

International Niemann-Pick Disease Registry

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As the global community comes together to mark Rare Disease Day 2026, the International Niemann-Pick Disease Registry (INPDR) reflects on the vital role that high-quality data, collaboration, and shared knowledge play in improving outcomes for individuals and families affected by Niemann-Pick diseases.

Conferences & Community Engagement

Over the past month, the INPDR has actively participated in several international conferences and community events, providing valuable opportunities to share progress, exchange knowledge, and reinforce partnerships across the rare disease ecosystem.

- World Orphan Drug Congress Europe 2025 – At this event, the INPDR team engaged with key stakeholders from across research, industry, and patient advocacy. A central theme throughout the congress was the growing recognition of disease registries as critical enablers of rare disease research and therapy development.
- Fundación Niemann-Pick de España International Family-Scientific Conference – During the meeting Maria Garrido Gutiérrez presented updates on Registry developments, new collaborations, and ongoing initiatives. The conference offered a meaningful space for dialogue between families and professionals, reinforcing the value of partnership in shaping research priorities and improving care.
- Asociación ASMD España – Niemann-Pick B Scientific and Family Conference - Similarly, participation in this conference enabled the INPDR to connect with the Spanish ASMD community, share Registry progress, and learn from the work being led by clinicians, researchers, and families to advance understanding and support.



- WORLDSymposium 2026 - A major highlight of the year, where the INPDR maintained a visible presence within the exhibition hall and engaged extensively with the global lysosomal disease community. Attendance by Solomon Mbuja and Shaun Bolton facilitated numerous conversations with clinicians, researchers, and partners, while the INPDR booth served as a focal point for information exchange and networking.

During the Symposium, the INPDR presented four posters exploring key areas of research and Registry development, including real-world patient experience, disease progression across the lifespan, neurological and psychiatric manifestations, and the development of the Global Unique Identifier (GUID) standard. These posters are available to view on the [INPDR website](#) and represent the growing impact of Registry-enabled research.



INPDR Forum Report

We are pleased to share the publication of the INPDR Forum Report, a document bringing together the key discussions, insights, and priorities identified during the INPDR Forum. Reflecting contributions from across the global Niemann-Pick community, the report highlights shared experiences, emerging needs, and opportunities to strengthen the Registry’s impact moving forward.

We warmly thank everyone who participated and contributed their perspectives, helping to shape this important piece of work. The full INPDR Forum Report is available to read [here](#).

Key Achievements — A Year of Progress for the INPDR

Over the past year, the INPDR has continued to grow as a global resource supporting research and improved understanding of Niemann-Pick diseases. Milestones included expansion of participant enrolment across both the Clinician Reported and Patient Reported Databases, activation of new sites and countries, and increasing engagement through research data access requests and international conference presentations.

Expanding the Global Network

Further strengthening its international reach, the INPDR is pleased to welcome Evelina London Children’s Hospital in the United Kingdom as the latest site to join the Clinician Reported Database.

Key Achievements for the INPDR in 2025

- 665 participants enrolled worldwide to date (CRD+PRD)
- 7 new sites and 3 new countries joined the CRD
- 18 new sites progressing through set-up pipeline
- 5 research data access requests supported
- 8 conference presentations, 1,600+ attendees

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Under the leadership of Principal Investigator Dr Berna Seker Yilmaz, this collaboration represents another meaningful step toward building a truly global Registry infrastructure and ensuring that more patients and families have the opportunity to contribute to collective knowledge generation.

The NP GUID – Secure Data Collaboration

Thanks to funding from the Ara Parseghian Medical Research Fund, the INPDR has launched a groundbreaking tool for global data collaboration — the Niemann-Pick Global Unique Identifier (GUID).

This system allows researchers and clinicians to generate a consistent, irreversible pseudonymised identifier for each patient across multiple studies. Developed under a published technical standard, the NP GUID ensures secure, ethical data sharing while protecting personal identity — enabling broader collaboration worldwide.

Access the tool here: [NP GUID Tool](#)

Thank you to all who continue to contribute, collaborate, and support the INPDR. With every step forward, we move closer to our shared goal: a better future for all individuals and families affected by Niemann-Pick diseases.

To learn more about the INPDR and how to get involved, please visit: www.inpdr.org