INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

England & Wales · Charity number 1175311

Details

Status	Registered
Legal form	Charitable company
Company number	10646129
Registered	2017-10-23
Register	View on the Charity Commission register

Contact

Address	Suite 2e North Sands Business Centre Liberty Way Sunderland SR6 0QA
Phone	01914150693
Email	info@inpdr.org
Website	www.inpdr.org

Activities

Objects: THE ONLY OBJECT FOR WHICH THE CHARITY IS ESTABLISHED IS TO RELIEVE SICKNESS AMONGST INDIVIDUALS AFFECTED BY NIEMANN-PICK DISEASES, THROUGH FACILITATING THE ADVANCEMENT OF EDUCATION IN ALL MATTERS CONCERNING SUCH DISEASES.

Activities: The INPDR aims to increase understanding of Niemann-Pick Disease through the collection of patient data on a global scale. It will encourage efficient and timely diagnosis, improve understanding of disease progression and influence patient care, whilst supporting global research efforts, facilitating recruitment of clinical studies and supporting development/access to new and emerging therapies.

Classification

- **How:** Provides Services, Acts As An Umbrella Or Resource Body
- **What:** Education/training, The Advancement Of Health Or Saving Of Lives
- **Who:** Other Defined Groups

Geography

- Throughout England And Wales

Finances

Period end	Income	Expenditure	Assets	Employees
2025-03-31	£447,821	£368,458	-	-
2024-03-31	£131,498	£307,686	-	-
2023-03-31	£173,361	£415,907	-	-
2022-03-31	£456,982	£279,140	-	-
2021-03-31	£390,375	£265,255	-	-

Trustees

Name	Role	Appointed
JAMES EDWARD GREEN	Chair	2017-03-01
TARAKEGN HIWOT		2017-02-28
TONI MATHIESON		2017-03-01

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

England & Wales - Charity number 1175311

Accounts

Digitally Signed Document

Document ID: A053FAC71F12E6

Document Details

Filename:	INP003-Charity accounts (final)-CS-20251218-172546-752.PDF
Client of:	James Anderson & Co

Signature Details

Name:	Jim Green
Email:	Jim.Green@inpdrgateway.com
Date & Time:	19/12/2025 10:23:12 AM (GMT)
IP Address:	109.148.232.23
Signing Statement:	I approve these accounts on behalf of the board.

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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

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

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.

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, Soutth 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.

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.

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

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.

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.

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.

Oragnisational 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

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 safe-uards.
- 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 capacit y
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.

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

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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').

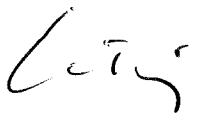
Having satisfied myself that the accounts of the Company are not required to be audited under Part 16 of the 2006 Act and are eligible for independent examination, I report in respect of my examination of your charity's accounts as carried out under Section 145 of the Charities Act 2011 ('the 2011 Act').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

	Notes	Unrestricted fund £	Restricted funds £	2025 Total funds £	2024 Total funds £
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		371,210	76,611	447,821	131,498
Expenditure on					
Charitable activities	4				
Charitable activities		273,031	95,427	368,458	307,686
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		90,750	-	90,750	11,387

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
		124,118	25,385
Creditors			
Amounts falling due within one year	11	(33,369)	(13,999)
Net current assets		90,749	11,386
Total assets less current liabilities		90,750	11,387
NET ASSETS		90,750	11,387
Funds	13		
Unrestricted funds		90,750	(7,429)
Restricted funds		-	18,816
Total funds		90,750	11,387

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

The notes form part of these financial statements

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.

continued...

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

continued...

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	-
	368,458	307,686

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
	171,022	129,563

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

	2025	2024
Coordination and administration	3	2
Registry Manager	1	1
	4	3

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

continued...

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	39,143	92,355	131,498
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	(7,429)	18,816	11,387

9. Fixed asset investments

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

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
1	100

	2025 £	2024 £
Aggregate capital and reserves	(42,247)	(49,446)
Profit/(loss) for the year	6,563	(52,160)

continued...

10. Debtors: amounts falling due within one year

	2025 £	2024 £
Trade debtors	55,000	22
Prepayments	1,111	-
	56,111	22

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
	33,369	13,999

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)
	90,750	-	90,750	11,387

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)	-
	18,816	(18,816)	-
TOTAL FUNDS	11,387	79,363	90,750

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)
	76,611	(95,427)	(18,816)
TOTAL FUNDS	447,821	(368,458)	79,363

continued...

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
	50,898	(32,082)	18,816
TOTAL FUNDS	187,575	(176,188)	11,387

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
	92,355	(124,437)	(32,082)
TOTAL FUNDS	131,498	(307,686)	(176,188)

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).

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

England & Wales - Charity number 1175311

Accounts

Digitally Signed Document

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Name:	Jim Green
Email:	Jim.Green@inpdrgateway.com
Date & Time:	24/12/2024 11:13:55 AM (GMT)
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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 2024

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Contents of the Financial Statements
for the Year Ended 31 March 2024

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Reference and Administrative Details
for the Year Ended 31 March 2024

Trustees	J E Green T Hiwot Mrs T A Mathieson Dr A Dardis Dr M Vanier
Co-opted Trustee - Ruben Giorgino	
Company secretary	Mrs T A Mathieson
Registered office	Suite 2 Vermont House Washington Tyne and Wear NE37 2SQ
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

Report of the Trustees
for the Year Ended 31 March 2024

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 2024. 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.

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

Background
The INPDR is 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 of patients diagnosed with either Acid Sphingomyelinase Deficiency (ASMD) or Niemann-Pick Type C (NPC). The active participation of clinicians and patients 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 have worked to implement the INPDR's global recruitment plan, to accelerate data collection, improve data quality assurance and to facilitate research with the ultimate goal of improving outcomes in Niemann-Pick diseases.

Clinical Data Collection

In 2023/24, INPDR collaborated with 22 clinical sites across Europe, North America, and South America to collect data on 450 patients (350 with NPC and 100 with ASMD) for clinical research. The registry is expanding with 44 new clinical sites in Europe, Australia, and the US.

Patient Reported Data

A revised patient-reported database (PRD) launched in 2023 saw 144 patients enrolled by March 31, 2024, marking a 40% growth. While growth has been slower than expected, improvements including promotional activities and user interface enhancements were initiated. By March 31, 2024, several questionnaires were completed, including 96 quality of life forms and 102 symptoms/severity assessments.

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.

Objectives and activities

Anonymised information sharing

INPDR supported multiple research projects focusing on various aspects of Niemann-Pick Disease, including disease progression, quality of life for patients and carers, seizure burden, and the management of associated conditions like inflammatory bowel disease.

Several anonymised data requests were processed, including genotype reports for the NPC population, an anonymized dataset was provided to C-Path, and a review of the All-Stripes database to assess its utility for a US-led sibling study was undertaken.

Publications, Reports and Posters

INPDR were also involved in reports and publications including, a 2024 publication in Orphanet Journal of Rare Diseases exploring the real-life impacts of olipudase alfa on ASMD patients and their families. In addition, INPDR published both quantitative and qualitative reports on the impact of olipudase alfa on adults with ASMD, with contributions from INPDA, NNPDF and NPUK.

INPDR also contributed to posters at several conferences including updates on Niemann-Pick Type C and its research at the WORLD Conference in February 2024, including findings on the impact of olipudase alfa on adults with ASMD and real-world study from the Early Access Program with Arimoclomol.

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)'.

Achievement and performance

Charitable activities

Grant funding in the form of unrestricted educational grants enabled the charity to carry out its activities. In addition, INPDR Gateway provided payment to INPDR to facilitate its services. Further funding came from the Ara Parseghian Medical Research 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 as INPDR Board and Scientific Advisory Committee Members.

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

Achievement and performance

Achievement and performance

In the last year we have worked to implement the INPDR's global recruitment plan, to accelerate data collection, improve data quality assurance and to facilitate research with the ultimate goal of improving outcomes in Niemann-Pick diseases.

- During 2023/24 INPDR worked with 22 clinical sites in Europe, North America, and South America to collect clinical data on 450 patients that will be used to support a range of clinical research studies. This included 350 people with NPC and 100 with ASMD.
- With increasing engagement from the global clinical community, we are also onboarding a further 44 clinical sites in several European countries, Australia, and the United States. This is the first step toward growing the registry and capturing critically important patient data.
-The registry also rolled out a revised patient-reported database (PRD) in 2023. Here patients can share their experience of Niemann-Pick Disease through a series of questionnaires. There were 144 patients in PRD by March 31st 2024 (33 people affected by ASMD and 111 with NPC), over 40% growth on the previous year. Increases were observed across several regions but particularly in US, Europe and UK.
- However, growth of the PRD has been slower than anticipated. Consequently, a number of improvements were initiated during the period including a range of promotional activities and user interface enhancements.
- Not all people registered in the PRD have completed questionnaires and some have completed questionnaires across different time points.
- By March 31st 2024, 96 quality of life questionnaires have been completed (81 NPC & 15 ASMD). 78 Carer quality of life questionnaires have been completed and 78 NPC quality of life forms have been completed. 102 symptoms / severity questionnaires have been completed (86 for NPC and 16 for ASMD).
- 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 2025.
- During the year, we embedded our data quality monitoring function in the US and UK with the support of the Ara Parseghian Medical Research Fund. Our Clinical Research Associates continue to visit clinical sites in the UK and the USA to undertake data monitoring. These visits are instrumental in building close relations with site staff and to ensure there has been an assessment of the accuracy of clinical data in the registry. In addition, we have established central data monitoring processes to provide a cost-effective monitoring of data completeness, timeliness and consistency.
- The INPDR achieved 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.
- In terms of data use in the INPDR, there have been several approved studies, research studies and data requests. 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."

Shared Information

- Genotype reports published for NPC population by Country - NNPDF, March 2024.
- Anonymised NPC extract released to C-Path, February 2024.
- Note review performed on All-Stripes database shared with INPDR to assess utility for US-led Sibling study.

