

# INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE

England & Wales · Charity number 1150256

## Details

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**Other names** INPDA

**Status** Registered

**Legal form** Charitable company

**Company number** [08287609](#)

**Registered** 2012-12-20

**Register** [View on the Charity Commission register](#)

## Contact

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North Sands Business Centre  
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**Email** [info@inpda.org](mailto:info@inpda.org)

**Website** [www.inpda.org](http://www.inpda.org)

## Activities

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**Objects:** TO RELIEVE SICKNESS AMONGST FAMILIES AFFECTED BY NIEMANN-PICK DISEASES, ANY DISTRESS WHICH MAY ARISE THEREFROM AND TO ADVANCE THE EDUCATION OF SUCH FAMILIES, INTERESTED PROFESSIONALS AND THE GENERAL PUBLIC IN ALL MATTERS CONCERNING SUCH DISEASES AS THE GOVERNING COUNCIL MAY IN ITS DISCRETION DETERMINE ("THE OBJECTS")

**Activities:** A network for non-profit organisations associated with the group of rare diseases known as Niemann-Pick Disease (NPD). The organisation aims to facilitate progress in research and understanding, family support and information services by providing an international forum. The organisation meets by teleconference every three months and bi-annually face to face.

## Classification

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- **How:** Provides Advocacy/advice/information, Acts As An Umbrella Or Resource Body
- **What:** Education/training, The Advancement Of Health Or Saving Of Lives
- **Who:** Other Defined Groups, The General Public/mankind

## Geography

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- Argentina
- Australia
- Brazil
- Canada
- China
- France
- Germany
- Italy
- Netherlands
- Norway
- Scotland
- Spain
- Switzerland
- United States
- Throughout England And Wales

## Finances

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Period end	Income	Expenditure	Assets	Employees
2025-03-31	£186,520	£184,220	-	-
2024-03-31	£214,845	£89,725	-	-
2023-03-31	£72,315	£57,616	-	-
2022-03-31	£20,112	£10,393	-	-
2021-03-31	£41,586	£8,052	-	-

## Trustees

Name	Role	Appointed
<b>Sandra Cowie</b>	Chair	2015-11-01
JIM GREEN		2012-12-18
Sarah Janetta		2023-11-01
TONI ANGELA MATHIESON		2012-12-18

**INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE**

England & Wales - Charity number 1150256

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# Accounts

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**REGISTERED COMPANY NUMBER: 08287609 (England and Wales)**  
**REGISTERED CHARITY NUMBER: 1150256**

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

**Report of the Trustees and**

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

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

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**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

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<b>Trustees</b>	Ms S Cowie President Ms J A Crowe Vice-President (resigned 10.11.24) Mrs T A Mathieson Ms L Chavez (resigned 10.11.24) J E Green Miss S Jannetta
<b>Company secretary</b>	Mrs T A Mathieson
<b>Registered office</b>	Suite 2E North Sands Business Centre Sunderland Tyne and Wear SR6 0QA
<b>Registered company number</b>	08287609 (England and Wales)
<b>Registered charity number</b>	1150256
<b>Independent examiner</b>	James Anderson & Co Chartered Accountants Pentland Estate Straiton Edinburgh EH20 9QH

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

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

**Objectives and activities**

**Our objectives and aims**

The objects for which the company is established are to relieve sickness amongst families affected by Niemann-Pick diseases, any distress which may arise therefrom and to advance the education of such families, interested professionals and the general public in all matters concerning such diseases as the governing council may in its discretion determine.

The following aims were agreed at the first meeting:

- To enhance progress and maximise the use of resources through international collaboration.
- To share available information.
- To facilitate strong networks of Foundations, Researchers, Clinicians and Industry representatives.
- To identify shared goals and address common challenges.
- To co-ordinate access to clinical trials and therapies.
- To be able to act as an international advocate for those affected these diseases.
- To act as an advocate, speaking with one voice re. health policy.

At each biennial INPDA Face-to-Face meeting, members agree on a set of strategic priorities to guide the organisation's work over the following two-year period. At the INPDA Face-to-Face meeting held in Tarragona in September 2023, the following priorities were established for 2023-2025:

**- Ensure sustainability**

Secure the financial resources, personnel, member engagement, skills, and organisational capacity required to deliver INPDA's planned activities.

**- Support and develop the membership**

Establish support mechanisms for new and developing groups, encourage increased support for ASMD, and introduce new associate members.

**- Strengthen communication**

Continue to enhance both internal and external communication through regular meetings; review and update materials in the INPDA Information Portal; encourage members to submit updated resources; expand the use of social media platforms, including LinkedIn, Facebook, Instagram and YouTube, and further grow newsletter distribution.

**- Raise awareness**

Deliver ongoing awareness-raising activities and engage with regulators and health policymakers. Refresh the Think Again. Think NPC campaign, develop ASMD awareness materials, and encourage engagement with International Niemann-Pick Disease Awareness Day (19 October).

**- Enable and accelerate research**

Facilitate collaboration on research projects, support earlier diagnosis (including use of the Suspicion Index), reduce duplication, and accelerate progress. Explore further opportunities for evidence generation, building on existing ASMD and NPC surveys and the development of consensus papers.

**- Support the International Niemann-Pick Disease Registry (INPDR)**

Continue to collaborate with and support the INPDR by facilitating global patient enrolment, encouraging community engagement, and promoting access to consensus clinical guidelines.

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

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**Review of activities**

The INPDA provides a collaborative forum for the sharing of information and experience regarding all aspects of Niemann-Pick diseases, including care and support, the provision and distribution of information and the furtherance of research. Our global network of non-profit organisations now extends to 27 members in 21 countries, with members united by a single aim - to improve outcomes for all those affected by Niemann-Pick diseases:

Argentina, Australia, Belgium, Brazil (2), Canada, China, Croatia, France, Germany, India, Italy, The Netherlands, Norway, Pakistan, Poland, Spain (3), Switzerland, Taiwan, Tunisia, United Kingdom, USA (4).

All our activities are undertaken in line with our aim of facilitating progress in the field of Niemann-Pick diseases. By providing a forum for mutual support, the INPDA aims to establish working links between all non-profit Niemann-Pick support groups and to raise awareness of the nature and operations of these, sometimes very different, organisations.

Our Executive Committee oversees the day-to-day management of the INPDA and reports regularly to the Full Member Council via bimonthly teleconferences.

This has been an extremely busy year for the INPDA with multiple projects targeting our priority objectives along with an increasing need to advocate for access to therapies for the global Niemann-Pick community, build linkages with industry, researchers, policymakers and payers and expand the global reach of the INPDA. The INPDA often serves as a point of contact for families, clinicians and researchers looking to connect with the broader Niemann-Pick community or to identify resources.

The INPDA works with our members to develop patient advocacy leadership, offering practical advice and support to newly established patient groups and sharing best practices. Our activities include:

- Supporting and developing patient advocacy leadership and offering practical advice and support to newly established patient groups.
- Using our shared experience to highlight and promote best practice in patient care and treatment
- Accelerating research through the creation of networks and sharing of information
- Encouraging a cross-sector collaborative approach
- Undertaking global advocacy activities in support of therapy development
- Engaging in advocacy with regulators and payers as new therapies are evaluated and approved
- Supporting global interaction and communication
- Fostering the effective use of resources and minimising duplication
- Evidence generation including collection of patient experience data and real-world evidence to support drug development, treatment strategies, plus discussions with regulatory bodies and health technology assessment authorities.

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

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**Objectives and activities**

**Significant activities**

Our activities this year have focused on the priorities that were established for the 2023-2025 period and include;

**Supporting new members**

INPDA continues to expand its membership and support the development of new patient organisations. In the past year, new members from Croatia and India have joined the alliance. INPDA has provided guidance, shared resources, and offered ongoing support to help these organisations become established and strengthen their capacity for long-term sustainability.

**Raising awareness**

Raising awareness of the collective challenges faced by the global Niemann-Pick community, and the significant unmet need that remains, is a core element of INPDA's work. The urgency of this need has never been greater. To accelerate progress, INPDA directs its awareness efforts toward multiple key audiences, including:

- **Regulators and the pharmaceutical industry**, to improve understanding of unmet needs and support progress toward approved therapies
- **Clinicians**, to help shorten the diagnostic odyssey for patients and families
- **Researchers and scientists**, to stimulate and accelerate research in Niemann-Pick diseases
- **Funders and policymakers**, to underline the importance of continued investment in advocacy, research, and initiatives such as newborn screening

**Awareness to support earlier diagnosis**

At the end of 2024, INPDA initiated a project to re-establish the Niemann-Pick disease type C Suspicion Index (NPC-SI) on an independent website. The NPC-SI is a screening tool developed by an international panel of clinical experts to support physicians unfamiliar with NPC in the early identification of patients with suspected disease. The tool generates a risk prediction score based on the presence of key clinical manifestations.

This is a two-phase project. Phase 1 focuses on reinstating the existing NPC-SI on an independent platform, together with supporting clinical information. Phase 2 will involve updating the tool to reflect advances in understanding of NPC since its original development. Phase 1 is scheduled for completion by September 2025.

**Advocacy and access to therapies**

While Niemann-Pick diseases have seen significant progress in research and drug development, therapies only deliver value if patients are able to access them. To better understand the current access environment, INPDA conducted a survey of its members on access to approved therapies for ASMD and Niemann-Pick disease type C, as well as clinical trials and expanded access programmes.

The survey revealed substantial geographical disparities in access to therapies, clinical trials, and expanded access programmes. These findings were presented in a poster at WORLDSymposium, a leading international scientific meeting focused on lysosomal storage diseases.

At a global level, INPDA continues to engage directly with regulatory authorities and health technology assessment (HTA) bodies to highlight unmet needs and communicate the broader impact of Niemann-Pick diseases on individuals, families, and communities.

INPDA has also identified a growing need to build understanding within the patient community of regulatory, reimbursement, and funding processes as new therapies emerge. To address this, INPDA is working to ensure members have access to the tools, knowledge, and resources required to advocate effectively at the national level, alongside INPDA's ongoing global advocacy efforts.

**Exploring digital health technologies**

INPDA has been working in collaboration with the International Niemann-Pick Disease Registry (INPDR), Niemann-Pick UK (NPUK), the National Niemann-Pick Disease Foundation (NNPDF), and the Ara Parseghian Medical Research Fund (APMRF) to explore the potential of digital health technologies as a means of complementing traditional outcome measures in Niemann-Pick disease type C.

**Supporting and enabling research**

INPDA continues to actively support research in Niemann-Pick diseases. The Reus Valley Meeting, a biennial scientific conference focused on NPC research, was held in Switzerland in October 2024. The meeting supports INPDA's objectives of building research networks and facilitating collaboration across the NPC research community. Planning is now underway for the 2026 Thames Valley Meeting.

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

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**Objectives and activities**

**Collecting real-world patient and carer data**

In collaboration with NPUK, INPDR, NNNPDF, Niemann-Pick Canada, Australian Niemann-Pick Disease Type C Foundation (Australia), and Aston University, INPDA has been developing an international survey to capture the lived experiences of people affected by Niemann-Pick disease type C and their carers. This study aims to strengthen understanding of the impact of NPC on quality of life. A second phase of the study is planned in additional languages to ensure global representation.

**Promoting best practice and clinical care**

In early 2025, INPDA initiated an update of the Clinical Management Guidelines for Niemann-Pick disease type C. Originally published in 2018, the guidelines are now being reviewed and updated by an international panel of NPC clinical experts to reflect advances in disease understanding and the evolving therapeutic landscape, including the FDA approval of two new therapies for NPC in September 2024. Once finalised, the updated guidelines will be submitted for publication in a peer-reviewed journal.

**INPDA Face-to-Face Meeting**

INPDA convenes its members in person every two years to strengthen collaboration, share knowledge, and set future priorities. The 2025 INPDA Face-to-Face Meeting will be held in Puerto Iguazú, Argentina, and hosted by Asociación Niemann-Pick de Argentina and Associação Niemann-Pick Brasil (ANPB).

All INPDA member organisations are invited to attend with up to two senior representatives, along with a researcher or clinician working in the field of Niemann-Pick diseases. In addition, expert clinicians, researchers, and representatives from the pharmaceutical industry with an interest in Niemann-Pick diseases will be invited to participate.

The Face-to-Face Meeting provides a vital forum for members to learn from one another, receive updates from leading experts, and build the relationships that are essential to accelerating progress in Niemann-Pick diseases. In collaboration with our hosts, the INPDA team is developing a programme that will ensure patient advocacy leaders are informed of current research and equipped with the knowledge needed to support and advocate for their communities.

The meeting will also serve as the forum for establishing INPDA's strategic priorities for the 2025-2027 period.

The previous Face-to-Face Meeting, held in 2023, was attended by approximately 80 delegates, including senior representatives from patient organisations, clinicians, researchers, and pharmaceutical industry representatives. The meeting strengthened relationships, encouraged collaboration, and supported the development of mutual support networks. We anticipate that the 2025 meeting will be equally dynamic and impactful.

**Collaboration and partnerships**

INPDA recognises that collaboration is essential to addressing shared challenges and ensuring that the voice of the Niemann-Pick community is represented in relevant discussions. INPDA is an active member of both EURORDIS and Rare Diseases International.

INPDA also acknowledges that many lysosomal disease communities face common challenges. To help address these collectively, INPDA is a member of the Global LSD Collaborative, which works to advance shared priorities, including:

- Access to charitable and compassionate use medicines
- Clinical harmonisation, including the identification and validation of biomarkers
- Regulatory challenges
- Achieving global reach and equity of access

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

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**Objectives and activities**

**Public benefit**

In shaping our objectives for the year, agreeing our priorities 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)'.

As a network for non-profit organisations associated with the group of rare diseases known as Niemann-Pick diseases, we aim to facilitate progress through the provision of an international collaborative forum for the sharing of information and experience regarding all aspects of Niemann-Pick disease, including care and support, the provision and distribution of information and the furtherance of research. We meet virtually every three months and bi-annually face to face, with ad hoc meetings as required.

