Insert Study Title

Parent/Legal Guardian Information Sheet [Version XX Date XX]

SPONSORS:

Chief Investigator :
[NAME]
[ADDRESS]
[TELEPHONE]
[FAX]
[EMAIL ADDRESS]

Site Investigator:
[NAME]
[ADDRESS]
[TELEPHONE]
[FAX]
[EMAIL ADDRESS]

Introduction

We would like to invite your child or legal ward (referred to as "child" in the remainder of the document) to take part in this single / multi centre research study at [Name of participating site to be inserted]. This study is conducted by the Sponsor, XXX

Before you decide if you would like your child to participate, we want you to know why we are doing the study. We also want you to know about any risks (anything unexpected that might happen) and what you and your child will be expected to do in the study.

This form gives you information about the study. The study doctor will talk to you and your child about the study and answer any questions you have. We encourage you to discuss this study with your family and anyone else you trust before making your decision. Once we have answered all your questions, and if you decide that your child should take part in this study, we will ask you to sign a consent form to show that you understand and agree for your child to participate in the study. We will give you a copy of this information sheet and the consent form to keep as they have important information and telephone numbers that you should refer to throughout the duration of the study.

It is important that you know:

- Your child does not have to join the study
- You may change your mind and stop your child being in the study any time you want, without giving a reason.
- If we make any important changes to the study we will tell you about it and make sure you still want your child to be in the study.

This study is registered on the internet with ClinicalTrials.gov. You can access this website to gain more information (www.clinicaltrials.gov).

Also add in this section:
General study information
Details of the drug being tested

What is the purpose of the study?

Provide general information about the study and describe the aims of the study

Why has my child been asked to take part?

Describe main inclusion criteria of the study

e.g. We believe that your son may be eligible to participate in this research study because he has been diagnosed with DMD, is age ….., and ….

Report expected number of participants in the study, number of sites/countries involved

What medication is being tested in the study?

The investigational drug to be used in this study is XXXX. Investigational means that XXXX has not yet been approved by regulatory authorities.

- *Describe the study medication (name, preparation, taste, modality and frequency of administration)*
- *Is a placebo used in the study (A placebo is a dummy drug that looks like the study drug but contains no active medication. The placebo will look and taste exactly the same as the active drugs so you and your son will not know which medication your son is taking. Your study doctor will also not know what medication he is taking).*
- *If applicable, provide information regarding different dose groups, study design (e.g. dose escalation plan), randomization (what is the chance for the child to receive study medication)*

e.g. For randomized studies

In order to compare the effect of XXX versus placebo it is important that boys receiving different treatments are evaluated in the same way.
For the comparison to be fair, we need to make sure that there is no difference between the boys in the groups that are being compared. We do this by putting boys into groups at random – like tossing a coin or throwing dice - rather than by choice. Add information about how randomization is done (e.g. computer)

e.g. For Placebo-controlled studies:
In research it is known that people sometimes report that they are feeling better if they know that they are being given a certain treatment that they believe will work for them. Research has also shown that results can be influenced if the doctor or researcher believes that a medication they give a study participant is better than another. The best way to solve this problem is to ensure that neither the boys nor their families taking the study medication nor their doctors know which treatment they are on. This type of research is called a **'double blind'** study, i.e. both participants and doctors are 'blind' to the medication taken.

Nor you, your son or the study doctor will know which medication your son has been receiving in the study until the last subject recruited in the study has completed the study and all data have been collected. The study doctor will however be able to ask which treatment your son has been receiving in case of a medical emergency.

What will happen if I agree for my child to take part and what tests will be carried out?
Describe duration of the study, number and frequency of visits, duration of the visits and whether they will be performed in one or more separate days, whether overnight stay (in hospital and/or hotel) will be required
Provide details of each visit, how long they will take and what will happen to the child. Mentioned whether the child will be familiar with some or all study procedures
Schedule of events and/or further details can be provided in an appendix

Other medications
Ask to report any concomitant medication
e.g. During screening, you should inform the study staff of all prescription and non-prescription drugs, supplements, vitamins and supplements that your child is taking.