Peer-reviewed Publications

Raebel, E.M., Wiseman, S., Donnelly, C., Mathieson, T., Pountney, J., Crowe, J. and Hopkin, J., 2024. Real-life impacts of olipudase alfa: The experience of patients and families taking an enzyme replacement therapy for acid sphingomyelinase deficiency. Orphanet Journal of Rare Diseases, 19(1), p.36.

Reports

- The impact of olipudase alfa on adults with ASMD - Part 1 - Quantitative Report. Solomon Mbua, INPDR Justin Hopkin, NNPDF Toni Mathieson, NPUK Joslyn Crowe, NNPDF Conan Donnelly, INPDR.
- The impact of treatment with olipudase alfa on adult patients with ASMD - Part 2 - Qualitative Report. Rare Disease Research Partners.

Achievement and performance

Posters

- WORLD Conference, February 2024, Niemann-Pick Type C: An Update by The International Niemann-Pick Disease Registry.
- WORLD Conference, February 2024, The impacts of olipudase alfa on adults with ASMD: The patient-reported experience.
- WORLD Conference, February 2024, Real World Data Collection in Niemann-Pick Disease Type C - Data from Early Access Program with Arimoclomol.

Communication and engagement

INPDR also collaborated internationally to undertake research in Niemann-Pick diseases. Results were presented at national and international meetings including:
- Niemann-Pick UK,
- The International Niemann-Pick Diseases Alliance,
- 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

The registry has worked hard to strengthen its profile and increase international engagement during the last year, in addition to presenting at international meetings, we established the INPDR Ambassador Programme, where community leaders work to promote recruitment to the registry in their country and to provide 360• feedback to the INPDR with a view to improving INPDR engagement and performance.

Financial review

Financial position

Unrestricted income in the year was £39,143 (2023 £109,381) and unrestricted expenditure was £183,249 (2023 £348,370).

A restricted grant totalling £61,321 (2023 £63,980) 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. A restricted grant of £11,534 was received from Sanofi Genzyme towards the INPDR forum 2023 and the National Lottery Community Fund advanced £19,500 for telling the story of NPDs in the UK.

Deficit on unrestricted fund

There is a deficit on the unrestricted funds. This reflected increased activity and corresponding expenditure following an underspend in these funds in previous years due to slower activity during COVID.

It is proposed to address the deficit in the unrestricted funds by increasing the contributions from "INPDR Gateway".

Reserves policy

There were no free reserves on 31 March 2024 as there was a deficit of £7,429 (2023 £136,677). The board aim is to hold six months running costs in reserve which would be £91,624. 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 alongside a trust and corporate grant strategy.

To support the commercial subscription, a commercial company, INPDR Gateway Ltd, was established. An appropriate governance model was established to allow the Board of Directors of INPDR Gateway to manage commercial relationships on behalf of INPDR and ensure that income generated through these activities is utilised by INPDR to serve the interests of the International Niemann-Pick community. INPDR Gateway contributed to £20,000 (2023 £104,276) to INPDR.

Future plans
The INPDR has developed and embedded a comprehensive model for collecting both clinician and patient-reported data, supported by systems that improve data quality and facilities for clinical and epidemiological research. Our sustainability plan, powered by the INPDR Gateway, offers commercial organizations the opportunity to invest in the registry, driving growth and broadening the services we provide.

In the coming year, the INPDR will build on these systems to further advance our mission, with continued investment in the following key areas:

1. Communication
A variety of resources will be developed to strengthen communication with all INPDR stakeholders. The registry will invest in communication expertise and enhance its presence at national and international conferences. We will also focus on building stronger partnerships with the global Niemann-Pick community to promote the INPDR among clinicians and patients.

2. Data Quality and Completeness
The INPDR team will collaborate with clinical sites worldwide to improve data completeness and accuracy, ensuring the registry effectively supports a wide range of clinical research initiatives. The registry management team will further develop this function and undertake further initiatives to enhance central data monitoring processes at INPDR.

3. Research Support
In the coming year, the INPDR aims to expand its portfolio of supported research studies. In partnership with our collaborators, we will facilitate approved research by offering clinicians, researchers, and industry access to anonymized data and analyses. Additionally, we will engage in specific research projects with clinicians and national and international patient organizations to deepen the understanding of the Niemann-Pick disease experience.

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.

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.

Oragnisational 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.

Structure, governance and management

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

Risk management

As part of the Quality Management System, 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 2022/23, 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 2023/24 and no data subject rights requests.

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

Statement of trustees' responsibilities

The trustees (who are also the directors of International Niemann-Pick Disease Registry for the purposes of company law) are responsible for preparing the Report of the Trustees and the financial statements in accordance with applicable law and United Kingdom Accounting Standards (United Kingdom Generally Accepted Accounting Practice).

Company law requires the trustees to prepare financial statements for each financial year which give a true and fair view of the state of affairs of the charitable company and of the incoming resources and application of resources, including the income and expenditure, of the charitable company for that period. In preparing those financial statements, the trustees are required to

- select suitable accounting policies and then apply them consistently;
- observe the methods and principles in the Charity SORP;
- make judgements and estimates that are reasonable and prudent;
- prepare the financial statements on the going concern basis unless it is inappropriate to presume that the charitable company will continue in business.

The trustees are responsible for keeping proper accounting records which disclose with reasonable accuracy at any time the financial position of the charitable company and to enable them to ensure that the financial statements comply with the Companies Act 2006. They are also responsible for safeguarding the assets of the charitable company and hence for taking reasonable steps for the prevention and detection of fraud and other irregularities.

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 24 December 2024 and signed on its behalf by:

J E Green - Trustee

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 2024.

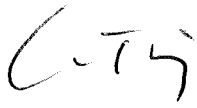
Responsibilities and basis of report
As the charity's trustees of the Company (and also its directors for the purposes of company law) you are responsible for the preparation of the accounts in accordance with the requirements of the Companies Act 2006 ('the 2006 Act').

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
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

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

24 December 2024

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Statement of Financial Activities
(Incorporating an Income and Expenditure Account)
for the Year Ended 31 March 2024

	Notes	Unrestricted fund £	Restricted funds £	2024 Total funds £	2023 Total funds £
Income and endowments from					
Donations and legacies	2	520	92,355	92,875	69,085
Charitable activities	3				
Charitable activities		38,623	-	38,623	104,276
Total		39,143	92,355	131,498	173,361
Expenditure on					
Charitable activities	4				
Charitable activities		183,249	124,437	307,686	415,907
NET INCOME/(EXPENDITURE)		(144,106)	(32,082)	(176,188)	(242,546)
Reconciliation of funds					
Total funds brought forward		136,677	50,898	187,575	430,121
Total funds carried forward		(7,429)	18,816	11,387	187,575

The notes form part of these financial statements

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Balance Sheet
31 March 2024

	Notes	2024 £	2023 £
Fixed assets			
Investments	9	1	1
Current assets			
Debtors	10	22	90,056
Cash at bank		25,363	116,772
		25,385	206,828
Creditors			
Amounts falling due within one year	11	(13,999)	(19,254)
Net current assets		11,386	187,574
Total assets less current liabilities		11,387	187,575
NET ASSETS		11,387	187,575
Funds	13		
Unrestricted funds		(7,429)	136,677
Restricted funds		18,816	50,898
Total funds		11,387	187,575

The charitable company is entitled to exemption from audit under Section 477 of the Companies Act 2006 for the year ended 31 March 2024.

The members have not required the company to obtain an audit of its financial statements for the year ended 31 March 2024 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 24 December 2024 and were signed on its behalf by:

J E Green - Trustee

The notes form part of these financial statements

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.

continued...

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 company has a deficiency of assets on its unrestricted fund. In response plans have been drawn up to address this. 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 £	2024 Total funds £	2023 Total funds £
Donations	520	-	520	5,105
Grants	-	92,355	92,355	63,980
	520	92,355	92,875	69,085

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

	2024 £	2023 £
Sanofi Genzyme	11,534	-
University of Notre Dame du Lac	61,321	63,980
National Lottery Community Fund	19,500	-
	92,355	63,980

3. Income from charitable activities

	Activity	2024 £	2023 £
Contribution to Research	Charitable activities	18,459	20,092
Recharge to INPDR Gateway Ltd	Charitable activities	20,000	84,184
Third party reimbursement	Charitable activities	164	-
		38,623	104,276

4. Charitable activities costs

	Direct Costs (see note 5) £
Charitable activities	307,686

continued...

5. Direct costs of charitable activities

	2024	2023
	£	£
Staff costs	129,563	103,305
Equipment costs	-	1,299
Registry hosting	21,740	47,913
Travel & meetings	29,722	38,583
Website costs	1,027	1,070
Other staff costs	-	689
Office expenses	9,520	8,184
Promotional expenses	5,826	158
Executive meetings	2,424	2,426
Independent examiner's fee	1,974	852
Other regulatory costs	3,713	60,001
Consultancy	71,669	89,619
Legal fees	-	23,572
Rebranding	-	9,024
Research	20,068	29,212
HIC maintenance & support	10,440	-
	307,686	415,907

6. Trustees' remuneration and benefits

There were no trustees' remuneration or other benefits for the period ended 31 March 2024 (2023 - nil)

Trustees' expenses

During the period one trustee was reimbursed £ 583 (2023 £103) for travel expenses. (2023 - 1)

7. Staff costs

	2024	2023
	£	£
Wages and salaries	121,223	97,527
Social security costs	5,993	3,897
Other pension costs	2,347	1,881
	129,563	103,305

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

	2024	2023
Coordination and administration	2	2
Registry Manager	1	1
	3	3

The Chief Executive Officer is key management and his total employee benefits (including NI and Pension) were £65,455. (2023 £53,915)

continued...

