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REGISTERED COMPANY NUMBER: 10646129 (England and Wales)
REGISTERED CHARITY NUMBER: 1175311

INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY
(A COMPANY LIMITED BY GUARANTEE)

Report of the Trustees and

Financial Statements for the Year Ended 31 March 2025

**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY**

**Contents of the Financial Statements
for the Year Ended 31 March 2025**

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**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY**

**Reference and Administrative Details
for the Year Ended 31 March 2025**

Trustees	J E Green T Hiwot Mrs T A Mathieson Dr A Dardis Dr M Vanier
Company secretary	Mrs T A Mathieson
Registered office	Suite 2e North Sands Business Centre Liberty Way Sunderland Tyne and Wear SR6 0QA
Registered company number	10646129 (England and Wales)
Registered charity number	1175311
Independent examiner	James Anderson & Co Chartered Accountants Pentland Estate Straiton Edinburgh EH20 9QH
Chief executive officer	Conan Donnelly

**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY (REGISTERED NUMBER: 10646129)**

**Report of the Trustees
for the Year Ended 31 March 2025**

The trustees who are also directors of the charity for the purposes of the Companies Act 2006, present their report with the financial statements of the charity for the year ended 31 March 2025. The trustees have adopted the provisions of Accounting and Reporting by Charities: Statement of Recommended Practice applicable to charities preparing their accounts in accordance with the Financial Reporting Standard applicable in the UK and Republic of Ireland (FRS 102) (effective 1 January 2019).

Objectives and activities

Background

The International Niemann-Pick Disease Registry (INPDR) is an independent, not-for-profit charitable company registered in the UK. Created by a collaboration of patients, health professionals and medical researchers, it offers a single, disease-specific global registry data resource that includes both clinical data and patient reported data for Acid Sphingomyelinase Deficiency (ASMD) Niemann-Pick disease, and Niemann-Pick disease type C.

Due to its rarity, registration for Niemann-Pick diseases is particularly important. Registration can provide sufficient data to understand its complex natural history, support accelerated recruitment to trials and facilitate the development and monitoring of care standards.

Mission, Vision and Principles

The mission of the INPDR is to document the Niemann-Pick patient experience; to improve diagnosis, treatment and care, to advance research and ultimately, to improve health outcomes for all those affected.

Our vision is the creation and ongoing development of a comprehensive, international data resource, specific to Niemann-Pick diseases which benefits patients by increasing understanding of these rare conditions, encourages efficient and timely diagnosis, enables progress in research and clinical trials, and facilitates the development of therapeutic interventions.

INPDR advances our vision by adhering to the following principles.

- o We respect and protect patient privacy, and build trust among our patient community.
- o We strive for excellence through continuous improvement, professionalism and expert support.
- o We operate to the highest levels of scientific integrity and promote high-quality research.
- o We are transparent in communications and take responsibility for our actions and decisions.
- o We foster an environment of collaboration to improve outcomes in Niemann-Pick diseases.
- o We embrace innovation and change as appropriate to our needs.
- o We shall remain independent of advocacy, policy and commercial interests and let the data tell the story of Niemann-Pick diseases.

A key strength of the INPDR is the collaborative relationships developed from the outset and enabling the powerful combination of patient leadership and professional implementation. This ensures the legitimacy of the patient perspective and ensures that it is at the heart of our organisation and mission.

**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY (REGISTERED NUMBER: 10646129)**

**Report of the Trustees
for the Year Ended 31 March 2025**

The INPDR will work to enhance and facilitate progress through the following aims and objectives:

Aims

- o To build a high quality, complete International population-based Niemann-Pick Disease Registry
- o Protect patient privacy and ensure compliance with data protection and the highest clinical standards
- o To facilitate clinical research through data sharing and provision of analytical services
- o Provide evidence to facilitate advocacy and inform public debate

Objectives

- o To describe the epidemiology and natural history of ASMD Niemann-Pick disease and Niemann-Pick Type C
- o To support development of standards of diagnosis, clinical management and quality of care
- o To establish genotype-phenotype correlations
- o To provide support for equal access to biochemical and genetic testing
- o To facilitate accelerated recruitment to interventional studies
- o To provide a regulatory compliant registry to satisfy post-marketing obligations
- o To support regulatory interaction between national/supranational medicines agencies and pharmaceutical companies
- o To accelerate progress in understanding NPD, by encouraging education of health professionals and researchers

The INPDR developed and maintains a regulatory-compliant, secure database of consenting patients, with appropriate security, management, and controls. A secure web-based electronic data capture (EDC) platform is used for the systematic and uniform collection of clinical data, patient-reported data and caregiver-reported data of patients diagnosed with either Acid Sphingomyelinase Deficiency (ASMD) or Niemann-Pick Type C (NPC). The active participation of clinicians, patients and caregivers from around the world facilitates the collection of demographic information, diagnostic results, treatment, and health outcomes data. The INPDR is a long-term initiative that will continue to grow and to create mechanisms to ensure that the data collected works hardest to serve patient needs.

Significant activities

In the last year we worked to continue improving INPDR's impact by increasing global recruitment, supporting data collection, enhancing data quality activities and facilitating and supporting meaningful research. All activities undertaken in the reporting period moved the charity forward in its mission to advance research and improve health outcomes in Niemann-Pick diseases.

