



NIEMANN-PICK UK

Company number 07775835

Charity registration numbers

1144406 (England & Wales)

SC045407 (Scotland)

REPORT AND FINANCIAL STATEMENTS

FOR THE YEAR ENDED 30 APRIL 2024

NPUK (Niemann-Pick UK) is a charitable organisation dedicated to making a positive difference to the lives of those affected by Niemann-Pick diseases their families and the health professionals caring for them. We raise much needed awareness, provide practical and emotional support, advice, information, and facilitate research into potential therapies.

We are the only UK patient group to provide specialist support to those affected by Niemann-Pick diseases and over 32 years, we have developed valuable insights into living with these conditions and the challenges they present.

- Our desired impact is to improve quality of life for UK patients and families, by supporting them in coping with the day-to-day challenges presented by Niemann-Pick diseases.
- Through use of our services, the families we support report better physical and mental wellbeing feeling less anxious and less isolated,

We rely entirely on voluntary donations, charitable grants and fundraising to support our work with those affected by Niemann-Pick diseases.

Reference & Administrative Details
For the Year ended 30 April 2024

<i>England & Wales Charity registration number:</i>	1144406
<i>Scotland Charity registration number:</i>	SC045407
<i>Company registration number:</i>	07775835
<i>Trustees (Directors):</i>	Helen Carter (Co-Chair) Joella Melville (Co-Chair) Dr William Evans Dave Roberts Janice Brooks Bill Owen Jacqueline Imrie Fiona Dunne Thomas Kirkegaard (Appointed 17 June 2023) Professor Frances Platt (Appointed 17 June 2023) Anthony Mead Wynn (Appointed 14 October 2023)
<i>Chief Executive:</i>	Toni Mathieson
<i>Co-opted Members:</i>	Claire Fyvie
<i>Bankers:</i>	HSBC 33 Town Square, Basildon, Essex SS14 1BA
<i>Investment Managers:</i>	Investec Wealth & Investment Limited 5 George Square Glasgow G2 1DY
<i>Independent Examiners:</i>	James Anderson & Co Pentland Estate Straiton Edinburgh EH20 9QH
<i>Registered address:</i>	Suite 2 Vermont House Concord Washington Tyne & Wear NE37 2SQ
<i>Governing document:</i>	Memorandum & Articles of Association

Trustees Annual Report**For the Year ended 30 April 2024**

The trustees present their annual report and financial statements of the charity for the year ended 30 April 2024. The directors of the charitable company are its trustees for the purposes of charity law.

The financial statements comply with the Charities Act 2011, the Charities and Trustee Investment (Scotland) Act 2005, the Charities Accounts (Scotland) Regulations 2006 (as amended), the Companies Act 2006, the Memorandum and Articles of Association, and 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).

Our Objectives and Activities.**Objectives,**

To relieve sickness amongst families afflicted by Niemann-Pick disease and any distress which may arise there from. To advance the education of such families, interested professionals and the general public in all matters concerning the disease as the committee may determine.

Review of Activities,

NPUK is dedicated to making a positive difference to the lives of those affected by Niemann-Pick diseases (NPD's) and their families, from diagnosis to bereavement and beyond. Our vision is a world where the burden of these diseases is minimised, where those affected have access to effective therapies, can meaningfully participate in society, reach their full potential and achieve the best possible quality of life.

NPD's are Lysosomal Storage Disorders (LSD's) and there are two recognised forms of NPD; they have similar clinical presentations, but quite separate causes:

- Acid Sphingomyelinase Deficiency (ASMD)

ASMD — also known as Niemann-Pick diseases type A, A/B and B — represent a spectrum of the same disease, characterised by a deficiency of an enzyme which causes a build-up of toxic materials in the body's cells. Many variations exist within this spectrum, in terms of clinical symptoms and rate of progression.

- Niemann-Pick Disease Type C (NPC)

NPC is caused by a protein deficiency, not an enzyme deficiency, but the end result is the same; an accumulation of materials (lipids) in the body's cells. The age of onset and rate of disease progression can greatly vary between individual patients.

Niemann-Pick diseases are progressive, life-limiting genetic conditions with complex and disabling symptoms that impact both physical and mental health. Due to their rarity, many affected individuals and their families experience isolation, a lack of awareness, and difficulty accessing expert care and services.

We provide a specialist care, support and advocacy service, aiming to minimise the burden of living with Niemann-Pick, including practical advice, emotional support and expert information. We aim to increase knowledge, confidence and resilience, and to ensure patients reach their full potential, can make informed decisions and achieve the best possible quality of life.

We offer clinical, advocacy and emotional support services, and connect our community through virtual and in-person events which helps to reduce feelings of isolation and despair. Our strong digital presence, with educational and inspiring content encourages mutual support and improves understanding of these ultra-rare diseases. Our care, support and information services are also used by the health, social and education professionals involved in their care.

We have strong governance, with strategic and operational leadership and the needs of the community and their voice at the heart of all we do. Our 11 Trustees have skills and experience including health care and scientific research, project management, company law and health communications. They have close connections to our community, and seven Trustees have first-hand experience of the impact of diagnosis and living with the disease on a daily basis.

Our office base located in Washington, Tyne and Wear, is the central hub for our activities, including collaboration and partnership working. From here, we develop and share supportive information on all aspects of Niemann-Pick diseases, via our website, a 24-hour help line, educational resources, regular newsletters, e-bulletins and social networking platforms.

Our Head of Communications coordinates our online presence and community engagement by developing informative resources for patients, family members, health professionals and the general public. This content includes but is not limited to; producing short films and videos, digital content, engaging our community through our website, social networking platforms and magazines/e-newsletters which provide current disease-specific information, plus practical advice for daily living and the latest developments in research. Special focus is given to including true community participation and representation in the development of our content and campaigns. This ensures we truly reflect their experience and that we fully understand and can meet their changing needs.

Our Clinical Nurse Specialist provides expert clinical care and practical advice, helping families to manage complex symptoms and disease progression and advocating on their behalf with health professionals, schools, and social services. Her experience and exceptional expertise in Niemann-Pick diseases enables her to recognize complex

Trustees Annual Report**For the Year ended 30 April 2024****Review of Activities (Continued)**

and changing individual support needs, pre-empting crisis points and providing appropriate advice and measures to improve quality of life and experiences for all. She helps families to navigate health services, including the transition from child to adult services, and supports them in making informed decisions about treatment and care.

Our Families Officer provides non-clinical advice, and emotional support, advocating for patients' needs and ensuring they receive appropriate support to fulfil their potential. Her individual advocacy service includes assistance and support at home, school or in clinic, help in exploring statutory rights and entitlements covering benefits, housing, education, and employment.

Our Fundraising Officer provides guidance, support, and resources for all those who wish to raise funds on our behalf, nurtures relationships with our current donors and supporters and explores corporate and community giving programmes to generate new income streams.

Our CEO provides leadership and is responsible for the day-to-day management of the Charity in line with the objectives and the strategic plans approved by the Board of Trustees. At a strategic level, our CEO is a member of the Clinical Reference Group for inherited metabolic disorders and, as active members of the Specialised Healthcare Alliance we continue to raise the profile of ultra-rare diseases across the UK, using our voice to influence policies relating to health and specialised services and to increase awareness that will bring lasting change for those affected. In addition, our CEO plays a key role within the International Niemann-Pick Disease Alliance (INPDA), the International Niemann-Pick Disease Registry (INPDR), the UK Lysosomal Disorder Patient Collaborative (LSD Collaborative) and the UK Newborn Screening Collaborative.

Public benefit statement

In shaping our objectives and planning our activities, the Trustees have considered the Charity Commission's guidance on public benefit, including the guidance on the Public Benefit Requirement (PB1), Public benefit: running a charity (PB2) and Public Benefit: Reporting (PB3). These matters are addressed throughout the body of the report.

Contribution made by volunteers.

We are extremely grateful to our registered volunteers (15) for their continuing and long-term support at community events. Their support is pivotal to the success of our events and meetings, and we would not be able to effectively achieve our objectives without their valued contribution.