If there are any changes to your child's medications (starting medications, stopping medications, changing medication doses), these should be reported to the study team at [Name of participating site to be inserted].

Mention any prohibited medication

What are the potential risks or discomforts?

Report any information about possible risks from participating in the study including

- *Known and potential side effects of study medication*
- *Risks associated with study procedures*

e.g. Risks of Blood Draws: Taking blood may cause soreness or bruising at the site of the needle insertion. This is a common risk. Rarely, lightheadedness, fainting, or a more serious injury such as hematoma (bleeding under the skin) may develop. If a cannula is used, soreness or bruising at the site of the cannula insertion is possible. To reduce the discomfort of taking blood or cannula insertion, a local numbing cream may be applied to the area. The side effects that may be associated with numbing cream include lack of sensation to the area where it is applied, with an increased chance of harm to the area because of lack of sensation.

Depending on the study visit, between XX and XX ml of blood will be collected at a single time point.

e.g. Risk of Electrocardiography: Rarely, this test may cause irritation to the skin under the electrodes.

e.g. Risk of DEXA scan and Spine X-Ray: During the DEXA (bone) scan and the spine X-ray, your child will be exposed to a small amount of radiation. The extra radiation your child will be exposed to because he is participating in this study is less than he would be exposed in a normal year. This is considered an acceptable amount of radiation for patients with a high risk of fractures as can be seen in DMD. There may be some minor discomfort from lying in the same position for up to 10 minutes while having the DEXA scan.

e.g. Risks of Muscle Strength, Functional, and Timed Tests: It is possible that these tests could make your child more tired than after a regular (non-research) doctor's visit or that he may have muscle soreness. These are common risks. There are also uncommon risks of falling or shortness of breath.

e.g. Risk of inappropriate use of study medication: The medication used in this study are FOR USE BY RESEARCH PARTICIPANTS ONLY. Please take care to keep them out of the reach

of children or people who have trouble reading or understanding written directions. Use of the study medicine by persons who have not been carefully screened could be dangerous.

e.g. Risk of Loss of Confidentiality: The confidentiality of all study-related records will be maintained in accordance with the country specific laws. All paper records containing identifying information will be kept in locked files accessible only to the study team and unlocked only while a study staff member is physically present. Results of the study procedures listed above will be entered (without any identifying personal data) into a secure study database for analysis. Your child will only be identified by a study ID number to protect his confidentiality. However, because this is a rare disease there is a very small risk of your child being identified by mutation your child has.

What are the potential benefits of taking part in this study?

Describe whether any benefit is expected

e.g. There may be no direct benefit to your child from participating in this study though others may possibly benefit from information that the doctors gain while treating your child).

Does my child have to take part?

It is your choice to have your child take part in this study. You can stop his participation at any time. If you decline to have your child participate or you decide to withdraw him from the study there will be no penalty or loss of benefit. The care you and your child receive will not be affected in any way if you decide not to take part in this study.

Your child's doctor may be involved as an investigator in this study. As both doctor and a research investigator, s/he is interested both in your child's medical care and the conduct of this research study. Before agreeing to participate in this study, or at any time during your study participation, you may discuss your child's care with another doctor who is not associated with this study. You are not under any obligation for your child to participate in any research study offered by your doctor.

If you decide to withdraw your child from the study, any data and blood samples collected up to the point of withdrawal may be used for study purposes. Your decision to withdraw your child's agreement for the use of your child's private health information for this research study will have no effect on you or your child's current or future medical care.

The study doctor may withdraw your child from the study for various reasons, including:

- if your child cannot or does not take study medication
- you or your child do not comply with study procedures
- safety blood test results indicate that continuing in the study could be harmful
- not being able to obtain blood samples from your child for safety monitoring
- your child experiences side effects that are not tolerable

- if the study is closed

If you wish to withdraw your informed consent, you will need to notify your study doctor to indicate that you are withdrawing consent and the date of withdrawal. In this case, no further study procedures will be performed and no additional data will be collected.