8. Comparatives for the statement of financial activities

	Unrestricted fund £	Restricted funds £	Total funds £
Income and endowments from			
Donations and legacies	5,105	63,980	69,085
Charitable activities			
Charitable activities	104,276	-	104,276
Total	109,381	63,980	173,361
Expenditure on			
Charitable activities			
Charitable activities	348,370	67,537	415,907
NET INCOME/(EXPENDITURE)	(238,989)	(3,557)	(242,546)
Reconciliation of funds			
Total funds brought forward	375,666	54,455	430,121
Total funds carried forward	136,677	50,898	187,575

9. Fixed asset investments

	Shares in group undertakings £
Market value	
At 1 April 2023 and 31 March 2024	1
Net book value	
At 31 March 2024	1
At 31 March 2023	1

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
1	100

	31.3.24 £	31.3.23 £
Aggregate capital and reserves	(49,446)	2,715
(Loss)/profit for the year	(52,160)	2,714

continued...

10. Debtors: amounts falling due within one year

	2024	2023
	£	£
Trade debtors	22	84,184
Prepayments	-	5,872
	22	90,056

11. Creditors: amounts falling due within one year

	2024	2023
	£	£
Social security and other taxes	2,854	2,532
Accruals and deferred income	11,145	16,722
	13,999	19,254

12. Analysis of net assets between funds

	Unrestricted fund	Restricted funds	2024 Total funds	2023 Total funds
	£	£	£	£
Investments	1	-	1	1
Current assets	6,569	18,816	25,385	206,828
Current liabilities	(13,999)	-	(13,999)	(19,254)
	(7,429)	18,816	11,387	187,575

13. 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
	50,898	(32,082)	18,816
TOTAL FUNDS	187,575	(176,188)	11,387

continued...

13. Movement in funds - continued

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
	92,355	(124,437)	(32,082)
TOTAL FUNDS	131,498	(307,686)	(176,188)

Comparatives for movement in funds

	At 1/4/22 £	Net movement in funds £	At 31/3/23 £
Unrestricted funds			
General fund	375,666	(238,989)	136,677
Restricted funds			
APMRF	54,455	(3,557)	50,898
TOTAL FUNDS	430,121	(242,546)	187,575

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

	Incoming resources £	Resources expended £	Movement in funds £
Unrestricted funds			
General fund	109,381	(348,370)	(238,989)
Restricted funds			
APMRF	63,980	(67,537)	(3,557)
TOTAL FUNDS	173,361	(415,907)	(242,546)

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

continued...

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 £3,014 (2023 £2,920). INPDA paid shared costs of £4,422 (2023 - £578). At 31 March 2024 there was a balance due to INPDA of £Nil (2023 £578).

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 £27,142 (2023 £20,991). INPDR received income in the year from NPUK of £7,981 (2023 - £10,899). At 31 March 2024 there was a balance due to NPUK of £2,650 (2023 £Nil).

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 (2023 £5,000) was lent by the Registry to the Gateway. In addition recharges and service charges were made to the Gateway in the year of £20,761 (2023 - £84,184). A sum of £22 was due from the Registry at 31 March 2024(2023 due to £84,184).

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Detailed Statement of Financial Activities for the Year Ended 31 March 2024

	Unrestricted funds £	Restricted funds £	2024 Total funds £	2023 Total funds £
Income and endowments				
Donations and legacies				
Donations	520	-	520	5,105
Grants	-	92,355	92,355	63,980
	520	92,355	92,875	69,085
Charitable activities				
Contribution to Research	18,459	-	18,459	20,092
Recharge to INPDR Gateway Ltd	20,000	-	20,000	84,184
Third party reimbursement	164	-	164	-
	38,623	-	38,623	104,276
Total incoming resources	39,143	92,355	131,498	173,361
Expenditure				
Charitable activities				
Wages	74,666	46,557	121,223	97,527
Social security	5,993	-	5,993	3,897
Employer pension	2,347	-	2,347	1,881
Equipment costs	-	-	-	1,299
Registry hosting	21,740	-	21,740	47,913
Travel & meetings	11,921	17,801	29,722	38,583
Website costs	1,027	-	1,027	1,070
Other staff costs	-	-	-	689
Office expenses	9,520	-	9,520	8,184
Promotional expenses	5,826	-	5,826	158
Executive meetings	2,424	-	2,424	2,426
Independent examiner's fee	1,974	-	1,974	852
Other regulatory costs	3,713	-	3,713	60,001
Consultancy	11,590	60,079	71,669	89,619
Legal fees	-	-	-	23,572
Rebranding	-	-	-	9,024
Research	20,068	-	20,068	29,212
HIC maintenance & support	10,440	-	10,440	-
	183,249	124,437	307,686	415,907
Total resources expended	183,249	124,437	307,686	415,907
Net (expenditure)/income	(144,106)	(32,082)	(176,188)	(242,546)

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

England & Wales - Charity number 1175311

Accounts

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 2023

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Contents of the Financial Statements
for the Year Ended 31 March 2023

Reference and Administrative Details for the Year Ended 31 March 2023

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 2 Vermont House Washington Tyne and Wear NE37 2SQ
Registered company number	10646129 (England and Wales)
Registered charity number	1175311
Independent examiner	James Anderson & Co Chartered Accountants Pentland Estate Straiton Edinburgh EH20 9QH

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 2023. 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
Aims and Objectives
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.

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.

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.

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 purpose of the registry
The INPDR was established to enhance and facilitate progress in Niemann-Pick diseases by providing a collaborative approach to the challenges faced by this rare disease community. The Registry purpose is:

a) To establish the natural history of the ASMD and Niemann-Pick Type C (their characteristics, management, and outcomes)
b) To provide an inventory of patients for recruitment to potential clinical trials
c) To establish genotype-phenotype correlations
d) To provide support for equal access to biochemical and genetic testing, education of health professionals, and patient empowerment.

Background

The INPDR is 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 of patients diagnosed with either Acid Sphingomyelinase Deficiency (ASMD) or Niemann-Pick Type C (NPC). The active participation of clinicians and patients 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 have worked to implement the INPDR's global recruitment plan, to accelerate data collection, improve data quality assurance and to facilitate research with the ultimate goal of improving outcomes in Niemann-Pick diseases.

- During 2022/23 INPDR worked with 20 clinical sites in Europe, North America, and South America to collect clinical data on over 400 patients that will be used to support a range of clinical research studies.
- We are also onboarding a further 30 clinical sites in several European countries, Australia, and the United States. This is the first step toward growing the registry and capturing critically important patient data.
- The registry also rolled out a revised patient-reported database (PRD). Here patients can share their experience of Niemann-Pick Disease through a series of questionnaires.
- The new PRD is being tested in English-language jurisdictions before translation into other languages to support greater global participation, a key priority for 2024.
- During the year, we developed our data quality monitoring function in the US with the support of our Clinical Research Associate (CRA), funded by the Ara Parseghian Medical Research Fund. We have now recruited a UK-based CRA to extend this critical function to the UK and Europe.
- The INPDR achieved ISO9001 accreditation for Quality Management Systems by the British Standards Institute during this period for all areas related data collection and management. This accreditation demonstrates the INPDR has robust system for managing the registry and supporting clinical research.
- The registry has actively supported three research studies during this period that will improve understanding of the natural history of Niemann-Pick diseases, establish genotype/phenotype correlations, provide evidence to support improved clinical care, and improve our understanding of NPD quality of life.
- The registry undertook a comprehensive rebranding and communications review. This enabled the registry to ensure consistency and clarity of messaging. In addition, the INPDR rolled out the INPDR Ambassador programme where community leaders work in partnership with the INPDR to develop the registry together.

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)'.

Achievement and performance

Charitable activities

Grant funding in the form of unrestricted educational grants enabled the charity to carry out its activities. In addition, INPDR Gateway provided payment to INPDR to facilitate its services. Further funding came from the Ara Parseghian Medical Research 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 as INPDR Board and Scientific Advisory Committee Members.

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

Achievement and performance

Recruitment to the INPDR

- At the end of March 2023, there were 20 clinical sites contributing data to the INPDR Clinician Reported Database (CRD) with a further 30 sites in the pipeline. As we move beyond the COVID-19 pandemic, clinical sites are moving more quickly through the onboarding process.

- Progress with consenting and registering new patients continued to be slow, increasing from 406 patients across 11 countries (318 NPC and 88 with ASMD) compared to 361 patients across 9 countries in March 2022.

- A revised Patient Reported Database (PRD) was rolled out to the community during 2022/23. This included new questionnaires to capture patient and carer quality of life, patient and carer needs as well as symptoms and severity of disease.

- The questionnaires are specific for NPC and ASMD as well as being tailored to the participant (patient, parent, or carer). The PRD was designed in partnership with INPDR stakeholders and in consultation with patients and their families.

-By the end of March 2023 there were 117 participants in the PRD (This included 28 people affected by ASMD and 89 by NPC) having increased from 98 in 2022.

Data collection and monitoring systems

- During 2021/22, with funding from the Ara Parseghian Medical Research Fund, the INPDR recruited a Clinical Research Associate (CRA) and established a data quality monitoring system in the USA.

- In 2022/23, data quality monitoring systems were embedded in the USA and the system was extended to the UK and Europe with a UK-based CRA recruited in December 2022.

- This system will be the basis for data quality improvement in the INPDR, a key priority for the coming year.

Governance

- The INPDR was awarded 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 that INPDR has a robust system for managing the registry and supporting clinical research.

- During 2021/22, the INPDR developed models for safe and secure data sharing. This system included statistical consultancy support and a secure safe-haven environment in the University of Dundee to provide secure access to anonymous record-level data. This has been rolled out to support research studies in 2022/23 for three studies.

Research Support

- The INPDR developed several collaborations during this period including a collaborative research project with the UK-based National Congenital Abnormalities and Rare Disease Registry System (NCARDRS).

- A jointly funded PhD study at Aston University, Birmingham to investigate quality of life in patients and families affected by Niemann-Pick diseases.

- A partnership with NNPDF, NPUK and INPDA to investigate the impact of olipudase alfa on quality of life of children and parents affected by ASMD and a subsequent study to investigate the impact of olipudase alfa on adults with ASMD.

- Studies to improve understanding of the natural history of Niemann-Pick diseases, establish genotype/phenotype correlations and provide evidence to support improved clinical care.

Communication and engagement

- INPDR also collaborated internationally to undertake research in Niemann-Pick diseases. Results were presented at national and international meetings including:
Niemann-Pick UK,
The International Niemann-Pick Diseases Alliance,
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

- The registry has worked hard to strengthen its profile and increase international engagement during the last year, in addition to presenting at international meetings, we established the INPDR Ambassador Programme, where community leaders work to promote recruitment to the registry in their country and to provide 360• feedback to the INPDR with a view to improving INPDR engagement and performance.

Financial review

Financial position

Unrestricted income in the year was £109,381 (2022 £402,705) and unrestricted expenditure was £348,370 (2022 £244,907)

A restricted grant totalling £63,980 (2022 £54,277) 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

The free reserves on 31 March 2023 amount to £136,677 (2022 - £375,666). The board aim is to hold six months running costs in reserve which would be £174,185. 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 alongside a trust and corporate grant strategy.

To support the commercial subscription, a commercial company, INPDR Gateway Ltd, was established. An appropriate governance model was established to allow the Board of Directors of INPDR Gateway to manage commercial relationships on behalf of INPDR and ensure that income generated through these activities is utilised by INPDR to serve the interests of international Niemann-Pick community. INPDR Gateway initiated its first project in 2022/23 and contributed to £104,276 to INPDR.

Future plans

The INPDR has developed and embedded a robust model for the collection of clinician and patient-reported data, systems to improve data quality, and facilities to support clinical and epidemiological research. We have implemented a sustainability plan, facilitated by INPDR Gateway, to provide the opportunity for commercial organisations to invest in the registry, enabling expansion and enhancing the range of services that can be offered.
Over the coming year, the INPDR will aim to employ these systems to deliver on our mission through further investment in:

1. Communication
A range of resources will be developed to enhance communication with all INPDR Stakeholders. The registry will invest in communications expertise and will strengthen its presence at national and international meetings. We will also seek to strengthen partnerships with national and international Niemann-Pick community to promote the INPDR to clinicians and the patient community.

2. Data quality and completeness
The INPDR team will work in partnership with clinical sites worldwide to enhance data completeness and accuracy ensuring that the registry can effectively support a broad range of clinical research studies.

3. Research support
Over the coming year, the INPDR will seek to expand the portfolio of research studies that we support. Alongside our partners, we will facilitate approved research by providing clinicians, researchers, and industry with controlled access to anonymised data and analyses. We will also undertake specific analysis and research in partnership with clinicians and national & international patient organisations to improve understanding of the Niemann-Pick disease experience.

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.

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.

Structure, governance and management
Oragnisational 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.

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 will meet the other Trustees.

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

Risk management
As part of the Quality Management System, 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 quarterly Management Review Meetings.

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.

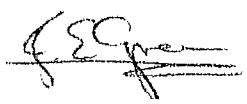
To manage risks related to data protection and ensure the privacy of registry participants, INPDR employs the services of a consultant Data Protection Officer. In addition, during the 2022/23, our technology partner, OpenApp was awarded ISO27001 accreditation for data security, providing further confidence that the INPDR are well-placed to ensure patient privacy.

As we move beyond the COVID-19 pandemic and the challenges that it created, we are now entering a new phase of growth and expansion, with this, new risks will emerge. INPDR will draw on our quality management system and the expertise of its Board to manage these risks and seize emerging opportunities to support the Niemann-Pick community.

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 22 December 2023 and signed on its behalf by:



J E Green - Trustee

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 2023.

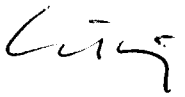
Responsibilities and basis of report
As the charity's trustees of the Company (and also its directors for the purposes of company law) you are responsible for the preparation of the accounts in accordance with the requirements of the Companies Act 2006 ('the 2006 Act').

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
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

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

22 December 2023

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Statement of Financial Activities
(Incorporating an Income and Expenditure Account)
for the Year Ended 31 March 2023

	Notes	Unrestricted fund £	Restricted fund £	2023 Total funds £	2022 Total funds £
Income and endowments from					
Donations and legacies	2	5,105	63,980	69,085	456,982
Charitable activities	3				
Charitable activities		104,276	-	104,276	-
Total		109,381	63,980	173,361	456,982
Expenditure on					
Charitable activities	4				
Charitable activities		348,370	67,537	415,907	279,140
NET INCOME/(EXPENDITURE)		(238,989)	(3,557)	(242,546)	177,842
Reconciliation of funds					
Total funds brought forward		375,666	54,455	430,121	252,279
Total funds carried forward		136,677	50,898	187,575	430,121

The notes form part of these financial statements

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

Balance Sheet
31 March 2023

	Notes	2023 £	2022 £
Fixed assets			
Investments	9	1	-
Current assets			
Debtors	10	90,056	842
Cash at bank		116,772	447,764
		206,828	448,606
Creditors			
Amounts falling due within one year	11	(19,254)	(18,485)
Net current assets		187,574	430,121
Total assets less current liabilities		187,575	430,121
NET ASSETS		187,575	430,121
Funds	13		
Unrestricted funds		136,677	375,666
Restricted funds		50,898	54,455
Total funds		187,575	430,121

The charitable company is entitled to exemption from audit under Section 477 of the Companies Act 2006 for the year ended 31 March 2023.

The members have not required the company to obtain an audit of its financial statements for the year ended 31 March 2023 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 22 December 2023 and were signed on its behalf by:

J E Green - Trustee

The notes form part of these financial statements

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.

continued...

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 £	2023 Total funds £	2022 Total funds £
Donations	5,105	-	5,105	211
Grants	-	63,980	63,980	456,771
	5,105	63,980	69,085	456,982

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

	2023 £	2022 £
Orphazyme	-	114,490
Sanofi Genzyme	-	269,979
University of Notre Dame du Lac	63,980	54,277
Cyclo Therapeutics Inc	-	18,025
	63,980	456,771

3. Income from charitable activities

	Activity	2023 £	2022 £
Contribution to Research	Charitable activities	20,092	-
Recharge to INPDR Gateway Ltd	Charitable activities	84,184	-
		104,276	-

4. Charitable activities costs

	Direct Costs (see note 5) £
Charitable activities	415,907

continued...

5. Direct costs of charitable activities

	2023	2022
	£	£
Staff costs	103,305	96,622
Equipment costs	1,299	1,540
Registry hosting	47,913	56,034
Travel & meetings	38,583	183
Website costs	1,070	949
Other staff costs	689	474
Office expenses	8,184	6,393
Promotional expenses	158	-
Executive meetings	2,426	742
Independent examiner's fee	852	1,320
Other regulatory costs	60,001	3,105
Consultancy	89,619	72,968
Legal fees	23,572	3,130
Rebranding	9,024	30,000
Research	29,212	5,680
	415,907	279,140

6. Trustees' remuneration and benefits

There were no trustees' remuneration or other benefits for the period ended 31 March 2023 (2022 - nil)

Trustees' expenses

During the period one trustee was reimbursed for travel expenses. (2022 - admin - 1)

7. Staff costs

	2023	2022
	£	£
Wages and salaries	97,527	91,421
Social security costs	3,897	3,507
Other pension costs	1,881	1,694
	103,305	96,622

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

	2023	2022
Coordination and administration	2	2
Registry Manager	1	1
	3	3

No employees received emoluments in excess of £60,000.

continued...

8. Comparatives for the statement of financial activities

	Unrestricted fund £	Restricted fund £	Total funds £
Income and endowments from			
Donations and legacies	402,705	54,277	456,982
Expenditure on			
Charitable activities			
Charitable activities	244,907	34,233	279,140
NET INCOME	157,798	20,044	177,842
Reconciliation of funds			
Total funds brought forward	217,868	34,411	252,279
Total funds carried forward	375,666	54,455	430,121

9. Fixed asset investments

	Shares in group undertakings £
Market value	
Additions	1
Net book value	
At 31 March 2023	1
At 31 March 2022	-

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
1	100

	2023 £	2022 £
Aggregate capital and reserves	2,715	1
Profit for the year	2,714	-

continued...

10. Debtors: amounts falling due within one year

	2023	2022
	£	£
Trade debtors	84,184	-
Prepayments	5,872	842
	90,056	842

11. Creditors: amounts falling due within one year

	2023	2022
	£	£
Social security and other taxes	2,532	2,368
Accruals and deferred income	16,722	16,117
	19,254	18,485

12. Analysis of net assets between funds

	Unrestricted fund	Restricted fund	2023 Total funds	2022 Total funds
	£	£	£	£
Investments	1	-	1	-
Current assets	155,930	50,898	206,828	448,606
Current liabilities	(19,254)	-	(19,254)	(18,485)
	136,677	50,898	187,575	430,121

13. Movement in funds

	At 1/4/22	Net movement in funds	At 31/3/23
	£	£	£
Unrestricted funds			
General fund	375,666	(238,989)	136,677
Restricted funds			
APMRF	54,455	(3,557)	50,898
TOTAL FUNDS	430,121	(242,546)	187,575

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

	Incoming resources	Resources expended	Movement in funds
	£	£	£
Unrestricted funds			
General fund	109,381	(348,370)	(238,989)
Restricted funds			
APMRF	63,980	(67,537)	(3,557)
TOTAL FUNDS	173,361	(415,907)	(242,546)

continued...

13. Movement in funds - continued

Comparatives for movement in funds

	At 1/4/21 £	Net movement in funds £	At 31/3/22 £
Unrestricted funds			
General fund	217,868	157,798	375,666
Restricted funds			
APMRF	34,411	20,044	54,455
TOTAL FUNDS	252,279	177,842	430,121

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

	Incoming resources £	Resources expended £	Movement in funds £
Unrestricted funds			
General fund	402,705	(244,907)	157,798
Restricted funds			
APMRF	54,277	(34,233)	20,044
TOTAL FUNDS	456,982	(279,140)	177,842

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.

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 £2,920 (2022 £1,696). INPDA paid shared costs of £578 (2022 - £Nil). At 31 March 2022 there was a balance due to INPDA of £578 (2022 £100).

Niemann-Pick UK (NPUK) is a member of the INPDA. During the year there were recharges for shared staff, travel and overheads by NPUK to INPDR of £20,991 (2022 £16,098). INPDA received income in the year from NPUK of £10,899 (2022 - £Nil). At 31 March 2023 there was a balance due to NPUK of £Nil (2022 £2,896).

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 £5,000 was lent by the Registry to the Gateway to finance its start up and this balance was still due back at 31 March 2023. In addition recharges and service charges were made to the Gateway in the year of £84,184 (2022 - £Nil). A sum of £84,184 was due to the Registry at 31 March 2023 (2022 - £Nil)

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

England & Wales - Charity number 1175311

Accounts

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 2022

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Contents of the Financial Statements for the Year Ended 31 March 2022

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Reference and Administrative Details for the Year Ended 31 March 2022

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 2 Vermont House Washington Tyne and Wear NE37 2SQ
Registered company number	10646129 (England and Wales)
Registered charity number	1175311
Independent examiner	James Anderson & Co Chartered Accountants Pentland Estate Straiton Edinburgh EH20 9QH

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 2022. 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).

Our Aims and Objectives

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.

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.

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.

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 purpose of the registry
The INPDR was established to enhance and facilitate progress in Niemann-Pick diseases by providing a collaborative approach to the challenges faced by this rare disease community. The more patients who are enrolled, the more effective the Registry will become. The Registry purpose is:

a) To establish the natural history of the 2 Niemann-Pick diseases (their characteristics, management and outcomes)
b) To provide an inventory of patients for recruitment to potential clinical trials
c) To establish genotype-phenotype correlations
d) To provide support for equal access to biochemical and genetic testing, education of health professionals, and patient empowerment.

The Registry is a regulatory compliant, secure database of consenting patients, with appropriate security, management, and controls. Data is collected via a secure web-based electronic data capture (EDC) platform for the systematic and uniform collection of clinical data of patients diagnosed with either ASMD or NPC. The active participation of clinicians and patients from around the world facilitate the collection of demographic information, diagnostic results, treatment, and health outcomes data.

The INPDR is a long-term initiative that will continue to grow data collection internationally and to create mechanisms to ensure that the data collected works hardest to serve patient needs.

Significant activities

During the year we have worked to implement the INPDR's global recruitment plan, to further develop and strengthen INPDR processes to support data collection, quality assurance and information sharing, all with the view to ensuring the registry is best place to support the Niemann-Pick community.

- As the clinical research community slowly moved into a new phase of the of Global COVID pandemic, the INPDR re-initiated onboarding processes with over 30 clinical sites worldwide as the first step in toward patient recruitment to the INPDR's longitudinal Clinician Reported Database (CRD). During this period, the registry opened up its second clinical site in the United States and made significant progress with onboarding of a further two US sites, sites in South America, Australia and Europe.

- Several new patients were added to the CRD during this period, comprehensive natural history data, leading to potential genotype/phenotype correlations; identifies patients eligible for clinical trials; supports post-marketing surveillance and therapy development; supports research; supports regulatory interaction between national/supranational agencies and industry and supports collaboration.

- We were also granted a letter of support from the European Medicines Agency (EMA) for the INPDR as a resource for clinical research. In addition, we implemented an improvement programme based on EMA recommendations that included registry governance, data collection, quality monitoring and data sharing.

- The INPDR was awarded ISO9001 accreditation for Quality Management Systems by the British Standards Institute during this period for all areas related data collection and management.

- We gave presentations and presented posters, both virtually and in-person, reporting progress on our work at several national and international meetings including Niemann-Pick UK, the International Niemann-Pick Diseases Alliance, Genetic Alliance UK, the LSD Collaborative plus the USA based WORLD Symposium, the National Niemann-Pick Disease Foundation Conference, and the Ara Parseghian Research Conference.

- We also initiated a comprehensive review of patient reported data collection in the INPDR to extend data collection to include the monitoring of quality of life, unmet need, and the financial burden of Niemann-Pick disease. The Patient Reported Database or PRD is separate from the Clinician Reported Database (CRD) and allows patients across the world to contribute their data without attending a medical facility. Importantly, patients are able to consent to the PRD without being enrolled into the CRD, although patients are able to enrol into both.

- The PRD review has led in development of specific questionnaires Niemann-Pick Type C and ASMD. In addition, questionnaires were tailored for different users (patients, parents, and carers). These questionnaires were reviewed by Niemann-Pick community representatives, including patient and their family members and carers, and amended based on their feedback. Ethical approval for the PRD registry has been granted by the Office of Research Ethics Northern Ireland.

Continuing impact of COVID-19

Whilst we saw a gradual easing of COVID restrictions during this year, the impact remained in regard to travel and meeting restrictions, limiting our ability to undertake some of our planned activities, particularly those due to take place in a health-related setting. COVID demonstrated the value and benefits of using digital technology to maintain communication, enabling us to stay connected with our stakeholders and continue to work on behalf of our rare disease community.

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)'.

Charitable activities

Grant funding in the form of unrestricted educational grants enabled the charity to carry out its activities. Further funding came from the Ara Parseghian Medical Research Fund. Throughout this year we have maximised use of virtual technology to mitigate the impact of the COVID-19 pandemic and in this way we have been able to maintain our activities but not without effects on our ability to undertake all planned activities.

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 as INPDR Board and Scientific Advisory Committee Members.

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

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.

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 period 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 taken into account.

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.

Achievement and performance

Recruitment to the INPDR

- At the end of March 2022, there were 16 clinical sites contributing data to the INPDR Clinician Reported Database (CRD) with a further 38 sites in the pipeline. Due to the COVID-19 pandemic, progress with consenting and registering new patients continued to be slow, increasing from 311 patients across 8 countries in March 2021 to 361 patients across 9 countries in March 2022. This growth was largely due to the onboarding of new sites in the US and Canada during this period.

- To facilitate clinical site onboarding, there were significant changes to the protocol and consent forms for the CRD to ensure compliance with GDPR and to ease the onboarding and patient recruitment process. The study materials were also translated in 12 different languages by an expert clinical research translators.

Data collection and monitoring systems

- We implemented significant amendments to the Clinician Reported Database (CRD) and rolled out these changes out to active clinical sites. These changes allowed the registry to support safety monitoring by capturing safety endpoints, co-morbidities and pregnancy as well as making improvements to the capturing of information related to monitoring disease progression.

- We also initiated a comprehensive review of the Patient Reported Database (PRD) in the INPDR to extend data collection to include the monitoring of quality of life, unmet need and the financial burden of Niemann-Pick disease. This involved key stakeholders including patients, their carers and family members and the results of the six-month pilot study (now underway) will inform further review.

- The PRD review has led to the development of specific questionnaires for Niemann-Pick type C and ASMD. In addition, questionnaires were tailored for different users (patients, parents and carers). These questionnaires were reviewed by community members and amended based on their feedback. Due to the significant development work undertaken to improve the PRD, this database was not promoted during this period. By end March 2022, there were 98 patients in the PRD from 20 countries.

- A major advance during this period was the development of data quality monitoring processes. These activities were rolled out to US clinical sites by a clinical research associate. This process was evaluated and will be extended across all clinical sites during 2022/23.

Governance

- During this period INPDR were also granted a letter of support from the European Medicines Agency (EMA) for the INPDR as a resource for clinical research. In addition, we implemented an improvement programme based on EMA recommendations that included registry governance, data collection, quality monitoring and data sharing.

- In December 2021, The British Standards Institute awarded INPDR ISO9001 accreditation for Quality Management Systems (for activities related to the collection and management of its data) demonstrating to clients and stakeholders that the INPDR has systems to ensure consistent and high-quality service delivery

- The INPDR developed models for safe and secure data sharing and processed three data requests. One request was approved during this period with a significant increase in research support activity anticipated for the 2022/23 year.

Research Support

- The INPDR initiated a number of collaborations during this period including a collaborative research project with the UK based National Congenital Abnormalities and Rare Disease Registry System (NCARDRS), a jointly funded PhD study at Aston University, Birmingham to investigate quality of life in patients and families affected by Niemann-Pick diseases, plus a partnership with NNPDF, NPUK and INPDA to investigate the impact of olipudase alfa on quality of life of families and patients affected by ASMD.

Communication
The implementation of a comprehensive communication plan has been implemented to stimulate use of the INPDR by patients and researchers within our existing global networks and to promote collaboration with the broader rare-disease community.

The INPDR Registry Management Team also presented virtually at the following events in 2021:

- Ara Parseghian Medical Research Foundation "2021 Michael, Marcia, and Christa Parseghian Scientific Conference for NPC Research - Virtual Symposiums" May 2021
- Genetic Alliance UK, Panel discussion on Patient Registries, June 2021
- FindaCure Workshop, Presentation on Patient Registries, June 2021
- National Niemann-Pick Disease Foundation Virtual Family Conference, July, 2021

The registry has also introduced a Community Ambassador model to involve patients and families directly in the development of the INPDR.

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.

Financial Review

Financial position
Donations and grants were received in the year of £456,982 (2021 £390,375).

The grants were received from Orphazyme, Sanofi Genzyme and Cyclo Therapeutics Inc. In addition, a restricted grant was received from 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 funds received have enabled the continuing development and growth of the Registry, and 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
The free reserves at 31 March 2022 amount to £375,666 (2021 £217,868). The board aim is to hold six months running costs in reserve. At 31 March 2022 there are sufficient reserves but as the registry activities grow a higher level of reserve will be required.

Sustainability
A sustainability plan for the INPDR has been introduced, including a commercial subscription model and costing model for the non-commercial sectors, plus a trust and corporate grant strategy, with the aim of diversifying income and continuing to operate as a non-profit organisation.

To support the commercial subscription, a commercial company, INPDR Gateway Ltd, has been established. An appropriate governance model was established to allow the Board of Directors of INPDR Gateway to manage commercial relationships on behalf of INPDR and ensure that income generated through these activities is utilised by INPDR to serve the interests of international Niemann-Pick community.

Risk management
The trustees risk management process comprises an annual review of the principal risks and uncertainties that the charity faces, the establishment of policies, systems and procedures to mitigate those risks. The trustees are satisfied that the major risks to which the charity is exposed have been reviewed and procedures have been established to manage these risks.

The Covid-19 pandemic created a challenging context for all clinical research and it presents challenges for the INPDR to undertake on-site data collection and quality assurance that may create delays in data collection. In addition, the Trustees will continue to monitor the impact of the UK leaving the European Union as well as other geopolitical changes on its activities and regulatory requirements. While these events are outside of the control of the INPDR, a strong risk management structure will ensure that the impact of such events on registry activities are minimised.

Future plans
The INPDR's Strategic Plan 2020-2022 is a flexible working document and will be regularly updated with the information and plans needed to achieve the successful implementation of the INPDR's aims and objectives during this three-year period. It is underpinned by Annual Deliverables and Milestones that will reflect priorities and critical requirements plus an annual operational plan which will guide the day to day work of the INPDR. The next phase of development will see the INPDR realise its potential to improve the outcomes for those affected by Niemann-Pick diseases and will focus on three primary themes:

1. Strengthening Governance

Building on the robust governance model developed since 2013, the INPDR shall embed policies to manage data sharing, support research and engage with industry. We shall develop a risk management programme including routine audit cycle and records management system to promote a culture of continuous improvement.

2. Planning for Sustainability

The INPDR will implement a sustainability plan that will encourage stakeholder support and interaction, enable Registry development and help to accelerate progress for this global rare disease community. A subscription and charging policy will provide the opportunity for commercial organisations to invest in the INPDR enabling expansion and enhancing the range of services that can be offered. A wholly owned commercial subsidiary company - INPDR Gateway Ltd - has been established to facilitate service delivery and further support expansion of the INPDR.

3. Extended data collection: promoting completeness and quality

The INPDR shall further develop our data quality management programme, including a comprehensive data review, to enable a broader range of data collection and seek to continue its expansion globally into new jurisdictions to increase the number of patients in the registry. A key aspect of this will be working closely with regulatory authorities to ensure our data meets user and regulatory requirements.

4. Promoting Research

For the Registry to realise its potential to improve patient outcomes, the collected data must be used for research to improve understanding of the disease and help support therapeutic and diagnostic development activity. The Registry is approaching a maturity that shall enable the data to be used in support of clinical and epidemiological research. Primarily the registry will achieve this through sharing data with experts in the Niemann-Pick research field, including clinicians, academics, and industry. The Registry has implemented a data request process to support research and will actively encourage use of registry data with the aim of facilitating at least 15 research studies over the next two years.

Statement of trustees' responsibilities
The trustees (who are also the directors of International Niemann-Pick Disease Registry for the purposes of company law) are responsible for preparing the Report of the Trustees and the financial statements in accordance with applicable law and United Kingdom Accounting Standards (United Kingdom Generally Accepted Accounting Practice).

Company law requires the trustees to prepare financial statements for each financial year which give a true and fair view of the state of affairs of the charitable company and of the incoming resources and application of resources, including the income and expenditure, of the charitable company for that period. In preparing those financial statements, the trustees are required to:

- select suitable accounting policies and then apply them consistently;
- observe the methods and principles in the Charity SORP;
- make judgements and estimates that are reasonable and prudent;
- prepare the financial statements on the going concern basis unless it is inappropriate to presume that the charitable company will continue in business.

The trustees are responsible for keeping proper accounting records which disclose with reasonable accuracy at any time the financial position of the charitable company and to enable them to ensure that the financial statements comply with the Companies Act 2006. They are also responsible for safeguarding the assets of the charitable company and hence for taking reasonable steps for the prevention and detection of fraud and other irregularities.

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 25 January 2023 and signed on its behalf by:

.....
J Green, Trustee

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 2022.

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').

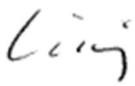
Having satisfied myself that the accounts of the Company are not required to be audited under Part 16 of the 2006 Act and are eligible for independent examination, I report in respect of my examination of your charity's accounts as carried out under Section 145 of the Charities Act 2011 ('the 2011 Act'). 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 registered member of ICAS 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
ICAS
James Anderson & Co
Chartered Accountants
Pentland Estate
Straiton
Edinburgh
EH20 9QH

Date: 25 January 2023

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Statement of Financial Activities
(Incorporating an Income and Expenditure Account)
for the Year Ended 31 March 2022

	Notes	Unrestricted fund £	Restricted fund £	2022 Total funds £	2021 Total funds £
Income and endowments from					
Donations and legacies	2	402,705	54,277	456,982	390,375
Expenditure on					
Charitable activities	3				
Charitable activities		244,907	34,233	279,140	265,255
NET INCOME		157,798	20,044	177,842	125,120
Reconciliation of funds					
Total funds brought forward		217,868	34,411	252,279	127,159
Total funds carried forward		375,666	54,455	430,121	252,279

The notes form part of these financial statements

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY
Company No. 10646129

Balance Sheet
31 March 2022

	Notes	2022 £	2021 £
Current assets			
Debtors	8	842	1,917
Cash at bank		447,764	262,905
		448,606	264,822
Creditors			
Amounts falling due within one year	9	(18,485)	(12,543)
Net current assets		430,121	252,279
Total assets less current liabilities		430,121	252,279
NET ASSETS		430,121	252,279
Funds	11		
Unrestricted funds		375,666	217,868
Restricted funds		54,455	34,411
Total funds		430,121	252,279

The charitable company is entitled to exemption from audit under Section 477 of the Companies Act 2006 for the year ended 31 March 2022.

The members have not required the company to obtain an audit of its financial statements for the year ended 31 March 2022 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 25 January 2023 and were signed on its behalf by:

.....
J Green, Trustee

The notes form part of these financial statements

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.

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.

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.

continued…

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 £	2022 Total funds £	2021 Total funds £
Donations	211	-	211	105
Grants	402,494	54,277	456,771	390,270
	402,705	54,277	456,982	390,375

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

	2022 £	2021 £
Orphazyme	114,490	76,000
Sanofi Genzyme	269,979	250,000
University of Notre Dame du Lac	54,277	59,277
Escape Bio	-	4,993
Cyclo Therapeutics Inc	18,025	-
	456,771	390,270

3. Charitable activities costs

	Direct Costs (see note 4) £
Charitable activities	279,140

continued…

4. Direct costs of charitable activities

	2022	2021
	£	£
Pension costs	1,694	936
Equipment costs	1,540	1,271
Wages & NIC	94,928	51,153
Registry hosting	56,034	28,647
Travel & meetings	183	459
Registry development and maintenence	-	84,790
Website costs	949	762
Other staff costs	474	440
Office expenses	6,393	3,660
Promotional expenses	-	439
Executive meetings	742	583
Independent examiner's fee	1,320	1,200
Other professional fees	3,105	204
Consultancy	72,968	65,095
Legal fees	3,130	25,616
Rebranding	30,000	-
Research	5,680	-
	279,140	265,255

5. Trustees' remuneration and benefits

There were no trustees' remuneration or other benefits for the period ended 31 March 2022 (2021 - nil)

Trustees' expenses

During the period there were no trustees expenses. (2021 nil)

6. Staff costs

	2022	2021
	£	£
Other pension costs	1,694	936
	1,694	936

	2022	2021
	£	£
Salaries and wages	91,419	50,336
Social security costs	3,508	817
Pension costs	1,694	936
	96,621	52,089

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

	2022	2021
Coordination and administration	2	1
Registry Manager	1	1
	3	2

No employees received emoluments in excess of £60,000.

continued…

7. Comparatives for the statement of financial activities

	Unrestricted fund £	Restricted fund £	Total funds £
Income and endowments from			
Donations and legacies	331,098	59,277	390,375
Expenditure on Charitable activities			
Charitable activities	240,389	24,866	265,255
NET INCOME	90,709	34,411	125,120
Reconciliation of funds			
Total funds brought forward	127,159	-	127,159
Total funds carried forward	217,868	34,411	252,279

8. Debtors: amounts falling due within one year

	2022 £	2021 £
Prepayments	842	1,917

9. Creditors: amounts falling due within one year

	2022 £	2021 £
Social security and other taxes	2,368	1,519
Accruals and deferred income	16,117	11,024
	18,485	12,543

10. Analysis of net assets between funds

	Unrestricted fund £	Restricted fund £	2022 Total funds £	2021 Total funds £
Current assets	394,151	54,455	448,606	264,822
Current liabilities	(18,485)	-	(18,485)	(12,543)
	375,666	54,455	430,121	252,279

continued…

11. Movement in funds

	At 1/4/21 £	Net movement in funds £	At 31/3/22 £
Unrestricted funds			
General fund	217,868	157,798	375,666
Restricted funds			
APMRF	34,411	20,044	54,455
TOTAL FUNDS	252,279	177,842	430,121

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

	Incoming resources £	Resources expended £	Movement in funds £
Unrestricted funds			
General fund	402,705	(244,907)	157,798
Restricted funds			
APMRF	54,277	(34,233)	20,044
TOTAL FUNDS	456,982	(279,140)	177,842

Comparatives for movement in funds

	At 1/4/20 £	Net movement in funds £	At 31/3/21 £
Unrestricted funds			
General fund	127,159	90,709	217,868
Restricted funds			
APMRF	-	34,411	34,411
TOTAL FUNDS	127,159	125,120	252,279

continued…

11. Movement in funds - continued

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

	Incoming resources £	Resources expended £	Movement in funds £
Unrestricted funds			
General fund	331,098	(240,389)	90,709
Restricted funds			
APMRF	59,277	(24,866)	34,411
TOTAL FUNDS	390,375	(265,255)	125,120

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.

12. 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 £864 (2021 £1,696). At 31 March 2022 there was a balance due to INPDA of £100 (2021 £22).

Niemann-Pick UK (NPUK) is a member of the INPDA. During the year there were recharges for shared staff, travel and overheads by NPUK to INPDR of £13,598 (2020 £7,799). At 31 March 2022 there was a balance due to NPUK of £2,896 (2021 £Nil).

continued…

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Detailed Statement of Financial Activities for the Year Ended 31 March 2022

	Unrestricted funds £	Restricted funds £	2022 Total funds £	2021 Total funds £
Income and endowments				
Donations and legacies				
Donations	211	-	211	105
Grants	402,494	54,277	456,771	390,270
	402,705	54,277	456,982	390,375
Total incoming resources	402,705	54,277	456,982	390,375
Expenditure				
Charitable activities				
Employer pension	1,694	-	1,694	936
Equipment costs	1,107	433	1,540	1,271
Wages & NIC	61,610	33,318	94,928	51,153
Registry hosting	56,034	-	56,034	28,647
Travel & meetings	129	54	183	459
Registry development and maintenence	-	-	-	84,790
Website costs	949	-	949	762
Other staff costs	474	-	474	440
Office expenses	6,393	-	6,393	3,660
Promotional expenses	-	-	-	439
Executive meetings	742	-	742	583
Independent examiner's fee	1,320	-	1,320	1,200
Other professional fees	3,105	-	3,105	204
Consultancy	72,540	428	72,968	65,095
Legal fees	3,130	-	3,130	25,616
Rebranding	30,000	-	30,000	-
Research	5,680	-	5,680	-
	244,907	34,233	279,140	265,255
Total resources expended	244,907	34,233	279,140	265,255
Net income	157,798	20,044	177,842	125,120

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

England & Wales - Charity number 1175311

Accounts

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 2021

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Contents of the Financial Statements
for the Year Ended 31 March 2021

Reference and Administrative Details for the Year Ended 31 March 2021

Trustees	J E Green T Hiwot Mrs T A Mathieson Dr A Dardis (appointed 12.10.20) M Vanier
Company secretary	Mrs T A Mathieson
Registered office	Suite 2 Vermont House Washington Tyne and Wear NE37 2SQ
Registered company number	10646129 (England and Wales)
Registered charity number	1175311
Independent examiner	James Anderson & Co Chartered Accountants Pentland Estate Straiton Edinburgh EH20 9QH

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 2021. 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

Aims and Objectives

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.

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.

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.

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

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

Objectives

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

The purpose of the registry

The INPDR was established in order to enhance and facilitate progress in Niemann-Pick diseases by providing a collaborative approach to the challenges faced by this rare disease community. The more patients who are enrolled, the more effective the Registry will become. The Registry purpose is:

a) To establish the natural history of the 3 Niemann-Pick diseases (their characteristics, management and outcomes)
b) To provide an inventory of patients for recruitment to potential clinical trials
c) To establish genotype-phenotype correlations
d) To provide support for equal access to biochemical and genetic testing, education of health professionals, and patient empowerment.

The purpose of the registry (continued)

The Registry is a regulatory compliant, secure database of consenting patients, with appropriate security, management and controls. Data is collected via a secure web-based electronic data capture (EDC) platform for the systematic and uniform collection of clinical data of patients diagnosed with either ASMD or NPC. The active participation of clinicians and patients from around the world facilitate the collection of demographic information, diagnostic results, treatment and health outcomes data.

The INPDR is a long term initiative that will continue to grow data collection internationally and to create mechanisms to ensure that the data collected works hardest to serve patient needs.

Significant activities

During the year we have worked to accelerate the INPDR's global recruitment, to further develop and strengthen the governance of the INPDR and to enable the collected data to be appropriately and securely shared to facilitate research, with the aim of achieving better outcomes for patients.

Although the achievement of our objectives has been slowed somewhat by the challenges of the COVID-19 pandemic, we remain focussed on the achievement of a sustainable patient-led database of at least 1000 patients, that includes, comprehensive natural history data, leading to potential genotype/phenotype correlations; identifies patients eligible for clinical trials; supports post-marketing surveillance and therapy development; supports research; supports regulatory interaction between national/supranational agencies and industry and supports collaboration.

Quality of life questionnaires are also collected directly from patients through our Patient Reported Dataset (PRD). This PRD registry is a separate module from the Clinical Reported Database (CRD) and allows for patients across the world to consent to data collect and contribute without attending a medical facility. Patients are able to consent to the PRD without being enrolled into the CRD, although patients are able to enrol into both. Currently work is in progress to link patients enrolled into the PRD and CRD to allow for clinical and quality of life data to be matched.

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)'.

Achievement and performance

This year, the INPDR has recruited key leadership roles, which has enabled the provision of essential support to clinical centres, with on-boarding of the registry and ongoing data entry. This will increase the completeness and quality of patient data to meet regulatory standards in research, which will facilitate the generation of new knowledge, leading to optimized clinical management and new therapeutic insights.

Recruitment to the INPDR

- At the end of March 2021, the number of patients actively engaging with the INPDR through the Patient Reported Data (PRD) platform stood at 73, from 15 different countries. Translations are currently underway to more effectively support users and encourage greater engagement.

- Recruitment to the Clinical Reported Database (CRD) has continued at a slower pace than expected, due to the COVID-19 pandemic, numbers stand at 311 patients across 8 countries as of March 31st 2021.

- A USA based Clinical Research Associate was successfully recruited in October 2020, enabling further progress in recruiting 10 US clinical sites. A global development plan has been initiated and will operate a phased approach to enable expansion into new jurisdictions to increase the number of patients in the registry.

Governance

- A Strategic Business Plan detailing work and plans for the period 2020-2022 was launched in October 2020. This is available to view on our website www.inpdr.org

- Building on our already robust governance model, we have developed and implemented policies to manage data sharing, support research and engage with industry. Our risk management programme has been revised to include a routine audit cycle and records management system to promote a culture of continuous improvement.

Achievement and performance (continued)

Governance (continued)

- Work has commenced to further develop our data quality management programme, including a comprehensive data review, to enable a broader range of data collection and enable the data to be used in support of clinical and epidemiological research, through the introduction of a data sharing model that will both protect sensitive patient data and also ensure that the data is accessible so it can be used effectively to improve understanding of Niemann-Pick disease.

- A key aspect of this will be working closely with regulatory authorities to ensure our data meets user and regulatory requirements. Therefore, we have continued to engage with regulators including the FDA and the European Medicines Agency (EMA), with whom the process to achieve a Qualification Opinion is underway.

Communication

- The implementation of a comprehensive communication plan has been implemented to stimulate use of the INPDR by patients and researchers within our existing global networks and to promote collaboration with the broader rare-disease community.

- An Abstract was successfully submitted for a platform presentation at the Virtual WORLD Symposium held in February 2021 “Niemann-Pick diseases and the International Niemann-Pick Disease Registry (INPDR)”.

- The INPDR Registry Management Team also presented virtually at the following events in 2020:
  - Ara Parseghian Medical Research Foundation “2020 Michael, Marcia, and Christa Parseghian Scientific Conference for NPC Research - Virtual Symposiums” May 2020
  - National Niemann-Pick Disease Foundation Virtual Family Conference, August 2020
  - Niemann-Pick UK Virtual Interactive Workshop on Niemann-Pick Diseases, September 2020
  - Niemann-Pick UK Family Conference, September 2020
  - Findacure Virtual Rare Disease Showcase “A Technological Revolution”, November 2020

Sustainability

A sustainability plan for the INPDR has been introduced, including a commercial subscription model and costing model for the non-commercial sectors, plus a trust and corporate grant strategy, with the aim of diversifying income and continuing to operate as a non-profit organisation.

Financial review

Donations and grants were received in the year of £390,375 (2020 £266,803).

The grants were received from Orphazyme, Sanofi Genzyme and Escape Bio. In addition, a restricted grant was received from 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 funds received have enabled the continuing development and growth of the Registry, and 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

The free reserves at 31 March 2021 amount to £217,868 (2020 £127,160). The board aim is to hold six months running costs in reserve. At 31 March 2021 there are sufficient reserves but as the registry activities grow a higher level of reserve will be required.

Future Plans - priorities for the year ahead include

The INPDR's Strategic Plan 2020-2022 is a flexible working document and will be regularly updated with the information and plans needed to achieve the successful implementation of the INPDR's aims and objectives during this three-year period. It is underpinned by Annual Deliverables and Milestones that will reflect priorities and critical requirements plus an annual operational plan which will guide the day to day work of the INPDR. The next phase of development will see the INPDR realise its potential to improve the outcomes for those affected by Niemann-Pick diseases and will focus on three primary themes:

1. Strengthening Governance

Building on the robust governance model developed since 2013, the INPDR shall embed policies to manage data sharing, support research and engage with industry. We shall develop a risk management programme including routine audit cycle and records management system to promote a culture of continuous improvement.

2. Planning for Sustainability

The INPDR will implement a sustainability plan that will encourage stakeholder support and interaction, enable Registry development and help to accelerate progress for this global rare disease community. A subscription and charging policy will provide the opportunity for commercial organisations to invest in the INPDR enabling expansion and enhancing the range of services that can be offered. A wholly owned commercial subsidiary company - INPDR Gateway Ltd - has also been established to facilitate service delivery and further support expansion of the INPDR.

