The Children's Hospital of Philadelphia®

# Informed Consent Form and HIPAA Authorization

**Study Title:** Magnetic Resonance Imaging and Biomarkers for Muscular Dystrophy

**IRB #:** 10-007802

Version Date: November 22, 2010 Version: 1.0 Control

Consent Name: MRI DMD Control

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|---|---|---|
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| **Affiliations:** | The Children's Hospital of Philadelphia<br>Department of Neurology | |
| **Study Sponsor:** | National Institutes of Health (NIMAS and NINDS), University of Florida | |

You, or your child, may be eligible to take part in a research study. This form gives you important information about the study. It describes the purpose of this research study, and the risks and possible benefits of participating.

If there is anything in this form you do not understand, please ask questions. Please take your time. You do not have to take part in this study if you do not want to. If you take part, you can leave the study at any time.

Parents or legal guardians, who are giving permission for a child, please note: in the sections that follow the word 'you' refers to 'your child.

## Why are you being asked to take part in this study?

You are being invited to take part in this research study because you are a healthy subject and are between 5 and 14 years of age that will be used for comparison purposes.

## What is the purpose of this research study?

Duchenne Muscular Dystrophy (DMD) is a genetic muscle condition seen only in boys. DMD causes weakness of large hip and shoulder muscles which later spreads to the small muscles of the body. This weakness is responsible for a decreased ability to walk and to perform other daily tasks. The purpose of this research study is to learn more about possible changes that occur in muscles of the lower leg in boys with DMD. In addition, the goal of this study is to develop an improved imaging procedure to monitor the progression of disease in children with DMD.

## How many people will take part?

About 200 people at 3 medical centers will take part in the study, including approximately 50 participants from CHOP.

## What is involved in the study?

This study aims to establish and validate (confirm) an improved imaging procedure to study muscle damage in patients with DMD. We will compare the muscles of boys with DMD with muscles of healthy children of the same age and determine whether this new technique can be used to monitor disease progression. Your healthy muscle will allow us to make comparisons with boys who have Duchenne Muscular Dystrophy. Comparisons will also be made related to your daily activities such as walking.

If you agree to take part, you will be expected to participate up to a total of two times in one month. On each testing day you are expected to participate for around three to six hours.

If you take part in this study you will have the following tests and procedures. Some of the procedures may be repeated several times.

**Magnetic Resonance Imaging (MRI):**

MRI is a procedure that allows doctors to look inside the body by using a scanner that sends out a strong magnetic field and radio waves. You will lie on a platform inside the magnet. A series of images will be taken from your thigh and lower leg. The scanning period will take approximately 90 minutes, during which you are required to lie still. During the entire procedure, one of the investigators will be present with you and will advise you of the progress of the study, making sure that you are comfortable. Although the measurement is painless, it will be somewhat noisy inside the magnet due to a hammering sound made when a large electrical current is pulsed through the magnet. Disposable earplugs will be provided to reduce the noise.

**Magnetic Resonance Spectroscopy (MRS)**

This procedure will give us information regarding the chemistry of your muscles and uses the same scanner. This technique will give us biochemical information about your muscles and will help us understand healthy muscle and fat inside of your muscles. These measurements will be performed at the same time as the MRI procedure.

**Muscle and Functional Performance Tests**

The muscle and functional performance tests will be performed at The Children's Hospital of Philadelphia. The testing session for muscle and functional performance should be 3-6 hours. You will first warm-up your muscles by performing simple exercises. Then the following tests will be performed:

- Range of motion (ROM) measurements will be taken for the joints in your legs.
- Your leg muscles will be tested using one of the strength testing devices. Depending on the results, we may ask you to repeat this procedure as many as three to eight times.
- We will test your muscle strength manually by demonstrating a movement and then asking you to perform the movement with your arms or legs.
- You may be required to perform simple tasks such as getting up from the floor, climbing steps, and walking at different speeds and distances for up to 6 minutes. If at any time you become tired you will be allowed to rest.

- We may also ask you to wear an accelerometer to collect data on your activity level over the course of several days. This device will measure how much activity you participate in during waking hours.