Achievements and Performance.*Care and Support.*

"Few people understand the heartbreak and heartache of being diagnosed with a rare condition. The NPUK team has made a huge difference to how we cope with this condition".

This year has seen a sustained increase in the demand for our services as the NHS, social care and voluntary sector continue to recover from the impact of Covid and disruption within the NHS. Increasingly, families and siblings are having to fill the gaps left by a shortage of social care, and social workers and newly diagnosed families and those experiencing the distress of disease progression are finding it more difficult to access necessary health services.

Our care and support team worked together to offer a high quality, professional and holistic support service to all those affected by Niemann-Pick diseases. Face-to-face visits with families and liaison with health and social care professionals enabled families to access more timely support from local health and statutory services and to reduce anxiety and stress.

Our Clinical Nurse Specialist has been vital in providing individualised patient-centred care and has provided urgent support and medication for newly diagnosed patients, and those experiencing disease progression. Bringing together and working with patients, families, clinicians and healthcare providers enabled access to vital support, helping to maintain patients' mobility, cognitive function, and physical and mental wellbeing. Working with schools, the Clinical Nurse Specialist has also provided information sessions for teaching staff and guidance on how to manage symptoms and aspects of Niemann-Pick in children attending school.

The work of our Families Officer has a huge impact on the emotional wellbeing of patients and their families. In an increasingly difficult economic environment, she has assisted patients and families in securing much-needed financial assistance, statutory benefits, and grant support. In so doing, she has helped to reduce anxiety and distress and has improved family relationships. Advocating for access to Special Educational Needs and Disabilities support, Education Health and Care Plans and home adaptations provided essential support to those finding it difficult to navigate these processes and ensured they achieved the desired outcome. Providing ongoing emotional support to newly diagnosed families and bereaved families also formed an essential part of her role.

Trustees Annual Report**For the Year ended 30 April 2024****Achievements and Performance (Continued)***Friendship and mutual support.*

'It's a great feeling to just be yourself amongst friends who know what you're going through and be able to share stories, benefit from each other's experiences and spend time away from the day-to-day worries and routines'

Regional Meetings.

Creating the opportunity for patients and families to meet and share their stories helps enormously when trying to make sense of this devastating disease and it also helps to reduce isolation and despair. During this financial year we were able to arrange six regional meetings, at places of interest in Dublin (2), Belfast, Glasgow, Cheshire, and Eastbourne. During these informal meetings, patients, their families, carers, and friends, were able to relax, make new friends and take a break from their daily routines. Most importantly, they were able to connect and share their experiences, make new friends and benefit from mutual support and to get to know the NPUK team.

Christmas Party

Our Annual Community Christmas Party took advantage of our Family Conference being held in November, offering an ideal opportunity for an early visit from Santa Claus. The party, in a winter wonderland setting, took place in the early evening of Saturday 25 November. It was a huge success with children and adults alike, enjoying the party games singing and dancing. Our team of volunteers were all in costume, adding to the excitement of the occasion, which included, a special visit from Santa Claus and his helpers, who brought smiles to faces and presents for all.

Family Conference

Our Annual Family Conference and Interactive Workshop for professionals took place at Wyboston Lakes over a 3-day period from 24 to 26 November 2024 and was attended by 218 people, including 157 community members from the UK (148) Spain (4) Switzerland (2) Australia (2) and Canada (1). This one event brought together patients, families, health and research professionals under one roof to create a unique opportunity for families to connect with each other and engage with researchers, scientists and health professionals. Professional attendees at this meeting tell us how valuable it is to meet the patients who may benefit from their work and to gain insights into the impact of the disease from the patient and family perspective.

Speakers from all over the world presented a variety of topics focused, in parallel sessions, on ASMD Niemann-Pick diseases and Niemann-Pick type C, plus combined sessions covering childhood dementia, developing emotional resilience and the International Niemann-Pick Disease Registry.

Patients and families were also able to connect with the NPUK staff and Board teams. Feedback received from those attending this event ensures that the whole Niemann-Pick community can shape our future work and events. In parallel to the main programme, we provided a varied and entertaining activity programme for children and young adults, helping to ensure parents and carers were able to attend the presentations and workshops, as well as enjoy social time together.

The Interactive Workshop on Niemann-Pick Diseases, specifically for research and health professionals, was attended by 97 delegates, including 21 speakers from 5 countries. Talks included the latest developments in the field of Niemann-Pick diseases, with a focus on basic science research, progress in clinical trials, the development of novel therapies and data collection and generation to support further progress and understanding of Niemann-Pick diseases.

These combined activities, with the help and support of our dedicated volunteers, helped to bring our community closer together, reduced stress and isolation and enabled our community to enjoy much-needed down time away from their daily, and often challenging activities.

Communications and Information

"NPUK is a massive community network which means I don't feel isolated and always get up to date information. To have this connectivity is something I feel really passionate about".

NPUK creates up to date and engaging content, across all platforms with the aim of raising awareness and increasing understanding of Niemann-Pick diseases. Our strong connections enable us to partner with our community to create content that they can identify with and that is relevant to them. This includes sharing their lived experience of Niemann-Pick and tips on how to deal with their day-to-day challenges, fundraising news and achievements and the latest research news plus practical help and resources, and interactive support opportunities.

Our community connections are therefore stronger, with shared experience, mutual support and empathy leading to long-lasting friendships and reduced loneliness and isolation.

Social media platforms and messaging services are our most active form of communication and our presence is growing across all platforms. Therefore, we tailor our content to build better engagement and interaction between our team, our community, our key stakeholders and the general public.

Our website consistently tracks well on Google Analytics and, as such, is one of the first sites accessed when searching for information about Niemann-Pick diseases. Launched in 2016, our website grew in line with information and progress in the field.

Trustees Annual Report**For the Year ended 30 April 2024****Communications and Information (Continued)**

This year, we identified the need to update this essential resource to simplify navigation and functionality. This work has commenced with our chosen partner and is expected to complete in late 2024.

Following the success of our award-winning short films and series of "Invisible Interviews", we are proud that, this year, "Graham's Story" received over 65,000 viewings and was nominated for "#1 Most Popular Festival in Western Europe" at the Cannes Film Awards. In addition, the short campaign film "A Rare Find" film developed with the UK Newborn Screening Collaborative received more than one million views on TikTok and won the Gold Award at the Smiley Charity Film Awards. Through our successful track record of producing award winning short films, such as "Imagine", (2017) and, "Go Make Memories" (2019), we have witnessed first-hand the impact and benefits of developing awareness and campaign films to engage with the wider general public, advocacy groups and key stakeholders.

The new part-time role of Communications Assistant has helped create the capacity for this work to take place and has provided our Head of Communications with much needed support.

Collaboration

"We share similarities with other rare disease charities and are stronger by working together rather than apart"

At a national level, we continue to actively engage with Genetic Alliance UK, the Specialised Healthcare Alliance and the UK National Screening Committee. As an active member of both the UK LSD Collaborative and the Newborn Screening Collaborative, we work to influence national policy for the benefit of all those affected by Niemann-Pick diseases, and to strengthen our collaborative campaign for better healthcare outcomes for the LSD and wider rare disease community.

On an organisational level, the UK LSD Collaborative has achieved charitable status, with the Charity Commission for England and Wales, enabling us to better utilise our combined resources and reduce overall costs. Formal discussions are in place to further develop these partnership opportunities with appropriate legal advice and in consultation with each of the charities involved. Agreement on the way forward is expected during 2024/2025 with tangible benefits beginning to emerge during 2025/2026.

We continued to support the International Niemann-Pick Disease Alliance (INPDA) in its global activities, and to provide hands-on support to the International Niemann-Pick Disease Registry (INPDR). The INPDR is a global database collecting information about the health and experiences of people living with Niemann-Pick diseases. It is supported by clinicians, scientists, researchers, and patient associations across the world and aims to collect clinical, genetic and diagnostic data to further understanding and enable research. Both the INPDA and the INPDR are independent charitable companies with NPUK representatives appointed as Board Members.

Research.