Clinical Data Collection

24 medical centres were included in the Clinician Reported Database (CRD) to collect clinical data from 488 patients in 2024/25, consisting of 120 patients with ASMD and 368 with NPC). As well as maintaining collaborations with centres in North America, South America and Europe, new centres in the Middle East and Brazil initiated data collection. 46 new sites have been approached to collaborate, with discussions in progress.

Patient Reported Data

The Patient Reported Database (PRD) had a total of 158 patients and caregivers enrolled in the 2024/25 period. Questionnaire responses were sought from participants, with 83 quality of life surveys and 101 symptom, severity and unmet need assessment returns obtained. Between December 2024 and March 2025, direct promotion of the PRD was paused to support a UK patient organisation research initiative, a comprehensive patient and caregiver survey (see 'Real World Experience of Niemann-Pick disease Type C Patients and Caregivers' below). The decision was made to encourage participation in the research initiative. The survey project supported the PRD by collating contact details of participants who were interested in joining the PRD.

The PRD is currently in English, but a multi-language solution is in development for 2025. Data quality monitoring was strengthened in the US and UK, supported by the Ara Parseghian Medical Research Fund, with visits to clinical sites to ensure data accuracy. INPDR also achieved ISO9001 accreditation for Quality Management Systems, confirming its strong registry and research support capabilities.

**Report of the Trustees
for the Year Ended 31 March 2025**

Objectives and activities

Anonymised information sharing

To meet the aim of facilitating clinical research through data sharing, INPDR has worked with researchers to submit requests for anonymised registry data. International applicants of differing areas of expertise and backgrounds have proposed research projects which explored differing aspects of Niemann-Pick, including disease mechanisms, movement disorders, patient and caregiver reported experiences, and disease management and progression.

Publications, Reports and Posters

INPDR was involved in reports and publications including, a 2024 publication in Orphanet Journal of Rare Diseases that explored the real-life impacts of olipudase alfa on ASMD patients and their families. Continuing work previously undertaken, INPDR published a second quantitative and qualitative report on the impact of olipudase alfa on adults with ASMD, with contributions from the INPDA, NNPDF and NPUK.

INPDR also produced and contributed to several conference presentations, including at the annual WORLD Symposium in February 2025, where three poster abstracts were accepted and presented. Additionally, presentations were delivered to multiple patient community meetings in Europe, North America and Australia to disseminate research findings and provide audience updates of INPDR progress.

Public benefit

In shaping our objectives for the year and planning our activities, the trustees have considered the Charity Commission's guidance on public benefit, including the guidance 'public benefit : running a charity (PB2)'.

Achievements and performance

Charitable activities

Funding in the form of unrestricted educational grants from industry stakeholders enabled the charity to carry out its activities. In addition, INPDR received reimbursement for the services it provided to the INPDR Gateway.. Further funding came from the Ara Parseghian Medical Research Fund and the National Lottery Community Fund.

We have continued to maintain a strong presence in the field of research and have collaborated on a global basis with other rare disease organisations, research and academic institutions, regulators, and industry to progress and further develop the registry, and to facilitate research and clinical development.

We continue to sustain appropriate relationships with the pharmaceutical industry, particularly those engaged in clinical programmes and activities in the field of Niemann-Pick diseases and to work closely with the global patient advocacy leaders supporting the Niemann-Pick community. This includes the International Niemann-Pick Disease Alliance, whose representatives are appointed to the INPDR Board of Trustees and Scientific Advisory Committee.

We continued to engage with the global Niemann-Pick community through virtual and in-person conferences and focus groups.

Report of the Trustees
for the Year Ended 31 March 2025

Achievements and performance

During the 2024/25 reporting period, we have worked to strengthen INPDR's impact and foster positive progress in Niemann-Pick research through the following achievements and performance:

- The number of participating clinical sites increased to 24 across Europe, North America and South America. Through INPDR's collaboration with these sites, 488 patient records were collected since the launch of the CRD, formed of 120 ASMD and 368 NPC records.
- 46 new clinical sites from across the globe, including Europe, North America, South America, Australia, Asia and Africa engaged with INPDR to initiate a collaboration to collect patient records. This laid the foundation for significant expansion of the INPDR's footprint, enabling representation from regions underserved by clinical research opportunities.
- The Patient Reported Database grew to include 158 patients and caregivers, with an acceptable number of questionnaire and survey responses obtained. A temporary halt on PRD promotion was imposed between December 2024 and March 2025 to support a patient organisation research initiative, which may have impacted on recruitment rates and survey responses. As the research initiative was designed to collect the contact details of participants interested in the PRD, we anticipate this will result in future PRD participant recruitment.
- While the PRD is currently available in English language, work has progressed significantly with our technology partner, OpenApp, to develop a multiple-language solution with the expectation that this will be rolled out in 2026.
- A key update to the PRD was the introduction of functionality allowing caregiver participants to indicate their loved one have passed away since enrolment. This ends the caregiver's study participation and prevents potentially distressing emails requesting new surveys to be completed from being sent to the caregiver.
- During the reporting period, INPDR's Clinical Research Associates (CRA) undertook several visits of CRD clinical sites to undertake data quality exercises, thereby supporting the accuracy and completeness of collected data. These visits also provided an opportunity to maintain and strengthen relationships with personnel at the clinical sites and allows for feedback and engagement from one of INPDR's key collaborator sector.
- Additionally, INPDR's CRA's supported the development of core data quality processes, with input from INPDR's Chief Operating Officer (COO). This included refining key data quality process documentation, and the development of a comprehensive Key Performance Indicator (KPI) dashboard, created by our Health IT partner.
- The INPDR maintained its ISO9001 accreditation for Quality Management Systems by the British Standards Institute during this period for all areas related to data collection and management. This accreditation demonstrates the INPDR has robust system for managing the registry and supporting clinical research.
- Changes to INPDR staffing occurred during 2024/25. Two new staff members joined INPDR: a full-time Chief Operating Officer (COO), who supports core registry operations and activities, and a part-time Communications Consultant who develops and delivers INPDR's communication approach to support the promotion of INPDR activity and progress to the patient community and stakeholders. The Chief Executive Officer (CEO) resigned and left INPDR as of 28th February 2025. In the CEO's absence, the COO, with the support of the Board of Trustees, has been maintaining business continuity. A replacement CEO will be sought during 2026.
- To support achieving the aim of facilitating research, INPDR have support several applications for anonymised research data to facilitate research projects. These are summarised below.

