



UK FSHD Patient Registry

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

Information for Parents

Your child is being invited to take part in a research database. Before you accept or decline the invitation on behalf of your child, it is important for you 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 (contact details overleaf). 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 Facioscapulohumeral Dystrophy (FSHD) and we are trying to collect more information on patients, like your child, with similar conditions. An information sheet for your child can be provided.

Why is this research being done?

The aim of this research is to establish a database, which will be a computer record of patients in the UK with FSHD. This will be a computer record from which we can identify groups of patients easily, who may be able to take part in clinical trials and help develop, or try out, new treatments for FSHD. The database will contain information needed by the researcher, including your child's diagnosis and other key details of their disease. The project team (data controller and data processor) are based at the John Walton Muscular Dystrophy Research Centre at Newcastle University and are responsible for the registry. The project is part of the TREAT-NMD neuromuscular network, and the project is supported by Muscular Dystrophy UK (MDUK), as well as 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 database. After reading this information sheet, you will be given time to think about the study, and to ask any additional questions that you may have (contact details overleaf). We will then ask if you wish for your child to be registered on the database. You will be asked to sign a consent form stating you agree for your child to join.

How will my child benefit from registering?

The database aims to benefit adults and children with FSHD. By holding clinical information on the database we will be able to identify suitable patients to help assess possible new treatments (clinical trials), and contact you to ask for your help. The results of research arising from the database may have business potential (if for example utilised by a pharmaceutical company to develop a new drug) but you will not receive financial benefits from such development. You, or your child, would not receive any payment or other financial benefit as a result of joining the database. Nevertheless, there may be other benefits to your child being involved, including:

- Informing you about suitable clinical trials that you may wish your child to join
- Updates on new developments on your child's condition
- Providing information for healthcare professionals interested in the best standards of care for your child's disease

Data linkage to the Newcastle MRC Centre Biobank for Rare and Neuromuscular Diseases

The Newcastle MRC Centre Biobank for Rare and Neuromuscular Diseases is a repository that creates a 'bank' of samples including: DNA and cells obtained from blood, urine, saliva, skin, muscle and nerve from healthy people, as well as from those with diagnosed diseases, for use in future research. A biobank is the term used to describe a collection of samples stored for research.

We are working to anonymously link your child's registration on the DM national registry to samples contained in the biobank should these be available. Linking the registry data with data that may be held in the biobank will help enhance the information available to researchers when accessing the samples or the registry, therefore facilitating research. Data would be linked via a unique identifier which ensures confidentiality is preserved.

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

Although the database will make it easier to identify patients suitable for recruitment to clinical trials, there is no guarantee that you and your child would be automatically approached to take part in clinical trial. Doctors organising clinical trials will review your child's details on the database and if they appear suitable will invite your child to join. All patients invited to join a clinical trial will be assessed in greater detail at this stage to ensure there are no reasons for them not to take part, which was not obvious from the information recorded on the database.

Will information about my child be kept confidential?

All information that we receive about your child will be treated confidentially. The information will be converted into code and stored on a secure computer located in the UK. We will make every effort to ensure your child's data is kept safe but we remind you to be vigilant when entering information online. Details of your child's specific diagnosis as well as personal information (name, age, address, gender) will be stored on the database. This information will allow us to identify your child easily should the need arise. Only specified members of Dr Marini Bettolo's team will be given access to look at this data. If we publish any research, or other studies, based upon information on the database this will not identify your child by name.

If you would like more information about how we manage personal data more generally, including your rights under law, and the contact details of the University's Data Protection Officer, please see our website: <https://www.ncl.ac.uk/data.protection/>.

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

Your participation in this project is voluntary. Should you wish to withdraw your child's data from the registry you will be free to do so without having to provide any explanation. If you wish to withdraw, you should contact the Registry team (Contact details are provided at the end of this document),

How will my child's data be used?

The main aim of us asking you to be part of the registry is to help recruitment into clinical trials. However the data may also be used to learn more about people with FSHD. The answers information you provide may be used to help researchers design future investigations.

Who is funding the research?

The project is funded by MDUK . No additional payments will be received by Dr Marini Bettolo, or other members of the research team, for adding your child's details on to the database.

Who has reviewed the project?

This research has been reviewed and approved by North East- Newcastle and North Tyneside 1 Research Ethics Committee.

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

Tel: 0191 241 8606

Email: Chiara.Marini-Bettolo@newcastle.ac.uk

If you feel that your child has been treated unfairly throughout the research, 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

If you are concerned about the management of your personal data, a complaint may be lodged with the Information Commissioner's Office, UK: <https://ico.org.uk>.

Thank you for taking the time to read this information sheet