"Research helps us to understand more about the disease and gives us hope for the future"

Throughout the year we have continued to maintain a presence in the field of research. We have attended a number of scientific conferences and presented a poster at the WORLD symposium, as well as maintaining contact and relationships with key academic, research and industry stakeholders working in the Niemann-Pick field. These activities enable us to convey the latest research updates to our community.

However, a decision by the National Institute for Health and Care Excellence (NICE) not to recommend Xenpozyme as a treatment for Niemann-Pick disease types AB and type B, has resulted in our scarce resources being redirected to support preparation of an appeal.

Due to the additional work and time required to contribute to the NICE process our plan to host the 2nd Oxford NPC Research Forum were put on hold, and Research Subcommittee meetings were postponed.

Four experimental therapies are being investigated for Niemann-Pick type C with two clinical trials recently completed and two more in progress. This is positive and encouraging news for our community and we await regulatory reviews that could herald the approval of effective therapies in the next financial year. We identified the need to provide independent evidence of the unmet needs within our community, which will support UK approvals and reimbursement. This year we have worked with the International Niemann-Pick Disease Registry to collect this data. Following analysis, we will publish the results of our work, which was also undertaken in partnership with the National Niemann-Pick Disease Foundation in the USA and the International Niemann-Pick Disease Alliance.

As part of our disease prevention strategy, we continue to campaign for a change in the way the National Screening Committee evaluate new applications for the Newborn Screening of rare diseases and we continue to promote the need for carrier testing for families affected by Niemann-Pick diseases. As members of the UK Newborn Screening Collaborative, we work to address the evident need for focused work to change outdated processes for the benefit of all relevant LSDs, to share knowledge and experience and to provide a strong, united voice with which we can represent our communities and avoid duplication of efforts. The Peter Carlton Jones Memorial Award 2023 was granted to Gareth Fenn, a final year PhD student within the Medicines Discovery Institute (MDI) at Cardiff University for his work in investigating the potential therapeutic effects of cannabidiol and copaiba in Niemann-Pick diseases. Gareth joined us at our Annual Conference, to present his findings and receive the Award.

Trustees Annual Report**For the Year ended 30 April 2024****Research (Continued)**

The William French Memorial Award was created as a lasting legacy in memory of William, who was diagnosed with NPC at just 9 months old. William led a fulfilling life despite his diagnosis and passed away peacefully in August 2023, aged just 22. The Award offers grants and bursaries to UK based young scientists and early career researchers working in the field of Niemann-Pick disease type C (NPC), with preference given to supporting their studies, and to research or activities that may lead to the advancement of progress in the study of NPC. The Award was launched at our Interactive Workshop on Niemann-Pick Diseases in November 2023, and the first application received shortly after. The successful applicant, Berna Seker-Vilmaz MD, a Research Associate at the Genetics and Genomics Medicine, Institute of Child Health, received an award to support a presentation of her work "Understanding the Infantile Phenotypes of Niemann-Pick Disease Type C (NPC)" at the SSIEM Annual Symposium taking place in Porto, Portugal in September 2024.

Fundraising

"Fundraising is our lifeblood and ensures continuity of the services we provide"

NPUK is a member of the Chartered Institute of Fundraising, and as such abides by the fundraising principles set out in the "Code of Fundraising Practice". This year, our part-time Fundraising Officer continues to support and nurture our fundraisers by providing advice, publicity, and fundraising resources. Fundraisers have undertaken the most amazing events and activities, running many miles, taking on physical challenges such as climbing mountains and baking non-stop for coffee mornings. Instead of receiving gifts, they raise funds in lieu of birthdays, anniversaries and in memory of their loved ones. NPUK also supported the 17th Annual Niemann-Pick UK Charity Golf Day and our 'Team NPUK' runners taking part in the Great North Run.

Our Fundraising Officer is also beginning to extend her reach by developing relationships with grant funders. This has led to some success with the Tesco Blue Token Scheme providing grants for the NPUK family support service, and a strong connection with the Rotary Clubs of District 1060, centred on Birmingham and the West Midlands.

In addition to our fundraisers, our regular donors have maintained their level of support and have provided a stable level of funding that has contributed to maintaining the services we provide. We are indebted to our loyal and generous supporters for their time, achievements, and ongoing patronage of our work.

Building on the outcomes from our Strategic Review in 2022, we have increased our capacity to write grant applications and over the year we submitted 13 applications to Grant Giving Organisations for funds totalling £419,752. Disappointingly, we received support from only 3 organisations totalling £27,000. We will review our grant application strategy, and in consideration of a now highly competitive environment and the lessons learned, will take action to increase the number of applications we submit in order to reach our financial objectives.

Regarding our donors and supporters' information, we adhere to the General Data Protection Regulation (GDPR) guidance on data use and retention, and we do not use direct marketing approaches. All communications with donors and supporters include the opportunity to opt out of receiving information or contact.

Impact

Our desired impact is to improve quality of life for UK patients and families, by supporting them in coping with the day-to-day challenges presented by Niemann-Pick diseases.

Niemann-Pick UK (NPUK) is a leading charity dedicated to supporting individuals and families affected by Niemann-Pick diseases, rare and life-limiting lysosomal storage disorders. Our work provides vital support services, including specialist care, advocacy, and community outreach, while promoting awareness and understanding of these conditions. Through partnerships with healthcare providers, researchers, and policymakers, we strive to improve access to treatment, advance research, and ensure that the voice of those impacted is at the forefront of healthcare discussions. Together, we are building a future with better care, hope, and quality of life for all affected by Niemann-Pick diseases.

We recognise the need to measure the outcomes and impact of the services we provide. However, due to the progressive nature of Niemann-Pick diseases and the serious and wide-ranging emotional and physical impact on the whole family, we have found that traditional impact measurement scales are not suitable for this purpose. Each disease type and stage of progression comes with different challenges and what has been described by our community as roller coaster of emotions. We are therefore working to develop our own combination of measures that accurately reflect the patient and family experience, as well as capture the difference we make through our work.

We are committed to making a meaningful difference, and recording our impact, in the following key areas:

- Care and Support Services: We measure the number of families reached through our work, assessing the effectiveness of our support through feedback surveys, case studies, and improvements in patient and family well-being. Tracking improvements in quality of life, reduced isolation, and mental health support is central to understanding our impact on the community.
- Advocacy and Access to Treatments: We measure our advocacy impact by tracking policy changes, lobbying for access to therapies, and participation in clinical trials.

Trustees Annual Report

For the Year ended 30 April 2024

Impact (Continued)

By engaging with government agencies, pharmaceutical companies, and healthcare providers, we assess how successful we are in influencing decisions that improve access to expert care, treatment availability and access, and reducing the time it takes for patients to receive a diagnosis.

- Community Engagement and Awareness: We evaluate the reach and success of our day-to-day communications and awareness campaigns by tracking engagement across multiple platforms (website, social media etc.). Metrics such as the growth in our social media following, website visits, attendance at educational events, and the number of new supporters joining NPUK help us to gauge our success in reaching community members, key stakeholders and the wider general public.

- Research and Knowledge Generation: Our impact is measured by the extent to which we encourage and facilitate research and collaboration, participate in research projects and activities and the ways in which our work influences scientific collaboration and partnership. In addition, feedback from the clinical, research and academic institutions we work with helps to understand the impact of our grant funding programmes, awareness and educational activities.

- Patient-Centric Initiatives: We assess our success in ensuring that the voice of the patient is central to discussions on treatments and care. This includes measuring patient and carer participation in surveys, advisory boards, and public consultations, as well as tracking how often their input is reflected in policy decisions, clinical trial designs, or treatment evaluations.

At our Annual Conference in 2023, we held workshops to help us understand our impact. With Trustees as facilitators, community members were invited to share their views and perspective regarding the impact of NPUK's work and services.

Members of our community told us that continued access to our care and support team, and the services we offer, makes a significant difference to their lives, with some describing it as a lifeline. Some of the positive differences experienced by those we support are summarised below;

- Newly diagnosed patients and their families, coming to terms with diagnosis, felt less isolated, more informed about Niemann-Pick diseases and felt more confident about facing the challenges of caring for their affected loved one.

