally by the body in the adrenal glands (located on each kidney). The exact mechanism of how steroids work is not known, but they have become part of the routine care of boys in the UK over the last 15 years. Currently, there are two types of steroids used in Duchenne muscular dystrophy (DMD). They are believed to work in a similar way and the choice of steroid depends upon availability in a particular country, their cost, the way it is taken and the known side-effects. Prednisolone is more widely used in the UK and America and Deflazacort in Europe.

Why are steroids prescribed for DMD?

Studies have shown that giving additional steroids can improve muscle strength in boys with DMD, especially those who are still walking. Deciding when steroid treatment should begin is an area where medical knowledge and understanding is evolving.

In general, the standard recommendation is that steroid treatment should be started when your child's motor function is in the 'plateau phase' which is when they still have good muscle strength. However, timings will vary and a decision to commence steroids is always made on a case by case basis.

What are the benefits of taking steroids for DMD?

The following benefits of taking steroids have been noted:

- Increased energy and stamina.
- Increased ability to engage in physical activities.
- Delayed 'loss of ambulation' (i.e loss of walking skills).
- Developing of additional skills, for example, jumping, hopping, running and riding a bike.
- Increased ability to concentrate/learn as less fatigued.

In addition, steroids can reduce the likelihood of your child requiring spinal surgery, help to maintain cardiac function and respiratory function, delaying the need for assistive ventilation. Steroids can also improve upper arm function, which is important for feeding, driving a wheelchair, self-care, using a phone or a computer.

However, any benefits need to be balanced against side-effects associated with taking steroids for a long period of time. Therefore, prior to your child starting steroids your neuromuscular (NM) consultant will talk to you about the benefits and side-effects of long-term steroid treatment and the different drugs and regimes.

Steroid treatment regimes

Which steroid regime your child is put on will be decided following a conversation(s) between parent(s) and NM consultant. The two drugs that are currently used are:

Prednisolone 0.75 mg/kg/day or Deflazacort 0.9 mg/kg/day.

North Star* data has shown that daily steroids are more effective than intermittent (ten days on, ten days off) in improving muscle strength. However, intermittent steroids may

have less side-effect.

Research into which regime is best, and alternative forms of steroids with fewer side-effects, is ongoing.

Monitoring and surveillance

Prior to starting steroids, your child will require a blood test to check vitamin D and calcium levels, and their immunity to chicken pox.

A record of their blood pressure, height and weight will be taken, and urine tested for sugar.

Once your child starts steroids, they will continue to be reviewed regularly at the NM clinic. At every NM appointment, your child will be reviewed for side-effects and have their motor skills assessed.

If you have any concerns in between appointments, you can always contact your child's NM Consultant for advice and, if needed, their steroid regime can be changed.

Consent for your child to be enrolled into the North Star* study will also be sought.

**North Star is a natural history study, which collects anonymised information about how your child's DMD progresses. It will also record any side effects experienced when on steroids and changes in muscle function, allowing the natural history of DMD to be systematically evaluated.*

*The North Star Project was set up in 2003 to help drive improvements in services and set national standards of care for children living with DMD. **The overall aim is to optimise the care of young patients with DMD** by achieving and practicing consensus on best clinical management, with agreed assessment and treatment protocols, no matter which clinical centre is attended.*

What are the side-effects and how can they be treated?

Behaviour

Steroids, especially prednisolone, can affect your child's mood, make them more irritable, hyperactive or have difficulty sleeping. With your NM consultant, it is helpful to identify your child's baseline mood, temperament and ADHD issues, prior to starting steroids.

Behaviour can often temporarily worsen in the initial six weeks of steroid treatment, then settle. Guidance on managing changes in your child's behaviour may be available through psychology services in your area.

Weight gain

Steroid medication, especially prednisolone, may stimulate your child's appetite, so that he will want to eat more. At each clinic appointment, they will be weighed. Excessive weight gain can make it harder to move around. Losing weight is very difficult for boys with DMD, so it is preferable to prevent weight gain.

To limit weight gain, it is important to consider what food is offered to your child; try to avoid extra crisps, sweets, fizzy drinks and frequent snacks. Instead, offer fruit, water, bread, pasta, cereals. Additional vitamins, including vitamin D and calcium, are often prescribed. If weight gain becomes a concern, a referral to a dietitian can be made and the steroid regime will be reviewed, weaned or may need to be stopped, if it is felt to impact negatively.