**Benefits of the INPDA**

As a global alliance, INPDA is uniquely positioned to communicate effectively with the international Niemann-Pick community, ensuring equity of access to information and promoting consistency, accuracy, and shared understanding across countries and regions.

**- Stronger communication and shared learning**

By sharing information and maximising communication, INPDA supports member organisations in strengthening leadership, reducing duplication of effort, and using time and resources more efficiently and effectively.

**- Equity and global inclusion**

INPDA help to reduce geographic disparities by ensuring that smaller, newer, or under-resourced organisations have access to the same information, tools, and opportunities as more established groups.

**- Access to the latest scientific and therapeutic developments**

INPDA ensures that member organisations are kept informed of emerging scientific, clinical, and therapeutic advances and, where appropriate, supported to respond or act on these developments.

**- Supporting and enabling research**

Wherever possible, INPDA encourages research by facilitating collaboration, supporting the formation of research networks, providing seed funding, and working in partnership to secure grant funding.

**- Patient and carer perspectives at the centre**

Through its network, INPDA ensures that patient and carer experience informs research priorities, clinical guidance, regulatory discussions, and health policy decision-making.

**- A stronger collective voice**

By working together, INPDA members amplify their collective voice, strengthening their ability to influence change and deliver meaningful improvements in care, access, and outcomes for people affected by Niemann-Pick diseases worldwide.

**- Credibility and engagement with external stakeholders**

INPDA provide a trusted, unified point of contact for researchers, industry, regulators, and policymakers seeking to engage with the global Niemann-Pick community.

**- Capacity-building for advocacy**

INPDA supports members to build knowledge and advocacy capability, particularly around regulatory, reimbursement, and access processes, strengthening national-level advocacy alongside INPDA's global efforts.

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

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**Achievements and performance**

**Charitable activities**

Building on the lessons learned during the COVID-19 pandemic, INPDA has maximised the use of virtual technologies to maintain regular communication, share information, and remain closely connected as a global network. These virtual connections enable member organisations to exchange experiences, stay informed of key developments, and continue to amplify a collective voice on behalf of individuals and families affected by Niemann-Pick diseases.

INPDA has maintained a strong and active presence in the research landscape, collaborating globally with rare disease organisations, research networks, and academic institutions. Through these partnerships, INPDA supports the advancement of research, facilitates the clinical development of promising therapies, and contributes to discussions aimed at influencing newborn screening policies.

INPDA continues to foster appropriate, transparent relationships with pharmaceutical companies engaged in clinical programmes and activities related to Niemann-Pick diseases. In parallel, INPDA provides high-level support, information, and guidance to patient advocacy leaders working within its member organisations to help them navigate an evolving therapeutic and regulatory environment.

INPDA also remains committed to supporting the International Niemann-Pick Disease Registry (INPDR) in its mission to collect high-quality clinical, genetic, diagnostic, and outcome data from individuals living with Niemann-Pick diseases. The INPDR operates as an independent charitable organisation, with INPDA representatives appointed to both its Board of Directors and Scientific Advisory Committee, ensuring strong alignment between patient priorities and registry governance.

**Financial review**

**Income**

Grant funding in the form of unrestricted grants enabled the charity to carry out its activities. Further funding came from the Roy & Murray Green Together Fund. Incoming funds were £186,520 as compared to £214,845 in the previous year.

**Expenditure**

Expenditure for the year was £184,220 as a result of the Face-to-Face Conference in comparison to £89,725 in the previous year.

**Financial Strategy**

Our Financial Strategy is based on levels of incoming funds matching the expenditure required to deliver our objectives and activities. Whilst income will be spent within a reasonable period of receipt to fund the operation of the Charity, we have a responsibility to manage risk, and to ensure the charity is financially sustainable. We will therefore hold a level of reserves to ensure the smooth running of the Charity during times of financial uncertainty.

**Reserves policy**

The free reserves at 31 March 2025 amounted to £175,414 (2024 £169,059). The Board aim to hold six months running costs in reserve and therefore this figure is considered higher than usual, due to the late receipt of grant funding, resulting in delays to activities which will now extend into the next fiscal year.

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

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**Looking to the future**

At the INPDA Face-to-Face Meeting held in September 2025, INPDA trustees and members agreed a set of strategic priorities to guide the organisation's work for the 2025-2027 period. These priorities are as follows:

**- Safeguarding sustainability**

Ensure INPDA has the financial resources, personnel, member engagement, skills, and organisational capacity required to deliver its planned activities.

**- Supporting and developing the membership**

Establish and strengthen support mechanisms for new and developing groups, encourage increased support for ASMD, and introduce new associate members.

**- Strengthening communication**

Enhance internal and external communication through regular meetings, effective use of the INPDA web portal, continued publication of the newsletter (February, June, and October), expanded use of social media, and a planned update of the INPDA website.

**- Raising awareness and influencing policy**

Continue to raise awareness through targeted activities and by educating and influencing regulators and health policymakers.

**- Advocacy for access to therapies**

Maintain a strong advocacy focus on equitable and timely access to therapies for people affected by Niemann-Pick diseases.

**- International Niemann-Pick Disease Awareness Day**

Continue to support and facilitate International Niemann-Pick Disease Awareness Day on 19 October.

**- Enabling and accelerating research**

Further support research by facilitating collaboration on research projects, minimising duplication, accelerating progress, exploring opportunities for evidence generation, and supporting the development of consensus papers.

**- Supporting the International Niemann-Pick Disease Registry (INPDR)**

Continue to support the INPDR to facilitate the enrolment of patients worldwide into this disease-specific registry.

**- Supporting scientific exchange**

Continue support for the Thames Valley NPC Research Meeting scheduled for September 2025.

**- Future planning and governance**

Plan for the next INPDA Face-to-Face Meeting, scheduled for June 2026 in the United States of America.

**- Key projects for 2025-2026**

Priority projects for the coming period include:

- o Completion of Phase 1 of the NPC lived experience survey
- o Completion of the NPC-SI website and initiation of Phase 2 to update the tool
- o Hosting the INPDA Face-to-Face Meeting in Argentina in September 2025
- o Publication of the updated Consensus Clinical Guidelines for NPC
- o Initiation of an update to the Consensus Clinical Guidelines for ASMD
- o Commencement of work to refresh the Think Again. Think NPC awareness campaign to reflect new knowledge and learnings

**Structure, governance and management**

**Governing document**

The charity is governed by its memorandum and articles of association and constitutes a limited company, limited by guarantee, as defined by the Companies Act 2006.

**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 (Governing Council). They are volunteers and each takes on responsibilities within the Alliance to co-ordinate/support an aspect of the Alliance functions.

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

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**Structure, governance and management**

**Organisational structure**

The Trustees and all other supporters of the INPDA are volunteers. The Trustees are directly responsible for the effective governance of the charity and for providing appropriate support and guidance and monitoring of all activities. This is carried out in accordance with statutory guidance and legislation as provided by the Charity Commission. Members are asked to 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 INPDA. An Executive Committee oversees the day-to-day management of the INPDA and reports regularly to the Full Member Council via quarterly virtual meetings. The Trustees and members meet face-to-face every two years with at least four additional virtual meetings each year in order to review reports and to carry out management and financial reviews.

**Induction and training of new trustees**

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

The Trustees are responsible for the day-to-day management of the INPDA, with the support of an Executive Committee. All trustees give of their time freely and no trustee received remuneration in the year, outside of travel and subsistence expenses.

**Pay policy for staff**

Currently, INPDA has no direct employees. Consultancy support in the areas of communications, governance, and project management is engaged on a contractual, as-needed basis to ensure the effective delivery of INPDA's programmes, projects, and strategic priorities.

In the event of direct employment, the Trustees are 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 small size of the organisation is also taken into account.

**Wider network**

The International Niemann-Pick Disease Alliance (INPDA) is an alliance of non-profit support organisations who are associated with the rare group of genetic diseases known collectively as Niemann-Pick Disease. The Alliance was formed with a view to providing a forum where, through the exchange of information, experience and knowledge, progress could be accelerated.

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE (REGISTERED NUMBER: 08287609)**

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

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**Structure, governance and management**

**Related parties**

None of our trustees receive remuneration or other benefit from their work with the charity. At present there are 27 separate foundations in the Alliance.

**Member Groups:**

Argentina - Asociaion Niemann Pick de Argentina  
Australia - Australian NPC Disease Foundation  
Brazil - Associação Niemann Pick Brasil - ANPB  
Canada - Niemann-Pick Canada  
China - China Niemann-Pick Disease Association  
France - Vaincres Les Maladies Lysosomales  
Germany - Niemann-Pick Selbsthilfegruppe  
Italy - Associazione Italiana Niemann Pick  
Netherlands - Volwassenen, Kinderen en Stofwisselingsziekten  
Norway - NP Foreningen I Norge  
Spain - Fundacion Niemann-Pick de Espana  
Switzerland - Niemann-Pick Suisse  
UK - Niemann-Pick UK  
USA - National Niemann-Pick Disease Foundation

**Associate Members:**

Belgium - Belgische Organisatie voor Kinderen en volwassenen met een Stofwisselingsziekte BOKS  
Brazil - Niemann-Pick B R-S  
Croatia - Niemann-Pick Hrvatska  
India - Niemann-Pick India Charitable Trust  
Pakistan - LSD Society Pakistan  
Poland- Stowarzyszenie Chorych na Chorobe Niemannna Picka i Choroby Rzadkie (Niemann-Pick and Rare Diseases Assoc. Poland)  
Spain - Asociación Niemann Pick de Fuenlabrada  
Spain - ASMD Espana  
Taiwan - Niemann-Pick Taiwan  
Tunisia - Association Tunisienne des Maladies Lysosomales  
USA - The Wylder Nation Foundation  
USA - Ara Parseghian Medical Research Foundation APMRF  
USA - Firefly Fund

Related party disclosures are shown at note 11.

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

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**Structure, governance and management**

**Principal risks and mitigation**

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

**- Funding environment pressures**

Risk: Increased competition for a diminishing pool of grant funding opportunities and a limited number of potential funders.

Mitigation: INPDA continues to strengthen relationships with existing funders, pursue collaborative funding opportunities with partners, and explore diversification of income streams where possible, including project-based funding and partnerships.

**- Economic and political uncertainty**

Risk: Ongoing economic and political pressures may negatively affect voluntary income, grant availability, and charitable giving.

Mitigation: INPDA maintains prudent financial planning, closely monitors expenditure, and prioritises activities aligned with strategic objectives to ensure resources are used effectively.

**- Capacity constraints**

Risk: Limited organisational capacity restricts the ability to increase the volume of grant applications and develop new income streams.

Mitigation: INPDA seeks to focus capacity on high-impact funding opportunities, explore shared funding applications with partner organisations, and utilise consultancy support where resources allow.

**- Reliance on volunteers**

Risk: A strong dependence on volunteers, with limited spare capacity to provide operational cover or support succession planning.

Mitigation: INPDA promotes shared leadership, documents key processes, and seeks to broaden engagement across the membership to reduce reliance on a small number of individuals.

**- Global reach and representation gaps**

Risk: Limited representation in countries where there is a known population of individuals affected by Niemann-Pick diseases but no local patient advocacy organisation.

Mitigation: INPDA actively supports outreach efforts, provides guidance to emerging groups, and encourages the development of new organisations or associate membership where appropriate.

**Additional Risks Considered**

**- Dependence on external partners**

Risk: Reliance on external organisations, collaborators, and consultants may affect the pace or continuity of key activities.

Mitigation: INPDA maintains clear agreements, fosters strong collaborative relationships, and seeks to ensure knowledge sharing and continuity across projects.

**- Regulatory and policy change**

Risk: Changes in regulatory, reimbursement, or policy environments may affect access to therapies, research priorities, or advocacy objectives.

Mitigation: INPDA maintains active engagement with regulators, policymakers, and partner organisations to monitor developments and respond proactively.

**- Reputational risk**

Risk: As a global alliance representing patient organisations, INPDA faces reputational risk related to advocacy positions, partnerships, or external communications.

Mitigation: INPDA applies clear governance processes, ensures transparency in decision-making, and aligns communications with agreed policies and patient-centred values.

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 17 December 2025 and signed on its behalf by:



Ms Sandra Cowie – Trustee

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

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**Independent examiner's report to the trustees of International Niemann-Pick Disease Alliance ('the Company')**

I report to the charity trustees on my examination of the accounts of the Company for the year ended 31 March 2025.