- Patients, their families, and carers, who were not able to see a doctor or clinician in person, felt less anxious and better able to cope after accessing urgent clinical support, medication advice and guidance from our Clinical Nurse Specialist.

- Families experiencing financial hardship reported feeling less anxious because of interventions and support from our Families Officer to access the NPUK Support Fund and wider statutory benefits.

- Patients and family members accessing our counselling and wellbeing support service developed more confidence and reported improved emotional resilience and mental wellbeing and reported an improvement in their relationships and ability to better communicate their feelings

- Loneliness and isolation were reduced, through contact with our team and opportunities to attend the Family Conference, Christmas Party and regional social events including the whole of Ireland, Scotland, Yorkshire, Cheshire and East Sussex.

- Harder to reach groups have become more integrated into the Niemann-Pick community, feel more comfortable in accessing support services and are participating in community events.

- Children and young adults affected by Niemann-Pick diseases have maintained fine motor and cognitive skills and participation in virtual social and community events through access to iPads supplied by NPUK.

- Parents, carers, siblings, and extended family members of affected children benefitted from one-to-one support and information helping to improve family wellbeing and quality of life

- Bereaved families and individuals felt supported and better able to cope with day-to-day life through being involved in the NPUK Reflections programme and access to counselling.

- Professionals tell us that access to our services helps patients and families to cope with the day-to-day challenges of living with Niemann-Pick diseases, boosting physical and emotional resilience, increasing confidence, and reducing anxiety

Governance.

The Trustees are committed to good governance and are guided by the "Charity Governance Code, 13 July 2017" and "The Essential Trustee, Updated 3 May 2018.

The Trustees work as an effective team to deliver its "objects" All decisions are agreed collectively and in the best interest of the Charity. Two co-opted members of the Board were appointed as full board members and one new Trustee was appointed in line with the recruitment and appointment procedures. The new board members bring with them skills and experience of medical research, scientific research, and business management. The new appointees bring the total number of Trustees to 11. Looking forward, there is a need to recruit a Treasurer and this post is now being widely advertised through NPUK channels and other appropriate Institute channels.

Trustees Annual Report**For the Year ended 30 April 2024****Governance (Continued)**

Succession Planning for the post of Chief Executive Officer remains a risk to the future success of the organisation and a lack of funds has prevented the recruitment of an Executive Assistant.

Trustees support the Chief Executive Officer, wherever possible, and continue to support the need to generate incoming funds to alleviate this risk.

A strong dependence on key NPUK members of staff with no spare capacity will increase the need to investigate further collaboration with the UK Lysosomal Storage Disease (LSD) Collaborative. (This newly formed charity could become an umbrella organisation for the existing LSD Charities with the ability to raise funds and share resources).

The outcomes and the actions arising from the Strategic Review of July 2022 are reviewed throughout the year and Trustees focus on the risks and challenges of achieving our aims and objectives. Two significant strategic decisions are summarised below;

- Arising from a family survey and informed views about the changing needs of our community, the Trustees discussed the evolving role of our care and support service and the role of the Families Officer and the Clinical Nurse Specialist. They considered an increasing number of mental health issues affecting children and families, the introduction of metabolic nurse specialists, in all NHS National Specialist Care Centres (5 Adult and 3 Paediatric) and our limited financial resources.

Trustees agreed that more information was required before deciding any further course of action. This would be further discussed at the next board meeting in June 2024.

- Following a proposal from the Finance Subcommittee, the Trustees agreed to transfer of endowment funds from Investec to fixed rate products. This followed a discussion about the performance of the endowment against the objectives, industry benchmarks, the return on investment and the underlying financial uncertainty for the year ahead.

Risk Management.

The Directors have a risk management strategy, which comprises:

- A risk management framework, risk identification and assessment.
- An annual risk assessment and review of the principal risks and uncertainties that NPUK faces.
- The establishment of policies, procedures, and systems to mitigate the risks identified.
- The implementation of actions and procedures designed to minimise or manage any potential impact on NP

Principal Risks.

The principal risks for the foreseeable future relate to:

- Increased demand and a highly competitive market for grant funding reduces the probability of achieving successful grant applications and may force the need to review our resource priorities.
- Uncertainties around incoming funds from the pharmaceutical companies will be a risk to our Family Conference.
- Further external economic and social pressures will adversely affect voluntary income.
- Not being able to use the recognised wellbeing and mental health impact measurement scales may affect the success of our grant applications.
- A strong dependence on key members of staff, with no spare capacity, will cause a disruption to the services we provide if they leave the organisation.

The actions we are taking to address these risks are to:

- Recruit an Executive Assistant to give high level support to the CEO, when funds permit.
- Further develop the role of Fundraising Officer to include developing sponsors for fundraising and social events.
- Further develop and update our Business Continuity Strategy to take into account the possible impact of reduced incoming funds and the risks we face as a charity.
- Review our Grant Funding strategy and be prepared to further increase our grant writing capacity.
- Commit the resources necessary to develop a suitable (bespoke) impact measurement tool that can be integrated with our existing data base.

Plans for Future Periods.

- We will regularly evaluate progress towards the outcomes agreed at our Strategic Review meeting and complete the related action plans to create a robust, resilient, and financially sustainable charity.
- We recognise that Succession Planning is a key priority, and we will recruit an Executive Assistant to support the CEO when funds become available.
- We will be aware of the changing needs of our community and will adapt and develop the charity and our services to meet their presenting needs.

Trustees Annual Report**For the Year ended 30 April 2024****Plans for Future Periods (Continued)**

- We will keep up to date with the latest research into Niemann-Pick diseases and focus on supporting the UK Newborn Screening Collaborative,
- We will collaborate with UK research and academic institutions and, if possible, provide small grants in support of relevant projects and we will actively engage with worldwide experts and emerging biotech companies to encourage an interest in developing new therapies for the treatment of Niemann-Pick diseases.
- We will build on our strong relationship with the UK LSD Collaborative and other relevant organisations to improve access to mental health services and influence national policy in those areas that impact the Niemann-Pick and wider LSD patient community.
- We will further develop our working relationship with the UK LSD Collaborative Charity to better utilise our combined resources to reduce overall costs.
- We will collaborate with pharmaceutical companies and NICE to provide evidence in support of appraisal processes for new therapies arising from clinical trials; Working to identify appropriate clinical endpoints and gather evidence that helps to demonstrate the effectiveness of new drugs will become a key challenge for our community.
- We will continue to support the INPDA in all its activities and continue our key role in supporting the INPDR.
- We will remain involved in community driven campaigns to raise awareness and improve the care of those individuals affected by rare diseases. We value our relationship with the clinical teams at the NHS England Specialist Centres and will continue to work with them to further improve standards of care for those affected by these disorders.
- We will strive to continually improve and address any shortfalls in our performance that we become aware of.

The trustees have considered their financial obligations over the next three -year period and have no concerns about the charity's ability to continue as a going concern.

FINANCIAL REPORT

The Financial Year 2023/2024 was a year of financial uncertainty shaped by the risk of insufficient incoming funds to meet our commitments and a low to medium probability of securing successful grant applications.

A financial deficit of £105,360 was approved by Trustees (June 2023) to maintain the services we provide. (The actual deficit was £82,504 at the year-end).

The following notes, relating to income and expenditure, are based on the Statement of Accounts and describe the performance against budget and, where relevant, the performance against the previous financial year 2022/2023.

Income.

Income for the year was £373,655 (£305,491 unrestricted funds and £68,164 unrestricted funds). This was against a budget of £394,203 (-£20,548). - Income for the previous financial year, 2022/2023, was £306,594.

