

Why join the registry?

The Myotubular and Centronuclear Myopathy Patient Registry aims to help researchers locate suitable patients for specific clinical trials. It can also improve our understanding of the condition and its prevalence, and even encourage new research.

This in turn will help to develop better standards of care for patients, families, and the medical community, and will help geneticists to better prepare and advise families affected by these conditions in the future.

Patients can register in order to:

- ✓ **Help** identify patients for clinical trials as they become available.
- ✓ **Support** research projects with specific MTM and CNM data.
- ✓ **Encourage** further research into the conditions thanks to the existence and availability of the data.
- ✓ **Provide** doctors with up-to-date information on managing MTM and CNM, to help them deliver better standards of care for their patients.
- ✓ **Receive** updates on relevant research, studies or clinical trials.

Strength in Numbers

Join today by registering online

www.mtmcnmregistry.org

Or email us at:

mtmcnmregistry@treat-nmd.eu



www.mtmcnmregistry.org

Reaching clinical trials

Research to find a treatment or cure for muscle conditions such as the myotubular and centronuclear myopathies is well underway, with some encouraging results so far.

Natural history studies and observational studies are already in progress, and arrangements for the first clinical trials to treat patients with X-linked myotubular myopathy are in the pipeline.

Research is also being done on other forms of the condition, known as centronuclear myopathy. Early signs are hopeful, and suggest that clinical trials could be a tangible distance away.

The main purpose of the Myotubular and Centronuclear Myopathy Patient Registry is to offer clinicians and researchers an easier path to clinical trials, and inform patients and families about research or new treatments.

www.mtmcnmregistry.org

Who can register?

We welcome the registration of:

- ✓ Patients with a myotubular or centronuclear myopathy diagnosis, confirmed via genetic testing or muscle biopsy.
- ✓ Female carriers of X-linked myotubular myopathy, especially those with myotubular myopathy type symptoms.
- ✓ Any patient who is deceased, but who had a confirmed diagnosis.

If you do not fit these descriptions but would like to receive updates and newsletters, please email us and we will add you to our mailing list.

What information is collected?

- ✓ Personal details such as name, address, date of birth and gender
- ✓ Clinical and genetic information such as symptoms and diagnosis

How do I register?

Register on our website and create an account so you can view or update your information at any time.

If a patient is under 18, a parent or guardian can register on their behalf.

Strength in numbers: join today

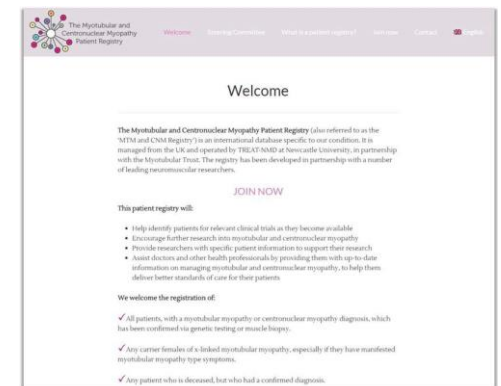
Is my data safe?

Your information will be stored on a secure server and looked after by the Registry Curator at Newcastle University, part of the TREAT-NMD Neuromuscular Network. Researchers can apply for anonymous data from the Registry, or ask the Curator to send information about their project to the relevant patients.

The registry steering committee (a panel of expert doctors, researchers, and patient representatives) will assess these requests to make sure they are suitable and ethical.

Patients' identifiable information or contact details will never be shared.

Visit our website for more information



www.mtmcnmregistry.org