UK Myotonic Dystrophy Patient Registry

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

Information for adult patients (age 16 and over)

You are being invited to take part in an ongoing research project known as a 'Patient Registry'. This would involve you answering some questions about how Myotonic Dystrophy (sometimes shortened to 'DM'), affects you and giving consent for the registry team to keep a record of your answers in a secure, password-protected computer database. You can join if you have Myotonic Dystrophy, even if you don't have any symptoms. You can also nominate your medical team to add some extra information from your medical notes on your behalf.

Before you accept or decline the invitation, it is important for you to understand why the research is being done and what it will involve. Please read the following information and discuss it with relatives, friends and your GP, if you wish. If there is anything that is not clear, or if you have any further questions, please ask us (our contact details are at the end of this document, or on the website here).

Take time to decide whether you would like to take part, or not.

Why is this research being done?

It is important to collect information from people with rare conditions like Myotonic Dystrophy to help researchers and scientists trying to better understand the condition. More research is likely to improve the care patients receive in future. Specific examples of how the registry data has been used to further research and support the Myotonic Dystrophy community in the UK can be viewed on the registry website.

Having a computer database of patients with Myotonic Dystrophy also means that patients that may be eligible to take part in clinical trials to help develop new treatments can be easily found, and this allows those who are interested to volunteer to take part if they wish. This helps to improve the equality of access to trials for patients living all over the UK. The registry is different to any mailing lists or contact databases you may be on with a patient organisation (for example the Myotonic Dystrophy Support Group, or Cure-DM) and you won't be signed up automatically. You need to either sign up to the registry online yourself or ask the registry team for support to sign up.

The John Walton Muscular Dystrophy Research Centre (Newcastle University), is responsible for maintaining this registry. The project is funded by the patient groups MDSG (Myotonic Dystrophy Support Group), CureDM (Cure Myotonic Dystrophy UK Charity) and MDUK (Muscular Dystrophy UK) and clinical data entry support is provided by many health professionals across the UK.

Before you consider registering your details, it is important that you understand what is involved and what will be done with the information you provide. This information sheet contains answers to some of the questions you might have about the registry. When you have read this document, and after you have had some time to think about it, we ask if

you wish to register. If you do, we ask you to sign the online consent form saying that you agree to join. If you have any questions or accessibility issues, please contact the registry team before signing the consent form.

How will I benefit from registering?

This database aims to benefit patients living with Myotonic Dystrophy in the UK by collecting useful information which can be used to improve patient care, to support research into potential treatments, and to show that there is a community of people living with DM in the UK who may not be otherwise counted. We will make contact with you when we are able to share information about possible new treatments (clinical trials), or other relevant news and information. By holding secure records of your clinical details, we will also be able to check whether such trials may be suitable for you to consider participating in. There may be other benefits to joining the registry, for example if data is used to improve national standards of care, in academic research, or to help with getting new treatments approved.

You will not receive any direct payments or any other financial benefit as a result of joining the registry. The results of research arising from the data may have business potential, but you will not receive financial benefits from such development.

Where can I find out more about how my information is used?

Details of any completed requests to use the registry data are available to view on the registry website, along with any publications, or other research outputs to which the registry has contributed data (where publicly available). You can also contact the Registry Manager, or visit at www.hra.nhs.uk/information-about-patients/ for more information.

What information will I be asked to provide?

The registry asks questions about you and how Myotonic Dystrophy affects you. You will receive an email reminder every year after you first join, to ask you to update your information again. You will also have the chance to provide the details of a neuromuscular specialist (normally a consultant doctor in a hospital) if you see one, and give them permission to add some information from your medical notes to the registry. You can view all of the questions on the registry website before taking part, including the questions your specialist would be asked to answer. You will be able to see the answers your specialist has provided, but you cannot edit these yourself. If you have any questions about any data entered by your specialist, please contact the Registry Project Manager.

The registry may add additional questionnaires in future to help inform research. If a new questionnaire is added this will always be optional and additional information specific to that questionnaire will be provided.

It is important that you keep your contact information up to date, particularly your email address as this is used as your registry username, and for any communications from the registry. If you do not have an email account or internet access, you can nominate a trusted family member or friend to help with your data entry and to pass on any email correspondence.

Sharing your Genetic Test Results with the registry

While it's not essential to share this with the registry, some of the most important data for research are the details of your genetic diagnosis, found in your genetic test results. There are currently two ways this can be shared with the registry:

1) your nominated neuromuscular specialist can input the data directly via the clinician form, or

2) a digital copy of the test results letter can be uploaded to the registry's secure upload site, to be entered by the registry team.

If you don't have a copy of your genetic report, you can ask at the hospital where your blood test was taken as part of your diagnosis, or your GP may also have access. Patient organisations like (Myotonic Dystrophy Support Group), CureDM (Cure Myotonic Dystrophy UK Charity) and MDUK (Muscular Dystrophy UK) can help you with this if you get stuck.

