REC reference number: 13/NE/0266
Committee: NRES Committee North East – Newcastle & North Tyneside 1

Global FKRP Registry

INFORMATION FOR PATIENTS

Principal Investigator: Prof. Dr. med. Maggie Walter M.A, Friedrich-Baur Institut, Munich University, Germany

Should you have questions relating to the registry, you can contact either your local doctor or the registry principal investigator Prof. Maggie Walter (you can find her contact information below).

Before you agree to register 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. At the end of the form there is a checkbox for you to click on to confirm that you agree to participate. If you have any questions after reading this form, please contact us before continuing.

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 have 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 Fukutin Related Protein (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.

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What do I have to do and what will happen with my data?

If you agree to take part in this project, you should read this patient information and click on the checkbox at the end. This confirms that you agree to participate. Then you should complete the online registration questionnaire, in which we ask you for some personal data and some information about your condition. Furthermore, your 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 registry steering committee. Your data will be stored securely and no unauthorised people will be able to gain any information about you. 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 TREAT-NMD registries 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 the form of a written report.

When researchers search 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 condition that will help them decide whether you might be suitable for the trial. If they think that you meet 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 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 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 take part in a particular trial, your 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 the 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 medical condition. We also ask you to inform us if there are any major changes in your 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 data?

Staff in charge of the registry have access to your 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 data, this is necessary because he/she will fill out the second part of the registration questionnaire.

How will I be identified in the registry?

Your 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 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 you personally from the information they have access to. Only the person in charge of the registry

(Prof. Dr Maggie Walter) and persons explicitly appointed by her will be able to "de-code" the data to get access to your personal details.

Will my data 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 Munich, under the responsibility of Prof. Dr. Maggie Walter.

If we publish any research or other documents based on the data from the registries, this research will never identify you 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 TREAT-NMD registries oversight committee. **Your data will not be made available to employers, government organisations, insurance companies or educational institutions, nor to other members of your family.**

How will I 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 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) you might be a suitable candidate for a certain clinical trial. We will also inform you if we receive any new information on your 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 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 disorder.

I want to take part in a clinical trial or research study. If I register, is this guaranteed?

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 details will ensure that you will be involved in a clinical trial or research study. If you are interested in receiving details of trials or studies that you 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 you might be eligible for that trial or study, based on the data about you stored in the registry, it is possible that at a later date it will turn out that you do not meet the inclusion criteria for the trial/study after all.

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

We hope that you will be interested in registering even if you do not want to take part in a clinical trial or research study. Your 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 disorder. If you do not want to receive any information about clinical trials or research studies that you might be eligible for, please select this option in the questionnaire.

Do I have to participate in this registry and can I withdraw from it if I change my mind?

Your participation in this project is completely voluntary. You can decide not to participate in this registry without having to give any reason. If you decide to join this registry, then the Data Protection Act grants you the right to access your own data and to view, rectify or update them at any time. Should you wish to withdraw data from the registry, you will be free to do so at any time without having to provide any explanation and without consequence on your treatment or the quality of care that will be provided to you. If you wish to withdraw, you 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 data, or if you wish to withdraw your data from the registry, please contact:

The registry staff using this email address: uk@fkrp-registry.org
Or the registry principal investigator Dr Maggie Walter at:
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