Research Studies

- St Mary's Hospital Manchester, UK, "Defining the seizure burden in Niemann Pick C."
- Aston University, UK, "Quality of life (QoL) in patients with Niemann-Pick Disease Type C (NPC) and their carers."
- University College London, "Disease progression in early and late infantile Niemann-Pick."
- University of Oxford, UK, "The manifestations and management of inflammatory bowel disease in Niemann-Pick disease type C (NPC) patients."
- University of Oxford, "Incorporation of ASIS into the INPDR for NPC cases."
- Hospital for Sick Children, Toronto, Canada, "Phenomenology of movement disorders in Niemann-Pick-Disease Type C (NPC): Findings from the International Niemann-Pick-Disease Registry (INPDR)"
- University of Oxford, "3D-Modelling of NPC1 mutations as a tool for dissecting disease mechanisms and phenotypic heterogeneity"

Report of the Trustees
for the Year Ended 31 March 2025

Achievements and performance

Conference Poster Presentations

- Development of a novel patient-reported outcome (PRO) measure for acid sphingomyelinase deficiency according to the United States Food and Drug Administration (USFDA) guidance - WORLDSymposium 2025, San Diego, United States of America
- A Multi-Disciplinary Collaboration to Investigate the Potential Contexts of Using SleepBased DHTs in Clinical Research and Practice - WORLDSymposium 2025, San Diego, United States of America
- Investigating the Real-World Experience of Patients with Niemann-Pick Disease Type C (NPC) and Their Carers - WORLDSymposium 2025, San Diego, United States of America

Reports

- The impact of treatment with olipudase alfa on adult patients with ASMD - Part 2 - Qualitative Report. Rare Disease Research Partners.

Communication and engagement

INPDR recruited a Communication Consultant to support the promotion and visibility of the work undertaken by the charity through the development of a communication strategy and stakeholder engagement plan. This has allowed INPDR to have a greater presence on social media channels worldwide, allowing communications to reach a global audience.

Reflecting the international scope of work, INPDR presented research findings and updates to national and global conferences, including patient community meetings:

- Niemann-Pick UK, UK
- Vaincre Les Maladies Lysosomales, France
- ASMD Espana, Segovia, Spain
- WORLD (We're Organising Research on Lysosomal Disease) Symposium, Florida, USA
- National Niemann-Pick Disease Foundation Conference, Florida, USA
- Ara Parseghian Research Conference, Arizona, USA

These conferences not only provided an opportunity to showcase the work of the INPDR but also facilitated direct engagement with patient community members and stakeholders working in Niemann-Pick diseases. This engagement, in turn, increased INPDR's visibility and performance in those countries.

Financial review

Financial position

Unrestricted income in the year was £371,210 (£2024 £39,143) and unrestricted expenditure was £273,031 (2024 £183,249).

A restricted grant totalling £76,611 (2024 £61,321) was awarded by the University of Notre Dame, through its Ara Parseghian Medical Research Fund and Michael, Marcia, and Christa Parseghian Endowment for Excellence in Niemann Pick Type C Research. The funding received has enabled the continuing development of the Registry. Alongside the high level of support and interaction from the patient and clinical communities, demonstrates the potential of the Registry to make a positive difference for the global Niemann-Pick patient community.

Reserves policy

There were free reserves of £11,387 on 31 March 2025 (2024 -£7,429) The board aim is to hold six months running costs in reserve which would be £136,515. Further as the registry activities grow a higher level of reserve will be required.

Sustainability & INPDR Gateway Ltd

A sustainability plan has been introduced with the aim of diversifying income and providing financial stability. This includes a commercial subscription model, a costing model for the non-commercial sectors plus a trust and corporate grant strategy.

To support the commercial subscription, INPDR Gateway Ltd was established. INPDR Gateway is a non-profit commercial company established to support and facilitate appropriate engagement between the International Niemann-Pick Disease Registry (INPDR) and industry partners. Its primary purpose is to provide a transparent, structured, and compliant mechanism through which industry can engage with registry-related activities, while protecting the independence, governance, and patient-centred mission of the INPDR.

