

MTM Registry Consent – Adult Participants

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Myotubular and Centronuclear Myopathy (MTM and CNM) Patient Registry

Principal Investigator/Data Controller: Dr Chiara Marini Bettolo, Translational and Clinical Research Institute, Newcastle University

You are being invited to be part of a registry (research database). Before you agree to register 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.

If you have any questions after reading this information, please contact the Registry Curator, at mtmcmregistry@newcastle.ac.uk.

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/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 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 or the impact of treatments.

2. Whose data are you collecting in this registry?

This registry is for patients living with myotubular myopathy or other centronuclear myopathies (MTM/CNM), and for female carriers of x-linked myotubular myopathy. 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 individuals who have been advised that they have MTM/CNM, even if they do not yet have a confirmed genetic diagnosis. However, only when an individual 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?

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 information. Only the staff in charge of the registry will have access to the data you provide. In addition, the doctor that you name during registration* will be able to view (but not edit) your data. This is necessary because they will be asked to fill out an extra part of your registration questionnaire. You will also be able to view (but not edit) any data about you that the doctor provides to your registration.

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 on the registry, please contact the Registry Curator, at mtmcmregistry@newcastle.ac.uk.

*You will not be asked to name a doctor if you are a female carrier with no symptoms.

4. How can I update my details if anything changes?

To make sure that the data in the registry is correct and up to date, we will send you a reminder once a year asking you to tell us about any changes in your medical condition. This can be done online by logging in with your username and password. 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 a change in the ability to walk.

5. How will I be identified in the registry?

Your personal details (name, address, email address, telephone number) must be stored in the registry so that we can contact you if we need to inform you about possible clinical trials or anything else relevant to your 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 data be kept confidential?

Yes. The registry is managed by the John Walton Muscular Dystrophy Research Centre at Newcastle University in the UK and the data is encrypted and stored on a secure server in the UK. All data held is subject to protection under the General Data Protection Regulation (GDPR) (EU) 2016/679) and the General Data Protection Act 2018. All information we receive from you will be treated confidentially. If we publish any research or other documents based on data from the registry, this will never identify you 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 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 for submitting your 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 are other benefits to participating, including the following:

- We will inform you if (based on the information you provide) you might be a suitable candidate for a certain clinical trial.
- We will inform you if we receive any new information on about MTM/CNM which might be of interest to you – for example if we find better ways of caring for patients with MTM/CNM.
- 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 the same condition, or by providing general 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 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 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 clinical trials. However, even if your 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 details will ensure you will be involved in a clinical trial. It is important to understand that even if the coordinators of a clinical trial believe you might be eligible for that trial, based on the data about you stored in the registry, it is still possible that later it will turn out that you do not meet the trial inclusion criteria after all.

9. 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. The information you provide will still be very 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 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 General Data Protection Regulation (GDPR) (EU) 2016/679EU and the Data Protection Act 2018 grants you the right to access your own data and to rectify it 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 contact the Registry Curator at mtmcmregistry@newcastle.ac.uk.

11. 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 at mtmcmregistry@newcastle.ac.uk.

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

There is no set time limit 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?

Newcastle University (UK) receives funding to manage this registry from The Myotubular Trust, Muscular Dystrophy UK (both patient organisations), and Astellas Gene Therapies (a company with a gene therapy in development for X-Linked MTM). Funders do not have access to data and no additional payments will be received by Dr Chiara Marini Bettolo, or other members of the research team, for adding your details on to the database. Even if providing funding, pharmaceutical companies will not be permitted to join the registry Steering Committee. Instead, they will be able to provide input or make suggestions to the Steering Committee through a separate Industry Advisory Panel.

14. Who has reviewed the project?

This research has been reviewed and given a favourable opinion by North East - Tyne & Wear South 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, at mtmcmregistry@newcastle.ac.uk.

For patients living in the UK: If you feel that you have been treated unfairly 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). You can [find your nearest PALS office](#) on the NHS website.

If you are concerned about the management of your personal data, a complaint may be lodged with the Information Commissioner's Office, UK: <https://ico.org.uk>.