**Responsibilities and basis of report**

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

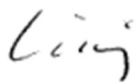
Having satisfied myself that the accounts of the Company are not required to be audited under Part 16 of the 2006 Act and are eligible for independent examination, I report in respect of my examination of your charity's accounts as carried out under Section 145 of the Charities Act 2011 ('the 2011 Act').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

**Independent examiner's statement**

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 Spalding  
The Institute of Chartered Accountants of Scotland

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

17 December 2025

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

	Notes	Unrestricted fund £	Restricted funds £	2025 Total funds £	2024 Total funds £
<b>Income and endowments from</b>					
Donations and legacies		<u>172,996</u>	<u>13,524</u>	<u>186,520</u>	<u>214,845</u>
<b>Expenditure on</b>					
<b>Charitable activities</b>	3				
Meetings		68,087	17,579	85,666	72,925
Charitable activities		<u>98,554</u>	<u>-</u>	<u>98,554</u>	<u>16,800</u>
<b>Total</b>		<u>166,641</u>	<u>17,579</u>	<u>184,220</u>	<u>89,725</u>
<b>NET INCOME/(EXPENDITURE)</b>		6,355	(4,055)	2,300	125,120
<b>Reconciliation of funds</b>					
Total funds brought forward		169,059	23,104	192,163	67,043
<b>Total funds carried forward</b>		<u>175,414</u>	<u>19,049</u>	<u>194,463</u>	<u>192,163</u>

The notes form part of these financial statements

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE (REGISTERED NUMBER: 08287609)**

**Balance Sheet  
31 March 2025**

		<b>2025</b>	<b>2024</b>
	<b>Notes</b>	<b>£</b>	<b>£</b>
<b>Current assets</b>			
Debtors	7	72,457	-
Cash at bank		<u>158,656</u>	<u>195,246</u>
		231,113	195,246
<b>Creditors</b>			
Amounts falling due within one year	8	(36,650)	(3,083)
		<u>194,463</u>	<u>192,163</u>
<b>Net current assets</b>			
		<u>194,463</u>	<u>192,163</u>
<b>Total assets less current liabilities</b>		194,463	192,163
		<u>194,463</u>	<u>192,163</u>
<b>NET ASSETS</b>			
		<u>194,463</u>	<u>192,163</u>
<b>Funds</b>	10		
Unrestricted funds		175,414	169,059
Restricted funds		<u>19,049</u>	<u>23,104</u>
<b>Total funds</b>		<u>194,463</u>	<u>192,163</u>

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

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

The trustees acknowledge their responsibilities for

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

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

The financial statements were approved by the Board of Trustees and authorised for issue on 17 December 2025 and were signed on its behalf by:



Sandra Cowie - Trustee

# INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE

## Notes to the Financial Statements for the Year Ended 31 March 2025

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### 1. Statutory information

International Niemann-Pick Disease Alliance is a private company, limited by guarantee with charitable status and registered in England and Wales. In the event of the charity being wound up, the liability in respect of the guarantee is limited to £1 per member of the charity. The company's registration number and registered office address can be found on the Reference and Administrative Details page.

The presentation currency of the financial statements is the Pound Sterling (£).

### 2. 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.

#### 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.

#### 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.

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

**2. Accounting policies - continued**

**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 at bank and in hand**

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

**3. Charitable activities costs**

	<b>Direct Costs (see note 4) £</b>
Meetings	85,666
Charitable activities	<u>98,554</u>
	<u>184,220</u>

**4. Direct costs of charitable activities**

	<b>2025 £</b>	<b>2024 £</b>
Finance & admin costs	18,548	7,314
IT costs	100	470
Meetings, conferences, travel and accommodation	85,666	72,925
Project costs - research	59,670	4,302
Think again: Think NPC	310	216
Other overheads	9,077	3,304
Independent examiner's fee	1,200	1,194
Rebranding costs	<u>9,649</u>	<u>-</u>
	<u>184,220</u>	<u>89,725</u>

**5. Trustees' remuneration and benefits**

There were no trustees' remuneration or other benefits for the year ended 31 March 2025 nor for the year ended 31 March 2024.

**Trustees' expenses**

During the period there one trustees was reimbursed expenses of £2,065 (2024 £921) for travel.

**6. Comparatives for the statement of financial activities**

	<b>Unrestricted fund £</b>	<b>Restricted funds £</b>	<b>Total funds £</b>
<b>Income and endowments from</b>			
Donations and legacies	<u>198,363</u>	<u>16,482</u>	<u>214,845</u>
<b>Expenditure on Charitable activities</b>			
Meetings	62,047	10,878	72,925
Charitable activities	<u>16,800</u>	<u>-</u>	<u>16,800</u>
<b>Total</b>	<u>78,847</u>	<u>10,878</u>	<u>89,725</u>
<b>NET INCOME</b>	119,516	5,604	125,120

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

**6. Comparatives for the statement of financial activities - continued**

	<b>Unrestricted fund £</b>	<b>Restricted funds £</b>	<b>Total funds £</b>
<b>Reconciliation of funds</b>			
Total funds brought forward	49,543	17,500	67,043
	<u>          </u>	<u>          </u>	<u>          </u>
<b>Total funds carried forward</b>	<u>169,059</u>	<u>23,104</u>	<u>192,163</u>

**7. Debtors: amounts falling due within one year**

	<b>2025 £</b>	<b>2024 £</b>
Trade debtors	<u>72,457</u>	<u>-</u>

**8. Creditors: amounts falling due within one year**

	<b>2025 £</b>	<b>2024 £</b>
Other creditors	35,450	1,943
Accruals and deferred income	<u>1,200</u>	<u>1,140</u>
	<u>36,650</u>	<u>3,083</u>

**9. Analysis of net assets between funds**

	<b>Unrestricted fund £</b>	<b>Restricted funds £</b>	<b>2025 Total funds £</b>	<b>2024 Total funds £</b>
Current assets	212,064	19,049	231,113	195,246
Current liabilities	<u>(36,650)</u>	<u>-</u>	<u>(36,650)</u>	<u>(3,083)</u>
	<u>175,414</u>	<u>19,049</u>	<u>194,463</u>	<u>192,163</u>

**10. Movement in funds**

	<b>At 1/4/24 £</b>	<b>Net movement in funds £</b>	<b>At 31/3/25 £</b>
<b>Unrestricted funds</b>			
General fund	169,059	6,355	175,414
<b>Restricted funds</b>			
Roy & Murray Green Together Fund	21,047	(7,724)	13,323
Brazilian Patient Organisations Travel Fund	<u>2,057</u>	<u>3,669</u>	<u>5,726</u>
	<u>23,104</u>	<u>(4,055)</u>	<u>19,049</u>
<b>TOTAL FUNDS</b>	<u>192,163</u>	<u>2,300</u>	<u>194,463</u>

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

**10. Movement in funds - continued**

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

	<b>Incoming resources £</b>	<b>Resources expended £</b>	<b>Movement in funds £</b>
<b>Unrestricted funds</b>			
General fund	172,996	(166,641)	6,355
<b>Restricted funds</b>			
Roy & Murray Green Together Fund	7,857	(15,581)	(7,724)
Brazilian Patient Organisations Travel Fund	<u>5,667</u>	<u>(1,998)</u>	<u>3,669</u>
	<u>13,524</u>	<u>(17,579)</u>	<u>(4,055)</u>
<b>TOTAL FUNDS</b>	<u><u>186,520</u></u>	<u><u>(184,220)</u></u>	<u><u>2,300</u></u>

**Comparatives for movement in funds**

	<b>At 1/4/23 £</b>	<b>Net movement in funds £</b>	<b>At 31/3/24 £</b>
<b>Unrestricted funds</b>			
General fund	49,543	119,516	169,059
<b>Restricted funds</b>			
Roy & Murray Green Together Fund	17,500	3,547	21,047
Brazilian Patient Organisations Travel Fund	<u>-</u>	<u>2,057</u>	<u>2,057</u>
	<u>17,500</u>	<u>5,604</u>	<u>23,104</u>
<b>TOTAL FUNDS</b>	<u><u>67,043</u></u>	<u><u>125,120</u></u>	<u><u>192,163</u></u>

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

	<b>Incoming resources £</b>	<b>Resources expended £</b>	<b>Movement in funds £</b>
<b>Unrestricted funds</b>			
General fund	198,363	(78,847)	119,516
<b>Restricted funds</b>			
Roy & Murray Green Together Fund	6,882	(3,335)	3,547
Brazilian Patient Organisations Travel Fund	<u>9,600</u>	<u>(7,543)</u>	<u>2,057</u>
	<u>16,482</u>	<u>(10,878)</u>	<u>5,604</u>
<b>TOTAL FUNDS</b>	<u><u>214,845</u></u>	<u><u>(89,725)</u></u>	<u><u>125,120</u></u>

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

The Roy & Murray Green Together Fund is to enhance the development and integration of the INPDA including the support of travel and staffing for the organization.

Brazilian Patient Organisations Travel Fund - to support travel costs of Niemann-Pick B-RS and ANPB patient groups in Brazil to attend INPDA Face 2 Face meetings.

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

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**11. Related party disclosures**

During the year salary, admin and travel costs etc were recharged by a member of the alliance, Niemann-Pick UK(NPUK) of £14,754 (2024 £15,455). Costs of £Nil (2024 £127) were incurred on behalf of NPUK. There was a balance owed by NPUK of £1,262 at 31 March 2025 (2024 £1,946).

The International Niemann-Pick Disease Alliance (INPDA) is a member of the International Niemann-Pick Disease Registry (INPDR). During the year costs were incurred on behalf of the INPDR and reimbursed of £6,736 (2024 £4,422). Costs were incurred by INPDR on behalf of the INPDA and reimbursed of £2,116 (2024 - £3,014). At 31 March 2025 there was a balance owed by INPDR of £4,619 (2024 £Nil).

During the year costs were recharged by a member of the alliance, National Niemann-Pick Disease Foundation of £496 (2024 £2,175). There was no balance outstanding at the year end.

**INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE**

England & Wales - Charity number 1150256

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# Accounts

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**REGISTERED COMPANY NUMBER: 08287609 (England and Wales)**  
**REGISTERED CHARITY NUMBER: 1150256**

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

**Report of the Trustees and**

**Financial Statements for the Year Ended 31 March 2024**

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

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**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

**Reference and Administrative Details  
for the Year Ended 31 March 2024**

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<b>Trustees</b>	Ms S Cowie President Ms J A Crowe Vice-President (resigned 10.11.24) Mrs T A Mathieson Ms I Hontanilla (resigned 12.11.23) Ms L Chavez (resigned 10.11.23) J E Green Miss S Jannetta (appointed 18.11.23)
<b>Company secretary</b>	Mrs T A Mathieson
<b>Registered office</b>	Suite 2 Vermont House Washington Tyne and Wear NE37 2SQ
<b>Registered company number</b>	08287609 (England and Wales)
<b>Registered charity number</b>	1150256
<b>Independent examiner</b>	James Anderson & Co Chartered Accountants Pentland Estate Straiton Edinburgh EH20 9QH

## INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE

### Report of the Trustees for the Year Ended 31 March 2024

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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

##### Our objectives and aims

The objects for which the company is established are to relieve sickness amongst families affected by Niemann-Pick diseases, any distress which may arise therefrom and to advance the education of such families, interested professionals and the general public in all matters concerning such diseases as the governing council may in its discretion determine.

The following aims were agreed at the first meeting:

- To enhance progress and maximise the use of resources through international collaboration.
- To share available information.
- To facilitate strong networks of Foundations, Researchers, Clinicians and Industry representatives.
- To identify shared goals and address common challenges.
- To co-ordinate access to clinical trials and therapies.
- To be able to act as an international advocate for those affected these diseases.
- To act as an advocate, speaking with one voice re. health policy.

#### Review of activities

The INPDA provides a collaborative forum for the sharing of information and experience regarding all aspects of Niemann-Pick disease, including care and support, the provision and distribution of information and the furtherance of research. Our global network of non-profit organisations working in the field of Niemann-Pick disease (NPD) now extends to 27 members in 20 countries, with members united by a single aim - to improve outcomes for all those affected by Niemann-Pick diseases:

Argentina, Australia, Belgium, Brazil (2), Canada, China, France (2), Germany, India, Italy, The Netherlands, Norway, Pakistan, Poland, Spain (3), Switzerland, Taiwan, Tunisia, United Kingdom, USA (4).

All our activities are undertaken in line with our aim of facilitating progress in the field of Niemann-Pick diseases. By providing a forum for mutual support, the INPDA aims to establish working links between all non-profit Niemann-Pick support groups and to raise awareness of the nature and operations of these, sometimes very different, organisations.

Our Executive Committee oversees the day-to-day management of the INPDA and reports regularly to the Full Member Council via quarterly teleconferences. This year, we have continued to facilitate progress by:

- Supporting and developing patient advocacy leadership and offering practical advice and support to newly established patient groups.
- Using our shared experience to highlight and promote best practice in patient care and treatment
- Accelerating research through the creation of networks and sharing of information
- Encouraging a cross-sector collaborative approach
- Undertaking global advocacy activities in support of therapy development
- Engaging in advocacy with regulators and payers as new therapies are evaluated and approved
- Supporting global interaction and communication
- Fostering the effective use of resources and minimising duplication
- Evidence generation including collection of patient experience data and real-world evidence to support drug development, treatment strategies, plus discussions with regulatory bodies and health technology assessment authorities.

#### Objectives and activities

##### Significant activities

A key element of our work is raising awareness of our collective challenges and the high level of unmet need that exists within the global Niemann-Pick community. Never has the need for awareness of Niemann-Pick diseases been more apparent, and in order to achieve our aim to accelerate progress, there are multiple targets for our awareness efforts, including:

- regulators and the pharmaceutical industry - to assist understanding of the unmet needs of our community and to accelerate progress towards approved therapies
- clinicians - to improve the diagnostic odyssey faced by patients and families
- researchers and scientists - to accelerate research in NPD
- funders - to underline the need for our efforts in advocacy and research to continue- newborn screening and its potential benefits

Representatives of the INPDA have attended and presented at key conferences around the world, including patient group events and wider rare disease events.

We have also maintained and further developed our relationships with clinical and research institutions, drug developers and other key stakeholders. The high-level work provides the opportunity for us to advocate for improvements to care and services, access to clinical standards of care, and access to treatments and therapies where they exist.

Representatives have also met with statutory bodies, health authorities and regulators around the world. These essential efforts support research and progress in clinical care, and address issues such as the current regulatory environment, working with the pharmaceutical industry and HTA bodies, including;

- Continuing to work with our members to provide information and evidence to support access to approved therapies, or early access to promising therapies
- Advocating on behalf of our global community directly with regulators
- Appropriate involvement in regulatory meetings
- Collection of evidence through surveys, information gathering
- The need to provide expert patient advocates who can speak on behalf of the whole patient community in activities to support disease understanding and therapy development, including regulator or industry led advisory boards
- Continuing to work with clinicians and researchers to develop patient reported outcome measures that effectively record disease progression and quality of life
- Working with the global Niemann-Pick and wider Lysosomal disease community to identify biomarkers and clinical endpoints
- Working with global health professional and patient representatives to identify digital health technologies that could support research and further understanding

We have continued to undertake our own community research studies, aiming to collect and develop evidence of impact and unmet need, and to provide information that will increase disease understanding, and support research and therapy development.

