

## UK FSHD Patient Registry

**Principal Investigator/Data Controller: Prof Chiara Marini Bettolo,  
Translational and Clinical Research Institute, Newcastle University**

### Information for Young People – Aged 11-15

**You are being asked to take part in an ongoing research project** known as a 'Patient Registry'. By sharing details about how you feel and letting your doctor add some medical information and test results, researchers will be able to learn more and do more work to help people living with FSHD. You can join if you have any type of FSHD, and you can ask as many questions as you like before deciding if you want to take part.

**Before you decide if you want to join in it is important to understand why the project is being done and what it means for you. Please read this sheet carefully and talk about it with your family and friends. There is a list at the end of this sheet to help explain what some words mean.**

#### Why are we doing this research?

It is important to collect data from people with rare conditions like FSHD so we can learn more about the condition and help scientists who are trying to make treatments. To do this we have set up a registry (a kind of *list* stored on a computer) made up of information about the way FSHD affects you. We ask you to answer some questions online once a year (with help from your parent/guardian) then we store the answers safely in the registry. When all the information is stored together is very useful for scientists and researchers who are trying to find out more about FSHD.

We want to invite you to take part, with your parents'/guardians' permission, and ask if it is OK for us to store some information about you. If you are happy to be involved then we will ask you and your parents/guardians to sign a form ('*consent*') so we can collect and keep the information.

Prof Marini-Bettolo is the PI (short for Principal Investigator) for the registry, which means she is the doctor in charge of looking after the registry at Newcastle University. The day-to-day running and management of the registry is done by a Registry Curator – please see the [registry website](#) for contact information.

#### Why have I been asked to take part?

You have been asked to be part of this project because you have a condition called FSHD. We are trying to invite everyone with FSHD if they would like to take part. The information you give us can then be used to help researchers looking at how to help people with this condition.

#### Do I have to take part?

You do not have to take part in this project. Deciding not to take part will not change how your doctors look after you. If you do want to take part then you, and your parents/guardians, will be asked to sign a *consent form* to say that you agree, and that we can keep information about you. You will also have a copy of this form to keep.

If you, or your parents/guardians change your minds and decide you don't want to be part of this project anymore then you can leave the project (*withdraw consent*) and we can remove the information about you from the registry. This will also not change the way the doctors look after you.

### **What will happen if I take part?**

If you are happy to be part of this project then we will invite your hospital doctor to join too, and ask them to share some information from your hospital notes. You will not be asked to have any extra tests done for this project, and you will not need any time off school.

Either you, your parents, your doctor (or someone in their team) or someone in Prof Marini Bettolo's team will put the information about you on to the database. Prof Marini-Bettolo is the doctor in charge of the registry at Newcastle University, this job is called being the PI (short for Principal Investigator). It is stored safely on a secure computer server that is protected by passwords, so nobody else will see your information.

### **Will this project help me?**

We hope that the information you give will be helpful for everyone living with FSHD in the future, because the more we know, the more the doctors can learn how to help. Medicines to help the condition may take a long time to be discovered and nobody knows when they will be found, so we cannot promise that anyone will receive them. But, the information gathered along the way helps to show that there are young people living with FSHD in the UK, and can encourage and support people to try and find medicines.

If new treatments are developed that may help you, we can then contact you to see if you wanted to try them in what is called a 'clinical trial'. These won't be run by the same people that run the registry, but we will make sure you are given the information for who to contact. We can't promise that this will happen, and if it does, you do not have to be involved - doctors would talk to you again at a different time if this was a possibility.

There may be other benefits to joining the registry, for example if data is used to improve national standards of care, in academic research, or to help with getting new treatments approved by the NHS. You will not receive any direct payments or any other financial benefit as a result of joining the registry.

### **Who is funding the project?**

The project is funded by MDUK ([Muscular Dystrophy UK](#)). No additional payments will be received by Prof Marini Bettolo, or other members of the registry team, for adding your details on to the database.

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

We will keep information about you private. This means we will only tell people who need to know you are taking part in the registry.

Only the people who help add your details (like your hospital doctor and your parents/guardians), and people allowed by the doctor in charge of the registry will know you are taking part and will be able to see the information about you. You can tell anyone you like, but you don't have to. Your parents/guardians will also need to know, as they need to sign the *consent form* and will help make sure your information is correct.

Researchers may ask for information from the registry to help them learn more about the condition. Your information might be shared with them if Prof Marini Bettolo and her team think they have a good reason to ask for the data and if their research might help you in future. We will never share personal details like your name, age or address when we share data from the registry. Nobody will ever know it is about you.

### **Who decided the project could be done?**

Before any *research* can be done it has to be checked by an *Ethics Committee*. This research has been reviewed and approved by North East- Newcastle and North Tyneside 1 Research Ethics Committee. This is to make sure that the *research* is a good idea and is safe to do. It has also been checked by support groups to make sure it is appropriate and useful.

There is another group of experts called the Registry Steering Committee who carefully check how the information collected by the registry is used and make sure we only share information with people who are trying to help.

This project is organised by the John Walton Muscular Dystrophy Research Centre (part of Newcastle University).

### **What happens after my 16<sup>th</sup> birthday?**

In the UK, once you are sixteen, you are able to choose for yourself whether or not you would like to participate in research, such as the UK FSHD Registry. It will no longer be your parents'/guardians' decision. Following your sixteenth birthday, the registry team will contact you to ask if you would like to continue participating in the registry. It is completely up to you and you can say yes or no. However, if you are happy to continue, we will transfer login details from your parents to yourself and ask you to sign a new consent form to show you still want to be part of the registry.

### **Contact Details:**

If you would like to talk to someone about taking part, your parents can contact either the Principal Investigator/Data Controller: Prof Chiara Marini Bettolo, or the Registry Curator on the details below:

#### **Principal Investigator:**

Prof Chiara Marini Bettolo

Tel: 0191 241 8737

Email: [Chiara.Marini-Bettolo@newcastle.ac.uk](mailto:Chiara.Marini-Bettolo@newcastle.ac.uk)

### **Registry Curator:**

Tel: 0191 241 8640

Email: [fshdregistry@newcastle.ac.uk](mailto:fshdregistry@newcastle.ac.uk)

Or you may contact the Patient Advice and Liaison Service (PALS) 0800 0320202.

### **GLOSSARY – What do these words mean?**

**Clinical trial** – a research project to test a new treatment, medicine, or therapy with patients living with a particular condition (like FSHD).

**Consent** – Saying yes to something, or agreeing it can be done. You can always change your mind after giving consent.

**Consent form** – A written record to show that you have given permission for us to store your data. This can be on paper or on a computer.

**Database** – a collection of information stored on a computer

**Ethics Committee** – a group of experts who look carefully at research projects to make sure they are planned fairly, and properly carried out

**Password protected** – a way of stopping people from looking at your information without permission

**Registry** – a special kind of database for collecting health information

**Research** – careful investigation of a particular subject, learning more about something

**Steering Committee** – a group of experts, including people from patient groups, who make sure the data collected is only shared with people or companies who are trying to help.

**Thank you for taking the time to read this information sheet.**

