

Frequently Asked Questions



Information for participants considering registering in a Patient Registry

Before you agree to register in a national patient registry affiliated with the TREAT-NMD global registry, it is important that you understand what is involved and what will be done with the information you provide. This page contains answers to some of the questions you might have. The registry coordinators in your country will also be able to answer your questions about your own national registry.

“What is a patient registry and why did TREAT-NMD set them up”?

Simply put, a patient registry is a database or collection of information about people affected by a particular condition. In the case of the TREAT-NMD registries, it is a collection of genetic and clinical information about people with neuromuscular diseases.

“Will I get paid for participating in the patient registry”?

No a participant will not get paid for participating in the registry.

“Why is a patient registry needed?”

Scientific advances over recent years have lead to substantial changes in the treatment of many neuromuscular diseases. New therapeutic strategies are being developed and, for some of these treatments, trials are already underway. Several new therapeutic strategies for neuromuscular diseases target specific genetic defects, some of which are so rare that only a few patients in the world will have the right profile for a clinical trial. When a clinical trial is being planned, it is very important that patients suitable for that trial can be found and contacted quickly.

The best way of ensuring this can happen is to make sure that patients' details are all collected together 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 their disease. The TREAT-NMD network is creating this kind of registry in countries across Europe, and thanks

to the support of colleagues and patients across the world, countries worldwide are joining this initiative.

As well as the individual national registries, we are also creating a single global registry which combines the information from each of the national registries, and this will ensure that patients who register in their national registry can be contacted if their profile fits a clinical trial. In addition, these registries will help researchers to answer questions such as how common these diseases are across the world and will support other activities to improve patient care, such as the assessment of standards of care.

“What are the objectives of the registries?”

The primary objectives of the registry are to:

- Accelerate and facilitate clinical trials by locating potential research participants quickly and efficiently
- Facilitate the planning of clinical trials
- Assist the neuromuscular community with the development of recommendations and standards of care
- Characterize and describe the specific neuromuscular disease (NMD) population as a whole, enhancing the understanding of NMD prevalence across Europe.

“Which disease are there registries for?”

TREAT-NMD global registries have been set up for the diseases spinal muscular atrophy (SMA) and Duchenne muscular dystrophy (DMD). A global registry for patients with myotonic dystrophy type 1 (DM1) will launch in 2011. Across the world a number of other registries for neuromuscular diseases have been set up, such as the CMD international registry for all individuals with any of the forms of congenital muscular dystrophy, which is run by the patient organization Cure CMD.

“Which patients are eligible?”

All patients with a confirmed diagnosis (or pending diagnosis) in one of the conditions for which registries exist are eligible for inclusion. For upload into the global registry, diagnosis must be confirmed via genetic testing results.

“What do I have to do to register and where will my data go?”

If you agree to register in your own national registry, you should read the participant/parent information sheet and sign the consent form associated with the New Zealand national registry.

The registry questionnaire asks you for some personal data and some information about your disease. The information you provide will be entered into the New Zealand national registry. Your data will be stored securely and no unauthorized people will be able to gain access to any information about you. Your data will be assigned a unique code, and the data about all patients in each country’s national registry will then be fed into the TREAT-NMD global registry, without including your personal information. When planning clinical

trials, researchers can ask the global registry to look at the data to find participants eligible for their trial, based on the patients' clinical and genetic data. Only researchers who have been approved by their own local ethics committee and by the TREAT-NMD governing board and ethics council are allowed to do this.

In the global registry, your data will only be identified by an anonymous code, not by your name. This means that people accessing the registry at the global level will not be able to find out your personal information (name, address etc.), but only the information they need about your disease that will help them decide whether you might be suitable for the trial. If they think you meet the criteria and might benefit from the trial, they will contact the person in charge of your national registry. Staff working for the national registry will "decode" the data to find out your personal details and will contact you to give you information about the trial or about any other issues relevant to your disease. They will not give your name or any personal information to the researchers. If you are interested in the information 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 my data if it changes?”

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, New Zealand Neuromuscular Disease Registry curator will contact you at least once a year asking you to tell it about any changes in your medical condition. We will also ask you to inform us about any major changes in your details that might occur in the period between updates, for example change of address or loss of ability to walk.