Our Biennial Face-to-Face meeting was held in Tarragona Spain in November 2023. The meeting was very well attended and provided a dynamic and impactful opportunity for our patient advocacy leadership representatives plus key figures in the clinical, research and scientific field of Niemann-Pick diseases to come together, share updates and build networks that will support the ongoing growth of the community. The program at the Face-to-Face meeting enabled the sharing of knowledge and experience and included presentations on the latest clinical and research updates. This meeting helps to ensure patient advocacy leaders are aware of current research, providing information that supports their ability to inform their communities and advocate on their behalf. This event was attended by approximately 80 delegates including senior representatives of patient associations, clinicians, researchers, and pharmaceutical industry representatives helping to strengthen relationships, encourage collaboration and enable the formation of mutual support networks. In addition, this meeting also enabled the INPDA to agree its strategic priorities, supporting the future direction of the organisation.

To assist capacity building, we have in place four voluntary Executive Liaison Roles to support progress in the INPDA's key priority areas. These roles were recruited internally (from INPDA Member Groups) and externally (individuals / organisations with the necessary experience/interest) and in line with the requirements of our Governing document:

- Research Liaison Officer, ASMD
- Research Liaison Officer, NPC
- Membership Liaison Officer
- Newborn Screening Liaison Officer

In support of wider collaboration, and to ensure our voice is heard at all levels, the INPDA is a member of EURORDIS and Rare Disease International.

## **INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE**

### **Report of the Trustees for the Year Ended 31 March 2024**

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#### **Objectives and activities**

##### **Key Projects**

The INPDA is currently engaged in a number of key projects. Each project aims to make a difference in the areas of scientific, clinical and therapeutic research, patient care, access to treatments and time to diagnosis.

The International Niemann-Pick Disease Registry (INPDR), which is an independently governed non-profit organisation, has the power to increase knowledge and understanding of NPD through the collection of much needed patient data a global scale. The INPDA was intrinsic in the realisation of this important resource and continues to support its development, by acting as an ambassador, sharing information and supporting the collection of data.

The Think Again. Think NP-C Campaign first launched in 2015, has done much to improve diagnosis of NPC by targeting specialist health care professionals who are currently unfamiliar with the condition and giving them the tools they need to recognise and act upon the symptoms of NPC. We are also currently exploring an opportunity to revitalize this campaign and redevelop a suspicion index tool to support earlier diagnosis for NPC.

The INPDA Information Portal was developed with the support of a Genzyme PAL grant. This open access Portal, which is hosted on the INPDA website, brings together a wide range of helpful information for patients, families, and professionals working in the NPD field, in a range of different languages and formats. The Portal reflects the collaborative spirit of the INPDA and continues to grow along with our respective member groups, who provide articles and documents, and translations where necessary, further boosting the ongoing global effort.

INPDA Biennial Face-to-Face meeting was held in Tarragona Spain with more than 80 delegates present.

Support and planning for the Reuss Valley NPC Scientific Meeting to be held in Switzerland in October 2024. This is a biennial scientific meeting focused on NPC research that supports our goal of building networks and facilitating research in NPC.

##### **Public benefit**

In shaping our objectives for the year, agreeing our priorities 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)'.

As a network for non-profit organisations associated with the group of rare diseases known as Niemann-Pick diseases, we aim to facilitate progress through the provision of an international collaborative forum for the sharing of information and experience regarding all aspects of Niemann-Pick disease, including care and support, the provision and distribution of information and the furtherance of research. We meet virtually every three months and bi-annually face to face, with ad hoc meetings as required.

##### **Benefits of the INPDA**

The INPDA has a global reach; therefore, it is ideally placed to effectively communicate information to the Niemann-Pick community, offering equity of access and ensuring consistency and accuracy of the information provided.

- By sharing information and maximising communication, we can help each organisation to develop their leadership and to use their time and resources efficiently and effectively.
- We aim to ensure that all member organisations are informed of, and if appropriate, able to act upon, the most recent scientific and therapeutic developments.
- Wherever possible, we will help to encourage research by supporting the formation of networks, providing seed funding and working in partnership to secure grant funding.
- By working together, members have a stronger voice with which to influence change and bring improvements for Niemann-Pick patients everywhere.

# INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE

## Report of the Trustees for the Year Ended 31 March 2024

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### Achievement and performance

#### Charitable activities

Following upon our learnings during the COVID 19 Pandemic we have maximised use of virtual technology to keep each other informed and to stay connected. This connection provides member groups the opportunity to share their experiences, stay informed and continue to raise our voice on behalf of those affected by Niemann-Pick diseases.

We have continued to maintain a strong presence in the field of research and have collaborated with other rare disease organisations, research and academic institutions on a global basis to progress research, facilitate clinical development of promising therapies and influence Newborn Screening.

We continue to sustain appropriate relationships with the pharmaceutical companies engaged in clinical programmes and activities in the field of Niemann-Pick diseases and to provide high level support and information to the patient advocacy leaders supporting our community.

We continue to support the International Niemann-Pick Disease Registry (INPDR) in its objectives to collect clinical, genetic, diagnostic and outcome data from patients with Niemann-Pick diseases. The INPDR is an independent charitable company with INPDA representatives appointed as Board and Scientific Advisory Committee Members.

### Financial review

#### Income

Grant funding in the form of unrestricted grants enabled the charity to carry out its activities. Further funding came from the Roy & Murray Green Together Fund. Incoming funds were higher in this year at £214,845 as compared to £72,316 in the previous year.

#### Expenditure

Expenditure for the year was £89,725 as a result of the Face-to-Face Conference in comparison to £57,617 in the previous year.

### Financial Strategy

Our Financial Strategy is based on levels of incoming funds matching the expenditure required to deliver our objectives and activities. Whilst income will be spent within a reasonable period of receipt to fund the operation of the Charity, we have a responsibility to manage risk, and to ensure the charity is financially sustainable. We will therefore hold a level of reserves to ensure the smooth running of the Charity during times of financial uncertainty.

### Reserves policy

The free reserves at 31 March 2024 amounted to £169,059 (2023 £49,543). The Board aim to hold six months running costs in reserve and therefore this figure is considered higher than usual, due to the late receipt of grant funding, resulting in delays to activities which will now extend into the next fiscal year.

### Future plans

At Face-to-Face meeting in November 2023, we agreed on a series of priorities for 2023-2025. Our priorities were agreed as follows:

- To safeguard sustainability by ensuring we have the resources we need (financial resources, personnel, member engagement, skills and capacity) to undertake our planned activities
- To establish support mechanisms for new and developing groups, encourage further support for ASMD, introduce new associate members.
- To strengthen our internal and external communication with regular meetings, use of our web portal, social media, and newsletter
- Continue to raise awareness through ongoing activities and influencing/educating regulators and health policy makers
- Continue to support and facilitate International Niemann Pick Disease Awareness Day on October 19
- To further enable research by providing opportunities to collaborate on research projects, minimize duplication and accelerate progress, explore opportunities for evidence generation such and facilitate development of consensus papers
- To continue our support for the International Niemann-Pick Disease Registry (INPDR) to facilitate the enrolment of patients around the globe into this disease-specific patient registry
- To continue our support for the Reus Valley NPC Research meeting scheduled for October 2024
- Plan for the next INPDA Face-to-Face meeting for September 2025 to be held in Argentina.

### Structure, governance and management

#### Governing document

The charity is governed by its memorandum and articles of association and constitutes a limited company, limited by guarantee, as defined by the Companies Act 2006.

## **INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE**

### **Report of the Trustees for the Year Ended 31 March 2024**

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#### **Structure, governance and management**

##### **Recruitment and appointment of new trustees**

Future Trustees shall be appointed by the Trustees from time to time following a nomination received from the Nominations Committee (Governing Council). They are volunteers and each takes on responsibilities within the Alliance to co-ordinate/support an aspect of the Alliance functions.

##### **Organisational structure**

The Trustees and all other supporters of the INPDA are volunteers. The Trustees are directly responsible for the effective governance of the charity and for providing appropriate support and guidance and monitoring of all activities. This is carried out in accordance with statutory guidance and legislation as provided by the Charity Commission. Members are asked to 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 INPDA. An Executive Committee oversees the day-to-day management of the INPDA and reports regularly to the Full Member Council via quarterly virtual meetings. The Trustees and members meet face-to-face every two years with at least four additional virtual meetings each year in order to review reports and to carry out management and financial reviews.

##### **Induction and training of new trustees**

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

##### **Pay policy for staff**

Currently, the INPDA has no paid staff members. The Trustees are responsible for the day-to-day management of the Charity, with the support of an Executive Committee. All trustees give of their time freely and no trustee received remuneration in the year, outside of travel and subsistence expenses. In the event of employment, the Trustees are 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 small size of the organisation is also taken into account.

##### **Wider network**

The International Niemann-Pick Disease Alliance (INPDA) is an alliance of non-profit support organisations who are associated with the rare group of genetic diseases known collectively as Niemann-Pick Disease. The Alliance was formed with a view to providing a forum where, through the exchange of information, experience and knowledge, progress could be accelerated.

## INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE

### Report of the Trustees for the Year Ended 31 March 2024

---

#### Structure, governance and management

##### Related parties

None of our trustees receive remuneration or other benefit from their work with the charity. At present there are 27 separate foundations in the Alliance.

##### Member Groups:

Argentina - Asociaion Niemann Pick de Argentina  
Australia - Australian NPC Disease Foundation  
Brazil - Associação Niemann Pick Brasil - ANPB  
Canada - Niemann-Pick Canada  
China - China Niemann-Pick Disease Association  
France - Vaincres Les Maladies Lysosomales  
Germany - Niemann-Pick Selbsthilfegruppe  
Italy - Associazione Italiana Niemann Pick  
Netherlands - Volwassenen, Kinderen en Stofwisselingsziekten  
Norway - NP Foreningen I Norge  
Spain - Fundacion Niemann-Pick de Espana  
Switzerland - Niemann-Pick Suisse  
UK - Niemann-Pick UK  
USA - National Niemann-Pick Disease Foundation

##### Associate Members:

Belgium - Belgische Organisatie voor Kinderen en volwassenen met een Stofwisselingsziekte BOKS  
Brazil - Niemann-Pick B R-S  
India - Niemann-Pick India Charitable Trust  
France - Niemann-Pick Francais  
Pakistan - LSD Society Pakistan  
Poland- Stowarzyszenie Chorych na Chorobe Niemannanna Picka i Choroby Rzadkie (Niemann-Pick and Rare Diseases Assoc. Poland)  
Spain - Asociacón Niemann Pick de Fuenlabrada  
Spain - ASMD Espana  
Taiwan - Niemann-Pick Taiwan  
Tunisia - Association Tunisienne des Maladies Lysosomales  
USA - The Wylder Nation Foundation  
USA - Ara Parseghian Medical Research Foundation APMRF  
USA - Firefly Fund

Related party disclosures are shown at note 11.

##### Principal risks

The principal risks for the foreseeable future relate to;

- Increased competition for a reducing number of grant funding opportunities and a limited number of funders
- Further economic and political pressures which will adversely affect voluntary income.
- Lack of capacity to increase the rate of grant applications and income streams.
- A strong dependence on volunteers with no spare capacity to provide cover / plan for succession

##### Statement of trustees' responsibilities

The trustees (who are also the directors of International Niemann-Pick Disease Alliance 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.

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

**Report of the Trustees  
for the Year Ended 31 March 2024**


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**Statement of trustees' responsibilities - continued**

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:



Ms S Cowie - Trustee

## Independent Examiner's Report to the Trustees of International Niemann-Pick Disease Alliance

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### Independent examiner's report to the trustees of International Niemann-Pick Disease Alliance ('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').

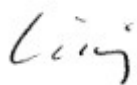
Having satisfied myself that the accounts of the Company are not required to be audited under Part 16 of the 2006 Act and are eligible for independent examination, I report in respect of my examination of your charity's accounts as carried out under Section 145 of the Charities Act 2011 ('the 2011 Act').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

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

24 December 2024

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

	Notes	Unrestricted fund £	Restricted funds £	2024 Total funds £	2023 Total funds £
<b>Income and endowments from</b>					
Donations and legacies		<u>198,363</u>	<u>16,482</u>	<u>214,845</u>	<u>72,316</u>
<b>Expenditure on</b>					
<b>Charitable activities</b>	3				
Meetings		62,047	10,878	72,925	45,484
Charitable activities		<u>16,800</u>	<u>-</u>	<u>16,800</u>	<u>12,133</u>
<b>Total</b>		<u>78,847</u>	<u>10,878</u>	<u>89,725</u>	<u>57,617</u>
<b>NET INCOME</b>		119,516	5,604	125,120	14,699
<b>Reconciliation of funds</b>					
Total funds brought forward		<u>49,543</u>	<u>17,500</u>	<u>67,043</u>	<u>52,344</u>
<b>Total funds carried forward</b>		<u>169,059</u>	<u>23,104</u>	<u>192,163</u>	<u>67,043</u>

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

**Balance Sheet  
31 March 2024**

	Notes	2024 £	2023 £
<b>Current assets</b>			
Debtors	7	-	25,822
Cash at bank		<u>195,246</u>	<u>43,498</u>
		195,246	69,320
<b>Creditors</b>			
Amounts falling due within one year	8	(3,083)	(2,277)
		<u>192,163</u>	<u>67,043</u>
<b>Net current assets</b>			
		192,163	67,043
<b>Total assets less current liabilities</b>		<u>192,163</u>	<u>67,043</u>
<b>NET ASSETS</b>		<u>192,163</u>	<u>67,043</u>
<b>Funds</b>	10		
Unrestricted funds		169,059	49,543
Restricted funds		<u>23,104</u>	<u>17,500</u>
<b>Total funds</b>		<u>192,163</u>	<u>67,043</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

- 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 24 December 2024 and were signed on its behalf by:



S Cowie - Trustee

**1. Statutory information**

International Niemann-Pick Disease Alliance is a private company, limited by guarantee with charitable status and registered in England and Wales. In the event of the charity being wound up, the liability in respect of the guarantee is limited to £1 per member of the charity. The company's registration number and registered office address can be found on the Reference and Administrative Details page.

