International Niemann-Pick Disease Registry and Mandos Health announce new research collaboration using real-world data to evaluate the treatment effects of VTS-270 to manage Niemann-Pick type C1

1 June 2023 – Tyne & Wear, England. The International Niemann-Pick Disease Registry (INPDR) and biopharmaceutical company Mandos Health today announce a new collaboration giving Mandos Health controlled access to registry information via INPDR's research "Gateway" platform. This partnership will provide Mandos Health with real-world evidence that will directly inform the development of its Niemann-Pick type C1 (NPC1) investigational product, VTS-270. INPDR is the largest active database about the diagnosis, management, and progression of the devastating inherited lysosomal storage disorder known as Niemann-Pick type C1.

In 2013, the INPDR was launched 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 300 patients with NPC1, across 20 clinical sites in 11 countries, which makes the largest source of real-world Niemann-Pick type C1 evidence ever collected.

INPDR's Chief Executive Officer, Conan Donnelly states "The INPDR is delighted to announce that registry data is working hard on behalf of the community to support clinical research. This collaboration with Mandos Health demonstrates that INPDR is serving the Niemann-Pick community in the way it was intended. It also supports our efforts to deepen understanding of Niemann-Pick diseases."

"We are committed to working with the Niemann-Pick community to ensure we have every piece of high-quality evidence incorporated in our analysis of the treatment effects of VTS-270," notes Cathy Traz, Mandos Health's VP of Patient & Community Engagement "We chose to partner with INPDR as it provides an unparalleled source of real-world data that represents the Niemann-Pick type C1 community and it enables us to address open questions and have further discussions with regulators about the treatment effects of VTS-270 on this ultra-rare condition."

Niemann-Pick UK's Chief Executive and INPDR Trustee, Toni Mathieson adds, "This work is only possible as patients, their families and clinicians have committed to participating in the registry, sharing important real-world information about diagnosis, management, and progression of Niemann-Pick diseases. We would like to express our sincere gratitude to the community by showing how their valuable data is accelerating research in the field. We enter this partnership 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."

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 and devastating inherited lysosomal storage disorders that can affect both children and adults:

Acid Sphingomyelinase Deficiency (ASMD) includes Niemann-Pick disease type A (NPA) and type B (NPB), which are caused by a lack of the enzyme acid sphingomyelinase leading to a build-up of toxic materials in the body. Niemann-Pick disease type C (NPC) is a hugely life-limiting neurodegenerative disease caused by an accumulation of lipids (fats) in the liver, brain, and spleen.

About Mandos Health

Mandos Health is a biopharmaceutical company committed to furthering development of VTS-270 for Niemann-Pick Type C, an ultra-rare genetic disease that affects the body's ability to metabolize cholesterol and lipids within cells. Niemann-Pick Type C disease can affect the brain, nerves, liver, spleen, bone marrow and lungs and there are currently no FDA approved treatments.

For more information, visit: www.mandoshealth.com.