WHY JOIN A PATIENT REGISTRY?

Patient registries are databases containing information about individuals who are affected by a specific condition or genetic mutation. In rare diseases, they play an important role in the development of new therapies. The neuromuscular registries listed below are coordinated from the John Walton Muscular Dystrophy Research Centre.

Registries can:
- Identify participants for clinical trials.
- Help develop care standards, to help improve the care people receive.
- Support specific research questions for doctors and scientists.
- Contribute to the natural history of a rare disease.
- Provide a link to the research community enabling people to receive information directly relevant to their condition (for example, newsletters or updates on standards of care).

UK National Patient Registries

- www.treat-nmd.org.uk/registry
- www.fshd-registry.org/uk
- www.dm-registry.org/uk

International Patient Registries

- Limb-girdle muscular dystrophy type R9 or 2I (LGMDR9 or LGMD2I)
- Congenital Muscular Dystrophy Type 1C (MDC1C)
- Muscle Eye Brain Disease
- Walker-Warburg Syndrome
  - www.fkrp-registry.org
- X-linked myotubular myopathy (MTM)
- Centronuclear myopathy (CNM)
  - www.mtmcnmregistry.org
- Ullrich congenital muscular dystrophy (UCMD)
- Bethlem myopathy
- Bethlem/Ullrich intermediate
  - www.collagen6.org
- GNE myopathy
- Hereditary inclusion body myopathy (HIBM)
- Nonaka myopathy
- Distal myopathy with rimmed vacuoles (DMRV)
  - www.gne-registry.org

Examples of how patient registries have been used successfully:

- The UK DM Registry has previously been used to support the recruitment onto a phase II clinical trial of tideglusib in teenagers and adults with congenital and childhood-onset DM. The registry has also been used to support a falls and fall-associated fractures survey in patients with DM1.
- The UK FSHD Registry was used to help a pharmaceutical company gain patient insight into their upcoming clinical trial protocol. This was captured via a survey sent out through the registry.
- The UK SMA Registry was used to distribute an EU wide survey regarding patient quality of life and pain.
- The Global FKRP Registry was used to support participant recruitment onto a phase III clinical trial of deflazacort in adults with LGMD2I.
- The GNE Myopathy Registry was used to support participant recruitment onto a phase III clinical trial of aceneuramic acid in adults with GNE myopathy.
- Many of the registries have collectively supported participant recruitment onto a research project investigating activity monitoring in patients with neuromuscular conditions.

For any questions please contact: registries@ncl.ac.uk