# MTM Registry Consent – For Deceased Patients

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## Myotubular and Centronuclear Myopathy (MTM and CNM) Patient Registry

Principal Investigator/Data Controller: Professor Hanns Lochmüller, Institute of Genetic Medicine, Newcastle University

You are being invited to enter information about a deceased family member into the Myotubular and Centronuclear Myopathy (MTM and CNM) Patient Registry. A registry is a type of research database.

We appreciate that completing this may be quite emotional. So that you are prepared, we would like you to be aware that the questions you will be asked will cover the patient's genetic diagnosis (if they had a confirmed one) and information on their condition (including mobility, breathing, and feeding). We would also like details of their physician, and we will ask you to upload copies of their genetic and muscle biopsy reports if these were carried out. If you don't have some of these details to hand now, you can save your information and log back in at a later time to complete it.

Before you agree to participate, it is important that you understand what is involved and what will be done with the data you provide. The information below contains answers to some of the questions you might have, and underneath this there are consent statements that you will need to complete, to confirm that you would like to participate.

If you have any questions after reading this page, please contact the Registry Curator, Jo Bullivant, at mtmcnmregistry@treat-nmd.eu. Take time to decide whether you would like 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 and CNM?

Scientific advances over recent years have led to substantial changes in the treatment of many diseases. New therapeutic strategies are being developed for MTM and CNM, 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 (or any research) is being planned, it is very important that the researchers have access to accurate information about the prevalence of the disease and how people are affected by it.

A good way of helping with this is to collect patient details together in a single database or "registry" containing 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 who suffer, or suffered from 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 will collect data on deceased patients who were advised that they had MTM or CNM, even if they did not have a confirmed genetic diagnosis.

**3. Who will be holding the information I provide about the patient?**

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 the patient 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 information is being used or would like to see a copy of the information which is held about you or the patient on the registry, please contact the Registry Curator, Jo Bullivant, at mtmcnmregistry@treat-nmd.eu.

**4. How will the patient be identified in the registry?**

All of the data you provide will be stored securely and given a unique, anonymous code. Any personal or identifiable details about you or the patient (name, address, email address, telephone number) will be stored separately. 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 and CNM Registry will be able to "de-code" the data to get access to personal details.

**5. Will the 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 the patient 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 for research.

**6. How will I 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 the patient'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.

The data we collect from you might provide benefits to other patients with MTM/CNM, for example by revealing statistics on how many people have had 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. If you would not like to receive communications like this from the registry, you can indicate this in the consent statements below.

**7. 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 data and to rectify it at any time. Should you wish to withdraw the patient's data from the registry you will be free to do so without having to provide any explanation. If you wish to withdraw, you can contact the Registry Curator, Jo Bullivant, at mtmcnmregistry@treat-nmd.eu.

**8. Who should I contact if I have any questions?**

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

**9. How long will the patient's information be kept on the registry?**

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

**10. Who is funding the research?**

The Myotubular Trust is providing funding to Newcastle University to manage this registry. No additional payments will be received by Professor Lochmüller, or other members of the research team, for adding your information on to the database.

**11. 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.

**12. 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.

If you feel that you have 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.