**Report of the Trustees
for the Year Ended 31 March 2025**

Financial review

INPDR Gateway enables industry partners to collaborate with the registry on activities such as feasibility assessments, data enquiries, and research support, in a manner that is consistent with ethical, legal, and data-protection requirements. By operating as a separate legal entity, the Gateway allows commercial interactions to be managed appropriately, ensuring clear separation between charitable registry activities and commercial services.

Importantly, INPDR Gateway does not compromise patient confidentiality or registry independence. All data access is governed by robust policies, with oversight from the INPDR governance structures, and no identifiable patient data are shared without appropriate approvals and consent.

Through this model, INPDR Gateway supports the long-term sustainability of the registry, facilitates responsible industry engagement, and helps accelerate research and therapy development for Niemann-Pick diseases, while maintaining trust with patients, families, and the wider community. This financial year, INPDR Gateway contributed £370,000 (£20,000 in 2024) to INPDR in reimbursement for services provided, maintaining the INPDR and enabling further development in line with priorities.

Future plans

The INPDR has established a solid foundation to achieve its aims and objectives through the development of its clinician-reported and patient-reported databases which encompasses its registry system. Through data collected within the registry system, high quality data can be leveraged to enable impactful clinical research for Niemann-Pick diseases for the benefit of patients and families globally. Our priority for the coming year is to strengthen this foundation further to increase INPDR's impact

For 2025/26, a strategic and operational plan has been defined, defining a roadmap of work that will further enhance the utility of INPDR's registry data. Core work streams include:

1. Research

INPDR will pursue a number of research opportunities, both internally initiated and in collaboration with partners, to generate new knowledge in Niemann-Pick diseases. While the primary focus will remain on leveraging registry data, INPDR will also support research projects that initially generate data outside the registry system, with plans to integrate those external datasets into INPDR's repository.

2. Global Development of CRD and PRD

Efforts will focus on increasing participant recruitment to both the Clinician Reported Database (CRD) and Patient Reported Database (PRD). For the CRD, enhanced support measures for clinical sites will be developed, and updates to the CRD IT system will be implemented to improve data utility. Materials will be produced to help patient community organisations expand CRD participation in their local territories, further enabling patient and caregiver involvement.

Currently, the PRD is available only in English. Work is scheduled to provide the PRD in six languages, enabling broader global participation. Complementing this, a PRD User Experience review will be conducted to identify improvements that enhance data utility and reduce barriers to participation.

3. Data Quality and Completeness

INPDR will strengthen registry data quality by improving its central data monitoring process to identify and correct data errors. A Key Performance Indicator (KPI) system will be developed and launched to allow for real-time monitoring and review of CRD data, thereby enabling the timely intervention of data errors. Regular data quality reports will be produced biannually to provide insights into data integrity. Ongoing efforts will focus on improving data completeness and accuracy through refining data points, source verification at clinical sites, and central data monitoring effort.

4. Communication

Materials to enable clear and meaningful communication outputs for INPDR's diverse stakeholder audience will be produced. Central to this will be the development of a 2025/26 Communication Plan and Strategy, which will define INPDR's approach in engaging with its stakeholders. A schedule of regular updates across all workstreams will be implemented, alongside targeted promotion of key outputs. INPDR will identify and attend major conferences and meetings to disseminate findings within the global Niemann-Pick community and foster collaborations.

Structure, governance and management

Status and Governing Document

INPDR is a company limited by guarantee governed by its Memorandum and Articles of Association. The company has one member, the International Niemann-Pick Disease Alliance.

INPDR is also registered as a charity with the Charity Commission.

**Report of the Trustees
for the Year Ended 31 March 2025**

Structure, governance and management

Recruitment and appointment of new trustees

Future Trustees shall be appointed by the Trustees from time to time following a nomination received from the Nominations Committee. Trustees are volunteers and each takes on responsibilities within the organisation to co-ordinate/support an aspect of its function.

Induction and training of new trustees

New Trustees are briefed on their legal obligations, the contents of the memorandum & articles of association, the board and decision making processes, the business plan and the recent financial performance of the charity. During the induction, they are mentored by other Trustees.

Organisational Structure

The Trustees are directly responsible for the effective governance of the organisation in accordance with statutory guidance and legislation as provided by the Charity Commissioners and Companies House. The Trustees meet quarterly with additional contact as required, in order to review reports and to carry out management and financial reviews.

Trustees follow a code of conduct, which provides clear guidelines as to the standards of behaviour, responsibilities, and best practice expected of those involved with the INPDR.

The Trustees delegate the day-to-day management of the organisation to the Registry Management Team, led by the Chief Executive Officer, who reports regularly to the Board at quarterly virtual meetings.

Key management remuneration

All trustees give of their time freely and no trustee received remuneration in the year. The board is responsible for setting employee salary levels. All salaries are benchmarked with similar roles in the voluntary sector, not with the public or private sectors. The size of the organisation is also considered.