The presentation currency of the financial statements is the Pound Sterling (£).

**2. 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.

**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.

**Income**

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

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

**Expenditure**

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

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

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

**Taxation**

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

**Fund accounting**

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

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

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

**Debtors and creditors receivable / payable within one year**

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

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

**2. Accounting policies - continued**

**Debtors and creditors receivable / payable within one year  
Cash in bank and in hand**

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

**3. Charitable activities costs**

	<b>Direct Costs (see note 4) £</b>
Meetings	72,925
Charitable activities	<u>16,800</u>
	<u>89,725</u>

**4. Direct costs of charitable activities**

	<b>2024 £</b>	<b>2023 £</b>
Finance & admin costs	7,314	1,653
IT costs	470	195
Meetings, conferences, travel and accommodation	72,925	45,484
Project costs - research	4,302	4,535
Think again: Think NPC	216	269
Social media & communications	-	2,719
Other overheads	3,304	1,694
Independent examiner's fee	<u>1,194</u>	<u>1,068</u>
	<u>89,725</u>	<u>57,617</u>

**5. Trustees' remuneration and benefits**

There were no trustees' remuneration or other benefits for the year ended 31 March 2024 nor for the year ended 31 March 2023.

**Trustees' expenses**

During the period there one trustees was reimbursed expenses of £921 (2023 £567) for travel.

**6. Comparatives for the statement of financial activities**

	<b>Unrestricted fund £</b>	<b>Restricted funds £</b>	<b>Total funds £</b>
<b>Income and endowments from</b>			
Donations and legacies	<u>66,266</u>	<u>6,050</u>	<u>72,316</u>
<b>Expenditure on</b>			
<b>Charitable activities</b>			
Meetings	45,484	-	45,484
Charitable activities	<u>12,133</u>	<u>-</u>	<u>12,133</u>
<b>Total</b>	<u>57,617</u>	<u>-</u>	<u>57,617</u>
<b>NET INCOME</b>	8,649	6,050	14,699
<b>Reconciliation of funds</b>			
Total funds brought forward	40,894	11,450	52,344

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

**6. Comparatives for the statement of financial activities - continued**

	Unrestricted fund £	Restricted funds £	Total funds £
<b>Total funds carried forward</b>	<u>49,543</u>	<u>17,500</u>	<u>67,043</u>

**7. Debtors: amounts falling due within one year**

	2024 £	2023 £
Trade debtors	-	<u>25,822</u>

**8. Creditors: amounts falling due within one year**

	2024 £	2023 £
Other creditors	1,943	1,209
Accruals and deferred income	<u>1,140</u>	<u>1,068</u>
	<u>3,083</u>	<u>2,277</u>

**9. Analysis of net assets between funds**

	Unrestricted fund £	Restricted funds £	2024 Total funds £	2023 Total funds £
Current assets	172,142	23,104	195,246	69,320
Current liabilities	<u>(3,083)</u>	-	<u>(3,083)</u>	<u>(2,277)</u>
	<u>169,059</u>	<u>23,104</u>	<u>192,163</u>	<u>67,043</u>

**10. Movement in funds**

	At 1/4/23 £	Net movement in funds £	At 31/3/24 £
<b>Unrestricted funds</b>			
General fund	49,543	119,516	169,059
<b>Restricted funds</b>			
Roy & Murray Green Together Fund	17,500	3,547	21,047
Brazilian Patient Organisations Travel Fund	-	<u>2,057</u>	<u>2,057</u>
	<u>17,500</u>	<u>5,604</u>	<u>23,104</u>
<b>TOTAL FUNDS</b>	<u>67,043</u>	<u>125,120</u>	<u>192,163</u>

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

**10. Movement in funds - continued**

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

	<b>Incoming resources £</b>	<b>Resources expended £</b>	<b>Movement in funds £</b>
<b>Unrestricted funds</b>			
General fund	198,363	(78,847)	119,516
<b>Restricted funds</b>			
Roy & Murray Green Together Fund	6,882	(3,335)	3,547
Brazilian Patient Organisations Travel Fund	<u>9,600</u>	<u>(7,543)</u>	<u>2,057</u>
	<u>16,482</u>	<u>(10,878)</u>	<u>5,604</u>
<b>TOTAL FUNDS</b>	<u><u>214,845</u></u>	<u><u>(89,725)</u></u>	<u><u>125,120</u></u>

**Comparatives for movement in funds**

	<b>At 1/4/22 £</b>	<b>Net movement in funds £</b>	<b>At 31/3/23 £</b>
<b>Unrestricted funds</b>			
General fund	40,894	8,649	49,543
<b>Restricted funds</b>			
Roy & Murray Green Together Fund	<u>11,450</u>	<u>6,050</u>	<u>17,500</u>
<b>TOTAL FUNDS</b>	<u><u>52,344</u></u>	<u><u>14,699</u></u>	<u><u>67,043</u></u>

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

	<b>Incoming resources £</b>	<b>Resources expended £</b>	<b>Movement in funds £</b>
<b>Unrestricted funds</b>			
General fund	66,266	(57,617)	8,649
<b>Restricted funds</b>			
Roy & Murray Green Together Fund	<u>6,050</u>	<u>-</u>	<u>6,050</u>
<b>TOTAL FUNDS</b>	<u><u>72,316</u></u>	<u><u>(57,617)</u></u>	<u><u>14,699</u></u>

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

The Roy & Murray Green Together Fund is to enhance the development and integration of the INPDA including the support of travel and staffing for the organization.

Brazilian Patient Organisations Travel Fund - to support travel costs of Niemann-Pick B-RS and ANPB patient groups in Brazil to attend INPDA Face 2 Face meetings.

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

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**11. Related party disclosures**

During the year salary, admin and travel costs etc were recharged by a member of the alliance, Niemann-Pick UK (NPUK) of £15,455 (2023 £8,750). Costs of £127 (2023 £243) were incurred on behalf of NPUK. There was a balance owed to NPUK of £1,945 at 31 March 2024 (2023 £965).

The International Niemann-Pick Disease Alliance (INPDA) is a member of the International Niemann-Pick Disease Registry (INPDR). During the year costs were incurred on behalf of the INPDR and reimbursed of £4,422 (2023 £578). Costs were incurred by INPDR on behalf of the INPDA and reimbursed of £3,014 (2023 - £2,920). At 31 March 2024 there was a balance owed by INPDR of £nil (2022 £578).

During the year costs were recharged by a member of the alliance, National Niemann-Pick Disease Foundation of £2,175 (2023 £39,543). There was no balance outstanding at the year end.

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

	<b>2024</b>	<b>2023</b>
	<b>£</b>	<b>£</b>
<b>Income and endowments</b>		
<b>Donations and legacies</b>		
Donations	204,628	72,316
INPDA Biennial Meetings	<u>10,217</u>	<u>-</u>
	<u>214,845</u>	<u>72,316</u>
<b>Total incoming resources</b>	214,845	72,316
<b>Expenditure</b>		
<b>Charitable activities</b>		
Finance & admin costs	7,314	1,653
IT costs	470	195
Meetings, conferences, travel and accommodation	72,925	45,484
Project costs - research	4,302	4,535
Think again: Think NPC	216	269
Social media & communications	-	2,719
Other overheads	3,304	1,694
Independent examiner's fee	<u>1,194</u>	<u>1,068</u>
	<u>89,725</u>	<u>57,617</u>
Total resources expended	<u>89,725</u>	<u>57,617</u>
<b>Net income</b>	<u><u>125,120</u></u>	<u><u>14,699</u></u>

**INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE**

England & Wales - Charity number 1150256

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# Accounts

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**REGISTERED COMPANY NUMBER: 08287609 (England and Wales)**  
**REGISTERED CHARITY NUMBER: 1150256**

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

**Report of the Trustees and**

**Financial Statements for the Year Ended 31 March 2023**

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

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**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

**Reference and Administrative Details  
for the Year Ended 31 March 2023**

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<b>Trustees</b>	Ms S Cowie President Ms J A Crowe Vice-President Mrs T A Mathieson Ms I Hontanilla Ms L Chavez J E Green
<b>Company secretary</b>	Mrs T A Mathieson
<b>Registered office</b>	Suite 2 Vermont House Washington Tyne and Wear NE37 2SQ
<b>Registered company number</b>	08287609 (England and Wales)
<b>Registered charity number</b>	1150256
<b>Independent examiner</b>	James Anderson & Co Chartered Accountants Pentland Estate Straiton Edinburgh EH20 9QH

## **INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE**

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

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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**

##### **Our objectives and aims**

The objects for which the company is established are to relieve sickness amongst families affected by Niemann-Pick diseases, any distress which may arise therefrom and to advance the education of such families, interested professionals and the general public in all matters concerning such diseases as the governing council may in its discretion determine.

The following aims were agreed at the first meeting:

- To enhance progress and maximise the use of resources through international collaboration.
- To share available information.
- To facilitate strong networks of Foundations, Researchers, Clinicians and Industry representatives.
- To identify shared goals and address common challenges.
- To co-ordinate access to clinical trials and therapies.
- To be able to act as an international advocate for those affected these diseases.
- To act as an advocate, speaking with one voice re. health policy.

#### **Review of activities**

The INPDA provides a collaborative forum for the sharing of information and experience regarding all aspects of Niemann-Pick disease, including care and support, the provision and distribution of information and the furtherance of research. Our global network of non-profit organisations working in the field of Niemann-Pick disease (NPD) now extends to 24 members in 18 countries, with members united by a single aim - to improve outcomes for all those affected by Niemann-Pick diseases:

Argentina, Australia, Brazil (2), Belgium, Canada, China, France (2), Germany, Italy, The Netherlands, Norway, Pakistan (1) Spain (3), Switzerland, Taiwan, Tunisia, United Kingdom (2), USA (2).

All our activities are undertaken in line with our aim of facilitating progress in the field of Niemann-Pick diseases. By providing a forum for mutual support, the INPDA aims to establish working links between all non-profit Niemann-Pick support groups and to raise awareness of the nature and operations of these, sometimes very different, organisations.

Our Executive Committee oversees the day-to-day management of the INPDA and reports regularly to the Full Member Council via quarterly teleconferences. This year, we have continued to facilitate progress by:

- Supporting and developing patient advocacy leadership and offering practical advice and support to newly established patient groups.
- Using our shared experience to highlight and promote best practice in patient care and treatment
- Accelerating research through the creation of networks and sharing of information
- Encouraging a cross-sector collaborative approach
- Undertaking global advocacy activities in support of therapy development
- Supporting global interaction and communication
- Fostering the effective use of resources and minimising duplication

# INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE

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

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### Objectives and activities

#### Significant activities

A key element of our work is raising awareness of our collective challenges and the high level of unmet need that exists within the global Niemann-Pick community. Never has the need for awareness of Niemann-Pick diseases been more apparent, and in order to achieve our aim to accelerate progress, there are multiple targets for our awareness efforts, including:

- regulators and the pharmaceutical industry - to assist understanding of the unmet needs of our community and to accelerate progress towards approved therapies
- clinicians - to improve the diagnostic odyssey faced by patients and families
- researchers and scientists - to accelerate research in NPD
- funders - to underline the need for our efforts in advocacy and research to continue
- newborn screening and its potential benefits

Representatives of the INPDA have attended and presented at key conferences around the world, virtually and in person, including patient group events and wider rare disease events.

We have also maintained and further developed our relationships with clinical and research institutions, drug developers and other key stakeholders. The high-level work provides the opportunity for us to advocate for improvements to care and services, access to clinical standards of care, and access to treatments and therapies where they exist.

Representatives have also met with statutory bodies, health authorities and regulators around the world. These essential efforts address issues such as the current regulatory environment, working with pharmaceutical industry and HTA bodies, including;

- Continuing to work with our members to provide information and evidence to support access to available therapies either approved or through Early Access Programmes (EAPs)
- Advocating on behalf of our global community directly with regulators
- Appropriate involvement in regulatory meetings
- Collection of evidence through surveys, information gathering
- The need to provide expert patient advocates who can speak on behalf of the whole patient community in activities to support disease understanding and therapy development, including regulator or industry led advisory boards
- Continuing to work with clinicians and researchers to develop robust tools to measure disease progression and meaningful change, clinical endpoints and patient reported outcomes

This year, we have continued to undertake steps to perform our own community research studies, aiming to collect and develop evidence of impact and unmet need, and to provide information that will increase disease understanding, and support research and therapy development. The data generated by these efforts is providing an excellent resource for the community as we develop a body of knowledge re disease impacts.

Our planned Face-to-Face meeting had been virtual in 2021. We held a Face to Face meeting in 2022 however with lingering travel restrictions still in place due to the COVID pandemic there were some members still unable to attend in person. As a result a hybrid meeting format was utilized to maximize reach of the meeting and to ensure key projects and priorities identified at the 2021 virtual meeting continued to move forward with input from all. This valuable opportunity to come together is essential for our patient advocacy leadership representatives plus key figures in the clinical, research and scientific field of Niemann-Pick diseases. An INPDA Face to Face biennial meeting is planned for November 2023 and this will allow us to return to our regular schedule for biennial meetings and provide an opportunity for all member organizations to participate in person in this important event.

To assist capacity building, we have successfully implemented three of four voluntary Executive Liaison Roles (Research Liaison officer ASMD, Research liaison officer NPC, and Newborn screening liaison officer) and are working to implement the fourth liaison officer role (Membership Liaison Officer) in order to support progress in the INPDA's key priority areas. These roles were recruited internally (from INPDA Member Groups) and externally (individuals / organisations with the necessary experience/interest) and in line with the requirements of our Governing document:

- Research Liaison Officer, ASMD
- Research Liaison Officer, NPC
- Membership Liaison Officer
- Newborn Screening Liaison Officer

In support of wider collaboration, and to ensure our voice is heard at all levels, the INPDA is a member of EURORDIS and Rare Disease International.

