

GLOBAL FKRP REGISTRY

REGISTER ONLINE:
www.fkrp-registry.org

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

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



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 known as LGMD2I, and a form of congenital muscular dystrophy known as MDC1C. In very rare instances, *FKRP* mutations can also cause the conditions 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*-related muscular dystrophies are such 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, delaying the testing of potential therapies.

Who should register?

- Patients with limb girdle muscular dystrophy type 2i (LGMD2I), congenital muscle disease type 1c (MDC1C), Muscle Eye Brain disease, or Walker-Warburg syndrome;
- Patients under the age of 18 years must be registered by their parent or guardian;
- This registry is for patients worldwide.

Why should I register?

There are several reasons for patients to register:

- Registries facilitate patient recruitment for clinical trials and registered patients may be able to participate in trials more easily;
- Registries aim to accelerate research into new therapies for *FKRP*-related MDs;
- 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 *FKRP*-related MDs;
- Registries may help achieve equal care for all patients with these conditions worldwide;
- Registries may help raise the urgently needed funds for further research.

The Global FKRP Registry is just one of TREAT-NMD's activities aiming to benefit patients worldwide. More information about the TREAT-NMD network 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 organisation focused on expediting the discovery of new treatments for LGMD2I.

Contact details

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