**Visit Schedule**

The table below provides a brief description of the purpose and duration of each study visit.

| Visit | Procedures | Duration |
|---|---|---|
| Visit 1- Baseline | MRI, MRS, medical history, medication log, Tanner staging | 3 - 6 hours |
| Visit 2- Follow-up | MRI, MRS, Strength and Functional testing, activity monitor | 3 – 6 hours |

## What are the risks of this study?

Taking part in a research study involves inconveniences and risks. If you have any questions about any of the possible risks listed below, you should talk to your study doctor or your regular doctor.

Under the conditions proposed for this project, MRI and MRS are not known to harm living systems. This procedure is used routinely for medical care and is very safe for most people, but you will be monitored during the entire MR scan in case any problems occur. However, the possibility of unforeseen hazards cannot be ruled out.

**Risks of MRI and MRS**

- The MRI scanner contains a very strong magnet. Therefore, you may not be able to have the MRI if you have any type of metal implanted in your body, for example, any pacing device (such as a heart pacer), any metal in your eyes, or certain types of heart valves or brain aneurysm clips. Someone will ask you questions about this before you have the MRI.
- There is not much room inside the MRI scanner. You may be uncomfortable if you do not like to be in close spaces ("claustrophobia"). During the procedure, you will be able to talk with the MRI staff through a speaker system, and, in the event of an emergency, you can tell them to stop the scan.
- The MRI scanner produces a loud hammering noise, which has produced hearing loss in a very small number of patients. You will be given earplugs to reduce this risk.

**Risks of Muscle and Functional Testing**

You may experience some muscles soreness and stiffness as a result of the muscle and functional performance testing.

This study may include risks that are unknown at this time.

## Are there any benefits to taking part in this study?

There is no direct benefit to subjects participating in this study. However, you will receive information in regard to the strength and composition of your lower extremity muscles as determined by MRI/MRS.

The potential benefit to others is through the information found in the study. By comparing information from your muscles with that of boys with Duchenne Muscular Dystrophy, a better and more specific understanding of the muscular changes associated with DMD may be obtained. In addition, since the goal of this study is to develop a better imaging procedure to study disease progression in children with DMD, the information gained in this study may be of benefit to future clinical trials in DMD.

## Do you need to give your consent in order to participate?

Once you read this form and had your questions answered, you will be asked to decide if you wish to participate. If you wish to participate in this study, you must sign this form. A copy will be given to you to keep as a record.

**What happens if you decide not to take part in this study?**

Participation in this study is voluntary; you do not have to take part in order to receive care at CHOP. If you decide not take part or if you change your mind there will be no penalties or loss of any benefits to which you are otherwise entitled. Your current and future medical care at CHOP will not be affected by your decision.

**Are there alternatives to participation in this study?**

The alternative to participating in this study is to not participate in the study.

## What about privacy, authorization for use of Personal Health Information (PHI) and confidentiality?

We need to collect health information about you in order to conduct this study. This includes information about you from medical records and from the procedures, interviews and tests that are part of this research. We will do our best to keep your personal information private and confidential. However, we cannot guarantee absolute confidentiality. Your personal information may be disclosed if required by law.

This information will be stored in locked filing cabinets or on computer servers with secure passwords, or encrypted electronic storage devices.

The results of this study may be shown at meetings or published in journals to inform other doctors and health professionals. We will keep your identity private in any publication or presentation about the study.

People and organizations that may inspect and/or copy your research records to conduct this research, assure the quality of the data and to analyze the data include:

- Members of the research team at CHOP;
- Medical staff who are directly or indirectly involved in your care related to this research;
- People who oversee or evaluate research and care activities at CHOP;
- Co-investigators who are directly involved with the study at other participating sites: University of Florida, Oregon Health and Science University and University of Pennsylvania
- People from agencies and organizations that perform independent accreditation and/or oversight of research; such as the Department of Health and Human Services, Office for Human Research Protections
- The Data Coordinating Center at the University of Florida
- Groups monitoring the safety of this study
- The National Institutes of Health who is sponsoring this research
- Public health authorities that are required by law to receive information for the prevention or control of disease, injury or disability

By law, CHOP is required to protect your health information. The research staff will only allow access to your health information to the groups listed above. By signing this document, you are authorizing CHOP to use and/or release your health information for this research. Some of the organizations listed above may not be required to protect your information under Federal privacy laws. If permitted by law, they may be allowed to share it with others without your permission.