In more detail, variances to budget of £5,000 or more were:

- Fundraising income through the Just Giving platform was £30,886 against a budget of £79,090 (-£48,204) due to fewer than expected people taking part in fundraising activities. The budget of £79,090 appeared realistic against last year's total of £83,253. However, some fundraisers may have transferred to the "Go Fund Me" platform which transfers funds through PayPal. (£5,716 against a budget of £500).
- Income from successful grant applications was a disappointing £9,569 against a modest budget of £23,539. (-13,970).
- A legacy of £12,500 was delayed by legal matters and this is now expected in 2024/2025. (-£12,500)
- An expected fundraising event (£10,000) did not take place (-£10,000)
- Gift Aid at £6,402 against a budget of £13,856 (-£7,454) reflected the reduction in fundraising activities.

These negative variances to budget were offset by:

- A restricted donation of £53,553 from the Niemann-Pick Research Foundation which represented stage 1 of the closing balance of the charity.
- A grant from the Hollie Foundation of £7,569 which was £5,569 better than budget (£2,000) but, sadly, represented the closing balance of the charity.
- PayPal Giving of £5,716 against a budget of £500 (+£5,216) and a previous year total of £634
- General donations which were £20,112 against a budget of £15,000(+£5,112)

Trustees Annual Report.**Financial Report (Continued)*****Expenditure.***

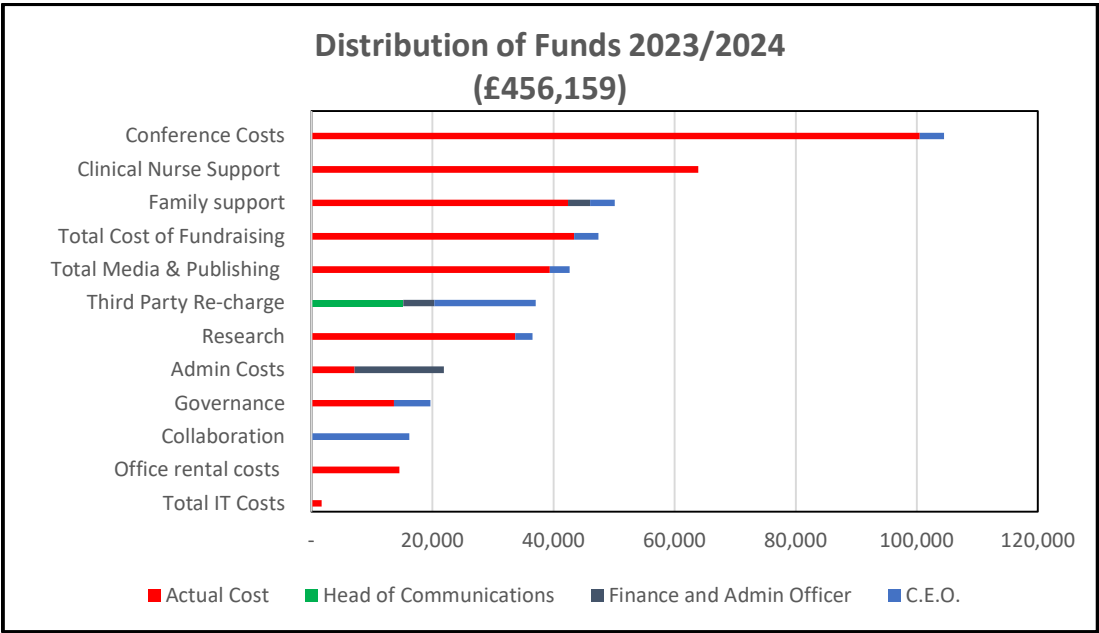
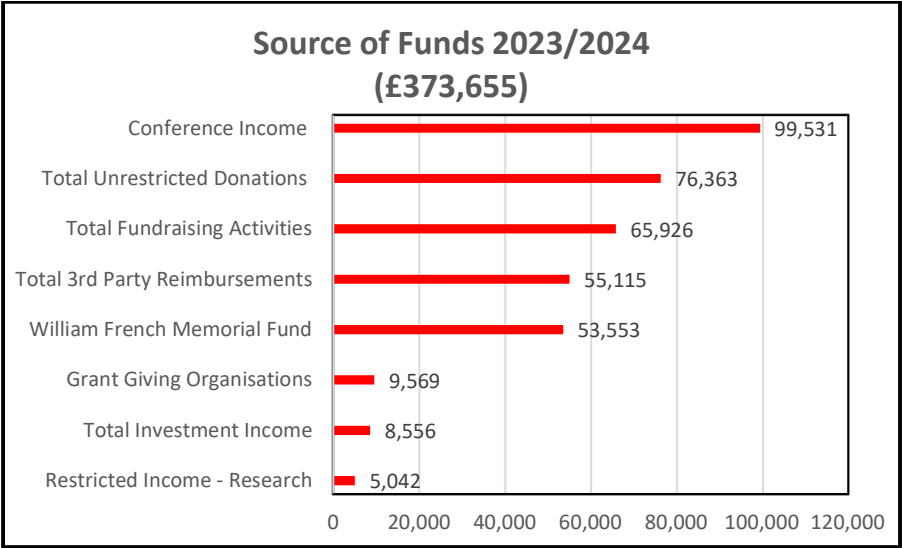
Expenditure for the year was £456,159. All budgets were tightly controlled, and these actions resulted in an overall underspend of £43,404 against a budget of £499,563. (Expenditure for the previous year, 2022/2023 was £440,372)

In more detail, there were two significant areas of overspend offset by savings across all other budgets.

- The costs of the Family Conference and the Interactive Workshop, over three days, was £100,477 against a budget of £76,953 (+£23,524). However, this also included the Children's Christmas party, and the total costs of the Conference and Interactive workshop (£100,477) was covered by the unrestricted conference income of £99,531.
- The "Cost of Fundraising Activities" was £43,367 (9.4%) against a budget of £31,665 (+£12,002). The principal overspends were attributed to the cost of researching and completing grant applications (+£9,580) and the costs of merchandise and event fees. (+£2,641). The cost attributed to Grant Applications was 5.2% and Fundraising Activities 4.2%. This compares to a reported average across all charities of; Activities for raising funds 6.6% and Grants 16.3% (NCVO/TSRC, Charity Commission 2021)

Trustees Annual Report.

Financial Report (Continued)



Sources of Information for charts.

- The Sources of Income were taken from the Statement of Accounts.
- The Expenditure was taken from the Statement of Accounts.
- The Allocation of costs for the Chief Executive, the Finance and Administration Officer and Head of Communications were taken from the Management Accounts and allocated in the basis of “hours spent” on each category.

Trustees Annual Report.**Financial Report (Continued)*****Financial Strategy.***

Our Financial Strategy is based on raising sufficient levels of incoming funds to match the expenditure required to deliver our objectives. 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, ensure the charity is able to meet its future commitments and to create financial sustainability. We therefore hold a level of reserves to ensure the smooth running of the Charity during times of financial uncertainty

Reserves Policy.

Our Reserves Policy is to ensure that NPUK has sufficient assets to consistently fund the services it provides to families affected by Niemann-Pick diseases and to hold sufficient funds to address the risks and contingencies identified by the Trustees. The required level of reserves will be maintained, by a contribution from unrestricted funds, when available, and will be reviewed by the Board of Trustees on a quarterly basis.

The Reserves Policy is reviewed by the Board of Trustees on an annual basis and conforms to the Charity Commissioners' guidance "Charity Reserves; Building Resilience" (CC19, published 29 January 2016).

Our Reserves Policy for the financial year, May 2024 to April 2025, takes into account:

- The risks associated with raising sufficient incoming funds during the financial year 2024/2025.
- A three-year financial forecast and cash flow forecast which considers the potential risks to income, the need to fund our succession planning strategy and the changing requirements of the families we support.
- The perceived move by grant giving Organisations towards funding local community projects and umbrella organisations in favour of small national charities.

Level of Reserves.

The Trustees have reviewed the Reserves Policy and have considered the key objectives and commitments for the period 2024/2025. They have considered the plans for future periods and the risks associated with the generation of income.

The objectives of the reserves policy for the financial year 2024/2025 are, therefore.

- To address a possible reduction in the incoming funds from pharmaceutical companies (£35,000).
- To address the risks associated with a reduced rate of success in obtaining grant funding (£36,250).
- To hold sufficient funds (8 weeks) to manage salaries and cash flow for the day-to-day operation of the Charity. (£69,890).

