UK FSHD Patient Registry

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

Information for parent(s) / guardian(s) of registry participants under 16 years

Your child is being invited to take part in an ongoing research project known as a 'Patient Registry.' This would involve you helping your child to answer some questions about how FSHD affects them and giving consent for the research team to keep a record of their answers in a secure, password-protected computer database. Your child can join if they have any type of FSHD, including if they do not have any symptoms. You can also nominate your child's medical team to add some extra information from their medical notes.

Before you accept or decline the invitation on behalf of your child, it is important for you both to understand why the research is being done and what it will involve. Please read the following information and discuss it with your child, and any friends or relatives, or your GP if you wish. If you have any further questions, please ask us (contact details at the end of this document, or on the website here).

Your child should also read the information sheet for their age range (6-10 or 11-15 years) and ask any questions they might have. Take time to decide whether you would like your child to take part or not.

Why has my child been asked to take part?

Your child is being invited to take part in this research because they have a rare neuromuscular condition known as Facioscapulohumeral dystrophy (FSHD) and we are trying to collect more information on patients, like your child, with the same condition.

Why is this research being done?

It is important to collect data from people with rare conditions like FSHD, to help researchers and scientists trying to better understand the condition. More research is likely to improve the care patients receive in future. Specific examples of how the registry data has been used to further research and support the FSHD community in the UK can be viewed on the registry website.

Having a computer database of patients with FSHD also means that patients that may be eligible to take part in clinical trials to help develop new treatments can be easily found, and this allows those who are interested to volunteer to take part if they wish. This helps to improve the equality of access to trials for patients living all over the UK.

The registry is different to any mailing lists or contact databases that you or your child may be on with a patient organization (for example the FSHD Society's contact database) and they will not be signed up automatically. You need to either sign your child up to the registry online yourself or ask the registry team for support to sign them up.

The John Walton Muscular Dystrophy Research Centre (Newcastle University), is responsible for maintaining this registry. The project is funded by the patient organisation MDUK (Muscular Dystrophy UK) and clinical data entry support is provided by many health professionals across the UK.

Before you consider registering your child’s details it is important that you understand what is involved and what will be done with the information that you provide. This information sheet contains answers to some of the questions you may have about the registry. When you have read this document, and after you have had some time to think about it, we will ask if you wish for your child to be registered on the database. You will be asked to sign an online consent form stating you agree for your child to join, and we also ask your child to read and sign an assent form.

If you or your child have any questions or accessibility issues, please contact the registry team before signing the consent form.

How will my child benefit from registering?

This database aims to benefit patients living with FSHD in the UK by collecting useful information which can be used to improve patient care, to support research into potential treatments, and to show that there is a community of people living with FSHD in the UK who may not otherwise be counted. We will contact you when we are able to share information about new treatments or possible new treatments (clinical trials), or other relevant news and information. By holding secure records of your child’s clinical details, we will also be able to check whether such trials may be suitable for you to consider enrolling your child in. 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.

Neither you nor your child will receive any direct payments or any other financial benefit because of joining the registry. The results of research arising from the data may have business potential, but you will not receive financial benefits from such development.

Details of any completed requests to use the registry data will be available to view on the registry website, along with any publications, or other research outputs to which the registry has contributed data (where publicly available).

Where can I find out more about how my child’s information is used?

Details of any completed requests to use the registry data are available to view on the registry website, along with any publications, or other research outputs to which the registry has contributed data (where publicly available). You can also contact the Registry Manager, or visit at www.hra.nhs.uk/information-about-patients/ for more information.

What information will we be asked to provide?

The registry asks questions about your child and how FSHD affects them. You will receive an email reminder every year after your child first joins, to ask you to update their information again. You will also have the chance to provide the details of a neuromuscular specialist (normally a consultant doctor in a hospital) if your child sees one and give them permission to add some information from their medical notes to the registry. All the questions can be viewed on the registry website before taking part, including the questions your child’s specialist would be asked to answer. You will be able to see the answers the specialist has provided, but you cannot edit these yourself. If you have any questions about any data entered by the specialist, please contact the Registry Project Manager.

The registry may add additional questionnaires in future to help inform research; if a new questionnaire is added this will always be optional and additional information specific to that questionnaire will be provided.

It is important that you keep contact information for yourself and your child up to date, particularly your email address as this is used as your registry username, and for any communications from the registry. If you do not have an email account or internet access, you can nominate a trusted family member or friend to help you with your child's data entry and to pass on any email correspondence.

Sharing Genetic Test Results with the registry

While it's not essential to share this with the registry, some of the most important data for research are the details of your child's genetic diagnosis, found in their genetic test results. There are currently two ways this can be shared with the registry:

1) your child's nominated neuromuscular specialist can input the data directly via the clinician form, or
2) a digital copy of the test results letter can be uploaded to the registry's secure upload site, to be entered by the registry team.

