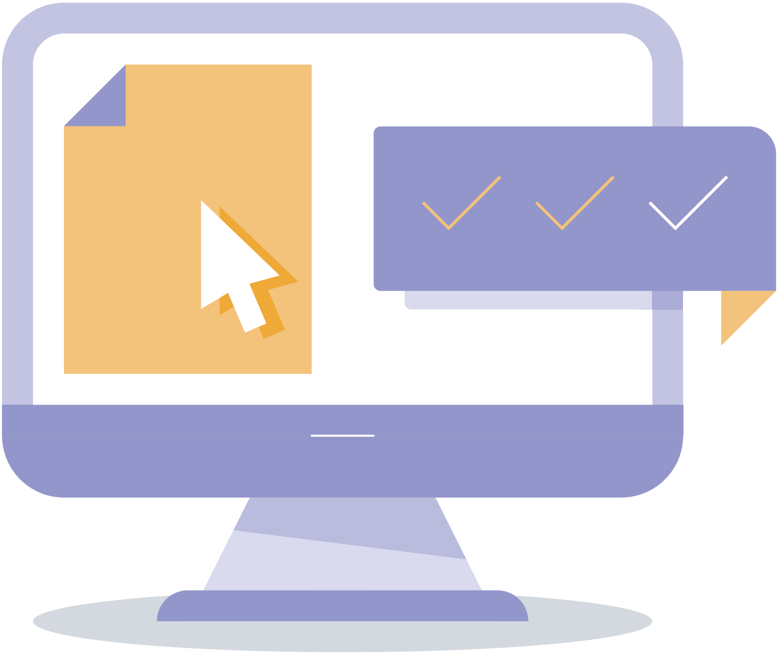
Understanding the MLD patient registry

(Metachromatic Leukodystrophy)

Information for Families and Healthcare Professionals



mld
SUPPORT
ASSOCIATION UK

What is a Registry?

A patient registry collects and stores patient medical information, family history and other related information for use in clinical research.

Part 1 of the Registry collects personal information, location and contact details, clinical history, diagnostic information, genetic information and contact details for clinicians. This only needs to be completed once, on enrolment.

Part 2 collects on-going Clinical information and will be completed once or twice a year.

Patient information in the registry will be used to create a natural history of MLD types to better understand rare diseases and to develop new treatments.

Why do we need a Registry?

Researchers studying MLD need accurate information to understand how the disease affects people.

In addition, the MLD registry can be used to locate people who may be eligible to participate in particular studies, such as trials to test new treatments.

Is the Registry secure?

The registry anonymised clinical data will be available to researchers while protecting your privacy. One way the registry protects your privacy is to remove your name, address and other "identifying" information from your clinical information before providing it to researchers.

Your registry information will be labelled with a code number and stored on secured computers and servers, and protected with encryption and passwords.

Only a few authorised people who work in the registry will have access to the key to the code and will be able to identify you if needed.

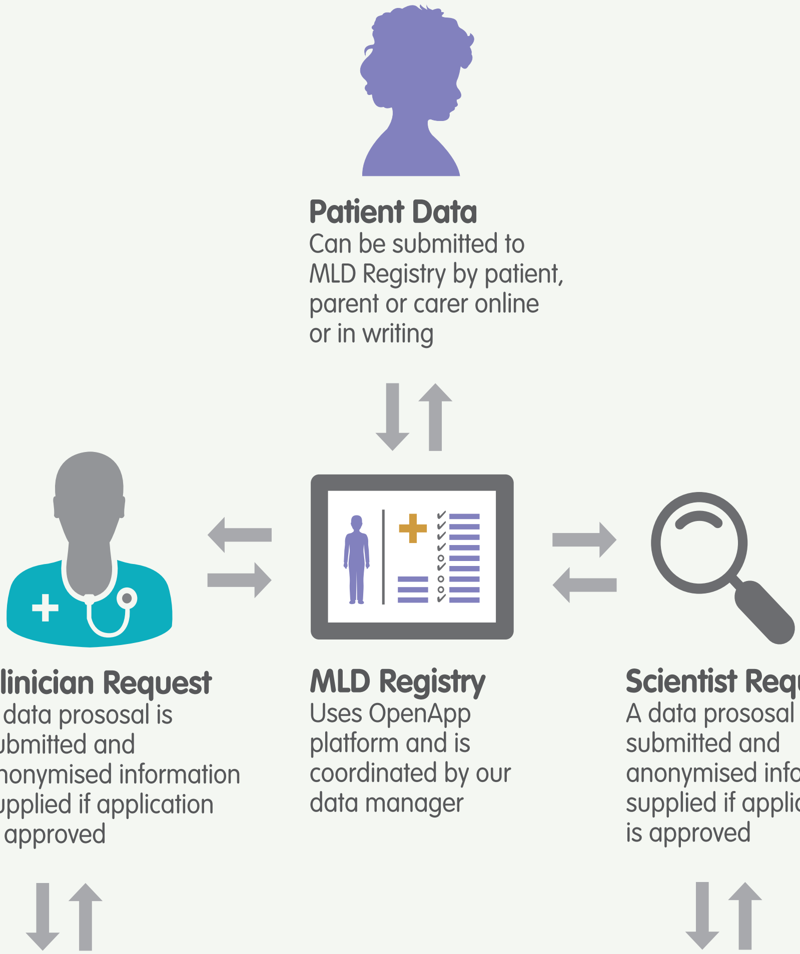
The registry will not share your identifiable information with anyone outside the registry (unless you give your permission to share it).

Approved researchers and clinicians will be allowed to see only the de-identified information. Approved researchers and clinicians may use de-identified information to conduct research, including research on diseases unrelated to your (the patient's) disease.

Governance

All data will be collected in line with GDPR guidelines and will only be collected on receipt of informed consent.

How the Registry works



Patient Data
Can be submitted to MLD Registry by patient, parent or carer online or in writing

Clinician Request
A data prososal is submitted and anonymised information supplied if application is approved

MLD Registry
Uses OpenApp platform and is coordinated by our data manager

Scientist Request
A data prososal is submitted and anonymised information supplied if application is approved

Data is used to measure, survey and compare aspects of a condition and its treatment to deepen the understanding of the condition and improve standards of care

What do I do to participate?

- **Contact MLD Support Association** (see details below).
- **You will be directed to or sent the information leaflet.**
- **Once you have read this and contacted us with any questions, you will be asked to sign the consent form.**
- **You will then be shown how to access the questionnaires.**

What is MLD?

MLD is an acronym for Metachromatic Leukodystrophy. MLD is an autosomal recessive genetic disorder which, at the moment, has no cure. MLD is directly caused by a deficiency of the enzyme Arylsulfatase-A. Without this enzyme sulfatides build up, eventually destroying the myelin sheath of the nervous system. The myelin sheath is a fatty covering that protects nerve fibres. Without the sheath, the nerves in the brain and the peripheral nerves cease to function properly.

About us

MLD Support Association UK was set up to bring hope to families in the fight to eradicate MLD (Metachromatic Leukodystrophy).

Our aim is to provide support to families, personally, through our Website and Facebook group and at annual Family Conferences and Fun Days.

To find out more about our work and the disease, see our **Living with MLD** and **Understanding MLD** leaflets, or visit our website.

Contact us

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Email: admin@mldsupportuk.org.uk

www.facebook.com/groups/MLDsupportUK

www.mldsupportuk.org.uk

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Registered Charity Number 1150542