Gastric irritation

Steroids can cause heart burn (reflux), irritate the lining of the stomach and cause ulceration of the stomach. Steroids should be taken after food to help minimise the effects on the stomach. Medication to protect the lining of the stomach may also be prescribed. Non-steroidal anti-inflammatory drugs (NSAIDs) such as aspirin, ibuprofen and naproxen should be avoided.

Growth and bone health

Long-term steroid use can cause loss of calcium from the bones making them weaker and fragile, and more likely to break (fracture) known as osteoporosis or bone thinning. Prior to starting steroids, your child will have blood tests to check the mineral and vitamin levels in their blood are normal. Compression fractures of the vertebral bones (which make up the spine) can occur in children on steroids. Also, fractures of other bones can occur as a result of a minor injury.

To monitor for the earliest signs of bone fragility the latest DMD Guidelines (Part 2) recommends your child has a spinal X-ray and spine DXA scan (which checks bone density) when starting steroids. While your child remains on steroids, the spinal X-ray and/or DXA scan will be repeated every one to two years, or sooner if they complain of back pain or DXA scan raises concern. A DXA scan is a painless, and relatively quick, scan that involves lying flat on the scanning table with legs supported on a cushion. It takes about 20 minutes to perform.

If your child complains of pain following a minor injury, or complains of pain in his back, please contact your GP or your NM team, as they may require an X-ray to rule out the possibility of a fracture.

Your child will also be referred to an endocrinologist if they stop growing, break a bone or their lateral X-ray or DXA scan raises concerns.

Puberty

Delayed puberty is a potential complication of steroids, especially Deflazacort, causing your child's growth to slow and delay the maturing of your child's body to that of an adult. At each NM clinic appointment their standing height or tibia length will be measured until completion of puberty. From the age of nine years, questions about your sons pubertal development will be asked at clinic. If required, a referral to an endocrinologist will be made as testosterone replacement therapy maybe required.

Exercise

It is important to encourage your child to be as active as possible. It will help to maintain bone density and provide opportunities to be out in the sunshine (which helps body to create vitamin D, improve mood and regulate internal body clock). Exercises, such as walking and swimming, appropriate to your child's motor functional level, are also recommended. Aids, such as walkers and standing frames, can also be used to extend ambulation and provide opportunities for weight bearing exercises. **Avoid excessive eccentric (squatting, trampoline) and resistive exercises (e.g. lifting weights).**

Adrenal suppression

When steroids are taken regularly, over a long period of time, the adrenal glands that produce steroids become inactive, and this can affect the body's ability to respond to stress by releasing more steroids into the body.

When your child is unwell (temperature above 38C), or having a surgical operation, or has a serious accident, they may become unwell very quickly unless additional steroid medication is given.

Signs your child may require additional steroids (adrenal crisis), can include any of the following signs:

- lethargy
- vomiting
- hypotension (low blood pressure)
- hypoglycaemia (low blood sugar)
- altered consciousness
- dizziness

If you are concerned about your child, please contact your GP or NM team. However, if your child is very unwell, you should take them to the local accident and emergency department and inform the medical staff that your child is taking regular steroid medication. Your child may require additional steroids as tablets or as an injection. Please see separate **“Emergency steroid management sheet”**.

Medical alert

It is recommended, that anyone providing treatment or caring for your child (e.g. medical, dental, family, teachers etc.) are informed your child is taking steroid medication and shown information on the steroid alert card and DMD alert card. Wearing a medic alert bracelet can also help to ensure your child has the correct treatment if they become sick or have an accident.

Body changes

Roundness of the face (Cushing's features), increase in body hair, especially on the arms, legs and back, thinning of the skin and acne (more noticeable in teenagers) can occur.

Infection

Steroids can increase your child's susceptibility to infection but this risk has not commonly been reported in studies for DMD boys. If your child has an infection, it is advisable that you seek medical advice early.

Immunisations

It is advised your child is immunised against flu each year. Children taking steroids should not have live vaccines such as MMR (measles, mumps, rubella) or nasal flu vaccine.

Blood pressure

Steroids can cause blood pressure to be raised. This will be monitored at your GP practice or by your community nurse, every month, for the first three months after starting steroids, and subsequently, every six months at each NM appointment.

Diabetes

There is an increased risk of developing diabetes; therefore, your child's urine will be checked for glucose at your GP practice, every month, for the first three months, and subsequently, every six months at each NM appointment.

Eyes

Children can develop cataracts (clouding of the lens in the eye) after some years on steroid medication. It is advisable your child has an eye test, once a year, at your local optician.

How do I give my child the steroids?

Prednisolone is available in tablet and liquid form.

Deflazacort is available in tablet form.

Steroids are taken once a day and are best taken in the morning, with food.

If a dose is missed, it can be taken at lunch time on the same day but no later. Do not take a double dose if one is missed.

Top tips for administering the steroids

- Be cheerful, patient and matter of fact when giving medicines.
- Explain what you are doing and involve your child; give them some choice in the process.
- It is best not to hide medication in food or drink.
- Check with a pharmacist, if the tablet can be crushed or given with food (e.g., spoonful of yoghurt or jam) or if flavours can be added to liquids (e.g., blackcurrant squash).
- Cold drinks or ice cream after medicines can help take away bad tastes.
- Reward charts and stickers can encourage children to take their medicine.

When do steroids stop?

The age steroids stop is decided individually. It may be necessary to change or stop the steroids because of side-effects or, very occasionally, one to two percent of boys do not respond to steroids. In other situations, steroids can be used for many years and continue beyond the loss of ambulation.

Prior to your child stopping steroids, they will be referred for a synacthen test.

A synacthen test uses a special chemical to test how well the adrenal glands make cortisol. It involves stimulating the adrenal glands and then checking to see if they respond.

Steroids must never be stopped suddenly. The dose has to be lowered gradually over several weeks to give time for your child's adrenal glands to start producing steroids again. Additional steroid medication may be required for up to one year after stopping steroids as the body adjusts to producing its own steroids.

Contact details

Non-urgent:

Bristol Neuromuscular Team: 0117 342 0502

Mon - Fri 9.00am to 4.00pm

Or contact your local paediatrician.

Out of hours:

Please contact your local out of hours service or go to your nearest accident and emergency department (A&E).

Notes and queries

Reference articles

- Action Duchenne A&E/Emergency Care for Patients with Duchenne Care Folder. www.actionduchenne.org
- 2008 Muscular Dystrophy UK Steroids and Duchenne muscular dystrophy Information sheet. www.muscular dystrophyuk.org
- 2013 Long-term benefits and adverse effects of intermittent versus daily glucocorticoid in boys with Duchenne muscular dystrophy. Ricotti V, Ridout DA, Scott E, et al J Neurol Neurosurg Psychiatry 84: 698-705.
- 2013 V1 Dubowitz Children's Neuromuscular Centre - Starting Steroids Flowchart
- 2013 Oct Steroids for Children with Neuromuscular disorders. Great Ormond Street Hospital
- 2018 Jan, 23 Lancet Neurology: Diagnosis and management of Duchenne muscular dystrophy, part1: Diagnosis and Neuromuscular, rehabilitation, endocrine and gastrointestinal and nutritional management. Prof DJ Bimkrant, Prof K Bushby et al.
- 2018 Jan23 Lancet Neurology: Diagnosis and management of Duchenne muscular dystrophy, part 2: respiratory, cardiac, bone health and orthopaedic management. Prof DJ Bimkrant, Prof K Bushby et al.
- 2018 Jan23 Lancet Neurology: Diagnosis and management of Duchenne muscular dystrophy, part 3: primary care, emergency management, psychosocial care, and transitions of care across the lifespan. Prof DJ Bimkrant, Prof K Bushby et al.

- Parents Project Muscular Dystrophy Factsheets Understanding and Using Glucocorticoids: What you need to know about giving your child steroids: Risk factors for Adrenal Crisis www.parentprojectmd.org
- 2010 March The Diagnosis and Management of Duchenne muscular dystrophy: A Guide for Families. Newcastle
- 2017 Feb V2 Clinical Guideline: Duchenne muscular dystrophy: Monitoring of Steroid Therapy. Bristol Royal Hospital for Children

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Please note that if for any reason you would value a second opinion concerning your diagnosis or treatment, you are entirely within your rights to request this.

The first step would usually be to discuss this with the doctor or other lead clinician who is responsible for your care.

Smoking is the primary cause of preventable illness and premature death. For support in stopping smoking contact **Smokefree Bristol** on **0117 922 2255**.

As well as providing clinical care, our Trust has an important role in research. This allows us to discover new and improved ways of treating patients.

While your child is under our care, you may be approached about them taking part in research. To find out more please visit: **www.uhbristol.nhs.uk/research-innovation**

For access to other patient leaflets and information please go to the following address:

www.uhbristol.nhs.uk/patients-and-visitors/information-for-patients/

Hospital switchboard: 0117 923 0000



Minicom: 0117 934 9869



www.uhbristol.nhs.uk



For an interpreter or signer please contact the telephone number on your appointment letter.



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