## **INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE**

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

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#### **Objectives and activities**

##### **Key Projects**

The INPDA is currently engaged in four key projects. Each aim to make a difference in the areas of scientific, clinical and therapeutic research, patient care, access to treatments and time to diagnosis.

The International Niemann-Pick Disease Registry (INPDR), which is an independently governed non-profit organisation, has the power to increase knowledge and understanding of NPD through the collection of much needed patient data a global scale. The INPDA was intrinsic in the realisation of this important resource and continues to support its development, by acting as an ambassador, sharing information and supporting the collection of data

The Think Again. Think NP-C Campaign first launched in 2015, has done much to improve diagnosis of NPC by targeting specialist health care professionals who are currently unfamiliar with the condition and giving them the tools they need to recognise and act upon the symptoms of NPC.

The INPDA Information Portal was developed with the support of a Genzyme PAL grant. This open access Portal, which is hosted on the INPDA website, brings together a wide range of helpful information for patients, families, and professionals working in the NPD field, in a range of different languages and formats. The Portal reflects the collaborative spirit of the INPDA and continues to grow along with our respective member groups, who provide articles and documents, and translations where necessary, further boosting the ongoing global effort.

##### **Public benefit**

In shaping our objectives for the year, agreeing our priorities 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)'.

As a network for non-profit organisations associated with the group of rare diseases known as Niemann-Pick diseases, we aim to facilitate progress through the provision of an international collaborative forum for the sharing of information and experience regarding all aspects of Niemann-Pick disease, including care and support, the provision and distribution of information and the furtherance of research. We meet virtually every two months and bi-annually face to face, with ad hoc meetings as required.

##### **Benefits of the INPDA**

The INPDA has a global reach; therefore, it is ideally placed to effectively communicate information to the Niemann-Pick community, offering equity of access and ensuring consistency and accuracy of the information provided.

- By sharing information and maximising communication, we can help each organisation to develop their leadership and to use their time and resources efficiently and effectively.
- We aim to ensure that all member organisations are informed of, and if appropriate, able to act upon, the most recent scientific and therapeutic developments.
- Wherever possible, we will help to encourage research by supporting the formation of networks, providing seed funding and working in partnership to secure grant funding.
- By working together, members have a stronger voice with which to influence change and bring improvements for Niemann-Pick patients everywhere.

## **INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE**

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

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#### **Achievement and performance**

##### **Charitable activities**

Grant funding in the form of unrestricted educational grants enabled the charity to carry out its activities. Further funding came from the Roy & Murray Green Together Fund.

Throughout this year, as we continue to work out way out of the impacts of the COVID 19 pandemic we have continued to maximise our use of virtual technology to keep each other informed and to stay connected. In this way we have been able to maintain our activities. Staying connected has provided member groups the opportunity to share their experiences, stay informed and continue to raise our voice on behalf of those affected by Niemann-Pick diseases. As the impacts of the COVID 19 pandemic decrease we are focused on rebuilding opportunities for networking and fundraising that were limited during the pandemic.

We have continued to maintain a strong presence in the field of research and have collaborated with other rare disease organisations, research and academic institutions on a global basis to progress research, facilitate clinical development of promising therapies and influence Newborn Screening.

We continue to sustain appropriate relationships with the pharmaceutical companies engaged in clinical programmes and activities in the field of Niemann-Pick diseases and to provide high level support and information to the patient advocacy leaders supporting our community.

We continued to support the International Niemann-Pick Disease Registry (INPDR) in its objectives to collect clinical, genetic, diagnostic and outcome data from patients with Niemann-Pick diseases. The INPDR is an independent charitable company with INPDA representatives appointed as Board and Scientific Advisory Committee Members.

#### **Financial review**

##### **Financial position**

##### **Income**

Grant funding in the form of unrestricted grants enabled the charity to carry out its activities. Further funding came from the Roy & Murray Green Together Fund. Incoming funds were higher in this year at £72,315 as compared to £20,112 in the previous year.

##### **Expenditure**

Expenditure for the year was £56,716 as a result of the Face to Face Conference in comparison to £10,393 in the previous year.

##### **Financial Strategy**

Our Financial Strategy is based on levels of incoming funds matching the expenditure required to deliver our objectives and activities. Whilst income will be spent within a reasonable period of receipt to fund the operation of the Charity, we have a responsibility to manage risk, and to ensure the charity is financially sustainable. We will therefore hold a level of reserves to ensure the smooth running of the Charity during times of financial uncertainty.

##### **Reserves policy**

The free reserves at 31 March 2023 amounted to £49,543 (2022 £40,894). This reserve is considered a sufficient reserve against variations in funding which can arise from time to time. The board aim is to hold six months running costs in reserve.

## **INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE**

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

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#### **Future plans**

At our Face to Face meeting in July 2022 the priorities established at the virtual meeting in 2021 were reviewed and updated. These will be reviewed again at the Face to Face meeting in November 2023. Our priorities were agreed as follows:

- To safeguard sustainability by ensuring we have the resources we need (financial resources, personnel, member engagement, skills and capacity) to undertake our planned activities
- To establish support mechanisms for new and developing groups, encourage further support for ASMD, introduce new associate members.
- To further develop the new Research and Newborn Screening Liaison Officer roles and strive to implement the Membership Liaison officer role
- Ensure committee structure continues to support progress toward identified priorities
- Communication - Continue to work to strengthen both our internal and external communication with regular meetings, use of our web portal, social media, and newsletter
- Continue to raise awareness through ongoing activities and influencing/educating regulators and health policy makers
- Continue to support and facilitate International Niemann Pick Disease Awareness Day on October 19
- To further enable research by providing opportunities to collaborate on research projects, minimize duplication and accelerate progress
- To continue our work with the International Niemann Pick Disease Registry (INPDR) to facilitate the enrolment of patients around the globe into this built for purpose patient registry
- To plan our next INPDA Face to Face meeting for November 2023

#### **Structure, governance and management**

##### **Governing document**

The charity is governed by its memorandum and articles of association and constitutes a limited company, limited by guarantee, as defined by the Companies Act 2006.

##### **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 (Governing Council). They are volunteers and each takes on responsibilities within the Alliance to co-ordinate/support an aspect of the Alliance functions.

##### **Organisational structure**

The Trustees and all other supporters of the INPDA are volunteers. The Trustees are directly responsible for the effective governance of the charity and for providing appropriate support and guidance and monitoring of all activities. This is carried out in accordance with statutory guidance and legislation as provided by the Charity Commission. Members are asked to 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 INPDA. An Executive Committee oversees the day-to-day management of the INPDA and reports regularly to the Full Member Council via quarterly virtual meetings. The Trustees and members meet face-to-face every two years with at least four additional virtual meetings each year in order to review reports and to carry out management and financial reviews.

##### **Induction and training of new trustees**

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

##### **Pay policy for staff**

Currently, the INPDA has no paid staff members. The Trustees are responsible for the day-to-day management of the Charity, with the support of an Executive Committee. All trustees give of their time freely and no trustee received remuneration in the year, outside of travel and subsistence expenses. In the event of employment, the Trustees are 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 small size of the organisation is also taken into account.

##### **Wider network**

The International Niemann-Pick Disease Alliance (INPDA) is an alliance of non-profit support organisations who are associated with the rare group of genetic diseases known collectively as Niemann-Pick Disease. The Alliance was formed with a view to providing a forum where, through the exchange of information, experience and knowledge, progress could be accelerated.

## **INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE**

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

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#### **Structure, governance and management**

##### **Related parties**

None of our trustees receive remuneration or other benefit from their work with the charity. At present there are 24 separate foundations in the Alliance.

##### **Member Groups:**

Argentina - Asociaion Niemann Pick de Argentina  
Australia - Australian NPC Disease Foundation  
Brazil - Associação Niemann Pick Brasil - ANPB  
Canada - Niemann-Pick Canada  
China - China Niemann-Pick Disease Association  
France - Vaincres Les Maladies Lysosomales  
Germany - Niemann-Pick Selbsthilfegruppe  
Italy - Associazione Italiana Niemann Pick  
Netherlands - Volwassenen, Kinderen en Stofwisselingsziekten  
Norway - NP Foreningen I Norge  
Spain - Fundacion Niemann-Pick de Espana  
Switzerland - Niemann-Pick Suisse  
UK - Niemann-Pick UK  
USA - National Niemann-Pick Disease Foundation

##### **Associate Members:**

Brazil - Niemann-Pick B R-S  
Belgium- Belgische Organisatie voor Kinderen in volwassenen met een Stofwisselingsziekte  
France - Niemann Pick Francais  
Pakistan - LSD Society Pakistan  
Spain - Asociación Niemann Pick de Fuenlabrada  
Spain - ASMD Espana  
Taiwan - Niemann-Pick Taiwan  
Tunisia - Association Tunisienne des Maladies Lysosomales  
UK - The Hollie Foundation  
USA - The Wylder Nation Foundation

Related party disclosures are shown at note 12.

##### **Principal risks**

The principal risks for the foreseeable future relate to;

- Increased competition for a reducing number of grant funding opportunities and a limited number of funders
- Further economic and political pressures which will adversely affect voluntary income.
- Lack of capacity to increase the rate of grant applications and income streams.
- A strong dependence on volunteers with no spare capacity to provide cover / plan for succession

##### **Statement of trustees' responsibilities**

The trustees (who are also the directors of International Niemann-Pick Disease Alliance 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  
ALLIANCE**

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

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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 20 December 2023 and signed on its behalf by:



.....  
Ms S Cowie – Trustee, President

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

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**Independent examiner's report to the trustees of International Niemann-Pick Disease Alliance ('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').

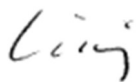
Having satisfied myself that the accounts of the Company are not required to be audited under Part 16 of the 2006 Act and are eligible for independent examination, I report in respect of my examination of your charity's accounts as carried out under Section 145 of the Charities Act 2011 ('the 2011 Act').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

Date: 21 December 2023

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

	Notes	Unrestricted fund £	Restricted funds £	2023 Total funds £	2022 Total funds £
<b>Income and endowments from</b>					
Donations and legacies		66,265	6,050	72,315	19,813
Other trading activities	3	-	-	-	299
<b>Total</b>		<u>66,265</u>	<u>6,050</u>	<u>72,315</u>	<u>20,112</u>
<b>Expenditure on</b>					
<b>Charitable activities</b>					
Meetings	4	45,483	-	45,483	-
Charitable activities		<u>12,133</u>	-	<u>12,133</u>	<u>10,393</u>
<b>Total</b>		<u>57,616</u>	-	<u>57,616</u>	<u>10,393</u>
<b>NET INCOME</b>		8,649	6,050	14,699	9,719
<b>Reconciliation of funds</b>					
Total funds brought forward		40,894	11,450	52,344	42,625
<b>Total funds carried forward</b>		<u>49,543</u>	<u>17,500</u>	<u>67,043</u>	<u>52,344</u>

The notes form part of these financial statements

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

**Balance Sheet  
31 March 2023**

	Notes	2023 £	2022 £
<b>Current assets</b>			
Debtors	8	25,822	101
Cash at bank		<u>43,498</u>	<u>54,267</u>
		69,320	54,368
<b>Creditors</b>			
Amounts falling due within one year	9	(2,277)	(2,024)
		<u>67,043</u>	<u>52,344</u>
<b>Net current assets</b>			
		<u>67,043</u>	<u>52,344</u>
<b>Total assets less current liabilities</b>		<u>67,043</u>	<u>52,344</u>
<b>NET ASSETS</b>		<u>67,043</u>	<u>52,344</u>
<b>Funds</b>	11		
Unrestricted funds		49,543	40,894
Restricted funds		<u>17,500</u>	<u>11,450</u>
<b>Total funds</b>		<u>67,043</u>	<u>52,344</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 20 December 2023 and were signed on its behalf by:



.....  
S Cowie – Trustee, President

# INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE

## Notes to the Financial Statements for the Year Ended 31 March 2023

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### 1. Statutory information

International Niemann-Pick Disease Alliance is a private company, limited by guarantee with charitable status and registered in England and Wales. In the event of the charity being wound up, the liability in respect of the guarantee is limited to £1 per member of the charity. The company's registration number and registered office address can be found on the Reference and Administrative Details page.

The presentation currency of the financial statements is the Pound Sterling (£).

### 2. 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.

#### 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.

#### Income

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

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

#### Expenditure

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

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

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

#### Taxation

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

#### Fund accounting

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

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

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

#### Debtors and creditors receivable / payable within one year

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

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

**2. Accounting policies - continued**

**Cash in bank and in hand**

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

**3. Other trading activities**

	<b>2023</b>	<b>2022</b>
	<b>£</b>	<b>£</b>
Fundraised income	<u>-</u>	<u>299</u>

**4. Charitable activities costs**

	<b>Direct Costs (see note 5)</b>
	<b>£</b>
Meetings	45,483
Charitable activities	<u>12,133</u>
	<u>57,616</u>

**5. Direct costs of charitable activities**

	<b>2023</b>	<b>2022</b>
	<b>£</b>	<b>£</b>
Finance & admin costs	1,653	1,943
IT costs	195	211
Face to face meetings - all INPDA members	42,875	-
Meetings, conferences, travel and accommodation	2,608	-
Project costs - research	4,535	1,670
Think again: Think NPC	269	332
Social media & communications	2,719	2,976
Other overheads	1,694	2,193
Independent examiner's fee	<u>1,068</u>	<u>1,068</u>
	<u>57,616</u>	<u>10,393</u>

**6. Trustees' remuneration and benefits**

There were no trustees' remuneration or other benefits for the year ended 31 March 2023 nor for the year ended 31 March 2022.

**Trustees' expenses**

During the period there were Trustees expenses of £567 (2022 nil) for travel.