The required free reserves are therefore £141,140 which is equivalent to 3.8 months of planned expenditure.

Designated Funds

- There are no commitments that require designated funds to be set aside.
- The total free reserves required for 2024/2025 are therefore £141,140 which is equivalent to 3.8 months of planned expenditure.

The free reserves at the year-end on 30 April 2024 were £86,664. However, a budget surplus of £23,125 for the financial year 2024/2025 (Total forecast reserves £109,789) means there is a £31,351 shortfall against the required level of reserves.

The Trustees will take action to increase income and mitigate the risks identified.

Endowment

NPUK has an expendable endowment, arising from a legacy, which is managed by Investec Wealth & Investment Limited to produce an expendable income.

The value of the endowment portfolio, on 30 April 2024, stood at £204,800 which is a slight increase (£2,807) from the previous value of £201,993 on 30 April 2023 and the income from the investment for 2023/2024 was £6,009 which was equivalent to 2.93% return on investment.

The Trustees have reviewed the endowment and confirmed the objective that we should aim to preserve the capital whilst generating a regular income. However, whilst the return on investment was consistent with prevailing financial uncertainties, the endowment did not meet our expectations and had consistently underperformed against the Investec benchmark figures.

The Trustees engaged with Investec to explore the economic outlook. Based on the current NPUK financial outlook, it was concluded that other fixed rate investment opportunities would better protect the capital and offer increased returns on the investment. For this reason, the Finance Subcommittee proposed that we should transfer the endowment funds to a fixed rate investment product.

**Trustees Annual Report
For the Year ended 30 April 2024*****Endowment (Continued)***

The Endowment will be reviewed on an annual basis to ensure we achieve our objectives and risk profile.

The endowment can be used to assist in financing of the family care and support service provided to families affected by Niemann-Pick diseases and Trustees have the discretionary power to transfer funds should it become necessary to do so.

If, at any time, the Charity is forced to close, this Endowment will be used to fund alternative care and support arrangements for affected families during a period of transition to new providers. Any assets remaining will be transferred to another charitable institution in line with the Articles of Association. The endowment is monitored by the Trustees on an annual basis.

Reference and Administrative Details.

The information relating to Niemann-Pick UK is on page 1.

Structure Governance and Management***Status***

The organisation is a charitable company limited by guarantee and the liability of each member is limited to £1. The organisation was registered as a charity in England and Wales on 24 October 2011 and in Scotland on 9 February 2015.

Governing Document

The charity is governed by its Memorandum and Articles of Association.

Appointment & Recruitment of new Trustees

New Trustees shall be appointed by the Trustees following a nomination received from the Nominations Committee pursuant to article 61 and in line with the Terms and Reference of the Nominations Committee. All Trustees are volunteers and have the skills and experience to take on responsibilities within the Group to co-ordinate/support an aspect of the Group requirements.

Trustees are elected for a period of three years after which they are required to retire. A retiring Trustee may be reappointed for a further term of three years, offering themselves for retirement after each three-year term.

Induction and Training of 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 and key members of staff.

Organisational Structure

The structure of the charity is governed by its objects and Articles of Association. At the end of the Financial Year, the Group had six employees, known as the Chief Executive, Finance and Administration Officer, Communications and Campaigns Manager, Counselling and Well-being Officer, Families Officer and Fundraising Officer. The Group also pays the full employment costs of a Clinical Nurse Specialist who is employed by the Salford Royal NHS Foundation Trust. The Trustees and all the other supporters of the Group are volunteers.

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

Pay Policy for Senior Staff

All trustees give of their time freely and no trustee received remuneration in the year. The Board of Trustees is responsible for setting employee salary levels. These are based on local government pay scales which are extensively used to benchmark roles in the voluntary sector. As a small charity, we take into account our levels of income and do not guarantee annual wage increases.

Related Parties

Niemann-Pick UK is a member of the International Niemann-Pick Disease Alliance (INPDA) and shares the services of the NPUK Chief Executive, Finance and Administration Officer and Communications and Campaigns Manager who are located at the Niemann-Pick UK Central Office.

**Trustees Annual Report
For the Year ended 30 April 2024**

Statement of Trustees Responsibilities.

The charity's trustees are responsible for preparing a trustees' annual report and 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 company and of the profit or loss of the 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 applicable Charities SORP;
- make judgments and estimates that are reasonable and prudent;
- prepare the financial statements on the going concern basis unless it is inappropriate to presume that the company will continue in business.

The trustees are responsible for keeping adequate accounting records which disclose with reasonable accuracy at any time the financial position of the company and to enable them to ensure that the financial statements comply with the Companies Act 2006, the Charities Act 2011, the Charities and Trustee Investment Scotland Act 2005 and the Charities Accounts (Scotland) Regulations 2006 (as amended). They are also responsible for safeguarding the assets of the company and hence for taking reasonable steps for the prevention and detection of fraud and other irregularities.

By Order of the Board



**H. Carter
Trustee**



**J. Melville
Trustee**

2 November 2024

I report on the accounts of the company for the year ended 30 April 2024 which are set out on pages 16 to 25.

Respective responsibilities of trustees and examiner

The trustees (who are also the directors of the company for the purposes of company law) are responsible for the preparation of the accounts. The trustees consider that an audit is not required for this year under section 144(2) of the Charities Act 2011 (the 2011 Act) or under Regulation 10 (1)(a) to (c) of The Charities Accounts (Scotland) Regulations 2006 (the 2006 Accounts Regulations) and that an independent examination is needed. The charity is required by company law to prepare accrued accounts and I am qualified to undertake the examination by being a qualified member of ICAS.

Having satisfied myself that the charity is not subject to audit under company law and is eligible for independent examination, it is my responsibility to:

- examine the accounts under section 145 of the 2011 Act and section 44(1)(c) of the Charities and Trustee Investment (Scotland) Act 2005 (the 2005 Act)
- to follow the procedures laid down in the general Directions given by the Charity Commission under section 145(5)(b) of the 2011 Act
- to state whether particular matters have come to my attention.

Basis of independent examiner's report

My examination was carried out in accordance with the general Directions given by the Charity Commission and is in accordance with Regulation 11 of the Charities Accounts (Scotland) Regulation 2006. An examination includes a review of the accounting records kept by the charity and a comparison of the accounts presented with those records. It also includes consideration of any unusual items or disclosures in the accounts, and seeking explanations from you as trustees concerning any such matters. The procedures undertaken do not provide all the evidence that would be required in an audit and consequently no opinion is given as to whether the accounts present a 'true and fair view' and the report is limited to those matters set out in the next statement.

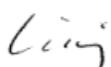
Independent examiner's statement

In connection with my examination, no matter has come to my attention:

(1) which gives me reasonable cause to believe that in any material respect the requirements:

- to keep accounting records in accordance with section 386 of the Companies Act 2006; and section 44(1)(a) of the 2005 Act and
- to prepare accounts which accord with the accounting records, comply with the accounting requirements of the Companies Act 2006, section 44(1)(b) of the 2005 Act and Regulation 8 of the 2006 Accounts Regulations and
- which are consistent with the methods and principles of the Statement of Recommended Practice: Accounting and Reporting by Charities
- have not been met or

(2) to which, in my opinion, attention should be drawn in order to enable a proper understanding of the accounts to be reached.



Christopher Spalding C.A. (ICAS)
James Anderson & Co
Chartered Accountants
Pentland Estate
STRAITON
Edinburgh
EH20 9QH

8 November 2024

Statement of Financial Activities
(Incorporating Income and Expenditure Account)
For the year ended 30 April 2024

	Notes	Unrestricted Funds £	Restricted Fund £	Endowment Fund £	Total Funds £	2023 £
Income and endowments from:						
Donations and legacies	3	175,894	7,042	-	182,936	105,425
Charitable activities	4	-	61,122	-	61,122	86,206
Other trading activities	5	121,041	-	-	121,041	108,370
Investments	6	8,556	-	-	8,556	6,593
Total income		<u>305,491</u>	<u>68,164</u>	<u>-</u>	<u>373,655</u>	<u>306,594</u>
Expenditure on:						
Raising funds		43,367	-	-	43,367	16,454
Charitable activities	7	397,931	14,861	-	412,792	423,918
Total expenditure		<u>441,298</u>	<u>14,861</u>	<u>-</u>	<u>456,159</u>	<u>440,372</u>
Net income / expenditure before losses on investments		(135,807)	53,303	-	(82,504)	(133,778)
Net gains / (losses) on investments		-	-	2,867	2,867	(16,400)
Net movement in funds		<u>(135,807)</u>	<u>53,303</u>	<u>2,867</u>	<u>(79,637)</u>	<u>(150,178)</u>
Reconciliation of funds:						
Total funds brought forward		<u>222,471</u>	<u>2,000</u>	<u>201,933</u>	<u>426,404</u>	<u>576,582</u>
Total funds carried forward	15	<u>86,664</u>	<u>55,303</u>	<u>204,800</u>	<u>346,767</u>	<u>426,404</u>

All income and expenditure derives from continuing activities.

The statement of financial activities includes all gains and losses recognised during the year.

Balance Sheet
As at 30 April 2024

	Notes	2024 £	2023 £
Fixed Assets			
Tangible fixed assets	8	-	-
Investments	9	204,800	201,933
		<u>204,800</u>	<u>201,933</u>
Current Assets			
Debtors	10	7,807	11,613
Bank & cash		160,244	227,489
		<u>168,051</u>	<u>239,102</u>
Creditors			
Amounts falling due within one year	11	26,084	14,631
Net Current Assets		<u>141,967</u>	<u>224,471</u>
Net Assets		<u>346,767</u>	<u>426,404</u>
Funds	15		
General fund		86,664	198,139
Designated fund		-	24,332
		<u>86,664</u>	<u>222,471</u>
Unrestricted funds		86,664	222,471
Endowment fund		204,800	201,993
Restricted funds		55,303	2,000
		<u>346,767</u>	<u>426,404</u>

The charitable company is entitled to exemption from audit under Section 477 of the Companies Act 2006 for the year ended 30 April 2024.

The members have not required the charitable company to obtain audit of its financial statements for the year ended 30 April 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 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 special provisions of Part 15 of the Companies Act 2006 relating to small charitable companies.

The financial statements on pages 16 to 25 were approved by and signed on behalf of the board of directors on 2 November 2024.



H. Carter Trustee

J. Melville Trustee

**Notes to the Financial Statements
For the Year ended 30 April 2024****1. Statutory information**

Niemann-Pick UK is a private company, limited by guarantee and incorporated in England. It is registered as a charity in England and Wales and Scotland. 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 at page 1 on the Reference and Administrative Details page.

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

2. Accounting policies

The principal accounting policies adopted, judgements and key sources of estimation uncertainty in the preparation of the financial statements are as follows:

a) Basis of preparation

The financial statements have been prepared in accordance with 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 (FRS102) issued in October 2019 - (Charities SORP (FRS 102), the Financial Reporting Standard applicable in the UK and Republic of Ireland (FRS 102) and the Companies Act 2006.

Niemann-Pick UK meets the definition of a public benefit entity under FRS 102.

The financial statements are prepared on a going concern basis under the historical cost convention, modified to include certain items at fair value.

b) Funds

Unrestricted income funds comprise those funds which the trustees are free to use for any purpose in furtherance of the charitable objects. Unrestricted funds include designated funds where the trustees, at their discretion, have created a fund for a specific purpose.

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.

Endowment funds represent those assets which are held permanently by the charity. Income arising on the endowment funds can be used in accordance with the objects of the charity and is included as unrestricted income. Any capital gains or losses arising on the investments form part of the fund. The trustees have the power to convert the fund and capital gains to an income fund.

c) 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.

Interest on funds held on deposit and is included upon notification of the interest paid or payable by the Bank. Dividends received are included upon notification of the distribution being paid.

No amount is included in the financial statements for volunteer time in line with the SORP (FRS 102). Further detail is given in the Trustees' Annual Report.

**Note to the Financial Statements
For the Year ended 30 April 2024 (continued)**

d) 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.

e) Tangible fixed assets & depreciation

Tangible fixed assets are stated at cost or valuation less accumulated depreciation and impairment losses. Cost includes costs directly attributable to making the asset capable of operating as intended.

Depreciation is provided on all tangible fixed assets, at rates calculated to write off the cost, less estimated residual value, of each asset on a systematic basis over its expected useful life as follows:

Computer equipment - 3 years straight line.

f) Investments

Investments are recognised initially at fair value which is normally the transaction price excluding transaction costs. Subsequently, they are measured at fair value with changes recognised in 'net gains / (losses) on investments' in the SoFA if the shares are publicly traded or their fair value can otherwise be measured reliably.

g) Impairment

Assets not measured at fair value are reviewed for any indication that the asset may be impaired at each balance sheet date. If such indication exists, the recoverable amount of the asset, or the asset's cash generating unit, is estimated and compared to the carrying amount. Where the carrying amount exceeds its recoverable amount, an impairment loss is recognised in profit or loss unless the asset is carried at a revalued amount where the impairment loss is a revaluation decrease.

h) 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.

i) Cash in bank and in hand

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

j) Pension costs

The company operates defined contribution schemes, the assets of which are held separately from those of the charity. The pension cost charge represents contributions payable by the charity to the scheme.

k) Taxation

No provision for corporation tax is necessary as the company has charitable status and does not trade. The company suffers input VAT on some of its expenditure which it does not recover.

l) Operating leases

Rentals payable under operating leases are charged to the Income and Expenditure Account on a straight line basis over the period of the lease.

m) 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.

Note to the Financial Statements
For the Year ended 30 April 2024 (continued)

	2024	2023
	£	£
3. Income from donations & legacies		
<i>General fund - unrestricted</i>		
Conference income and grants	99,531	64,993
Voluntary donations	76,363	36,432
<i>Restricted fund</i>		
Voluntary donations	5,042	4,000
	<u>182,936</u>	<u>105,425</u>

Included above were unrestricted grants received from pharmaceutical companies:

Sanofi Genzyme, Astex Therapeutics, Cyclo Therapeutics, Zevra Denmark, Azafaros BV

NPUK remains transparent, independent and impartial in all of its dealings with pharmaceutical companies. Unrestricted donations used for Core activities, the Interactive Workshop and Family Conference are provided without any control or influence over the content of the programme or the selection of speakers.

	2024	2023
	£	£
4. Income from charitable activities		
<i>General fund - unrestricted</i>		
Kickstart	-	10,990
<i>Restricted fund</i>		
Charities Aid Foundation	-	41,472
BBC Children in Need	-	31,743
Hollie Foundation	7,569	2,000
Hospital Saturday Fund	2,000	
William French Memorial Awards	53,553	-
	<u>63,122</u>	<u>86,205</u>

5. Income from other trading activities		
<i>General fund - unrestricted</i>		
Fundraising events	65,926	108,370
Recovery of costs charged to related parties (note 18)	55,115	-
	<u>121,041</u>	<u>108,370</u>

6. Investment income		
<i>General fund - unrestricted</i>		
Bank interest	2,547	1,487
Dividend income	6,009	5,106
	<u>8,556</u>	<u>6,593</u>

Note to the Financial Statements
For the Year ended 30 April 2024 (continued)

	2024	2023
	£	£
7. Expenditure on charitable activities		
Helpline / family support / travel	106,277	137,287
Information - social media & publications	54,605	47,332
Conference costs	100,477	71,562
Research costs	32,448	37,785
William French Memorial Award	1,250	-
Central office wages	80,658	73,113
Upkeep computer	1,719	977
Rent & rates	14,531	12,042
Other administration costs	4,321	14,503
Insurance & subscription	2,725	2,932
Bank charges	146	188
Executive & management meetings	11,199	5,135
Strategic review meeting	-	15,844
Management & staff meetings	168	2,696
Independent examiner's fee	2,268	2,520
	<u>412,792</u>	<u>423,918</u>
Charged to - <i>General fund - unrestricted</i>	397,931	254,146
- <i>Restricted fund</i>	<u>14,861</u>	<u>169,772</u>
8. Tangible assets		
	Equipment	
	£	
Cost		
At 30 April 2023 and 2024	<u>4,158</u>	
Depreciation		
At 30 April 2023 and 2024	<u>4,158</u>	
Net Book Value		
At 30 April 2023 and 2024	<u>-</u>	
9. Fixed asset investments		
	2024	2023
	£	£
Balance brought forward	201,933	218,333
(Impairment) / Increase in value	<u>2,867</u>	<u>(16,400)</u>
Market value at 30 April 2024	<u>204,800</u>	<u>201,933</u>
Historical cost of investments held at 30 April 2024	<u>220,000</u>	<u>220,000</u>
Investments at fair value comprise:		
	2024	
Fixed interest	39.63%	
Securities	33.19%	
Property	7.30%	
Alternatives	18.73%	
Cash	1.15%	

The fair value of investments is determined by reference to the quoted price for identical assets in an active market at the balance sheet date.

