



Further Information about the NPC Quality of Life Research Project

This Research Project

This research project is part of a PhD programme focusing on understanding the quality of life in patients with Niemann-Pick disease type C (NPC) and their caregivers.

The project will test whether the questionnaire designed specifically to assess quality of life in NPC patients and caregivers (known as the NPCQLQ) is capable of measuring this accurately and consistently.

Once there is evidence to support the accuracy of the questionnaire, data collected can then be used in clinical trials and other studies to assess the impact of potential new treatments on patient quality of life.

Your help is needed to make sure the questionnaire collects information accurately, and that it is able to reflect the impact of NPC on quality of life for patients and their carers.

About Jackson Pountney

Jackson graduated from Bangor University in 2017 with a BSc in Clinical and Health Psychology, he then worked as a support worker for individuals with brain injuries for three years. In 2021 Jackson graduated from Aston University with an MSc in Health Psychology. Currently, Jackson is a Health Psychology PhD student studying at Aston University.

Jackson's research is supported by Niemann-Pick UK, the National Niemann-Pick Disease Foundation, the International Niemann-Pick Disease Alliance and the International Niemann-Pick Disease Registry.

About the INPDR

This study is using patient-reported data from the International Niemann-Pick Disease Registry (INPDR).

The INPDR is the first and only global database of its kind to collect information about the health and experiences of people living with Niemann-Pick diseases. The INPDR encourages and enables research with the hope that it will lead to effective treatment options for this community.

The INPDR was established *by* the community *for* the community. It is a community-governed registry that is completely independent of commercial influence. This means its supporters can be assured that decisions are taken in the interest of people affected by Niemann-Pick diseases.



The INPDR Patient Reported Database (PRD)

The INPDR collects information directly from patients, caregivers, and clinicians via an anonymised Clinician Reported Database (CRD) and a Patient Reported Database (PRD). Through the CRD, clinicians can supply diagnostic, genetic, or treatment-related information, as well as clinical history and test results.

Through the PRD, patients and caregivers can self-enroll online and contribute their data through a series of questionnaires including disease severity, burden of illness and quality of life. Collecting data from both clinical and community perspectives helps to build a unique, holistic picture of Niemann-Pick diseases.

By joining the PRD, patients and caregivers can help the Niemann-Pick community to document how these diseases change over time and to highlight the impact of existing clinical and care management approaches. This will give researchers a better understanding of the impact of new treatments, helping to improve the effectiveness and long-term safety of future therapies.

For more information

For more information about this research project contact Jackson at 200216137@aston.ac.uk
To find out more about the INPDR, please email info@inpdr.org or visit www.inpdr.org