Wider network

The charity's member is the International Niemann-Pick Disease Alliance (INPDA) which is an alliance of non-profit support organisations who are associated with the rare group of genetic diseases known collectively as Niemann-Pick Diseases. The Alliance provides a forum where, through the exchange of information, experience and knowledge, global progress can be accelerated

Report of the Trustees
for the Year Ended 31 March 2025

Structure, governance and management

Risk management

As part of the Quality Management System, which has ISO9001 accreditation, the INPDR maintains a risks and issues log. Risks are managed on an ongoing basis by the Registry Management Team and reviewed by Board Members at a quarterly Management Review Meeting.

The registry operates in a highly regulated clinical research environment and must ensure compliance with data protection law and good clinical practice. INPDR operates in compliance with the European Medicine Agency (EMA) Guidelines on the Management of Patient Registries and the registry has received a letter of support from EMA as a resource for clinical research.

To manage risks related to data protection and ensure the privacy of registry participants, INPDR employs the services of a consultant Data Protection Officer. In addition, during the year, our technology partner, OpenApp was awarded ISO27001 accreditation for data security, providing further confidence that the INPDR are well-placed to ensure patient privacy.

With regard to data protection, there were no adverse events, incidents or breaches reported during 2024/25 and no data subject rights requests.

Principal Risks and Mitigation - INPDR

The principal risks anticipated in the foreseeable future, together with mitigating actions, include:

- Sustainability and funding risk

Risk: Limited and uncertain long-term funding may affect the ongoing operation, maintenance, and development of the registry.

Mitigation: INPDR actively seeks diversified funding sources, including grants, partnerships, and collaborative projects, and prioritises activities that maintain core registry functionality and data quality.

- Patient enrolment and geographic representation

Risk: Insufficient patient enrolment or uneven geographic representation may limit the robustness, generalisability, and utility of registry data.

Mitigation: INPDR works closely with patient organisations, clinicians, and advocacy networks (including INPDA members) to encourage enrolment, raise awareness of the registry, and support participation across diverse regions.

- Data quality, completeness, and consistency

Risk: Variability in data entry, incomplete datasets, or inconsistent reporting may reduce the scientific and clinical value of registry outputs.

Mitigation: INPDR applies defined data standards, provides guidance and training to participating sites, and undertakes regular data quality monitoring and validation.

- Data protection, privacy, and cybersecurity

Risk: Breach of data protection regulations or cybersecurity incidents could compromise sensitive patient data and damage trust.

Mitigation: INPDR operates in compliance with relevant data protection regulations (including GDPR), employs robust data security measures, and regularly reviews governance, consent processes, and technical safeguards.

- Dependence on clinician and site engagement

Risk: Reliance on clinician participation and site engagement may affect data submission rates and long-term sustainability.

Mitigation: INPDR maintains active engagement with clinical sites, demonstrates the value of registry participation through outputs and feedback, and seeks to reduce administrative burden where possible.

- Regulatory and ethical complexity

Risk: Variability in national regulatory and ethical requirements may slow site onboarding or limit participation in certain jurisdictions.

Mitigation: INPDR provides support to sites navigating local approvals and adapts processes where feasible to accommodate differing regulatory environments.

- Governance and operational capacity

Risk: Limited operational capacity may constrain registry development, responsiveness to research requests, or expansion of scope.

Mitigation: INPDR maintains clear governance structures, including Board and Scientific Advisory Committee oversight, and prioritises activities aligned with strategic objectives and available resources.

Additional Risks Considered

- Stakeholder alignment and expectations

Risk: Differing expectations among patients, clinicians, researchers, and industry partners may create challenges in prioritisation and data use.

Mitigation: INPDR applies transparent governance processes, clear data access policies, and inclusive stakeholder engagement to align expectations.

**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY (REGISTERED NUMBER: 10646129)**

**Report of the Trustees
for the Year Ended 31 March 2025**

Structure, governance and management

- Reputational risk

Risk: As a trusted source of disease-specific data, INPDR faces reputational risk related to data use, partnerships, or perceived conflicts of interest.

Mitigation: INPDR maintains independence, applies rigorous review of data access requests, and ensures transparency in partnerships and communications.

Recognising the contribution of volunteers

We are extremely grateful to those who give their time as volunteers in support of the INPDR. Without their dedicated and continued support, we would not be able to achieve all that is required on behalf of our organisation and community

This report has been prepared in accordance with the special provisions of Part 15 of the Companies Act 2006 relating to small companies.

Approved by order of the board of trustees on 19 December 2025 and signed on its behalf by:

J E Green - Trustee

**Independent Examiner's Report to the Trustees of
International Niemann-Pick Disease
Registry**

Independent examiner's report to the trustees of International Niemann-Pick Disease Registry ('the Company')
I report to the charity trustees on my examination of the accounts of the Company for the year ended 31 March 2025.

Responsibilities and basis of report

As the charity's trustees of the Company (and also its directors for the purposes of company law) you are responsible for the preparation of the accounts in accordance with the requirements of the Companies Act 2006 ('the 2006 Act').

Having satisfied myself that the accounts of the Company are not required to be audited under Part 16 of the 2006 Act and are eligible for independent examination, I report in respect of my examination of your charity's accounts as carried out under Section 145 of the Charities Act 2011 ('the 2011 Act'). In carrying out my examination I have followed the Directions given by the Charity Commission under Section 145(5) (b) of the 2011 Act.

