MTM Registry Consent – Parent or Guardian

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Myotubular and Centronuclear Myopathy (MTM and CNM) Patient Registry

Principal Investigator/Data Controller: Dr Chiara Marini Bettolo, Institute of Genetic Medicine, Newcastle University

Information for Parents or Guardians of MTM or CNM patients

Your child is being invited to be part of a registry (research database). Before you agree to register your child in the Myotubular and Centronuclear Myopathy (MTM and CNM) Patient Registry, it is important that you understand what is involved and what will be done with the information you provide.

The information below contains answers to some of the questions you might have, and underneath this information there are consent statements that you will need to complete, to confirm that you would like to participate. Underneath this, information is also available for your child.

If you or your child have any questions after reading this information, please contact the Registry Curator, Jo Bullivant, at mtmcnmregistry@treat-nmd.eu.
Take time to decide whether you would like your child to take part or not.

You can also use the link above to download or print a copy of this information to keep.

1. What is a patient registry and why is one needed for MTM/CNM?

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. 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. In addition, these registries will help researchers to answer questions such as how common diseases like MTM/CNM are globally and will support other activities to improve patient care, such as the assessment of standards of care.

2. Whose data are you collecting in this registry?

This registry is for patients suffering from the disease myotubular myopathy or centronuclear myopathy (MTM/CNM). Because one of the research aims is to understand the progress of the disease, the registry will be used to collect information on patients currently living with MTM/CNM, and, as a record, on those who have died.

The registry is intended to collect data from patients who have been advised that they have MTM/CNM even if they do not yet have a confirmed genetic diagnosis. However, only when a patient has a confirmed genetic diagnosis will they be eligible for consideration for certain clinical

trials. Even if you do not have a confirmed genetic diagnosis at this point you will still receive updates on the best ways of caring for patients.

3. Who will be holding the information I provide about my child?

The registry is managed by the John Walton Muscular Dystrophy Research Centre at Newcastle University in the UK, and they are responsible for managing and securing your child's information. They are part of the TREAT-NMD Neuromuscular Network, and the registry is funded by The Myotubular Trust. Only the staff in charge of the registry will have access to the data you provide. If you have any queries on how your child's information is being used or would like to see a copy of the information which is held about your child on the registry, please contact the Registry Curator, Jo Bullivant, at mtmcnmregistry@treat-nmd.eu.

4. How can I update my child's data if it changes?

To make sure that the data in the registry is correct and up to date, we will send you a reminder about once a year asking you to tell us about any changes in your child's medical condition. This can be done on-line by logging in with your username and password. We also ask that you inform us about any major changes in your child's details that might occur between the yearly updates, for example a change of address or a change in the ability to walk.

5. How will my child be identified in the registry?

Your and your child's personal details (name, address, email address, telephone number) have to be stored in the 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 their disease. This data will be stored in a secure manner and your records will be assigned a unique, anonymous code. It is this unique code that will be used to identify your record if data is being analysed. Only the staff in charge of the MTM / CNM registry will be able to "de-code" the data to get access to personal details.

6. Will my child's data be kept confidential?

Yes. The registry is managed by the John Walton Muscular Dystrophy Research Centre at Newcastle University in the UK. All data held is subject to protection under the Data Protection Act 1998 (derived from 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. If we publish any research or other documents based on data from the registry, this research will never identify you or your child by name.

We will share general (anonymous) statistical information and patient numbers from the registry with the myotubular and centronuclear myopathy medical community to add to their knowledge and improve information available to them as a pre-cursor to planning clinical trials.

7. How will my child benefit from registering?

This registry is intended as a public service for the benefit of patients living with MTM/CNM. 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 ultimately 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) your child might be a suitable candidate for a certain clinical trial
- We will also inform you if we receive any new information on about MTM/CNM which might be of interest to you or your child – for example if we find better ways of caring for patients with MTM/CNM.
- The data we collect from you might also provide benefits to other patients with MTM/CNM, for example by revealing statistics on how many people have the same condition, or by providing general statistical information for researchers interested in the best standards of care and to help them make plans for clinical trials and other research initiatives.
- We will publish some general statistical information from the registry, so you will be able to find out information about how MTM/CNM affects other people.

8. I want my child to be involved in a clinical trial. If I register, is this guaranteed?

No. Only patients with a confirmed genetic diagnosis will be eligible for certain clinical trials, if and when they become available in the future. However, even if your child's genetic diagnosis is not yet confirmed you will still receive updates on the best ways of caring for patients and other issues of relevance.

Even with a confirmed genetic diagnosis there is no guarantee that registering your child's details will ensure they will be involved in a clinical trial. It is also important that you understand that even if the coordinators of a clinical trial believe that your child might be eligible for that trial, based on the data about them stored in the registry, it is still possible that later on it will turn out that they do not meet the trial inclusion criteria after all.

9. I don't want my child 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 your child to take part in a trial. The information you provide will still be useful to researchers who are trying to find out more about patients living with MTM/CNM, and we will still provide you with other information that might be relevant to your child's disease.

10. 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 1998 (derived from EU Directive 95/46) grants you the right to access your child's data and to rectify it at any time. Should you wish to withdraw your child's data from the registry you will be free to do so without having to provide any explanation. If you wish to withdraw, you should contact the Registry Curator, Jo Bullivant, at mtmcnmregistry@treat-nmd.eu.

11. Who should I contact if I have any questions?

If you would like any additional information or if you wish to withdraw your child's data from the registry, please contact the Registry Curator, Jo Bullivant, at mtmcnmregistry@treat-nmd.eu.

12. How long will my child's information be kept on the registry?

There is no set time period for removing your data. Unless you ask us to remove your information from the registry, we shall keep it for as long as we consider necessary for the purposes described in this form. However you can contact the Registry Curator to remove your data at any time.

13. Who is funding the registry?

The Myotubular Trust is providing funding to Newcastle University to manage this registry. No additional payments will be received by Dr Chiara Marini Bettolo, or other members of the research team, for adding your child's details on to the database.

14. Who has reviewed the project?

This research has been reviewed and given a favourable opinion by North East- Newcastle and North Tyneside 1 Research Ethics Committee.

15. What if I have any concerns or further questions?

If you have any concerns, or other questions about this study or the way it has been carried out, you should contact the Registry Curator Jo Bullivant, at mtmcnmregistry@treat-nmd.eu.

For patients living in the UK: If you feel that your child has been treated unfairly throughout the research, or would like to comment on the conduct of any aspect of this research, you can also contact the Patient Advice and Liaison Service (PALS) 0800 0320202.