



Diagnosis and treatment



Steroids and Duchenne Muscular Dystrophy (DMD)

This leaflet has been produced to help you think about whether your child with Duchenne muscular dystrophy should have steroids or not. It includes some of the main questions people have asked when in this situation and also gives you some further information to look up. It is not intended to replace discussion with the muscle clinic team, so feel free to ask them any questions you may have after reading this.

Why are steroids used in DMD?

It has been known for a number of years now that steroids have an effect on muscle strength in DMD. If they are used in boys who are still walking, they **may** have an effect on stabilising or even improving muscle strength for a period of time. Not all boys respond to steroids and the way that steroids have this effect on slowing the dystrophic process is not known.

What steroids are used?

The main steroid that is used is called prednisolone (prednisone in the USA). Deflazacort is also used in some countries. These are **not** "anabolic steroids" which is what athletes use illegally to build up muscle- these do not have an effect in DMD.

What are the possible beneficial effects?

The studies which are now being reported on steroids are showing that overall boys who are treated with steroids walk for longer than those who are not. This effect varies from child to child but there are some studies coming out which are showing that some boys may carry on walking for years longer than they otherwise would. These children also seem to develop other complications of the condition less frequently too – for example they may develop breathing difficulties later than they might have and have fewer problems with their spines.

What are the possible risks?

The down side of steroid treatment, and the reason that people are still very cautious about using them, is that they may have side effects. Steroids have many side effects, but the chances of getting these vary from person to person, and on the dosage of the regime used. The most common side effects reported in the studies of using steroids in DMD in the short term are weight gain and mood changes. Weight gain seems to be most of a problem just at the time that the boys start taking the steroids, so it is a really good idea to keep a close eye on food intake at that time to avoid running into problems. If you would like further information on this, please ask the muscle team or your local dietician in the longer term (after many years of treatment) there may be growth suppression, the development of cataracts and thinning of the bones. The risk of these long-term effects cannot be measured. You should discuss these fully with your doctor. There are whole lists of other possible side effects, which include raised blood pressure, diabetes, thinning of the skin and poor wound healing and increased susceptibility to infection. A rare side effect of taking steroids in DMD is stomach irritation. It is important not to take non steroidal anti inflammatory tablets or medicines like neurofen or aspirin while you are on steroids. If your son develops tummy pain, or there is any sign of bleeding, you should contact your GP. This may not mean that they have to stop taking the steroids but it is important that this is noted. It is also very important that steroids are not stopped suddenly but tapered off as the body becomes used to their effect and needs time to adjust if they are withdrawn.

How do the benefits and risks balance out?

This is a difficult question. The reason that steroids are often used is because the studies which are coming out now are showing some significant benefits. In some studies some boys with DMD are still walking at the ages of 14 or 15. But there is no doubt that there can be important side effects, and the worst of these include significant growth delay and weak bones. The muscle team aim to minimise the risk of side effects by checking for them when you go to clinic. If significant side effects were picked up then the dose of the steroids could be altered, or tapered off completely. This may also be done if it seemed like they were not having a positive effect. It is important to realise that if the decision to use steroids is made this is not an "all or nothing" thing, but that the regime would be carefully worked out on an individual basis and changed if required. It is possible to change your mind at any time, though you must realise that steroids cannot be stopped suddenly.

What dose would be used and how often?

There are two alternatives. All of the studies that have shown a useful benefit of steroids have given them on a daily basis. However, it is possible that using steroids intermittently (i.e. not all the time) could be effective while reducing the risk of side effects. If you were particularly worried about the side effects this might be a good alternative. However, it has not been proven in controlled studies that this is as effective as using steroids every day.

What happens if we decide to use steroids?

If the decision to use steroids is made, then the muscle team will need to be keeping a close eye on your son and organise more tests than would usually be done. It will be very important that you attend these appointments. Your son will need to have some blood and urine tests to check everything is OK to use the steroids, and to check that he is immune to chicken pox (if not he will need to be immunised against it as chicken pox can be very serious in children who are on steroids). He will also need a scan of his bones and an eye check (these will need to be repeated at intervals while he is on the steroids), and a good assessment of how he is getting on at the moment.

Once the blood test results are back, and providing they are all OK, your GP will be asked to prescribe the steroids and you should be seen by your muscle team approximately three months after. At this appointment the team will check for any side effects and see if there is any difference in your son's physical performance and discuss with you if the dose seems right. He will then be seen as usual at six monthly intervals, though the GP will be asked to check his blood pressure and his urine for sugar in between appointments as well.

At every appointment, you can discuss the dose etc and what you want the plans for the future to be. It is possible that if your son responds well to the steroids that he will need to be on them for a long time. New information is becoming available every year about the long term use of steroids in DMD, and this should be fed back to you at the clinic.

What happens if we decide not to use steroids?

Your son will continue to be followed-up in the usual way and the muscle team will discuss with you all other options, as they become relevant. If you change your mind and want to think about steroids again in the future, you just need to mention this at one of your routine appointments.

Where can I find out more?

There are lots of sites on the web that discuss the use of steroids in DMD.

Here is one example, but you will be able to find more. Not all of them are completely up to date and it is important to realise that some of the most recent studies have not been formally published yet.

<http://www.parentprojectmd.org/treatment/supplements.html>

If the information in this fact sheet has been of use then you may like to consider becoming a member of the Muscular Dystrophy Campaign. Membership is free and is open to anybody over 16. By becoming a member you will receive Target MD magazine, which contains news and information on conditions, and you can also receive research and condition up-dates as soon as they are available.

To become a member please call **020 7803 4800** or freephone **0800 652 6352** or visit our website at **www.muscular-dystrophy.org**.

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Published: July 2004

Updated: April 2008

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