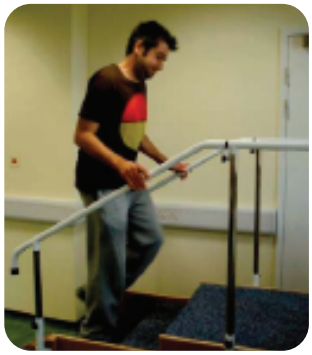
TREAT-NMD Neuromuscular Network

LGMD2iFund Limb Girdle Muscular Dystrophy 2I Research Fund

Register online:
www.FKRP-registry.org

GLOBAL FKRP PATIENT REGISTRY

An opportunity for patients with LGMD2I, MDC1C and other FKRP-related conditions to participate in clinical trials and obtain the best possible care.



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What is a patient registry?

A patient registry collects information about patients who are affected by a particular condition. The Global FKRP registry collects genetic and clinical data about patients affected by a mutation in the FKRP gene. Mutations in this gene cause one of the forms of limb girdle muscular dystrophy (LGMD) known as LGMD2I, and one of the forms of congenital muscular dystrophy (CMD) known as MDC1C. In very rare instances, FKRP mutations can also cause the conditions known as Muscle Eye Brain disease and Walker-Warburg syndrome. For research into treatments for all of these conditions, it is important that researchers have precise information about the genetic mutation that is responsible. In anonymous form, valuable medical data from the registry will be made available to selected researchers around the world, thereby accelerating the research into FKRP mutations and their possible treatments.

Additionally, with the advent of clinical trials for some neuromuscular conditions, patient registries mean that patients who may be eligible for certain clinical trials are readily identifiable. Because FKRP mutations are rare conditions, without a patient registry to gather patient details from all patients worldwide in one place, finding enough patients for a meaningful trial can take years, and this delays the testing of potential therapies.

Who should register?

Patients with limb girdle muscular dystrophy 2I (LGMD2I) or congenital muscular dystrophy 1C (MDC1C) or another condition with a confirmed mutation in the FKRP gene can register. Young people under the age of 16 years must be registered by their parents or guardians. This registry is for all patients worldwide.

Since patients with FKRP mutations are rare, every single person counts!

Why should I register?

There are several reasons for patients to register:

- Registries facilitate patient recruitment for the clinical trials and registered patients may be able to participate in trials more easily
- Registries aim to accelerate research into new therapies for LGMD2I, MDC1C and other FKRP-related conditions.
- Registered patients are kept informed about research results, such as new treatments for these conditions, as well as about TREAT-NMD activities.
- Registries help specialists gain more knowledge about the prevalence, the epidemiology and the natural history of LGMD2I, MDC1C and other FKRP-related conditions.
- Registries may help achieve equal care for all patients with these conditions worldwide.
- Registries may help raise the urgently needed funds for further research.

How do I register?

Registration is voluntary and is done by the patients themselves or by their parents/guardians. Patients can register online (over the internet), which allows them to view and update their data at any time.
Register online at: www.FKRP-registry.org

Information that you enter is encrypted while being transferred, so that it can not be intercepted.

If you can not register online, contact us at the address on the side.

The Global FKRP patient registry is just one of TREAT-NMD Alliance's activities aiming to benefit patients worldwide. More information about the TREAT-NMD Alliance and its activities is available at www.treat-nmd.eu.

More information on FKRP-related diseases can be found at the LGMD2I Research Fund website (www.lgmd2ifund.org), a non-profit organization focused in expediting the discovery of new treatments for LGMD2I.