3. Extended data collection: promoting completeness and quality

The INPDR shall further develop our data quality management programme, including a comprehensive data review, to enable a broader range of data collection and seek to continue its expansion globally into new jurisdictions to increase the number of patients in the registry. A key aspect of this will be working closely with regulatory authorities to ensure our data meets user and regulatory requirements.

Promoting Research

For the Registry to realise its potential to improve patient outcomes, the collected data must be used for research to improve understanding of the disease and help support therapeutic and diagnostic development activity. The Registry is approaching a maturity that shall enable the data to be used in support of clinical and epidemiological research. Primarily the registry will achieve this through sharing data with experts in the Niemann-Pick research field, including clinicians, academics and industry. The Registry has implemented a data request process to support research and will actively encourage use of registry data with the aim of facilitating at least 15 research studies over the next two years.

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.

Recruitment and appointment of new trustees
Up to three trustees may be appointed by the Member. Each partner may nominate up to one Trustee for appointment by the Board.

Oragnisational Structure
The Trustees and all the other supporters of the Group are volunteers.

The Trustees are directly responsible for the effective governance of the charity and for providing appropriate support and guidance and monitoring of all staff and activities. This is carried out in accordance with statutory guidance and legislation as provided by the Charity Commissioners. The Trustees meet on average every two years with at least four additional teleconferences each year in order to review reports and to carry out management and financial reviews.

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 will meet the other Trustees.

Key management remuneration
All trustees give of their time freely and no trustee received remuneration in the period 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 taken into account.

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 Disease. The Alliance was formed with a view to providing a forum where, through the exchange of information, experience and knowledge, progress could be accelerated.

Risk management
The trustees risk management process comprises an annual review of the principal risks and uncertainties that the charity faces, the establishment of policies, systems and procedures to mitigate those risks. The trustees are satisfied that the major risks to which the charity is exposed have been reviewed and procedures have been established to manage these risks.

The current Covid-19 pandemic has created a challenging context for all clinical research and it presents challenges for the INPDR to undertake on-site data collection and quality assurance that may create delays in data collection. In addition, the Trustees will also closely monitor the impact of the departure of the UK from the European Union as well as other geopolitical changes on its activities and regulatory requirements. While these events are outside of the control of the INPDR, a strong risk management structure will ensure that the impact of such events on registry activities are minimised.

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 22 December 2021 and signed on its behalf by:

James Green
Trustee

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 2021.

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').

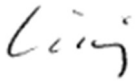
Having satisfied myself that the accounts of the Company are not required to be audited under Part 16 of the 2006 Act and are eligible for independent examination, I report in respect of my examination of your charity's accounts as carried out under section 145 of the Charities Act 2011 ('the 2011 Act'). 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 registered member of ICAS 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
ICAS
James Anderson & Co
Chartered Accountants
Pentland Estate
Straiton
Edinburgh
EH20 9QH

Date: 22 December 2021

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Statement of Financial Activities
(Incorporating an Income and Expenditure Account)
for the Year Ended 31 March 2021

	Notes	Unrestricted fund £	Restricted fund £	2021 Total funds £	2020 Total funds £
Income and endowments from					
Donations and legacies	2	331,098	59,277	390,375	266,803
Expenditure on					
Charitable activities	3				
Charitable activities		240,389	24,866	265,255	139,398
NET INCOME		90,709	34,411	125,120	127,405
Reconciliation of funds					
Total funds brought forward		127,159	-	127,159	(246)
Total funds carried forward		217,868	34,411	252,279	127,159

The notes form part of these financial statements

Balance Sheet
31 March 2021

	Notes	2021 £	2020 £
Current assets			
Debtors	8	1,917	-
Cash at bank		262,905	135,042
		264,822	135,042
Creditors			
Amounts falling due within one year	9	(12,543)	(7,883)
Net current assets		252,279	127,159
Total assets less current liabilities		252,279	127,159
NET ASSETS		252,279	127,159
Funds	11		
Unrestricted funds		217,868	127,159
Restricted funds		34,411	-
Total funds		252,279	127,159

The charitable company is entitled to exemption from audit under Section 477 of the Companies Act 2006 for the year ended 31 March 2021.

The members have not required the company to obtain an audit of its financial statements for the year ended 31 March 2021 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 22 December 2021 and were signed on its behalf by:

James Green
Trustee

The notes form part of these financial statements

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.

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.

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.

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.

continued...

1. Accounting policies - continued

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.

2. Donations and legacies

	Unrestricted funds £	Restricted funds £	2021 Total funds £	2020 Total funds £
Donations	105	-	105	267
Grants	330,993	59,277	390,270	266,536
	331,098	59,277	390,375	266,803

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

	2021 £	2020 £
Together Strong Foundation	-	56,536
Orphazyme	76,000	60,000
Sanofi Genzyme	250,000	150,000
University of Notre Dame du Lac	59,277	-
Escape Bio	4,993	-
	390,270	266,536

3. Charitable activities costs

	Direct Costs (see note 4) £
Charitable activities	265,255

4. Direct costs of charitable activities

	2021 £	2020 £
Equipment costs	1,271	-
Wages & NIC	52,089	9,085
Registry hosting	28,647	45,971
Travel & meetings	459	6,349
Registry development and maintenance	84,790	-
Website costs	762	835
Other staff costs	440	3,558
Office expenses	3,660	922
Promotional expenses	439	215
Conferences and awareness raising	-	12,132
Fundraising	-	395
Executive meetings	583	3,053
Independent examiner's fee	1,200	852
Carried forward	174,340	83,367

continued...

4. Direct costs of charitable activities - continued

	2021	2020
	£	£
Brought forward	174,340	83,367
Other professional fees	204	396
Consultancy	65,095	55,635
Legal fees	25,616	-
	265,255	139,398

5. Trustees' remuneration and benefits

There were no trustees' remuneration or other benefits for the period ended 31 March 2021 (2020 - nil)

Trustees' expenses

During the period no trustees were reimbursed for travel expenses. (2020 nil)

6. Staff costs

	2021	2020
	£	£
Salaries and wages	50,336	9,085
Social security costs	817	-
Pension costs	936	-
	52,089	9,085

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

	2021	2020
Coordination and administration	2	1

No employees received emoluments in excess of £60,000.

7. Comparatives for the statement of financial activities

	Unrestricted fund
	£
Income and endowments from	
Donations and legacies	266,803
Expenditure on	
Charitable activities	
Charitable activities	139,398
NET INCOME	127,405
Reconciliation of funds	
Total funds brought forward	(246)

continued...

7. Comparatives for the statement of financial activities - continued

	Unrestricted fund £
Total funds carried forward	127,159

8. Debtors: amounts falling due within one year

	2021 £	2020 £
Prepayments	1,917	-

9. Creditors: amounts falling due within one year

	2021 £	2020 £
Social security and other taxes	1,519	843
Accruals and deferred income	11,024	7,040
	12,543	7,883

10. Analysis of net assets between funds

	2021 Unrestricted fund £	2020 Total funds £
Current assets	230,411	135,042
Current liabilities	(12,543)	(7,883)
	217,868	127,159

11. Movement in funds

	At 1/4/20 £	Net movement in funds £	At 31/3/21 £
Unrestricted funds			
General fund	127,159	90,709	217,868
Restricted funds			
APMRF	-	34,411	34,411
TOTAL FUNDS	127,159	125,120	252,279

continued...

11. Movement in funds - continued

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

	Incoming resources £	Resources expended £	Movement in funds £
Unrestricted funds			
General fund	331,098	(240,389)	90,709
Restricted funds			
APMRF	59,277	(24,866)	34,411
TOTAL FUNDS	390,375	(265,255)	125,120

Comparatives for movement in funds

	At 1/4/19 £	Net movement in funds £	At 31/3/20 £
Unrestricted funds			
General fund	(246)	127,405	127,159
TOTAL FUNDS	(246)	127,405	127,159

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

	Incoming resources £	Resources expended £	Movement in funds £
Unrestricted funds			
General fund	266,803	(139,398)	127,405
TOTAL FUNDS	266,803	(139,398)	127,405

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.

continued...

12. 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 £1,696 (2020 £4,477). At 31 March 2021 there was a balance due to INPDA of £22 (2020 £1,241).

Niemann-Pick UK (NPUK) is a member of the INPDA. During the year there were recharges for shared staff, travel and overheads by NPUK to INPDR of £7,799 (2020 £10,334). At 31 March 2021 there was a balance due to NPUK of £Nil (2020 £573).

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Detailed Statement of Financial Activities
for the Year Ended 31 March 2021

	Unrestricted funds £	Restricted funds £	2021 Total funds £	2020 Total funds £
Income and endowments				
Donations and legacies				
Donations	105	-	105	267
Grants	330,993	59,277	390,270	266,536
	331,098	59,277	390,375	266,803
Total incoming resources	331,098	59,277	390,375	266,803
Expenditure				
Charitable activities				
Equipment costs	583	688	1,271	-
Wages & NIC	52,089	-	52,089	9,085
Registry hosting	19,598	9,049	28,647	45,971
Travel & meetings	459	-	459	6,349
Registry development and maintenance	84,790	-	84,790	-
Website costs	762	-	762	835
Other staff costs	134	306	440	3,558
Office expenses	3,660	-	3,660	922
Promotional expenses	439	-	439	215
Conferences and awareness raising	-	-	-	12,132
Fundraising	-	-	-	395
Executive meetings	583	-	583	3,053
Independent examiner's fee	1,200	-	1,200	852
Other professional fees	204	-	204	396
Consultancy	50,272	14,823	65,095	55,635
Legal fees	25,616	-	25,616	-
	240,389	24,866	265,255	139,398
Total resources expended	240,389	24,866	265,255	139,398
Net income	90,709	34,411	125,120	127,405