What are other choices my child has besides participation in this study?

If you choose not to have your child participate in this study, other choices may include:

- standard medical care
- participation in other research for DMD

You should further discuss these options with the study doctor or your child's regular doctor.

What if new information becomes available?

If any information is learned that might affect your willingness to continue your child's participation in this research, you will be informed.

What happens when the research stops?

Insert sponsor's exit strategy

What happens to the results of the study?

The results of this study will be provided to you in language that you will be able to understand. They will be published in medical journals, which are read by doctors, and presented at conferences to be shared with other healthcare professionals, families with DMD boys and people and organisations that work with boys with DMD. Results of the study may be used by the Sponsor to seek regulatory approvals in various countries so that XXX is available to other boys with DMD.

Will I get paid for being in the study?

Neither you nor your child will be paid for his participation in the study. We will provide you and your child with reimbursements for any travel costs related to your study visits at [Name of participating site to be inserted] and the study medicine will be provided free of charge.

Who do I call if I have any questions?

We want you to ask questions about any part of this study or consent form either now or at any time in the future. If you have any questions about this study, call the Study Doctor, [Insert PI Name], at [Insert Area Code and Number]. If you have any questions or concerns about your child's rights in this research study at any time, please call [Name and telephone number of participating site ethics or patient advocacy to be inserted].

How will the confidentiality be maintained?

Your child's participation in the study means that you agree to the investigator collecting data about you and your child.

You are entitled to ask the investigator what data are being collected about your child and how it will be used. These data concern your child's current clinical situation but also some of his background, the results of examinations carried out within the context of care of your child's health in accordance with the current standards, and the results of examinations required by the protocol. You have the right to inspect the data and correct them if necessary.

The investigator has a duty of confidentiality with regards to the data collected. This means that he/she undertakes to never reveal your child's name in the context of a publication or conference and that he/she will encode your child's data (your child's identity will be replaced by an ID code in the study) before sending to the sponsor.

The investigator, his/her team and a person designated by the Sponsor to ensure that data are entered correctly will be the only ones able to establish a link between the data transmitted throughout the study and your child's medical records.

The personal data transmitted will not contain any combination of elements that might allow your child to be identified.

The data transmitted to the sponsor will not allow your child to be identified. The sponsor is responsible for collecting the data gathered by all investigators taking part in the study, processing them and protecting them in accordance with the requirements of the law on the protection of privacy.

To verify the quality of the study, it is possible that your child's medical records will be examined by persons subject to professional confidentiality and designated by the Regulatory Authority, the Sponsor of the study or an independent audit body. In any event, this examination of your medical records may only take place under the responsibility of the investigator and under the supervision of one of the collaborators designated by him/her.

The (encoded) study data may be sent to regulatory authorities, to the relevant ethics committees, to other doctors and/or to organisations working in collaboration with the Sponsor. The data may also be sent by the Sponsor to other countries where the standards in terms of the protection of personal data may be different or less stringent to this country. As explained above, the transmitted data are encoded.

Your consent for your child to take part in this study, therefore, also implies your consent to the use of your child's encoded medical data for the purposes described in this information form and to the transmission of data to the aforementioned people and authorities.

The Sponsor will use the data collected within the context of the study in which your child is taking part, but would also like to be able to use it in connection with other research concerning the same disease as your child has. Any use of your child's data outside the context described in this document is only possible with the approval of an ethics committee.

If you withdraw your consent for your child to take part in the study, to guarantee the validity of the research, the data encoded up to the point at which you withdraw your child from the study will be retained. No new data may be sent to the Sponsor.

With your permission, we will let your child's GP, and other healthcare professionals involved in his care, know that he is taking part in the study.

What happens to the biological samples collected during the study?

Insert information about what is going to happen to any samples taken. Where will the testing take place? What will happen to any leftover samples?