

C-Path Forms Data Collaboration with International Niemann-Pick Disease Registry (INPDR) to Propel Niemann-Pick Disease Research Forward

TUCSON, Ariz. April 17, 2024 — [Critical Path Institute](#) (C-Path), a leader in advancing rare disease research, today announced a significant new partnership with the International Niemann-Pick Disease Registry (INPDR), focusing on the integration of data to support and accelerate rare disease research through C-Path's Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®). This collaboration leverages the comprehensive data collection efforts of the INPDR to enrich RDCA-DAP's capabilities in facilitating the development of therapies for Niemann-Pick disease type C (NPC).

RDCA-DAP, renowned for its robust data aggregation and analysis capabilities, provides an efficient and standardized infrastructure to support rare disease characterization and clinical development. The strategic partnership with INPDR will enhance RDCA-DAP's integrated database and analytics hub, allowing for the aggregation of valuable patient data, thereby enabling more efficient and effective interrogation of data critical for advancing research and therapy development.



In light of the INPDR collaboration with RDCA-DAP, C-Path's [Critical Path for Lysosomal Diseases \(CPLD\) pre-consortium, stands to benefit from this collaboration](#), as this is CPLD's first milestone in future work in NPC. CPLD is committed to uniting industry leaders, researchers, regulators, and patient advocacy groups to advance diagnostic and therapeutic approaches for lysosomal diseases. Amanda Klein, Pharm.D., Executive Director of CPLD, commended this effort, stating, "INPDR's data sharing with RDCA-DAP enhances our pursuit of new treatments and underscores the impact of collaborative innovation in rare disease research."



Conan Donnelly, CEO of INPDR, emphasized the importance of this collaboration, "INPDR is delighted to partner with C-Path and contribute anonymized data to support research and analysis aimed at improving outcomes in Niemann-Pick Type C. By pooling INPDR data with other valuable patient information, C-Path will enhance the power of our registry data and create the possibility of establishing new insights into Niemann-Pick Type C. We anticipate this collaboration will mark the beginning of a long-term, fruitful, and productive partnership with the C-Path team."

INPDR Board Trustee, Toni Mathieson, stated that, "This partnership marks a pivotal moment for the INPDR; it supports our mission to drive meaningful progress in research and will pave the way for improved care and therapies for the global Niemann-Pick type C community."

This data-sharing partnership aligns with C-Path's mission to catalyze the development of new treatments by fostering collaborations that leverage the power of data. The integration of INPDR data with RDCA-DAP

underscores a shared commitment to advancing the understanding and treatment of rare diseases through collaborative research and data analysis.

“Partnering with the International Niemann-Pick Disease Registry marks a significant milestone for RDCA-DAP,” said Alexandre Bétourné, Pharm.D., Ph.D., Executive Director, RDCA-DAP. “Integrating the comprehensive data from INPDR into our platform enriches our ability to propel forward research in Niemann-Pick diseases. This collaboration directly contributes to accelerating the pace of discovery for new treatments and significantly improving patient outcomes.”

For more information about contributing data to RDCA-DAP or to learn more about this collaboration, please visit c-path.org/rdca-dap or contact rdcadap@c-path.org.

About Critical Path Institute

Critical Path Institute (C-Path) is an independent, nonprofit established in 2005 as a public-private partnership, in response to the [FDA’s Critical Path Initiative](#). **C-Path’s mission is to lead collaborations that advance better treatments for people worldwide.** Globally recognized as a pioneer in accelerating drug development, C-Path has established numerous international consortia, programs and initiatives that currently include more than 1,600 scientists and representatives from government and regulatory agencies, academia, patient organizations, disease foundations and pharmaceutical and biotech companies. With dedicated team members located throughout the world, C-Path’s global headquarters is in Tucson, Arizona and C-Path’s Europe subsidiary is headquartered in Amsterdam, Netherlands. For more information, visit c-path.org.

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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 anonymized 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: 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.

Contacts:

Roxan Triolo Olivas

C-Path

520.954.1634

rolivas@c-path.org

Kissy Black

C-Path

615.310.1894

kblack@c-path.org