Your PHI will be collected until the end of the study. There is no set time for destroying the information that will be collected for this study. Your permission to use and share the information and data from this study will continue until the research study ends and will not expire. Researchers continue to analyze data for many years and it is not possible to know when they will be completely done.

## What are my rights and responsibilities as a research subject?

Taking part in a research study involves time and responsibilities. You need to follow the study doctor's instructions, keep all study appointments. Please consider the study time commitments and responsibilities as a research subject when making your decision about participating in this study.

You may change your mind and take back your authorization to use and disclosure your health information at any time. Even if you take back your authorization, we may still use and disclose the health information we have already obtained about you as necessary

to maintain the integrity or reliability of the current research. To take back your authorization, you must send a letter to Dr. Finkel at The Children's Hospital of Philadelphia. In the letter, you must say that you changed your mind and do not want us to collect any more health information about you. If you ask that we no longer collect your health information you will have to leave the study.

Your health is important to us. We will talk to you if we need to make changes to the study to protect your health. We will also tell you about any new information that may change whether you want to stay in the study.

A Data Safety and Monitoring Board, an independent group of experts, will be reviewing the data from this research throughout the study. We will tell you about the new information from this or other studies that may affect your health, welfare, or willingness to stay in this study.

## Financial Information

**Will there be any costs to you?**
The study sponsor is providing financial support and material for this study and the procedures described above. There will be no cost to you.

**Will you be paid for taking part in this study?**

You will receive a $5.00 gift certificate after completing each testing session in the study. The total compensation for two visits will be $10.00.

You will be reimbursed $15.00 at each visit to cover parking and one meal for you and one parent/legal guardian.

**Who is funding this research study?**

The National Institutes of Health is providing funding for this study.

Please ask Dr. Finkel if you have any questions about how this study is funded.

## What if you have questions about the study?

If you have questions about the study, call the study doctor, Dr. Finkel at 215-590-2763. You may also talk to your own doctor if you have questions or concerns.
The Institutional Review Board (IRB) at The Children's Hospital of Philadelphia has reviewed and approved this study. The IRB looks at research studies like these and makes sure your rights and welfare are protected. You can talk to a person from this group if you have questions about your rights as someone taking part in a research study. You can call the IRB Office at 215-590-2830 if you have questions or complaints about the study.

## Consent for Use of Data or Specimens for Future Research

As part of the study, we will collect information on you from the MRI and muscle and functional testing. This information will be given a unique code and will include no information that can identify you. Information that can identify you may be kept

permanently in a secure database at Children's Hospital of Philadelphia and the University of Florida. Only the study doctors and those working with them on this study will be able to see information that can identify you.

If you leave the study, you can ask to have the data collected about you removed or your samples destroyed. You can also ask us to remove information that identifies you from the data or samples.

We may wish to use your stored information in a future study related to DMD. Please let us know if you will let us do that by putting your initials next to one of the following choices:

_____ (initials) My information may be used for this study only.

_____ (initials) Provided that my identity stays private, my information may be used for other future research studies.

## Consent to Take Part in this Research Study and Authorization to Disclose Health Information

The research study and consent form have been explained to you by:

| Person Obtaining Consent | Signature of Person Obtaining Consent<br>Date: |
|---|---|

By signing this form, you are indicating that you have had your questions answered, you agree to take part in this research study and you are legally authorized to consent to your child's participation. You are also agreeing to let CHOP use and share your health information as explained above. If you don't agree to our collecting, using and sharing your health information, you cannot participate in this study. **NOTE:** *A foster parent is not legally authorized to consent for a foster child's participation.*

| Name of Subject | |
|---|---|
| Name of Authorized Representative | Relation to subject:<br>☐ Parent ☐ Legal Guardian |
| Signature of Authorized Representative | Date |
| Witness | Date |

## Child Assent to Take Part in this Research Study

**For children capable of providing assent:**

I have explained this study and the procedures involved to _____ in terms he/she could understand and that he/she freely assented to take part in this study.

_____
Person Obtaining Assent

_____ _____
Signature of Person Obtaining Assent Date

This study has been explained to me and I agree to take part.

_____ _____
Signature of Subject (optional) Date

**For children unable to assent:**

I certify that _____ was not capable of understanding the procedures involved in the study sufficiently to assent to study participation.

_____
Person Responsible for Obtaining Assent

_____ _____
Signature of Person Responsible Date