“Who will have access to my medical records?”

The Curator of the New Zealand Neuromuscular Disease Registry will have access to the information you provide. They might also need to gain access to your medical records to obtain information necessary to the project (for example, they might need to ask your geneticist to give them a copy of your genetic report). If this happens, you will be asked by the Curator to consent to this.

“How will I be identified in the registry?”

Your personal details (name, address etc.) have to be stored in your national registry so that they can contact you if they need to inform you about possible clinical trials or anything else that might be relevant to your disease. This data will be stored in a secure manner and your records will be assigned a unique

code. When the national registry transfers your data to the global registry, it will not transfer any of your personal details, and your records will only be identifiable by the code they have been assigned. Researchers accessing the global registry therefore cannot identify you personally from the information they have access to. Only the person in charge of your own national registry (the curator) will be able to "de-code" the data to get access to your personal details.

“Where will my data be kept and it will be kept confidential?”

Your data will be kept for an indefinite period under the responsibility of the curator of the New Zealand Neuromuscular Disease Registry. 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 kept in the national registry will be treated confidentially. If any research or other documents based on data from the registries is published, this research will never identify you by name. Third parties wishing to have access to data in the global registry (such as researchers or companies planning clinical trials or conducting research on new therapies) will only have access to anonymous information identifiable only by a code. Before they are granted access even to this anonymous information, they have to have the approval of an Ethics Committee. Your data will not be made available to employers, governmental organizations, insurance companies or educational institutions, or to your spouse, other members of your family or your doctor, unless you have given the Curator consent to do so..

“Will you ever give my name and address to a company running a clinical trial?”

No, we will never pass on any personally identifiable data to anyone outside the registry. If a company asks us to find patients who might be suitable for their trial, you will be sent a letter giving you details about the trial and the name of the person to contact if you are interested (usually the doctor who is running the trial at the centre nearest you). That is the only legal way we can help recruit patients for trials, and we think it is also the best way, because you have time to consider whether it is something you are interested in without being put under any pressure.

“How will I benefit from registering?”

The TREAT-NMD registries are intended as a public service for the benefit of patients living with neuromuscular diseases. 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 may be other benefits to participating, including the following:

- We will inform you if (on the basis of the information you 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 disease which might be of interest to you - for example if we find better ways of caring for patients with your condition.
- The data collected might also provide benefits to other patients with your disease, for example by revealing statistics on how many people across the world have the same condition, or providing information for researchers interested in the best standards of care for your disease.
- We will publish some general statistical information from the global registries on our website, so you will be able to find out information about how many other patients in the global registry have the same disease as you.

“I want to be involved in a clinical trial. If I register, is this guaranteed?”

Although one of the main aims of the TREAT-NMD registries is to make it easier for patients to be recruited for clinical trials, there is no guarantee that registering your details will ensure you will be involved in a clinical trial. TREAT-NMD has no control over where companies decide to run their trials or which patients are enrolled - we can provide companies with information and advice, but it remains their own decision. However, it is important that you understand that even if a trial is happening in your country and the people in charge of the trial believe that you might be eligible for that trial, based on the data about you stored in the global registry, it is still possible that once they have assessed you fully, it will turn out that you do not meet the trial inclusion criteria after all.

“I don’t want to be involved in a clinical trial. Should I still register?”

We hope you will be interested in registering even if you don’t want to take part in a trial. Your information will still be useful to researchers who are trying to find out more about people living with your disease, and we will still provide you with other information that might be relevant to your disease.

“Do I have to participate in the registry and can I withdraw if I change my mind?”

Your participation in this project is completely voluntary. National data protection laws grant you the right to access your own data and to rectify it or withdraw it completely at any time. Should you wish to withdraw your data from the registry you will be free to do so without having to provide any explanation? If you wish to withdraw, you should get in touch with the staff in charge of your own national registry.

“Who should I contact if I have any questions?”

If you would like any additional information about the New Zealand Neuromuscular Disease Registry, please contact the registry curator on 0800 800 337 or 09 815 0247 or via email on registry@mda.org.nz For information

about the global registries, contact Professor Hanns Lochmüller by email at hanns.lochmuller@ncl.ac.uk.