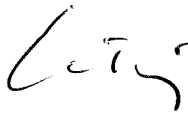
Independent examiner's statement

Since your charity's gross income exceeded £250,000 your examiner must be a member of a listed body. I can confirm that I am qualified to undertake the examination because I am a member of the Institute of Chartered Accountants of Scotland, which is one of the listed bodies.

I have completed my examination. I confirm that no matters have come to my attention in connection with the examination giving me cause to believe:

1. accounting records were not kept in respect of the Company as required by Section 386 of the 2006 Act; or
2. the accounts do not accord with those records; or
3. the accounts do not comply with the accounting requirements of Section 396 of the 2006 Act other than any requirement that the accounts give a true and fair view which is not a matter considered as part of an independent examination; or
4. the accounts have not been prepared in accordance with the methods and principles of the Statement of Recommended Practice for accounting and reporting by charities (applicable to charities preparing their accounts in accordance with the Financial Reporting Standard applicable in the UK and Republic of Ireland (FRS 102)).

I have no concerns and have come across no other matters in connection with the examination to which attention should be drawn in this report in order to enable a proper understanding of the accounts to be reached.



Christopher T Spalding
The Institute of Chartered Accountants of Scotland

James Anderson & Co
Chartered Accountants
Pentland Estate
Straiton
Edinburgh
EH20 9QH

19 December 2025

**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY**

**Statement of Financial Activities
(Incorporating an Income and Expenditure Account)
for the Year Ended 31 March 2025**

		Unrestricted fund £	Restricted funds £	2025 Total funds £	2024 Total funds £
	Notes				
Income and endowments from					
Donations and legacies	2	-	76,611	76,611	92,875
Charitable activities	3				
Charitable activities		371,210	-	371,210	38,623
Total		<u>371,210</u>	<u>76,611</u>	<u>447,821</u>	<u>131,498</u>
Expenditure on					
Charitable activities	4				
Charitable activities		<u>273,031</u>	<u>95,427</u>	<u>368,458</u>	<u>307,686</u>
NET INCOME/(EXPENDITURE)		98,179	(18,816)	79,363	(176,188)
Reconciliation of funds					
Total funds brought forward		(7,429)	18,816	11,387	187,575
Total funds carried forward		<u><u>90,750</u></u>	<u><u>-</u></u>	<u><u>90,750</u></u>	<u><u>11,387</u></u>

The notes form part of these financial statements

INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY (REGISTERED NUMBER: 10646129)

Balance Sheet
31 March 2025

	Notes	2025 £	2024 £
Fixed assets			
Investments	9	1	1
Current assets			
Debtors	10	56,111	22
Cash at bank		68,007	25,363
		<u>124,118</u>	<u>25,385</u>
Creditors			
Amounts falling due within one year	11	(33,369)	(13,999)
Net current assets		<u>90,749</u>	<u>11,386</u>
Total assets less current liabilities		<u>90,750</u>	<u>11,387</u>
NET ASSETS		<u>90,750</u>	<u>11,387</u>
Funds	13		
Unrestricted funds		90,750	(7,429)
Restricted funds		-	18,816
Total funds		<u>90,750</u>	<u>11,387</u>

The charitable company is entitled to exemption from audit under Section 477 of the Companies Act 2006 for the year ended 31 March 2025.

The members have not required the company to obtain an audit of its financial statements for the year ended 31 March 2025 in accordance with Section 476 of the Companies Act 2006.

The trustees acknowledge their responsibilities for

- (a) ensuring that the charitable company keeps accounting records that comply with Sections 386 and 387 of the Companies Act 2006 and
- (b) preparing financial statements which give a true and fair view of the state of affairs of the charitable company as at the end of each financial year and of its surplus or deficit for each financial year in accordance with the requirements of Sections 394 and 395 and which otherwise comply with the requirements of the Companies Act 2006 relating to financial statements, so far as applicable to the charitable company.

These financial statements have been prepared in accordance with the provisions applicable to charitable companies subject to the small companies regime.

The financial statements were approved by the Board of Trustees and authorised for issue on 19 December 2025 and were signed on its behalf by:

J E Green - Trustee

1. **Accounting policies**

Basis of preparing the financial statements

The financial statements of the charitable company, which is a public benefit entity under FRS 102, have been prepared in accordance with the Charities SORP (FRS 102) 'Accounting and Reporting by Charities: Statement of Recommended Practice applicable to charities preparing their accounts in accordance with the Financial Reporting Standard applicable in the UK and Republic of Ireland (FRS 102) (effective 1 January 2019)', Financial Reporting Standard 102 'The Financial Reporting Standard applicable in the UK and Republic of Ireland' and the Companies Act 2006. The financial statements have been prepared under the historical cost convention, with the exception of investments which are included at market value.

International Niemann-Pick Disease Registry meets the definition of a public benefit entity under FRS 102.

Income

All income is recognised once the charity has entitlement to the income, there is sufficient certainty of receipt and so it is probable that the income will be received, and the amount of income receivable can be measured reliably.

Donations and grants are recognised when they have been communicated in writing with notification of both the amount and settlement date. In the event that a donation or grant is subject to conditions that require a level of performance before the charity is entitled to the funds, the income is deferred and not recognised until either those conditions are fully met, or the fulfilment of those conditions is wholly within the control of the charity and it is probable that those conditions will be fulfilled in the reporting period.

Expenditure

Expenditure is recognised as soon as there is a legal or constructive obligation committing the charity to that expenditure, it is probable that a transfer of economic benefits will be required in settlement and the amount of the obligation can be measured reliably.

All expenditure is accounted for on an accruals basis. All expenses, including support costs and governance costs, are allocated or apportioned to the applicable expenditure headings in the statement of financial activities.

Expenditure on charitable activities includes any VAT which cannot be recovered and is reported as part of the expenditure to which it relates and comprises those costs incurred by the charity in the delivery of its activities and services for its beneficiaries. It includes both costs that can be allocated directly to such activities and those costs of an indirect nature necessary to support them.

Taxation

The charity is exempt from corporation tax on its charitable activities.

Fund accounting

Unrestricted funds can be used in accordance with the charitable objectives at the discretion of the trustees.

Restricted funds can only be used for particular restricted purposes within the objects of the charity. Restrictions arise when specified by the donor or when funds are raised for particular restricted purposes.

Further explanation of the nature and purpose of each fund is included in the notes to the financial statements.

Pension costs

The company operates a defined contribution scheme, the assets of which are held separately from those of the charity. The pension cost charge represents contributions payable by the charity to the scheme.

Investments

The investment in the group undertaking is measured at cost less impairment on the basis that it represents shares in an entity that is not publicly traded and the fair value cannot otherwise be measured reliably.

Debtors and creditors receivable / payable within one year

Debtors and creditors with no stated interest rate and receivable or payable within one year are recorded at transaction price. Any losses arising from impairment are recognised in expenditure.

Cash in bank and in hand

Cash at bank and cash in hand includes cash and short term highly liquid investments.

INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY

Notes to the Financial Statements - continued
for the Year Ended 31 March 2025

1. Accounting policies - continued

Going concern

The financial statements have been prepared on a going concern basis as the trustees believe that no material uncertainties exist. The trustees have considered the level of funds held and the expected level of income and expenditure for 12 months from authorising these financial statements. The budgeted income and expenditure is sufficient with the level of reserves for the charity to be able to continue as a going concern.

2. Donations and legacies

	Unrestricted funds £	Restricted funds £	2025 Total funds £	2024 Total funds £
Donations	-	-	-	520
Grants	-	76,611	76,611	92,355
	-	76,611	76,611	92,875

Grants received, included in the above, are as follows:

	2025 £	2024 £
Sanofi Genzyme	-	11,534
University of Notre Dame du Lac	76,611	61,321
National Lottery Community Fund	-	19,500
	76,611	92,355

3. Income from charitable activities

	Activity	2025 £	2024 £
Contribution to Research	Charitable activities	-	18,459
Recharge to INPDR Gateway Ltd	Charitable activities	370,000	20,000
Third party reimbursement	Charitable activities	1,210	164
		371,210	38,623

4. Charitable activities costs

	Direct Costs (see note 5) £
Charitable activities	368,458

**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY**

**Notes to the Financial Statements - continued
for the Year Ended 31 March 2025**

5. Direct costs of charitable activities

	2025	2024
	£	£
Staff costs	171,022	129,563
Sundries	5	-
Equipment costs	1,860	-
Registry hosting	48,663	21,740
Travel & meetings	21,349	29,722
Website costs	1,295	1,027
Other staff costs	1,947	-
Office expenses	9,011	9,520
Promotional expenses	-	5,826
Executive meetings	3,413	2,424
Independent examiner's fee	1,512	1,974
Other regulatory costs	4,606	3,713
Consultancy	75,840	71,669
Research	10,749	20,068
HIC maintenance & support	11,520	10,440
Patient Facing Activities	5,643	-
INPDR Gateway	23	-
	<u>368,458</u>	<u>307,686</u>

6. Trustees' remuneration and benefits

There were no trustees' remuneration or other benefits for the period ended 31 March 2025 (2024 - nil)

Trustees' expenses

During the period one trustee was reimbursed £140 (2024 £583) for travel expenses. (2024 - 1)

7. Staff costs

	2025	2024
	£	£
Wages and salaries	159,320	121,223
Social security costs	9,022	5,993
Other pension costs	2,680	2,347
	<u>171,022</u>	<u>129,563</u>

The average monthly number of employees during the year was as follows:

	2025	2024
Coordination and administration	3	2
Registry Manager	1	1
	<u>4</u>	<u>3</u>

The Chief Executive Officer is key management and his total employee benefits (including NI and Pension) were £60,465. (2024 £65,455)

INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY

Notes to the Financial Statements - continued
for the Year Ended 31 March 2025

8. Comparatives for the statement of financial activities

	Unrestricted fund £	Restricted funds £	Total funds £
Income and endowments from			
Donations and legacies	520	92,355	92,875
Charitable activities			
Charitable activities	38,623	-	38,623
Total	<u>39,143</u>	<u>92,355</u>	<u>131,498</u>
Expenditure on			
Charitable activities			
Charitable activities	183,249	124,437	307,686
NET INCOME/(EXPENDITURE)	(144,106)	(32,082)	(176,188)
Reconciliation of funds			
Total funds brought forward	136,677	50,898	187,575
Total funds carried forward	<u>(7,429)</u>	<u>18,816</u>	<u>11,387</u>

9. Fixed asset investments

	Shares in group undertakings £
Market value	
At 1 April 2024 and 31 March 2025	<u>1</u>
Net book value	
At 31 March 2025	<u>1</u>
At 31 March 2024	<u>1</u>

There were no investment assets outside the UK.

