

# INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY (INPDR): Building Bridges for the future



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## BACKGROUND:

The INPDR, a Niemann-Pick disease-specific registry, is a not-for-profit registered company based in the UK, with an independent Board of Trustees and Scientific Advisory Committee.

## METHODS:

The INPDR was created by a collaboration of patient groups, health and research professionals working in the field of Niemann-Pick disease. It aims to improve standards of diagnosis, care, and treatment for the global patient community. It includes both clinical reported data and patient reported data for Acid Sphingomyelinase deficient (ASMD) Niemann-Pick disease, and Niemann-Pick disease type C.

## RESULTS:

- 7 European countries are entering clinical data. Global roll-out is underway, supported by a robust Recruitment Plan encouraging further participation.
- Patient reported data has been entered in 9 countries; this allows patients to self-enrol in the INPDR and contribute to progress, independent of clinician input. This is an important option in countries where clinical data entry is yet to go live.
- A Communications Plan supports and consolidates recruitment and sustainability; identifying stakeholders, evaluating communications tools, raising the Registry profile, and encouraging two-way communication, emphasising the patient experience.

Continued development will provide a sustainable patient-led database of at least 1000 patients.

## IT WILL:

- Contain comprehensive natural history data, leading to potential genotype/ phenotype correlations
- Identify patients eligible for clinical trials
- Support post-marketing surveillance and therapy development
- Be a valuable research tool
- Support regulatory interaction between national/supranational medicines agencies and pharmaceutical companies
- Support a collaborative network that delivers objectives in a professional, efficient way

## DISCUSSION:

The INPDR is a regulatory compliant registry with appropriate security, management and controls, which will act as a model for all rare diseases, and obviates the need for multiple registries.

