

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**

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for the Year Ended 31 March 2023**

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**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY**

**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

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

**Report of the Trustees
for the Year Ended 31 March 2023**

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.

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

**Report of the Trustees
for the Year Ended 31 March 2023**

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.

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

**Report of the Trustees
for the Year Ended 31 March 2023**

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 (NCARDS).

- 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 NNPDR, 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.

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

**Report of the Trustees
for the Year Ended 31 March 2023**

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.

**Report of the Trustees
for the Year Ended 31 March 2023**

Structure, governance and management

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.

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**

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').

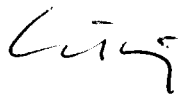
Having satisfied myself that the accounts of the Company are not required to be audited under Part 16 of the 2006 Act and are eligible for independent examination, I report in respect of my examination of your charity's accounts as carried out under Section 145 of the Charities Act 2011 ('the 2011 Act').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

		Unrestricted fund £	Restricted fund £	2023 Total funds £	2022 Total funds £
	Notes				
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		<u>109,381</u>	<u>63,980</u>	<u>173,361</u>	<u>456,982</u>
Expenditure on					
Charitable activities	4				
Charitable activities		<u>348,370</u>	<u>67,537</u>	<u>415,907</u>	<u>279,140</u>
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		<u><u>136,677</u></u>	<u><u>50,898</u></u>	<u><u>187,575</u></u>	<u><u>430,121</u></u>

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
		<u>206,828</u>	<u>448,606</u>
Creditors			
Amounts falling due within one year	11	(19,254)	(18,485)
		<u>187,574</u>	<u>430,121</u>
Net current assets			
		<u>187,575</u>	<u>430,121</u>
Total assets less current liabilities			
		<u>187,575</u>	<u>430,121</u>
NET ASSETS		<u>187,575</u>	<u>430,121</u>
Funds	13		
Unrestricted funds		136,677	375,666
Restricted funds		50,898	54,455
		<u>187,575</u>	<u>430,121</u>
Total funds		<u>187,575</u>	<u>430,121</u>

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

- ensuring that the charitable company keeps accounting records that comply with Sections 386 and 387 of the Companies Act 2006 and
- 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

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 2023**

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
	<u>5,105</u>	<u>63,980</u>	<u>69,085</u>	<u>456,982</u>

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
	<u>63,980</u>	<u>456,771</u>

3. Income from charitable activities

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

4. Charitable activities costs

	Direct Costs (see note 5) £
Charitable activities	<u>415,907</u>

**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY**

**Notes to the Financial Statements - continued
for the Year Ended 31 March 2023**

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
	<u>415,907</u>	<u>279,140</u>

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
	<u>103,305</u>	<u>96,622</u>

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

	2023	2022
Coordination and administration	2	2
Registry Manager	1	1
	<u>3</u>	<u>3</u>

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

**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY**

**Notes to the Financial Statements - continued
for the Year Ended 31 March 2023**

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	2023 £	2022 £
1	100		
Aggregate capital and reserves		2,715	1
Profit for the year		2,714	-

**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY**

**Notes to the Financial Statements - continued
for the Year Ended 31 March 2023**

10. Debtors: amounts falling due within one year

	2023	2022
	£	£
Trade debtors	84,184	-
Prepayments	5,872	842
	<u>90,056</u>	<u>842</u>

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
	<u>19,254</u>	<u>18,485</u>

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)
	<u>136,677</u>	<u>50,898</u>	<u>187,575</u>	<u>430,121</u>

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

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>

**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY**

**Notes to the Financial Statements - continued
for the Year Ended 31 March 2023**

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	<u>252,279</u>	<u>177,842</u>	<u>430,121</u>

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	<u>456,982</u>	<u>(279,140)</u>	<u>177,842</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.

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).