**7. Comparatives for the statement of financial activities**

	<b>Unrestricted fund</b>	<b>Restricted funds</b>	<b>Total funds</b>
	<b>£</b>	<b>£</b>	<b>£</b>
<b>Income and endowments from</b>			
Donations and legacies	14,000	5,813	19,813
Other trading activities	<u>299</u>	<u>-</u>	<u>299</u>
<b>Total</b>	<u>14,299</u>	<u>5,813</u>	<u>20,112</u>

**Expenditure on**

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

**7. Comparatives for the statement of financial activities - continued**

	Unrestricted fund £	Restricted funds £	Total funds £
<b>Charitable activities</b>			
Charitable activities	<u>10,393</u>	-	<u>10,393</u>
<b>NET INCOME</b>	3,906	5,813	9,719
<b>Reconciliation of funds</b>			
Total funds brought forward	<u>36,988</u>	<u>5,637</u>	<u>42,625</u>
<b>Total funds carried forward</b>	<u>40,894</u>	<u>11,450</u>	<u>52,344</u>

**8. Debtors: amounts falling due within one year**

	2023 £	2022 £
Trade debtors	<u>25,822</u>	<u>101</u>

**9. Creditors: amounts falling due within one year**

	2023 £	2022 £
Other creditors	1,209	956
Accruals and deferred income	<u>1,068</u>	<u>1,068</u>
	<u>2,277</u>	<u>2,024</u>

**10. Analysis of net assets between funds**

	Unrestricted fund £	Restricted funds £	2023 Total funds £	2022 Total funds £
Current assets	51,820	17,500	69,320	54,368
Current liabilities	<u>(2,277)</u>	-	<u>(2,277)</u>	<u>(2,024)</u>
	<u>49,543</u>	<u>17,500</u>	<u>67,043</u>	<u>52,344</u>

**11. Movement in funds**

	At 1/4/22 £	Net movement in funds £	At 31/3/23 £
<b>Unrestricted funds</b>			
General fund	40,894	8,649	49,543
<b>Restricted funds</b>			
Roy & Murray Green Together Fund	<u>11,450</u>	<u>6,050</u>	<u>17,500</u>
<b>TOTAL FUNDS</b>	<u>52,344</u>	<u>14,699</u>	<u>67,043</u>

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

**11. Movement in funds - continued**

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

	<b>Incoming resources £</b>	<b>Resources expended £</b>	<b>Movement in funds £</b>
<b>Unrestricted funds</b>			
General fund	66,265	(57,616)	8,649
<b>Restricted funds</b>			
Roy & Murray Green Together Fund	6,050	-	6,050
	<u>72,315</u>	<u>(57,616)</u>	<u>14,699</u>
<b>TOTAL FUNDS</b>	<u>72,315</u>	<u>(57,616)</u>	<u>14,699</u>

**Comparatives for movement in funds**

	<b>At 1/4/21 £</b>	<b>Net movement in funds £</b>	<b>At 31/3/22 £</b>
<b>Unrestricted funds</b>			
General fund	36,988	3,906	40,894
<b>Restricted funds</b>			
Roy & Murray Green Together Fund	5,637	5,813	11,450
	<u>42,625</u>	<u>9,719</u>	<u>52,344</u>
<b>TOTAL FUNDS</b>	<u>42,625</u>	<u>9,719</u>	<u>52,344</u>

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

	<b>Incoming resources £</b>	<b>Resources expended £</b>	<b>Movement in funds £</b>
<b>Unrestricted funds</b>			
General fund	14,299	(10,393)	3,906
<b>Restricted funds</b>			
Roy & Murray Green Together Fund	5,813	-	5,813
	<u>20,112</u>	<u>(10,393)</u>	<u>9,719</u>
<b>TOTAL FUNDS</b>	<u>20,112</u>	<u>(10,393)</u>	<u>9,719</u>

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

The Roy & Murray Green Together Fund is to enhance the development and integration of the INPDA including the support of travel and staffing for the organization.

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

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

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**12. Related party disclosures**

During the year salary, admin and travel costs etc were recharged by a member of the alliance, Niemann-Pick UK(NPUK) of £8,750 (2022 £6,528). Costs of £243 were incurred on behalf of NPUK. There was a balance owed to NPUK of £965 at 31 March 2023 (2022 £401).

The International Niemann-Pick Disease Alliance (INPDA) is a member of the International Niemann-Pick Disease Registry (INPDR). During the year costs were incurred on behalf of the INPDR and reimbursed of £578 (2022 £764). Costs were incurred by INPDR on behalf of the INPDA and reimbursed of £2,920 (2022 - £nil). At 31 March 2023 there was a balance owed by INPDR of £578 (2022 £nil).

During the year costs were recharged by a member of the alliance, National Niemann-Pick Disease Foundation of £39,543 (2022 £1,288). Of this £39,182 was in relation to the costs of the 2022 Face to Face Conference. There was no balance outstanding at the year end.

**INTERNATIONAL NIEMANN-PICK DISEASE  
ALLIANCE**

**Detailed Statement of Financial Activities  
for the Year Ended 31 March 2023**

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	<b>2023</b>	<b>2022</b>
	<b>£</b>	<b>£</b>
<b>Income and endowments</b>		
<b>Donations and legacies</b>		
Donations	72,315	19,813
<b>Other trading activities</b>		
Fundraised income	<u>-</u>	<u>299</u>
<b>Total incoming resources</b>	72,315	20,112
<b>Expenditure</b>		
<b>Charitable activities</b>		
Finance & admin costs	1,653	1,943
IT costs	195	211
Face to face meetings - all INPDA members	42,875	-
Meetings, conferences, travel and accommodation	2,608	-
Project costs - research	4,535	1,670
Think again: Think NPC	269	332
Social media & communications	2,719	2,976
Other overheads	1,694	2,193
Independent examiner's fee	<u>1,068</u>	<u>1,068</u>
	<u>57,616</u>	<u>10,393</u>
Total resources expended	<u>57,616</u>	<u>10,393</u>
<b>Net income</b>	<u>14,699</u>	<u>9,719</u>

**INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE**

England & Wales - Charity number 1150256

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# Accounts

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**REGISTERED COMPANY NUMBER: 08287609 (England and Wales)**  
**REGISTERED CHARITY NUMBER: 1150256**

**INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE**

**(A COMPANY LIMITED BY GUARANTEE)**

**Report of the Trustees and**

**Financial Statements for the Year Ended 31 March 2021**

**INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE**

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

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<b>Independent Examiner's Report</b>	7
<b>Statement of Financial Activities</b>	8
<b>Balance Sheet</b>	9
<b>Notes to the Financial Statements</b>	10 to 13
<b>Detailed Statement of Financial Activities</b>	14

## INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE

### Reference and Administrative Details for the Year Ended 31 March 2021

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<b>Trustees</b>	Ms S Cowie President Ms J A Crowe Vice-President (appointed 4.10.20) Mrs T A Mathieson Ms I Hontanilla Ms L Chavez (appointed 4.10.20) J E Green
<b>Company secretary</b>	Mrs T A Mathieson
<b>Registered office</b>	Suite 2 Vermont House Washington Tyne and Wear NE37 2SQ
<b>Registered company number</b>	08287609 (England and Wales)
<b>Registered charity number</b>	1150256
<b>Independent examiner</b>	Christopher Spalding James Anderson & Co Chartered Accountants Pentland Estate Straiton Edinburgh EH20 9QH

## INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE

### Report of the Trustees for the Year Ended 31 March 2021

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The trustees who are also directors of the charity for the purposes of the Companies Act 2006, present their report with the financial statements of the charity for the year ended 31 March 2021. The trustees have adopted the provisions of Accounting and Reporting by Charities: Statement of Recommended Practice applicable to charities preparing their accounts in accordance with the Financial Reporting Standard applicable in the UK and Republic of Ireland (FRS 102) (effective 1 January 2019).

#### Objectives and activities

##### Our objectives and aims

The objects for which the company is established are to relieve sickness amongst families affected by Niemann-Pick diseases, any distress which may arise therefrom and to advance the education of such families, interested professionals and the general public in all matters concerning such diseases as the governing council may in its discretion determine.

The following aims were agreed at the first meeting:

- To enhance progress and maximise the use of resources through international collaboration.
- To share available information.
- To facilitate strong networks of Foundations, Researchers, Clinicians and Industry representatives.
- To identify shared goals and address common challenges.
- To co-ordinate access to clinical trials and therapies.
- To be able to act as an international advocate for those affected these diseases.
- To act as an advocate, speaking with one voice re. health policy.

#### Significant activities

At our Face to Face Meeting in Lyon 2019, the Governing Council agreed the following priorities for the period June 2019-July 2021. Although progress towards achieving our priorities has been significantly slowed during this financial year, due to the COVID-19 pandemic, we have been able to move forward positively in the areas of communication, enabling research and raising awareness:

**Sustainability** – We need to ensure we have the personnel and financial resources, skills and capacity to undertake our planned activities, therefore we will:

- Further develop and strengthen our Board and Staff Team
- Increase fundraising and grant applications

**Membership** – As we grow, we will:

- Establish support mechanisms for new and developing groups
- Encourage participation in our networks, the sharing of information through the INPDA Information Portal
- Increase opportunities to learn from each other, share experience and mentor newly formed groups
- Expand support to the ASMD community in countries where this is not currently available

**Communication** – We will continue to work to strengthen both our internal and external communication, by:

- Maximising use of technology to support communication
- Hosting regular and interactive meetings
- Establishing a newsletter
- Maintaining appropriate relationships with industry and researchers
- Encouraging attendance at member group and external meetings
- Sharing information regarding scientific symposia
- Participating in Eurordis and RDI activities
- Maintaining the INPDA Information Portal, website and social media channels

**Raising awareness** - Through ongoing awareness activities, we will work to influence positive change and improvement to care, treatment and services for our global community, including:

- Supporting and further developing an annual Niemann-Pick awareness day on October 19
- Establishing an Expert Patient Board
- Raising our voice through social media and on-line activities
- Maintaining the Think Again. Think NPC Campaign
- Developing an ASMD awareness Campaign

## INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE

### Report of the Trustees for the Year Ended 31 March 2021

---

**Enabling Research** - By sharing information, providing networking opportunities and supporting key initiatives, including:

- Supporting the ongoing development and implementation of the International Niemann-Pick Disease Registry (INPDR)
- Encouraging collaboration on research projects, to minimize duplication and accelerate progress
- Supporting Newborn Screening initiatives
- Interacting with regulators and health policy makers
- Developing global networks
- Supporting the Gene Therapy Activation and Loire Valley meetings
- Actively seeking and sharing relevant information with our membership
- Attend relevant conferences and symposia

#### **Key Projects**

The INPDA currently undertakes a number of key activities aiming to make a difference in the areas of scientific, clinical and therapeutic research, patient care, access to treatments and time to diagnosis.

The International Niemann-Pick Disease Registry (INPDR), which is an independently governed non-profit organisation, has the power to increase knowledge and understanding of NPD through the collection of much needed patient data on a global scale. The INPDA was intrinsic in the realisation of this important resource and aims to support its continued development.

The Think Again. Think NPC Campaign aims to improve diagnosis of NPC by targeting specialist health care professionals who are currently unfamiliar with the condition and giving them the tools they need to recognise and act upon the symptoms of NPC.

The INPDA Information Portal is an open access and hosted on the INPDA website, it brings together a wide range of helpful information for patients, families, and professionals working in the Niemann-Pick field, in a range of different languages and formats. The Portal reflects the collaborative spirit of the INPDA and continues to grow along with our respective member groups, who provide articles and documents, and translations where necessary, further boosting the ongoing global effort.

#### **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)'.

We are a network for non-profit organisations associated with the group of rare diseases known as Niemann-Pick Disease. We aim to facilitate progress through the provision of an international collaborative forum for the sharing of information and experience regarding all aspects of Niemann-Pick disease, including care and support, the provision and distribution of information and the furtherance of research. We meet virtually every three months and bi-annually face to face, with an Executive Committee overseeing the day-to-day management of the organisation.

## **INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE**

### **Report of the Trustees for the Year Ended 31 March 2021**

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#### **Achievement and performance**

##### **Charitable activities**

Various grants were received from funders in the year including Mallinckrodt ARD to enable the charity to carry out its activities.

Throughout this year our Board and membership have worked hard to help our vulnerable community to cope with the impact of the COVID-19 pandemic. We have maximised use of virtual technology to keep each other informed and to stay connected. In this way we have been able to maintain our activities but not without effects on our ability to network and to raise funds.

Staying connected has provided member groups the opportunity to share their experiences during this difficult time and has demonstrated the resilience of our community spirit as we continue to raise our voice on behalf of those affected by Niemann-Pick diseases.

We have continued to maintain a strong presence in the field of research and have collaborated with other rare disease organisations, research and academic institutions on a global basis to progress research, facilitate clinical development of promising therapies and influence Newborn Screening.

We continue to sustain appropriate relationships with the pharmaceutical companies engaged in clinical programmes and activities in the field of Niemann-Pick diseases and to provide high level support and information to the patient advocacy leaders supporting our community.

We continued to support the International Niemann-Pick Disease Registry (INPDR) in its objectives to collect clinical, genetic, diagnostic and outcome data from patients with Niemann-Pick diseases. The INPDR is an independent charitable company with INPDA representatives appointed as Board and Scientific Advisory Committee Members.

#### **Financial review**

##### **Reserves policy**

The free reserves at 31 March 2021 amounted to £36,988 (2020 £6,821). This reserve is considered a sufficient reserve against variations in funding which can arise from time to time. The board aim is to hold six months running costs in reserve. The 2020 figure of £15,364 is considered a more realistic benchmark as 2021 costs were much reduced due to Covid.

#### **Future plans**

Future Plans - priorities for the year ahead include

Sustainability - Organisational strengthening and development, to ensure we have the resources we need (financial resources, personnel, member engagement, skills and capacity) to undertake our planned activities

Membership - To further establish support mechanisms for new and developing groups, encourage and develop support for ASMD communities and introduce new associate members.

