International Niemann-Pick Disease Registry announces new research collaboration using real-world data to evaluate the treatment effects of olipudase alfa to manage Acid Sphingomyelinase Deficiency (ASMD)

24 January 2024 – Tyne & Wear, England. The International Niemann-Pick Disease Registry (INPDR) today announced a new collaboration giving Sanofi controlled access to anonymized registry information via INPDR's research "Gateway" platform. This collaboration will provide Sanofi with real-world evidence that will support evaluation and decision making on the use of olipudase alfa to manage Acid Sphingomyelinase Deficiency (ASMD). INPDR is the largest active database about the diagnosis, management, and progression of the inherited lysosomal storage disorder known as Acid Sphingomyelinase Deficiency (ASMD).

The INPDR was created to collect real-world data and allow patients, families, and healthcare providers worldwide to record and share experiences. The INPDR database includes information relating to more than 100 patients with ASMD, across 13 clinical sites in 9 countries, which makes the largest source of real-world Acid Sphingomyelinase Deficiency evidence ever collected.

INPDR's Chief Executive Officer, Conan Donnelly states "The INPDR is pleased to share that the registry data is actively contributing to the advancement of clinical research for the community. The collaboration with Sanofi reinforces our commitment to enhancing the understanding of Niemann-Pick diseases and showcases the importance of the Registry for the Niemann-Pick community."

"We hope that this collaboration between INPDR and Sanofi will provide valuable real-world evidence of the clinical management of patients living with ASMD, potentially giving clinicians, patients and caregivers the opportunity to improve patient outcomes over time," said Antonio Santos, M.D., Ph.D., Global Head of Rare Hematology Medical Affairs, Sanofi.

INPDR Trustee, Toni Mathieson adds, "We enter this collaboration guided by our founding principles of integrity and independence, and our mission to advance research and improve health outcomes by accurately documenting the Niemann-Pick patient experience. This work is only possible thanks to the support of patients, their families and clinicians, who have committed to participating in the registry, sharing important real-world information about diagnosis, management, and progression of Niemann-Pick diseases. This collaboration demonstrates how their valuable data is accelerating research in the field."

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-enroll 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, autosomal recessive, lysosomal lipid storage disorders, affecting both children and adults. These can be further divided 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, historically 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. Niemann-Pick disease type C (NPC), a cellular lipid trafficking disorder, is a hugely life-limiting neurodegenerative disease with a variable clinical presentation and characterized by progressive, disabling neurological symptoms.