

**Duchenne Muscular Dystrophy: Double-Blind Randomized Trial to Find Optimum Steroid Regimen (FOR DMD study)**

**Patient information sheet for children aged six to eight years**

We would like you to be in the **FOR DMD study**.

***What is the FOR DMD study?***

This study is going to help Doctors to answer a very important question: what is the best medicine to help boys with Duchenne Muscular Dystrophy?

***Why have you asked me to take part?***

We would like 300 boys like you to take part. You have been asked because you are attending one of the special clinics at the hospitals that are helping with this study. You are also old enough to take part, if you want to.

***Did anyone else check the study is OK to do?***

Before this study started it was approved by the South Central - Southampton B Research Ethics Committee. This was done to make sure that the study is fair.

***Do I have to take part?***

It is up to you and your parents whether or not you take part. If you decide you don't want to, no one will get cross with you. Your Doctors and Nurses will still look after you as they do now. Your parents have an information sheet to read too. We would like you to decide with them if you will take part.

***What will happen to me if I take part in the study?***

If you will take part in the study, we will ask you to take a number of tests. You should show most of them already as you have done them at your visits to the clinic. All boys in this study will have the same tests. At the beginning of the study, you will come to the hospital, with your parents, more often than if you weren't in the study, and the first time you come in will be quite long, to make sure that it is OK for you to be in the study.

When you will come to the clinic for the study, we will ask you and your parents some questions about how you are doing and any medicines you are taking. Sometime, we will take some blood and urine to test, and will look at your skin and eyes. If you want, you can have some cream to make sure that the needle used to take some blood doesn't hurt. We will ask you what you do and what you eat and drink.



We will ask you to do some activities like getting up from lying down on the floor, walking for six minutes and moving around.



We will test to see how your lungs are working.



We will test to see how your heart is working.



We will look at your wrist and whole body with x-rays.



We will check to see that you can swallow the tablets that you will be taking in the study, with pretend tablets.



You will also be asked to answer some questions and write your answers down, with your parents. Your parents will also answer some questions, on their own forms.

None of these tests will hurt. When you are in the study, you will take the medicine every morning with your breakfast. We will see if there are any effects of the medicine on you. If you feel unwell, tell your parents or your doctor, so that we can help.

***Will the medicine upset me?***

If the medicine you take in the study upsets you your Doctor will take you off it straight away and treat you in the best way that he or she can.

***Will joining in help me?***

We cannot promise the study will help you but the information we get might help treat boys with DMD with better medicines in the future.

***What happens when the research stops?***

When the study is over your Doctor will use the results of it to treat you in the best way that he or she can.

***What if something goes wrong during the study?***

If this happens then we have a way of stopping your treatment and letting your Doctor know what medicines you have taken. He or she will then decide the best way to treat you.

***Will anyone else know I'm doing this?***

The Doctors and Nurses at the hospital will know who is in the study. Your GP (Doctor at home) will need to know that you are taking part too. In the study you get a special code number. The researchers use this in the study rather than your name.

***What if I don't want to be in the study any more?***

You can leave the study at any time. Just tell your parents or the Doctor or Nurse that you want to stop. No one will be cross with you. Your Doctor will look after you in the best way that he or she can.

***What if I have any more questions?***

If you have any other questions, don't be afraid to ask. Doctors and nurses get asked questions all the time and won't mind. You can always write down your questions and bring them with you the next time you visit the hospital, and they will be answered there.