



Patient Information and Informed Consent

Jennifer Trust/TREAT-NMD Patient Registry for Spinal Muscular Atrophy

Information for patients

Before you agree to register in the SMA Patient Registry, it is important that you understand what is involved and what will be done with the information you provide. This form contains answers to some of the questions you might have. At the end of the form there is a section for you to sign to confirm that you agree to participate. If you have any questions after reading this form, please contact us before signing the form. You will find our contact details on page 4.

“What is a patient registry and why do you want to create one?”

Scientific advances over recent years have led to substantial changes in the treatment of many diseases. 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 diseases like SMA target specific genetic defects. 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. In the UK, we are working with the Jennifer Trust to create a registry of all patients with SMA. As well as each national registry, we are also creating a single European registry which will combine 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 diseases like SMA are across Europe and will support other activities to improve patient care, such as the assessment of standards of care.

“Whose data are you collecting in this registry?”

This registry is for patients suffering from the disease spinal muscular atrophy (SMA). We are also working on a different registry for Duchenne muscular dystrophy (DMD), and in future we will be creating registries for other neuromuscular diseases.

Because it is primarily designed to register patients who might be suitable for treatment in future clinical trials of new therapies, and to help researchers find the best ways of caring for patients with SMA, this registry is intended for patients currently living with SMA, and not as a record of those who have already died. If you would like to make a record of a relative who has already died, you might like to consider taking part in Richard Finkel's “SMA parent survey” – see http://www.jtsma.org.uk/sma_parent_survey_aug07.html or the Jennifer Trust's “inspirations” stories – see <http://www.jtsma.org.uk/inspirations.html>.

“Who should fill in this form?”

If you are the patient, you can fill in and sign the form yourself if you are over 16. If you are younger than 16 but can understand this information, you can sign the form yourself, but we would also like your parent or guardian to sign it too. Whatever your age, please talk it over with your family and don't hesitate to contact us if you have any questions. If you are the parent or guardian of a child who is not old enough to understand this form, please sign the form yourself if you want your child's data to be included in the registry.

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

If you agree to take part in this project, you should read this patient information and sign the consent form at the end. Then you should complete the registration questionnaire, with the help of your doctor if necessary, and return it to us. In the questionnaire we ask you for some personal data and some information about your disease. The information you provide will be entered into a national registry in the UK which is supervised by TREAT-NMD and the Jennifer Trust. Your data will be stored securely and no unauthorized people will be able to gain access to any information about you. The data about all patients in each country's national registry will then be fed into the TREAT-NMD European registry, which is accessible to researchers worldwide. When planning clinical trials, researchers can search the European registry for 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 access the registry.

In the European registry, your data will only be identified by an anonymous code, not by your name. This means that 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 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 the UK registry. Staff working for the UK registry will “de-code” 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, we will send you follow-up forms once a year asking you to tell us about any changes in your medical condition. We 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 ambulation.

“Who will have access to my medical records?”

Staff in charge of the UK registry might need to gain access to your medical records to obtain information necessary to the project (for example, we might need to ask your geneticist to give us a copy of your genetic report).

“How will I be identified in the registry?”

Your personal details (name, address etc.) have to be stored in the UK registry so that we can contact you if we 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 we transfer your data to the European registry, we will not transfer any of your personal details, and your records will only be identifiable by the code they have been assigned. Researchers searching in the European registry therefore cannot identify you personally from the information they have access to. Only the person in charge of the UK registry (Professor Hanns Lochmüller of TREAT-NMD and Newcastle University) or a person explicitly appointed by him will be able to “de-code” the data to get access to your personal details.

“Will my data be kept confidential?”

Your data will be kept for an indefinite period at Newcastle University in the UK, under the responsibility of Professor Hanns Lochmüller.

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 located at Newcastle University in the UK.

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

Third parties wishing to have access to data in the European 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, nor to your spouse, other members of your family or your doctor.

“How will I benefit from registering?”

This registry is intended as a public service for the benefit of patients living with SMA. 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 SMA.
- The data collected might also provide benefits to other patients with your

disease, for example by revealing statistics on how many people in Europe 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 registry and from our other European registries on our website, so you will be able to find out information about how many other patients in Europe 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 this registry 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. If you are interested in receiving details of trials you might be eligible for, please tick the appropriate box at the end of this form. However, it is important that you understand that even if the coordinators of a clinical trial believe that you might be eligible for that trial, based on the data about you stored in the European registry, it is still possible that later on 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 patients living with SMA, and we will still provide you with other information that might be relevant to your disease. If you do not want to receive any information about clinical trials that you might be eligible for, please tick “no” in question 3 of the informed consent section at the end of this form.

“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. The Data Protection Act grants 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 the UK registry. Contact details are provided below.

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

If you would like any additional information or need to tell us about any change in your data, or if you wish to withdraw your data from the registry, please contact Professor Hanns Lochmüller on tel. 0191 241 8602 or email hanns.lochmuller@ncl.ac.uk.

Informed Consent

1. Do we have your permission to store your data in the national registry in the UK and to transfer it (in a form identifiable only by a code) to the European registry where it may be used for research and for the planning of clinical trials?

- NO
- YES

2. If we receive information on TREAT-NMD projects or other information related to your disease which might be relevant to you, would you like to be informed about this?

- NO
- YES

3. If we receive information about a clinical trial which you might be eligible for, would you like to be informed about this?

- NO
- YES

(Please note that even if the coordinators of a clinical trial believe that you might be eligible for the trial, based on the data about you stored in the European registry, it is still possible that later on it will turn out that you do not meet the trial inclusion criteria after all. Please also be aware that if we inform you about the existence of a trial, this does not imply that we endorse it. In order to participate in any trial, you will need to fill out a separate informed consent form.)

4. So that we can keep the registry up to date, we will need to contact you once a year and ask about any changes in your medical condition. Do you agree to receive follow-up forms once a year which you will be asked to fill in to register any changes in your medical condition?

- NO
- YES

5. If there are any major changes in your data (for example change of address, or changes in your medical condition, such as loss of ambulation) that occur in the period between updates, are you willing to inform us?

- NO
- YES

The nature of the registry has been fully explained to me. I have understood the patient information and informed consent form and have received a copy to take away with me. I have had the opportunity to ask questions, and all my questions have been answered to my satisfaction. Upon reflection, I agree to participate in this registry.

Signature of participant

Date

Signature of parent/guardian
(for a child under 16)

Date

First name:

Family name:

Address:

Telephone:

Email:
