

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**

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for the Year Ended 31 March 2022**

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**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

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Report of the Trustees for the Year Ended 31 March 2022

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.

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Report of the Trustees for the Year Ended 31 March 2022

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.

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Report of the Trustees for the Year Ended 31 March 2022

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.

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Report of the Trustees for the Year Ended 31 March 2022

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 NNPDR, NPUK and INPDA to investigate the impact of olipudase alfa on quality of life of families and patients affected by ASMD.

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Report of the Trustees for the Year Ended 31 March 2022

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.

INTERNATIONAL NIEMANN-PICK DISEASE REGISTRY

Report of the Trustees for the Year Ended 31 March 2022

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.

**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY**

**Report of the Trustees
for the Year Ended 31 March 2022**

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:

A handwritten signature in black ink, appearing to be 'J Green', written over a horizontal line.

.....
J 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 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**

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

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		<u>447,764</u>	<u>262,905</u>
		448,606	264,822
Creditors			
Amounts falling due within one year	9	(18,485)	(12,543)
		<u>430,121</u>	<u>252,279</u>
Net current assets			
		<u>430,121</u>	<u>252,279</u>
Total assets less current liabilities		430,121	252,279
		<u>430,121</u>	<u>252,279</u>
NET ASSETS		<u>430,121</u>	<u>252,279</u>
Funds	11		
Unrestricted funds		375,666	217,868
Restricted funds		<u>54,455</u>	<u>34,411</u>
Total funds		<u>430,121</u>	<u>252,279</u>

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

- 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 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.

**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY**

**Notes to the Financial Statements - continued
for the Year Ended 31 March 2022**

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	<u>402,494</u>	<u>54,277</u>	<u>456,771</u>	<u>390,270</u>
	<u>402,705</u>	<u>54,277</u>	<u>456,982</u>	<u>390,375</u>

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	<u>18,025</u>	<u>-</u>
	<u>456,771</u>	<u>390,270</u>

3. Charitable activities costs

	Direct Costs (see note 4) £
Charitable activities	<u>279,140</u>

**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY**

**Notes to the Financial Statements - continued
for the Year Ended 31 March 2022**

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 maintenance	-	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	-
	<u>279,140</u>	<u>265,255</u>

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

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

	2022	2021
Coordination and administration	2	1
Registry Manager	<u>1</u>	<u>1</u>
	<u>3</u>	<u>2</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 2022**

7. Comparatives for the statement of financial activities

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

8. Debtors: amounts falling due within one year

	2022 £	2021 £
Prepayments	<u>842</u>	<u>1,917</u>

9. Creditors: amounts falling due within one year

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

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	<u>(18,485)</u>	<u>-</u>	<u>(18,485)</u>	<u>(12,543)</u>
	<u>375,666</u>	<u>54,455</u>	<u>430,121</u>	<u>252,279</u>

**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY**

**Notes to the Financial Statements - continued
for the Year Ended 31 March 2022**

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

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>

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	<u>127,159</u>	<u>125,120</u>	<u>252,279</u>

**INTERNATIONAL NIEMANN-PICK DISEASE
REGISTRY**

**Notes to the Financial Statements - continued
for the Year Ended 31 March 2022**

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	<u>390,375</u>	<u>(265,255)</u>	<u>125,120</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.

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).

**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	<u>402,494</u>	<u>54,277</u>	<u>456,771</u>	<u>390,270</u>
	<u>402,705</u>	<u>54,277</u>	<u>456,982</u>	<u>390,375</u>
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 maintenance	-	-	-	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	<u>5,680</u>	<u>-</u>	<u>5,680</u>	<u>-</u>
	<u>244,907</u>	<u>34,233</u>	<u>279,140</u>	<u>265,255</u>
Total resources expended	<u>244,907</u>	<u>34,233</u>	<u>279,140</u>	<u>265,255</u>
Net income	<u>157,798</u>	<u>20,044</u>	<u>177,842</u>	<u>125,120</u>

This page does not form part of the statutory financial statements