The Ara Parseghian Medical Research Fund awards grant to the International Niemann-Pick Disease Registry

29 July 2024 – Tyne & Wear, England.

The International Niemann-Pick Disease Registry (INPDR) is pleased to announce that it has been awarded a two-year grant totalling $200,000 from the Ara Parseghian Medical Research Fund (APMRF) at the University of Notre Dame. This grant will facilitate the expansion of INPDR's activities in the United States and Canada. A portion of the funding will also be allocated to the development of a Niemann-Pick Disease specific Global Unique Identifier (GUID), which will enhance global data sharing and collaboration efforts.

The INPDR collects real-world data across 23 clinical sites in 12 countries allowing patients, families, and healthcare providers worldwide to record and share their experiences. With the inclusion of information relating to over 400 patients with Niemann-Pick Disease Type C (NPC) and Acid Sphingomyelinase Deficiency (ASMD), the INPDR is the largest source of real-world evidence ever collected.

INPDR's Chief Executive Officer, Conan Donnelly stated "We are deeply honoured to receive this generous grant from the Ara Parseghian Medical Research Fund, which will play a crucial role in advancing our research on Niemann-Pick Diseases. Critically, this funding will be instrumental in developing essential infrastructure to link clinical data, supporting more robust research and analysis. Additionally, it will help expand our data collection efforts in North America and beyond."

Sean Kassen, Director of the APMRF said, "Supporting the International Niemann-Pick Disease Registry is not just about collecting data—it's about connecting lives, driving research, and offering hope to families around the world. Every contribution brings us one step closer to understanding, treating, and ultimately curing this devastating disease."

INPDR CRA Consultant, Solomon Mbua, adds, "This funding will greatly enhance our ability to collect and analyse critical data, facilitating a deeper understanding of Niemann-Pick Diseases. By supporting our research, the Ara Parseghian Medical Research Fund is playing a crucial role in advancing our knowledge and helping us to identify potential pathways for future treatments. This partnership underscores the importance of collaboration in driving progress and hope for the Niemann-Pick community."

Notes to Editors

About the INPDR

The INPDR is a web-based disease-specific registry, collecting information about ASMD Niemann-Pick Disease (types A & B), and Niemann-Pick Disease Type C, via, an anonymised Clinician Reported Database (CRD) and a Patient Reported Database (PRD). The PRD enables patients to self-enrol online and to contribute their data through a series of questionnaires including disease impact, health economics and quality of life. The INPDR is actively supported by patients, clinicians, patient advocates and researchers from over 20 countries across five continents.

For more information, visit: www.inpdr.org.

About Niemann-Pick disease

Niemann-Pick Diseases are a group of rare, inherited disorders, affecting both children and adults. They happen because the body can't properly break down certain fats. These diseases are split into two distinct subgroups: Acid Sphingomyelinase Deficiency (ASMD) and Niemann-Pick Disease Type C (NPC).

Acid Sphingomyelinase Deficiency (ASMD) is an extremely rare, progressive genetic disease, previously known as Niemann-Pick Disease Type A (NPA), Type A/B and Type B (NPB). ASMD represents a spectrum of disease, caused by a lack of the enzyme acid sphingomyelinase and resulting in potentially life-limiting illness in children and young adults. NPC is a cellular lipid trafficking disorder, is a significantly life-limiting neurodegenerative disease that can present in different ways. NPC is progressive, with disabling neurological symptoms.

About the Ara Parseghian Medical Research Fund at the University of Notre Dame

The Ara Parseghian Medical Research Fund at the University of Notre Dame in the U.S. is a non-profit organization dedicated to finding a treatment or cure for NPC Disease—a genetic, cholesterol storage disorder that primarily strikes children with death occurring before or during adolescence. The Parseghian Fund was founded by Cindy and Mike Parseghian whose three youngest children were diagnosed with NPC in 1994 and the APMRF is named for Ara Parseghian, the much beloved and well-known Notre Dame American football coach and grandparent of the children.