



Global FKRP Registry

INFORMATION FOR PARENTS

Principal Investigator: Prof. Volker Straub, Newcastle University, UK

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

Before you agree to register your child in the Global FKRP Registry, it is important that you understand what is involved and what will be done with the information that you provide. This form contains the answers to the questions that you might have. Take time to decide whether you would like to take part or not. At the end of the form there are checkboxes for you to click on to confirm that you agree to your child participating. If you have any questions after reading this form, please contact the registry staff before continuing: coordinator@fkrrp-registry.org

What is a patient registry and why do we want to create one?

Scientific advances over recent years have led to substantial changes in the treatment of many disorders. New therapeutic strategies are being developed and, for some of these treatments, plans for large studies involving patients from more than one country are already in place.

Several new therapeutic strategies for neuromuscular disorders like LGMD2I target specific gene defects. When a scientific study or clinical trial is being planned, it is very important that patients suitable for that trial can be found and contacted quickly. The best way to ensure that this happens is to make sure that patients' details are all collected in a single database or "registry" that contains all the information that researchers will need, including each patient's particular genetic defect and other key information about that disorder. The TREAT-NMD network has created this kind of international registry, which means that all patients who register will be contacted if their profile fits the requirements for a particular study or clinical trial. In addition the registry will help researchers answer questions such as how common disorders like LGMD2I are distributed internationally, and will support other activities to improve patient care and establish a good standard of care worldwide.

Whose data are we collecting in this registry?

The Global FKRP Registry is for patients affected by LGMD2I (Limb Girdle Muscular Dystrophy 2I), MDC1C (Congenital Muscular Dystrophy 1C) and other conditions caused by a mutation in the FKRP gene. The Global FKRP Registry is primarily designed to register patients who might be suitable for participation in future research studies or clinical trials of new therapies, and to help the researchers find the best way of caring for patients with

LGMD2I/MDC1C and other FKRP-related conditions. This registry is intended for patients currently living with the condition and not as a record for those who have already died.

What does my child have to do and what will happen with their data?

If you agree to your child taking part in this project, then you should read this information and click on the checkboxes at the end. This confirms that you agree to your child participating. Then you should complete the online registration questionnaire, in which we ask you for some of your child's personal data and some information about their condition. Furthermore, your child's doctor will complete the professional part of the questionnaire. The information that you enter will be entered into an international registry which is supervised by the Global FKRP Registry steering committee. Your child's data will be stored securely and no unauthorised people will be able to gain any information about your child. When planning a scientific study or clinical trial, researchers can make enquires to the registry to obtain anonymous information about participants who are potentially eligible for that study or trial, based on the patients' clinical and genetic data that is stored in the registry. Hereby, only researchers whose registry enquiry has been approved by the Global FKRP Registry oversight committee and – depending on the nature of the enquiry – by their local ethics committee, will have access to specific registry information, which is usually given in form of a written report.

When researchers ask for the information from the registry they will not be able to find out your personal information (name, address, etc.), but only the information they need to know about your child's condition that will help them decide whether they might be suitable for the trial. If they think that your child meets the criteria and might benefit from the trial, they will contact the person in charge of the registry. Staff working for the registry will "de-code" the data to find out the personal details and will contact you to give you information about the trial or about any other issues relevant to your child's condition. They will not give your name or personal information to the researchers. If you are interested in the information that you receive about a particular clinical trial, you will be given information about how you can contact the researchers running the trial. If you decide that you would like your child to take part in the trial, you will need to review and sign a separate consent form. You are completely free to make your own decision about any trial we inform you about. If you decide not to let your child take part in a particular trial, their data will still be kept in the registry and we will continue to inform you about other trials unless you tell us not to. Please note that if we tell you about the existence of a trial, this does not imply that we endorse it.

How can I update my child's data if something has changed?

To make sure that the data in the registry is correct and up-to-date, it is essential that we update it regularly. To do this, we will send you emails once a year asking you to tell us about any changes in your child's medical condition. We also ask you to inform us if there are any major changes in your child's details that might occur in the period between updates, for example a change of address or the loss of ambulation.

Who will have access to my child's data?

