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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**

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**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

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

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.

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Report of the Trustees for the Year Ended 31 March 2024

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.

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Report of the Trustees for the Year Ended 31 March 2024

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.

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Report of the Trustees for the Year Ended 31 March 2024

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 - NNPDR, 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, NNPDR Toni Mathieson, NPUK Joslyn Crowe, NNPDR Conan Donnelly, INPDR.
- The impact of treatment with olipudase alfa on adult patients with ASMD - Part 2 - Qualitative Report. Rare Disease Research Partners.

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Report of the Trustees for the Year Ended 31 March 2024

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.

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Report of the Trustees for the Year Ended 31 March 2024

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.

Organisational Structure

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

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

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

Key management remuneration

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

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Report of the Trustees for the Year Ended 31 March 2024

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').

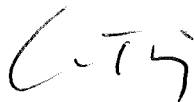
Having satisfied myself that the accounts of the Company are not required to be audited under Part 16 of the 2006 Act and are eligible for independent examination, I report in respect of my examination of your charity's accounts as carried out under Section 145 of the Charities Act 2011 ('the 2011 Act').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

		Unrestricted fund £	Restricted funds £	2024 Total funds £	2023 Total funds £
	Notes				
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		<u>39,143</u>	<u>92,355</u>	<u>131,498</u>	<u>173,361</u>
Expenditure on					
Charitable activities	4				
Charitable activities		<u>183,249</u>	<u>124,437</u>	<u>307,686</u>	<u>415,907</u>
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		<u>(7,429)</u>	<u>18,816</u>	<u>11,387</u>	<u>187,575</u>

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
		<u>25,385</u>	<u>206,828</u>
Creditors			
Amounts falling due within one year	11	(13,999)	(19,254)
Net current assets		<u>11,386</u>	<u>187,574</u>
Total assets less current liabilities		11,387	187,575
NET ASSETS		<u>11,387</u>	<u>187,575</u>
Funds	13		
Unrestricted funds		(7,429)	136,677
Restricted funds		18,816	50,898
Total funds		<u>11,387</u>	<u>187,575</u>

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.

INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY

Notes to the Financial Statements - continued
for the Year Ended 31 March 2024

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
	<u>520</u>	<u>92,355</u>	<u>92,875</u>	<u>69,085</u>

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	-
	<u>92,355</u>	<u>63,980</u>

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	-
		<u>38,623</u>	<u>104,276</u>

4. Charitable activities costs

	Direct Costs (see note 5) £
Charitable activities	<u>307,686</u>

**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY**

**Notes to the Financial Statements - continued
for the Year Ended 31 March 2024**

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	-
	<u>307,686</u>	<u>415,907</u>

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
	<u>129,563</u>	<u>103,305</u>

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

	2024	2023
Coordination and administration	2	2
Registry Manager	1	1
	<u>3</u>	<u>3</u>

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

INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY

Notes to the Financial Statements - continued
for the Year Ended 31 March 2024

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	<u>109,381</u>	<u>63,980</u>	<u>173,361</u>
Expenditure on			
Charitable activities			
Charitable activities	<u>348,370</u>	<u>67,537</u>	<u>415,907</u>
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	<u><u>136,677</u></u>	<u><u>50,898</u></u>	<u><u>187,575</u></u>

9. Fixed asset investments

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

There were no investment assets outside the UK.

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

INPDR Gateway Ltd

Registered office: United Kingdom 12706306

Nature of business: Data provider for research

Class of share:	% holding		
1	100	31.3.24 £	31.3.23 £
Aggregate capital and reserves		(49,446)	2,715
(Loss)/profit for the year		<u>(52,160)</u>	<u>2,714</u>

INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY

Notes to the Financial Statements - continued
for the Year Ended 31 March 2024

10. Debtors: amounts falling due within one year

	2024 £	2023 £
Trade debtors	22	84,184
Prepayments	-	5,872
	<u>22</u>	<u>90,056</u>

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
	<u>13,999</u>	<u>19,254</u>

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)
	<u>(7,429)</u>	<u>18,816</u>	<u>11,387</u>	<u>187,575</u>

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

INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY

Notes to the Financial Statements - continued
for the Year Ended 31 March 2024

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

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	<u>430,121</u>	<u>(242,546)</u>	<u>187,575</u>

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	<u>173,361</u>	<u>(415,907)</u>	<u>(242,546)</u>

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

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

The Sanofi fund was towards the INPDR 2023 Forum

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

**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY**

**Notes to the Financial Statements - continued
for the Year Ended 31 March 2024**

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)