Communication - Continue to work to strengthen both our internal and external communication with regular meetings, use of our website, Information Portal and social media (INPDA.org, @INPDA), and newsletter

Raising awareness- Through ongoing awareness activities and interactions with regulators and health policy makers, plus support for the Think Again. Think NPC Campaign, and the development of specific ASMD awareness activities and support for the International Niemann Pick Disease Awareness Day on October 19

Enable Research - Support and facilitate global research and therapeutic development, by providing opportunities to collaborate on research projects, minimize duplication and accelerate progress

International Niemann Pick Disease Registry - Work with the INPDR to facilitate the enrolment of patients around the globe into this new built for purpose patient registry

#### **Structure, governance and management**

##### **Governing document**

The charity is governed by its memorandum and articles of association and constitutes a limited company, limited by guarantee, as defined by the Companies Act 2006.

##### **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 (Governing Council). They are volunteers and each takes on responsibilities within the Alliance to co-ordinate/support an aspect of the Alliance functions.

## **INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE**

### **Report of the Trustees for the Year Ended 31 March 2021**

---

#### **Organisational structure**

The Trustees and all the other supporters of the Organisation are volunteers.

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

In order to achieve our identified priorities, a committee structure has been introduced to support the work involved. Committees will meet virtually at least once between each council meeting. The committee structure is intended to encourage the involvement of INPDA member organizations. Initially, four committees will be established and will report progress regularly to the Governing Council:

- Finance
- Communication
- Organization and membership
- Research

#### **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.

#### **Pay policy for staff**

The trustees are the key management. All trustees give of their time freely and no trustee received remuneration in the year. The Trustees are 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 small size of the organisation is also taken into account.

#### **Wider network**

The International Niemann-Pick Disease Alliance (INPDA) is an alliance of non-profit support organisations who are associated with the rare group of genetic diseases known collectively as Niemann-Pick Disease. The Alliance was formed with a view to providing a forum where, through the exchange of information, experience and knowledge, progress could be accelerated.

## INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE

### Report of the Trustees for the Year Ended 31 March 2021

---

#### Structure, governance and management

##### Related parties

None of our trustees receive remuneration or other benefit from their work with the charity.

At present there are 23 separate foundations in the Alliance.

##### Member Groups:

Argentina - Asociaion Niemann Pick de Argentina  
Australia - Australian NPC Disease Foundation  
Brazil - Associação Niemann Pick Brasil - ANPB  
Canada - Niemann-Pick Canada  
China - China Niemann-Pick Disease Association  
France - Vaincres Les Maladies Lysosomales  
Germany - Niemann-Pick Selbsthilfegruppe  
Italy - Associazione Italiana Niemann Pick  
Netherlands - Volwassenen, Kinderen en Stofwisselingsziekten  
Norway - NP Foreningen I Norge  
Spain - Fundacion Niemann-Pick de Espana  
Switzerland - Niemann-Pick Suisse  
UK - Niemann-Pick UK  
USA - National Niemann-Pick Disease Foundation

##### Associate Members:

Brazil - Niemann-Pick B R-S  
France - Niemann Pick Francais  
Pakistan - LSD Society Pakistan  
Spain - Asociacion Niemann Pick de Fuenlabrada  
Spain - ASMD Espana  
Taiwan - Niemann-Pick Taiwan  
Tunisia - Association Tunisienne des Maladies Lysosomales  
UK - The Hollie Foundation  
USA - The Wylder Nation Foundation

Related party disclosures are shown at note 10.

##### Risk management

The trustees have identified and addressed the main risk which is the financial risk arising from our low level of funding and our limited number of funders.

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.

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 13 December 2021 and signed on its behalf by:



Mrs T A Mathieson – Trustee

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

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**Independent examiner's report to the trustees of International Niemann-Pick Disease Alliance ('the Company')**  
I report to the charity trustees on my examination of the accounts of the Company for the year ended 31 March 2021.

**Responsibilities and basis of report**

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

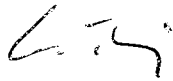
Having satisfied myself that the accounts of the Company are not required to be audited under Part 16 of the 2006 Act and are eligible for independent examination, I report in respect of my examination of your charity's accounts as carried out under section 145 of the Charities Act 2011 ('the 2011 Act').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

**Independent examiner's statement**

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 Spalding (ICAS)  
James Anderson & Co  
Chartered Accountants  
Pentland Estate  
Straiton  
EH20 9QH

Date: 13 December 2021

**INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE**

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

	Notes	Unrestricted fund £	Restricted funds £	2021 Total funds £	2020 Total funds £
<b>Income and endowments from</b>					
Donations and legacies		37,040	3,367	40,407	34,178
Other trading activities	3	<u>1,179</u>	<u>-</u>	<u>1,179</u>	<u>1,250</u>
<b>Total</b>		38,219	3,367	41,586	35,428
<b>Expenditure on</b>					
Raising funds		20	-	20	396
<b>Charitable activities</b>					
Charitable activities		8,032	-	8,032	33,418
<b>Total</b>		<u>8,052</u>	<u>-</u>	<u>8,052</u>	<u>33,814</u>
<b>NET INCOME</b>		30,167	3,367	33,534	1,614
<b>Reconciliation of funds</b>					
<b>Total funds brought forward</b>		<u>6,821</u>	<u>2,270</u>	<u>9,091</u>	<u>7,477</u>
<b>Total funds carried forward</b>		<u><u>36,988</u></u>	<u><u>5,637</u></u>	<u><u>42,625</u></u>	<u><u>9,091</u></u>

INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE

Balance Sheet  
31 March 2021

	Notes	2021 £	2020 £
<b>Current assets</b>			
Debtors	6	22	1,241
Cash at bank		<u>43,764</u>	<u>11,671</u>
		43,786	12,912
<b>Creditors</b>			
Amounts falling due within one year	7	<u>(1,161)</u>	<u>(3,821)</u>
<b>Net current assets</b>		<u>42,625</u>	<u>9,091</u>
<b>Total assets less current liabilities</b>		<u>42,625</u>	<u>9,091</u>
<b>NET ASSETS</b>		<u>42,625</u>	<u>9,091</u>
<b>Funds</b>	9		
Unrestricted funds		36,988	6,821
Restricted funds		<u>5,637</u>	<u>2,270</u>
<b>Total funds</b>		<u>42,625</u>	<u>9,091</u>

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

The members have not required the company to obtain an audit of its financial statements for the year ended 31 March 2021 in accordance with Section 476 of the Companies Act 2006.

The trustees acknowledge their responsibilities for

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

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

The financial statements were approved by the Board of Trustees and authorised for issue on 13 December 2021 and were signed on its behalf by:

  
L.A. Mathieson – Trustee

## INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE

### Notes to the Financial Statements for the Year Ended 31 March 2021

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#### 1. Statutory Information

International Niemann-Pick Disease Alliance is a private company, limited by guarantee with charitable status and registered in England and Wales. In the event of the charity being wound up, the liability in respect of the guarantee is limited to £1 per member of the charity. The company's registration number and registered office address can be found on the Reference and Administrative Details page.

The presentation currency of the financial statements is the Pound Sterling (£).

#### 2. 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.

##### **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.

##### **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.

**INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE**

**Notes to the Financial Statements - continued  
for the Year Ended 31 March 2021**

**2. Accounting policies - continued**

**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.

**3. Other trading activities**

	<b>2021</b>	<b>2020</b>
	<b>£</b>	<b>£</b>
Fundraised income	<u>1,179</u>	<u>1,250</u>

**4. Trustees' remuneration and benefits**

There were no trustees' remuneration or other benefits for the year ended 31 March 2021 nor for the year ended 31 March 2020.

**Trustees' expenses**

During the period the Trustees were reimbursed £nil (2020 £1,082) for travel and accommodation expenses.

**5. Comparatives for the statement of financial activities**

	<b>Unrestricted fund £</b>	<b>Restricted funds £</b>	<b>Total funds £</b>
<b>Income and endowments from</b>			
Donations and legacies	20,000	14,178	34,178
Other trading activities	<u>1,250</u>	-	<u>1,250</u>
<b>Total</b>	21,250	14,178	35,428
<b>Expenditure on</b>			
Raising funds	396	-	396
<b>Charitable activities</b>			
Charitable activities	18,425	14,993	33,418
<b>Total</b>	<u>18,821</u>	<u>14,993</u>	<u>33,814</u>
<b>NET INCOME/(EXPENDITURE)</b>	2,429	(815)	1,614
<b>Reconciliation of funds</b>			
<b>Total funds brought forward</b>	4,392	3,085	7,477
<b>Total funds carried forward</b>	<u>6,821</u>	<u>2,270</u>	<u>9,091</u>

INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE

Notes to the Financial Statements - continued  
for the Year Ended 31 March 2021

<b>6. Debtors: amounts falling due within one year</b>			<b>2021</b>	<b>2020</b>
			£	£
Trade debtors			<u>22</u>	<u>1,241</u>
<b>7. Creditors: amounts falling due within one year</b>			<b>2021</b>	<b>2020</b>
			£	£
Other creditors			93	1,253
Accruals and deferred income			<u>1,068</u>	<u>2,568</u>
			<u>1,161</u>	<u>3,821</u>
<b>8. Analysis of net assets between funds</b>			<b>2021</b>	<b>2020</b>
	<b>Unrestricted fund</b>	<b>Restricted funds</b>	<b>Total funds</b>	<b>Total funds</b>
	£	£	£	£
Current assets	38,149	5,637	43,786	12,912
Current liabilities	<u>(1,161)</u>	<u>-</u>	<u>(1,161)</u>	<u>(3,821)</u>
	<u>36,988</u>	<u>5,637</u>	<u>42,625</u>	<u>9,091</u>
<b>9. Movement in funds</b>			<b>Net movement in funds</b>	<b>At 31/3/21</b>
		<b>At 1/4/20</b>	<b>£</b>	<b>£</b>
<b>Unrestricted funds</b>				
General fund		6,821	30,167	36,988
<b>Restricted funds</b>				
Roy & Murray Green Together Fund		2,270	3,367	5,637
		<u>9,091</u>	<u>33,534</u>	<u>42,625</u>
<b>TOTAL FUNDS</b>				
Net movement in funds, included in the above are as follows:				
		<b>Incoming resources</b>	<b>Resources expended</b>	<b>Movement in funds</b>
		£	£	£
<b>Unrestricted funds</b>				
General fund		38,219	(8,052)	30,167
<b>Restricted funds</b>				
Roy & Murray Green Together Fund		3,367	-	3,367
		<u>41,586</u>	<u>(8,052)</u>	<u>33,534</u>
<b>TOTAL FUNDS</b>				

**INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE**

**Notes to the Financial Statements - continued  
for the Year Ended 31 March 2021**

**9. Movement in funds - continued**

**Comparatives for movement in funds**

	At 1/4/19 £	Net movement in funds £	At 31/3/20 £
<b>Unrestricted funds</b>			
General fund	4,392	2,429	6,821
<b>Restricted funds</b>			
New Member Travel	3,085	(3,085)	-
Roy & Murray Green Together Fund	<u>-</u>	<u>2,270</u>	<u>2,270</u>
	<u>3,085</u>	<u>(815)</u>	<u>2,270</u>
<b>TOTAL FUNDS</b>	<u><u>7,477</u></u>	<u><u>1,614</u></u>	<u><u>9,091</u></u>

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

	Incoming resources £	Resources expended £	Movement in funds £
<b>Unrestricted funds</b>			
General fund	21,250	(18,821)	2,429
<b>Restricted funds</b>			
New Member Travel	-	(3,085)	(3,085)
Loire Valley Meetings	11,908	(11,908)	-
Roy & Murray Green Together Fund	<u>2,270</u>	<u>-</u>	<u>2,270</u>
	<u>14,178</u>	<u>(14,993)</u>	<u>(815)</u>
<b>TOTAL FUNDS</b>	<u><u>35,428</u></u>	<u><u>(33,814)</u></u>	<u><u>1,614</u></u>

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

The Roy & Murray Green Together Fund is to support travel by representatives of INDPA member organisations in order to stimulate understanding, collaboration and friendship.

The New Member travel fund is towards new member travel to allow face to face meetings.

The Loire Valley meeting fund is towards the Loire Valley meeting.

**10. Related party disclosures**

During the year costs were recharged by a member of the alliance, Niemann-Pick UK, for administration, social media, office and travel costs etc of £5,361 (2020 £10,798). There was a balance owed to Niemann-Pick UK of £Nil at 31 March 2021 (2020 £1,253).

The International Niemann-Pick Disease Alliance is a member of the International Niemann-Pick Disease Registry (INPDR). During the year costs were incurred on behalf of the INPDR and reimbursed of £1,696 (2020 £4,477). At 31 March 2021 there was a balance owed by INPDR of £22 (2020 £1,241).

**INTERNATIONAL NIEMANN-PICK DISEASE ALLIANCE****Detailed Statement of Financial Activities  
for the Year Ended 31 March 2021**

	2021 £	2020 £
<b>Income and endowments</b>		
<b>Donations and legacies</b>		
Donations	40,407	34,178
<b>Other trading activities</b>		
Fundraised income	<u>1,179</u>	<u>1,250</u>
<b>Total incoming resources</b>	41,586	35,428
<b>Expenditure</b>		
<b>Raising donations and legacies</b>		
Fundraising costs	20	396
<b>Charitable activities</b>		
Finance & admin costs	2,335	2,270
IT costs	79	-
Face to face meetings - all INPDA members	-	23,267
Meetings, conferences, travel and accommodation	204	2,951
Think again: Think NPC	330	610
Social media & communications	2,639	2,027
Other overheads	1,044	1,225
Independent examiner's fee	1,068	1,068
Legal fees	<u>333</u>	<u>-</u>
	<u>8,032</u>	<u>33,418</u>
<b>Total resources expended</b>	<u>8,052</u>	<u>33,814</u>
<b>Net income</b>	<u>33,534</u>	<u>1,614</u>