Staff in charge of the registry have access to your child's data to obtain information necessary to a project and are able to contact you, for example to inform you about an upcoming clinical trial. Also the doctor that you choose during registration will have access to your child's data, this is necessary because he/she will fill out the second part of the registration questionnaire.

How will my child be identified in the registry?

Your child's personal details (name, address etc.) have to be stored in the registry so that we can contact you if we need to inform you about possible clinical trials or research studies or

anything else that might be relevant to your child's disorder. This data will be stored in a secure manner and your records will be assigned a unique code. Your records will only be identified by this unique code. Researchers searching in the registry therefore cannot identify your child personally from the information they have access to. Only the person in charge of the registry - Prof Volker Straub and persons explicitly appointed by him will be able to "de-code" the data to get access to your personal details.

Will information about my child be kept confidential?

Creating a registry requires the existence of a file containing a patient's personal and medical data. This file will be subject to the regulations on data protection (national laws related to EU directive 95/46). All information we receive from you will be treated confidentially. The information will be encrypted and stored on a secure server.

Your data will be kept for an indefinite period on a server located in the UK, under the responsibility of Prof. Volker Straub at Newcastle University.

If we publish any research or other documents based on the data from the registries, this research will never identify your child by name.

Third parties wishing to have access to the data in the registry (such as researchers or companies planning clinical trials or conducting research on new therapies) will only have access to anonymous information identifiable by a code. Before they are granted access even to this anonymous information, they have to have permission from the Global FKRP Registry steering committee. Your child's data will not be made available to employers, government organisations, insurance companies or educational institutions, nor to other members of your family.

How will my child benefit from registering?

The registry is intended as a public service for the benefit of patients living with LGMD2I, MDC1C and other FKRP-related conditions. You will not receive any payment or any other financial benefit as a result of submitting your child's data to the registry. The results of research facilitated by the registry may be patentable or may have commercial potential. However, you will not receive patent rights and will not receive financial benefits from future commercial development. Nevertheless, there are other benefits from participating, including the following: We will inform you if (on the basis of the information that you and your doctor provide) your child might be a suitable candidate for a certain clinical trial. We will also inform you if we receive any new information on your child's disorder which might be of interest to you - for example if we find better ways for caring for patients with LGMD2I, MDC1C and other FKRP-related conditions. The data collected might also provide benefits for other patients with your child's disorder, for example by revealing statistics on how many people worldwide have the same condition, or providing information for researchers interested in the best standards of care for your child's disorder.

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

Although one of the main aims of this registry is to make it easier for patients to be recruited for trials or studies, there is no guarantee that registering your child's details will ensure that they will be involved in a clinical trial or research study. If you are interested in receiving details of trials or studies that your child may be eligible for, please select this option in the questionnaire. However, it is important that you understand that even if the coordinators of a clinical trial or research study believe that your child might be eligible for that trial or study, based on the data about your child stored in the registry, it is possible that at a later date it will turn out that your child does not meet the inclusion criteria for the trial/study after all.

I do not want my child to take part in a clinical trial or research study. Should we still register?

We hope that you will be interested in registering even if your child will not take part in a clinical trial or research study. Their information will still be useful to researchers who are trying to find out more information about patients living with FKRP-related conditions, and we will still provide you with other information that might be relevant to your child's disorder. If you do not want to receive any information about clinical trials or research studies your child might be eligible for, please select this option in the questionnaire.

Does my child have to participate in the registry? Can we change our mind?

Your child's participation in this project is completely voluntary. You can decide for your child not to participate in this registry without having to give any reason. If you wish for your child to join this registry, then the Data Protection Act grants you the right to access your child's data and to view, rectify or update it at any time. Should you wish to withdraw your child's data from the registry, you will be free to do so at any time without having to provide any explanation and without consequence on their treatment or the quality of care that will be provided to them. If you wish to withdraw your child's data, you will need to get in touch with the staff in charge of the registry. Contact details are provided below.

Who should I contact if I have any other questions?

If you would like any additional information about the registry, if you need to tell us about any changes in your child's data, or if you wish to withdraw your child's data from the registry, please contact:

The registry staff using this email address: coordinator@fkrp-registry.org

Or the registry principal investigator

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