If you don't have a copy of your child's genetic report, you can ask at the hospital where their blood test was taken as part of their diagnosis, or their GP may also have access. Patient organisations like MDUK (Muscular Dystrophy UK) can also help you will this if you get stuck.

The registry team are working on a new collaboration with the national FSHD testing laboratory so that patients will have the option for the registry to receive a copy of their genetic test results directly. This is still in the planning stages, and this will only happen if you decide to give permission for this in future by signing a separate consent form.

I want my child to be involved in a clinical trial – is this guaranteed if we register?

Although one of the main aims of the registry is to make it easier for patients to be informed about clinical trials, there is no guarantee that registering your child's details will mean they will be able to take part in a clinical trial. The registry does not run or recruit directly to clinical trials. We will circulate information about relevant clinical trials that we think patients might meet the eligibility criteria for if they are recruiting in the UK, but these will always be subject to additional eligibility and screening criteria by trial site teams.

I'm not interested in my child getting involved in clinical trials, so why should I register them?

The registry is not only for recruiting to trials, but also for collecting vital information like patient numbers which helps build an understanding of how many people in the UK live with FSHD. This can help to improve clinical treatment for patients. By registering you are ensuring your child is counted, this helps us to demonstrate to big research companies (for example) that the UK FSHD community is worth investing time into.

How will my child's information be kept confidential and protected?

All information we receive about your child will be treated confidentially and stored on a secure computer server located in the UK, specified on the registry website. Details of your child's specific diagnosis as well as personal information (name, age, address, gender, etc.) will be stored on the database. This information is required to help show how the condition affects people in a particular way, to improve understanding that can then be passed into clinical care. It also enables us to match your child with eligibility criteria for clinical trials, and for other research purposes. Only members of Dr Marini Bettolo's team with specific permission will be allowed to look at this information. If we publish any research or other documents based on information from the database, this will not identify you by name.

The registry has a Steering Committee responsible for governing the use of registry data. The committee includes academic, medical, and genetic experts, as well as patient advocates and representatives. The committee review and approve or reject all requests for the registry to support specific projects, or requests for for data, ensuring that we only provide support to projects which may be beneficial to the community.

Does my child have to register? Can we change our decision?

Joining the registry is voluntary. Should you wish to withdraw your child from the registry you will be free to do so at any time without having to provide any explanation. If you wish to withdraw your child, you should contact the staff in charge of the registry. Joining or leaving the registry will in no way affect the care your child receives for their condition. Please note is not possible to withdraw data already used for other studies etc.

If you have chosen to give your hospital doctor permission to add clinical data to your registry record, this permission can be revoked at any time by contacting the Registry Manager. This permission will also be revoked automatically if you decide to withdraw from the registry completely.

How will my child's data be collected and updated?

You will be able to update most of your child's details via the registry website, and you can contact the registry team at any time if you need to amend any other information. The registry will automatically send you an email reminder after a year asking you to check or update your child's details. If your email address stops working, we will try to telephone you or send a letter to get an updated email contact for you. If you nominate your child's neuromuscular specialist, we will also ask them to update your child's clinical information once a year.

If you are unable to enter your child's information to the registry yourself due to physical disability or lack of internet access for example, you can nominate a trusted family member, carer, or friend to enter the answers for you, to pass on email messages etc. Please contact the registry team before signing up to learn more about this. The registry curator may also be able to help with this.

Once your child turns sixteen, they can choose for themselves whether they would like to continue to participate in the UK FSHD Registry. They will be contacted by the Registry Curator and asked to re-register using their own email address, and to sign a new consent form. The curator will arrange for their childhood data to be merged with their new adult record.

Who is funding the registry?

The registry is currently funded by MDUK (Muscular Dystrophy UK). No additional payments will be received by Dr Marini Bettolo, or other members of the registry team, for adding your child's details on to the database.

Who has reviewed the project?

Although the registry is managed and run from Newcastle University, we receive approval from an NHS ethics committee to make sure we are not doing anything harmful to you or your data. This research has been reviewed by North East – Newcastle and North Tyneside 1 Research Ethics Committee who have given approval for this registry to continue. Our ethical approval is renewed at least every three years.

What if I have any concerns or further questions?

If you have any concerns, or other questions, about this study or the way it has been carried out, you should contact the Principal Investigator/Data Controller: Dr Chiara Marini Bettolo, or the Registry Curator.

Principal Investigator:

Dr Chiara Marini Bettolo
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Registry Curator:

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If you feel that your child has been treated unfairly, or would like to comment on the conduct of any aspect of this research, please contact the Patient Advice and Liaison Service (PALS) 0800 0320202

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

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.

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

Consent form – agreeing to something in writing. This can be on a computer or a paper form.

Database – a collection of information stored safely on a computer.

Ethics Committee – a group of people 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.

Research companies – a business that pays for investigation of a particular subject.

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.