



Global FKRP Registry

INFORMATION FOR PATIENTS AGED 11-18 YEARS

Principal Investigator/ Data Controller:

Prof. Volker Straub, Newcastle University, UK.

If you have questions relating to the registry, you or your parents can contact either your local doctor or the registry principal investigator Prof. Volker Straub (you can find his contact information below).

You are being asked to take part in a research project so that we can learn more about your condition. Before you decide if you want to join in it is important to understand why the project is being done and what it will mean for you. Please read this sheet carefully and talk about it with your family and friends. Take time to decide whether you would like to take part or not. There is a glossary at the end of this information sheet to help explain what some words mean. If you want to contact the registry team, please use this email address: coordinator@fkrp-registry.org

Why are we doing this research?

We would like to set up *clinical trials* and *research* studies to develop and test potential treatments that could help you keep your strength for as long as possible. We would like as many patients as possible, who have a muscular dystrophy like yours, to participate in *research* studies and *clinical trials*, so we are setting up a registry for patients all over the world who have a similar condition to yours. With the help of this registry we can also find out how many children and adults there are in the world with your condition and if they are all receiving the right level of care.

If new treatments are made that may help you, we can then contact you to see if you wanted to try them ('*clinical trial*'). You do not have to be involved in any *clinical trials* and we would talk to you again at a different time if this was a possibility. You and your parents would need to sign a different *consent form* at that time as well.

Why have I been asked to take part?

As you probably know, you have a condition that is caused by a defect in a gene which affects your muscles. This condition has either been called "congenital muscular dystrophy" or "limb girdle muscular dystrophy". You might have noticed that you are not as strong as your friends or your brothers and sisters and that you are not able to run or do sports as well as they do. The reason is due to the faulty gene, the so-called FKRP gene. This means that your muscles do not work as well as they should.

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 we look after you. If you do want to take part then you, and your parents, will be asked to sign a *consent form* to say that you agree to take part and that we can keep information about you. You will also have a copy of this form to keep.

If you change your mind and decide you don't want to be part of this project anymore then we will remove the information about you from the *database*. Again, this will not change the way we look after you.

What will happen to me if I take part?

During the registration process, you will be asked to complete a few personal details and to answer a questionnaire together with your parents. You will not be asked to have any extra tests done for this project. You will not need any time off school other than to come to your usual clinic appointments. Your personal details, such as your name and address, will be stored on a secure computer that is protected by passwords and is looked after by the registry staff at Newcastle University. For further information about how Newcastle University manages personal data, including participant 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/>. People who are planning the *clinical trials* or *research* studies can ask the registry team to give them information about the numbers of patients with your condition and in which region they live to see if it is a good idea to do a clinical trial or research study in a certain place. But those people will never get direct access to your data, nor will they be given your name or address so that they will not be able to contact you directly. If you would like to take part in a specific *clinical trial* or *research* study, and are eligible to do so, you would then be contacted by the registry. In any case, you can always let us know if you have changed your mind and do not want to be on the registry anymore, and this would not affect your care in the future.

Will this project help me?

The project may not help you specifically, but the information we collect should help us learn more about FKRPs-related conditions and help develop new treatments in the future.

What happens when the project stops?

The LGMD2I Research Fund provides money for this project and we hope to be able to keep the *database* running and keep information about you for as long as possible.

What if something goes wrong with the project?

We do not think that anything will go wrong. The only people who can see the information about you are members of Professor Straub's team who help with the project. The *database* is secure and *password protected* and is kept on a secure server in the UK looked after by the registry staff at Newcastle University.

Will anyone else know I'm doing this?

The doctors and nurses at the hospital who look after you will know you are part of this project, but only people allowed by Professor Straub will be able to see the information about you. Your parents will also need to know, as they too need to sign the *consent form*.

Some people from *research companies* may ask for information about you if they think they have a treatment that may help you. If Professor Straub thinks it may be of help he will write to you to ask if you would like to take part. This would be your decision.

Who decided the project could be done?

Before any *research* can be done it has to be checked by an *Ethics Committee*. This is to make sure that the *research* is a good idea and is safe to do. The *database* will be kept on a secure computer in the UK and will be looked after by staff at Newcastle University.

Contact Details:

If you would like to talk to someone about taking part, you or your parents can contact Professor Volker Straub (Telephone: +44 (0) 191 241 8655 or email: volker.straub@newcastle.ac.uk or the registry staff (coordinator@fkrp-registry.org).

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>.

GLOSSARY –What do these words mean?

Anonymous information – information which does not contain any names or contact details

Clinical trial – an experiment involving patients with a particular disease

Consent – giving permission for something, or agreeing it can be done

Database – a collection of information

Ethics Committee – a group of people who look carefully at research projects to make sure they are properly carried out

Password protected – a way of stopping people from looking at your information

Research – careful investigation of a particular subject

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

Thank you for reading this information



ASSENT FORM for Children

Title of Study:

Global FKRP Registry

Principal Investigator/ Data Controller:

Prof. Volker Straub
John Walton Muscular Dystrophy Research Centre
Institute of Genetic Medicine
Newcastle University
International Centre for Life
Newcastle upon Tyne, NE1 3BZ
United Kingdom

Name of patient: _____

Date: _____

Please read or have read out to you by your parent/ guardian the sentences below and then tick (or ask your parent/ guardian to tick on your behalf) each box next to what you agree with:

- ☐ I confirm that I have read/had read to me, and understand the information sheet for the above study. I have had the opportunity to consider the information, ask questions and have had these answered in a way I understand.
- ☐ I understand that I may ask for my details to be removed from this database at any time, without giving any reason, without my medical care or legal rights being affected.
- ☐ By ticking this box, I understand that I give consent for the storage of data on myself in the Global FKRP Registry.
- ☐ I understand that the storing of data will allow contact to be made with me if a suitable clinical trial becomes available.
- ☐ However, I accept that allowing my data to be stored on this database does not mean I will automatically be entered into future clinical trials.
- ☐ I understand that the results from future research may not have any direct benefit to me.
- ☐ I am happy for doctors in charge of my medical care to add relevant information to my database entry on my behalf.
- ☐ I am happy to assent to be included in this registry.