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

Although one of the main aims of the registry is to make it easier for patients to be informed about clinical trials, there is no guarantee that registering your details will mean you will be a candidate to take part in a clinical trial. The registry does not run or recruit directly to clinical trials. We will circulate information about relevant clinical trials that we think you might meet the eligibility criteria for if they are recruiting in the UK, but these will always be subject to additional eligibility and screening criteria by trial site teams.

I'm not interested in getting involved in clinical trials, so why should I register?

The registry is not only for recruiting trials, it is also for collecting important information like patient numbers which helps build an understanding of how many people in the UK live with Myotonic Dystrophy. This in itself can help to improve clinical treatment for patients. By registering you are ensuring you are counted, this helps us to demonstrate to research companies (for example) that the UK DM community is worth investing time into.

How will my information be kept confidential and protected?

All information we receive from you will be treated confidentially and stored on a secure computer server located in the UK, specified on the registry website. Details of your specific diagnosis as well as personal information (name, age, address, gender, etc.) will be stored on the database. This information is required to help show how the condition affects people in a particular way, to improve understanding that can then be passed into clinical care. It also enables us to match you with eligibility criteria for clinical trials, and for other research purposes,. Only members of Prof. Marini Bettolo's team with specific permission will be allowed to look at this information. If we publish any research or other documents based on information from the database, this will not identify you by name.

The registry has a Steering Committee responsible for governing the use of registry data. The committee includes academic, medical and genetic experts, and patient advocates and representatives. The committee review and approve or reject all requests for the registry to support specific projects, or requests for data, ensuring that we only provide support to projects which may be beneficial to the community.

Do I have to join the registry and can I withdraw if I change my mind?

Joining the registry is voluntary. Should you wish to withdraw your information you will be free to do so at any time without having to provide any explanation. If you wish to withdraw, you should contact the staff in charge of the registry. Joining or leaving the registry will in no way affect the care you receive for your condition. Please note is not possible to withdraw data already used for other studies etc.

If you have chosen to give your hospital doctor permission to add clinical data to your registry record, this permission can be revoked at any time by contacting the Registry Manager. This permission will also be revoked automatically if you decide to withdraw from the registry completely.

How will my details be updated?

You will be able to update most of your details via the registry website, and you can contact the registry team at any time if you need to amend any other information (for example if you have changed your name). The registry will automatically send you an email reminder a year after joining, asking you to check or update your details. If your email address stops working we will try to telephone you or send a letter to get an updated email contact for you. If you nominate your neuromuscular specialist we will also ask them to update your clinical information once a year.

If you are unable to enter information to the registry yourself due to physical disability or lack of internet access for example, you can nominate a trusted family member, carer or friend to enter your answers for you, to pass on email messages etc. Please contact the registry team before signing up to learn more about this. The registry curator may also be able to provide assistance with this.

Who is funding the registry?

The registry is currently funded by MDSG, CureDM and MDUK. No additional payments will be received by Prof. Marini Bettolo, or other members of the registry team, for adding your details on to the database.

Who has reviewed this project?

Although the registry is managed and run from Newcastle University, we receive approval from an NHS ethics committee to make sure we are not doing anything harmful to you or your data. This research has been reviewed by North East – Newcastle and North Tyneside 1 Research Ethics Committee who have given approval for this registry to continue. Our ethical approval is renewed at least every three years.

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 Principal Investigator or Registry Curator:

Principal Investigator:
Dr Chiara Marini Bettolo
Tel: 0191 241 8737
Email: Chiara.Marini-Bettolo@newcastle.ac.uk

Registry Curator:
Tel: 0191 241 8640
Email: myotonicdystrophyregistry@newcastle.ac.uk

If you feel that you have been treated unfairly, or would like to comment on the conduct of any aspect of this research, please contact the Patient Advice and Liaison Service (PALS) 0800 0320202.

Thank you for taking the time to read this information sheet.

GLOSSARY – What do these words mean?

Clinical trial – a research project to test a possible new treatment, medicine, or therapy with patients living with a particular condition

Consent – Saying yes to something, or agreeing it can be done. You can always change your mind after giving consent.

Consent form – agreeing to something in writing. This can be on a computer or a paper form.

Database – a collection of information stored safely on a computer

Ethics Committee – a group of people who look carefully at research projects to make sure they are planned fairly, and properly carried out

Password protected – a way of stopping people from looking at your information without permission

Registry – a special kind of database for collecting health information

Research – careful investigation of a particular subject, learning more about something

Research companies – a business that pays for investigation of a particular subject

Steering Committee – a group of experts, including people from patient groups, who make sure the data collected is only shared with people or companies who are trying to help.