Note to the Financial Statements
For the Year ended 30 April 2024 (continued)

	2024	2023
	£	£
10. Debtors		
Prepayments	5,789	3,354
Amounts due from INPDA (note 18)	127	2,699
Amounts due from INPDR (note 18)	-	2,497
Other debtors	1,891	3,063
	<u>7,806</u>	<u>11,613</u>
11. Creditors: amounts falling due within one year		
Amounts due to INPDR	-	-
Amounts due to INPDA	-	243
Other tax & social security	2,123	2,296
Accruals and deferred income	23,961	12,092
	<u>26,084</u>	<u>14,631</u>
12. Employee information	No	No
The average monthly number of employees was:	<u>6</u>	<u>7</u>
	£	£
Staff Costs		
Salaries	133,237	160,007
Social security costs	13,812	16,210
Pension costs	2,805	3,273
	<u>149,854</u>	<u>179,490</u>

No staff member is remunerated at a level in excess of £60,000 per annum.

The key management personnel of the charity comprise the chief executive and the finance and administration officer.

Their total employee benefits were £83,492 (2023: £82,678).

13. Operating Leases

The total minimum future lease payments under non-cancellable operating leases are as follows:

	2024	2023
	£	£
<i>Expiry date</i>		
Not later than one year	10,800	10,800
Later than one and not later than five years	1,800	12,600
	<u>12,600</u>	<u>23,400</u>

14. Pension Costs

The company operates a money purchase (defined contributions) pension scheme. The assets of the scheme are held separately from those of the company in independently administered funds. The pension cost represents contributions payable by the company to the funds and amounted to £2,805 (2023: £3,273).

Note to the Financial Statements
For the Year ended 30 April 2024 (continued)

15. Statement of Funds

	Balance 30/4/2023 £	Income £	Expenditure £	Transfer £	Gains / (Losses) £	Balance 30/4/2024 £
2023-2024						
General fund	210,305	305,491	441,298	12,166	-	86,664
Designated fund	12,166	-	-	(12,166)	-	-
Total unrestricted funds	222,471	305,491	441,298	-	-	86,664
Endowment fund	201,933	-	-	-	2,867	204,800
Restricted funds						
Stewardship Fund	-	4,042	4,042	-	-	-
Hollie Foundation	-	7,569	7,569	-	-	-
Lesley Carlton Jones	2,000	1,000	1,000	-	-	2,000
Hospital Saturday Fund	-	2,000	1,000	-	-	1,000
William French Memorial Award	-	53,553	1,250	-	-	52,303
Total restricted funds	2,000	68,164	14,861	-	-	55,303
Total funds	426,404	373,655	456,159	-	2,867	346,767

	Balance 30/4/2022 £	Income £	Expenditure £	Transfer £	Gains / (Losses) £	Balance 30/4/2023 £
2022-2023						
General fund	241,360	227,379	270,600	-	-	198,139
Designated fund	24,332	-	-	-	-	24,332
Total unrestricted funds	265,692	227,379	270,600	-	-	222,371
Endowment fund	218,333	-	-	-	(16,400)	201,933
Restricted funds						
BBC Children in Need	-	31,743	31,743	-	-	-
Charities Aid Foundation 1	65,490	36,472	101,962	-	-	-
Charities Aid Foundation 2	8,975	5,000	13,975	-	-	-
Stewardship Fund	-	3,000	3,000	-	-	-
Hollie Foundation	-	2,000	2,000	-	-	-
Lesley Carlton Jones	2,000	1,000	1,000	-	-	2,000
Orphazyme	16,092	-	16,092	-	-	-
Total restricted funds	92,557	79,215	169,772	-	-	2,000
Total funds	576,482	306,594	440,372	-	(16,400)	426,404

Note to the Financial Statements
For the Year ended 30 April 2024 (continued)

15. Statement of Funds continued

Fund descriptions

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

The **designated funds** are funds set aside for the specific purposes outlined in the Trustees Annual Report on page 12.

The **endowment fund** is expendable and is available to be used, when required, to assist in financing the family care and support service provided to families affected by Niemann-Pick Diseases. Should the Charity be forced to close, this endowment will be used to fund alternative care and support arrangements during a period of transition to new providers.

The **restricted funds** are for use in terms of the grants awarded by the donors as follows:

BBC Children in Need	Continuation funding towards the post of Clinical Nurse Specialist
Charities Aid Foundation 1	Towards continuation and development of services
Charities Aid Foundation 2	Towards strengthening organisation
Stewardship fund	Research
Hollie Foundation	Funding of Family Officer
Lesley Carlton Jones	Peter Carlton Jones award
Orphazyme	Towards the collection of patient evidence to support submissions to regulator in support of drug efficacy
Hospital Saturday Fund	To support care and support services
William French Memorial Award	(managed by NPUK on behalf of NRPF) to support UK based scientists / researchers working in the field of NPC

16. Analysis of net assets between funds

	Unrestricted Funds £	Restricted Funds £	Endowment Funds £	Total Funds £
2024				
Fixed asset investments	-	-	204,800	204,800
Current assets	112,748	55,303	-	168,051
Current liabilities	(26,084)	-	-	(26,084)
Net assets at 30 April 2024	86,664	55,303	204,800	346,767

	Unrestricted Funds £	Restricted Funds £	Endowment Funds £	Total Funds £
2023				
Fixed asset investments	-	-	201,933	201,933
Current assets	237,102	2,000	-	239,102
Current liabilities	(14,631)	-	-	(14,631)
Net assets at 30 April 2023	222,471	2,000	201,933	426,404

Note to the Financial Statements
For the Year ended 30 April 2024 (continued)

17. Trustees

No remuneration was paid to Trustees during the financial period. During the year 10 trustees (2023: 9) were reimbursed travel and administration expenses in pursuit of charitable objectives and this amounted to £6,317 (2023: £4,385). Of this amount, £3,520 was paid for accommodation for the two-day Strategic Review meeting.

18. Related Party Transactions

Niemann-Pick UK is a member of the International Niemann-Pick Disease Alliance (INPDA). It also works closely with the International Niemann-Pick Disease Registry (INPDR). INPDR is the sole shareholder of INPDR Gateway Ltd. Various costs were incurred in the year by Niemann-Pick UK on behalf of these organisations and recharged.

The transactions in the year and the year end balances are as follows:

	2024	2023
	£	£
With INPDA		
Wages	4,681	4,393
Other costs	1,404	7,162
Reimbursement of research costs paid	(7,911)	(8,611)
Reimbursement of PhD study received	1,665	-
Sum due from NPUK	127	2,699
Sum due to NPUK (note 10)	-	(243)
With INPDR		
Wages	14,608	11,113
Other costs	3,240	3,365
Reimbursement of research costs paid	(24)	-
Reimbursement of PhD study received	2,500	-
Sum due to NPUK	-	2,497
With INPDR Gateway Ltd		
Wages	31,182	-