The company's investments at the balance sheet date in the share capital of companies include the following:

INPDR Gateway Ltd

Registered office: United Kingdom 12706306
Nature of business: Data provider for research

Class of share:	% holding	2025 £	2024 £
1	100		
Aggregate capital and reserves		(42,247)	(49,446)
Profit/(loss) for the year		<u>6,563</u>	<u>(52,160)</u>

INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY

Notes to the Financial Statements - continued
for the Year Ended 31 March 2025

10. Debtors: amounts falling due within one year

	2025 £	2024 £
Trade debtors	55,000	22
Prepayments	1,111	-
	<u>56,111</u>	<u>22</u>

11. Creditors: amounts falling due within one year

	2025 £	2024 £
Social security and other taxes	2,421	2,854
Accruals and deferred income	30,948	11,145
	<u>33,369</u>	<u>13,999</u>

12. Analysis of net assets between funds

	Unrestricted fund £	Restricted funds £	2025 Total funds £	2024 Total funds £
Investments	1	-	1	1
Current assets	124,118	-	124,118	25,385
Current liabilities	(33,369)	-	(33,369)	(13,999)
	<u>90,750</u>	<u>-</u>	<u>90,750</u>	<u>11,387</u>

13. Movement in funds

	At 1/4/24 £	Net movement in funds £	At 31/3/25 £
Unrestricted funds			
General fund	(7,429)	98,179	90,750
Restricted funds			
APMRF	534	(534)	-
National Lottery Community Fund	18,282	(18,282)	-
	<u>18,816</u>	<u>(18,816)</u>	<u>-</u>
TOTAL FUNDS	<u>11,387</u>	<u>79,363</u>	<u>90,750</u>

Net movement in funds, included in the above are as follows:

	Incoming resources £	Resources expended £	Movement in funds £
Unrestricted funds			
General fund	371,210	(273,031)	98,179
Restricted funds			
APMRF	76,611	(77,145)	(534)
National Lottery Community Fund	-	(18,282)	(18,282)
	<u>76,611</u>	<u>(95,427)</u>	<u>(18,816)</u>
TOTAL FUNDS	<u>447,821</u>	<u>(368,458)</u>	<u>79,363</u>

INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY

Notes to the Financial Statements - continued
for the Year Ended 31 March 2025

13. Movement in funds - continued

Comparatives for movement in funds

	At 1/4/23 £	Net movement in funds £	At 31/3/24 £
Unrestricted funds			
General fund	136,677	(144,106)	(7,429)
Restricted funds			
APMRF	50,898	(50,364)	534
National Lottery Community Fund	-	18,282	18,282
	<u>50,898</u>	<u>(32,082)</u>	<u>18,816</u>
TOTAL FUNDS	<u>187,575</u>	<u>(176,188)</u>	<u>11,387</u>

Comparative net movement in funds, included in the above are as follows:

	Incoming resources £	Resources expended £	Movement in funds £
Unrestricted funds			
General fund	39,143	(183,249)	(144,106)
Restricted funds			
APMRF	61,321	(111,685)	(50,364)
Sanofi	11,534	(11,534)	-
National Lottery Community Fund	19,500	(1,218)	18,282
	<u>92,355</u>	<u>(124,437)</u>	<u>(32,082)</u>
TOTAL FUNDS	<u>131,498</u>	<u>(307,686)</u>	<u>(176,188)</u>

The general fund is free to use in accordance with the objects of the charity.

The APMRF fund is to accelerate progress by facilitating registry recruitment in the USA.

The Sanofi fund was towards the INPDR 2023 Forum

The National Lottery Community Fund is for telling the story of Niemann-Pick Diseases in the UK

14. Related party disclosures

The International Niemann-Pick Disease Alliance (INPDA) is the only member of the company. During the year costs were charged by INPDA to INPDR of £Nil (2024 £3,014). INPDA paid shared costs of £4,991 (2024 - £4,422). At 31 March 2025 there was a balance due to INPDA of £Nil (2024 £Nil).

Niemann-Pick UK (NPUK) is a member of the INPDR. During the year there were recharges for shared staff, travel and overheads etc by NPUK to INPDR of £40,047 (2024 £27,142). INPDR received income in the year from NPUK of £Nil (2024 - £7,981). At 31 March 2025 there was a balance due to NPUK of £3,560 (2024 £2,650).

The INPDR (Registry) fully owns a company, INPDR Gateway Ltd (Gateway), which carries out the commercial operations of the Registry. During the year a sum of £Nil (2024 £Nil) was lent by the Registry to the Gateway. In addition recharges and service charges were made to the Gateway in the year of £370,000 (2024 - £20,761). A sum of £55,000 was due from the Registry at 31 March 2025 (2024 due to £22).