

Who has access to my data?

The Registry is governed by an oversight committee that includes doctors, scientists and patient organisations. It is their responsibility to monitor the registry appropriately and to review any requests for data, for example from a company planning a clinical trial.

If a researcher or a company applies for access to the data in the Registry, personally identifiable information such as your name, address, etc, does not go with it – the data about your condition is identified only by a code.

Your contact details are never given to the company by the Registry.

When a company wants help with recruiting patients to a trial, the Registry acts as a kind of 'trusted intermediary'. The Registry curator contacts everyone in the Registry who meets the criteria for the trial and gives them details of how to proceed if they are interested. Usually this would involve you contacting the clinic running the trial to arrange an appointment to discuss the trial and what it involves. You would only need to do anything if you were interested in taking part.

Further information

More information about patient registries can be found on the following websites:

www.muscular-dystrophy.org/research/patient_registries

www.treat-nmd.eu/resources/patient-registries/

If you have any questions please contact TREAT NMD, the Muscular Dystrophy Campaign, the Myotonic Dystrophy Support Group, or the Registry Curator - contact details are on the back of this leaflet.

The UK Myotonic Dystrophy Patient Registry is managed by TREAT-NMD - a network which brings together scientists, healthcare professionals, pharmaceutical companies and patient organisations to create the infrastructure to ensure that new therapies reach patients as quickly as possible.

Ben Porter (Registry Curator)

Institute of Genetic Medicine
Newcastle University
International Centre for Life
Newcastle upon Tyne, NE1 3BZ

myotonicdystrophyregistry@treat-nmd.eu
0191 2418640

Registry Principal Investigator:
Dr Chiara Marini Bettolo

Jointly supported and co-sponsored by:
Myotonic Dystrophy Support Group



www.myotonicdystrophysupportgroup.org
0115 987 5869
contact@mdsguk.org

Muscular Dystrophy Campaign



www.muscular-dystrophy.org
020 7803 4800
research@muscular-dystrophy.org

UK Myotonic Dystrophy Patient Registry

An opportunity for patients with Myotonic Dystrophy type 1 to participate in clinical trials and obtain the best possible care



What is a patient registry?

A patient registry collects information about people who are affected by a particular condition. Registries focus on the information that is needed to find people eligible for clinical trials, but they also have many other benefits. The information they hold can help clinicians develop care standards for a condition which helps to improve the care people receive in their local clinic. Registered persons can link to the research community and have the opportunity to receive information directly relevant to their condition.

It should be noted that submitting your data to a patient registry does not mean that you are obliged to enrol in clinical trials, nor will you be guaranteed a place on a clinical trial; it just gives you the option of taking part.

Why do we need a registry for Myotonic Dystrophy?

Patient registries allow people who may be eligible for certain trials to be contacted quickly and easily. This is especially important for Myotonic Dystrophy type I since it is a rare condition and without a patient registry, finding enough patients for a trial can take years, and this can delay the testing of potential therapies. Research in the laboratory has resulted in the development of potential new therapies for Myotonic Dystrophy type I which means that clinical trials are on the horizon.

Doctors and scientists can also access the medical data in the registry to learn more about the condition, how it progresses and how best to care for people with Myotonic Dystrophy type I.

Who can register?

Anyone in the UK with a diagnosis of Myotonic Dystrophy type I can register. Confirmation by a genetic test is the most reliable method, but if you haven't had a test yet then don't worry as you can have one later. You are still free to register without a confirmed genetic diagnosis. Young people under the age of 16 years must be registered by their parent/guardian.

Why should I register?

- Registering may open up opportunities to take part in clinical trials and other studies.
- Registries aim to speed up the development of new therapies for Myotonic Dystrophy type I.
- You will receive newsletters containing information relevant to your condition, such as the latest research developments.
- Registries help specialists gain more knowledge about the number of people affected by Myotonic Dystrophy and how the condition progresses - information that is vital for planning appropriate medical care.

How do I register?

Patients can register online (over the internet), which allows them to view and update their data at any time. If you don't have access to the internet you can phone or email the registry curator to have a paper version of the form sent to you.

<https://www.dm-registry.org/uk/>

Registration is voluntary and is done by the person themselves or by the parent/guardian.

What data are stored in the Registry?

The UK Myotonic Dystrophy Patient Registry contains patients' personal details, such as name, address, date of birth and gender, so that they can be identified and contacted by the Registry staff where appropriate.

The Registry also contains details of the symptoms people experience, such as the level of motor function and fatigue. Clinical data such as heart and lung function test results may also be added to the Registry by the person's doctor.

Results of genetic tests will also be stored in the Registry because to develop treatments it is important that researchers have precise information about the genetic mutation that is responsible.

Who enters your data?

Information entered into the Registry is provided by either the patient themselves or the professionals involved in their care, after the patient has given consent for them to do so. A curator is employed to manage the Registry and monitor the data.

Is my data safe?

All the data are stored in a secure server (protected in a similar way to online bank accounts) accessible to only specially appointed registry staff.

At all times the data remain your property and you have the right to withdraw your information at any time.

