



**NIEMANN-PICK UK**

**Company number 07775835**

**Charity registration numbers**

**1144406 (England & Wales)**

**SC045407 (Scotland)**

**REPORT AND FINANCIAL STATEMENTS**

**FOR THE YEAR ENDED 30 APRIL 2025**

**NPUK (Niemann-Pick UK)** is a charitable organisation dedicated to improving the lives of everyone affected by Niemann-Pick diseases - patients, their families, and the health professionals who support them. We raise awareness, provide expert practical and emotional support, share reliable information, and facilitate vital research into new and emerging therapies.

As the **only UK patient organisation** offering specialist support for those affected by Niemann-Pick diseases, we have, over the past 34 years, gained unique insight into what it means to live with these complex conditions and the challenges they present.

Through our work, we aim to **enhance quality of life** by helping patients and families manage the daily realities of Niemann-Pick diseases. Those we support consistently report improved physical and emotional wellbeing, reduced anxiety and isolation, and greater confidence in navigating care and treatment.

Our **vision** is a world where the burden of Niemann-Pick diseases is minimised, where everyone affected has access to effective therapies, and where individuals can participate fully in society, reach their potential, and live the best quality of life possible.

NPUK relies entirely on voluntary donations, charitable grants and fundraising to support the services we provide, and we are hugely grateful to all those who donate funds to support the services we provide for those affected by Niemann-Pick diseases.

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**Reference & Administrative Details  
For the Year ended 30 April 2025**

<i>England &amp; 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 Professor Frances Platt Anthony Mead Wynne
<i>Chief Executive:</i>	Toni Mathieson
<i>Co-opted Members:</i>	Dr. Claire Fyvie Dr. Uma Ramaswami (Appointed, 2/11/2024)
<i>Bankers:</i>	HSBC 33 Town Square, Basildon, Essex SS14 1BA
<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 2025

The trustees present their annual report and financial statements of the charity for the year ended 30 April 2025. 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 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.

Niemann-Pick diseases are a group of rare, inherited Lysosomal Storage Disorders (LSDs) that cause harmful levels of cholesterol and other lipids (fatty substances) to accumulate within the body's cells. This buildup leads to progressive damage in vital organs such as the liver, spleen, lungs, and brain. There are two distinct forms of Niemann-Pick disease, each with different genetic causes and patterns of progression but overlapping clinical features.

### Acid Sphingomyelinase Deficiency (ASMD – Niemann-Pick types A, A/B and B)

ASMD is caused by a deficiency of the enzyme **acid sphingomyelinase**, which leads to toxic lipid buildup in cells. It represents a clinical spectrum ranging from the severe, infantile **Type A** form to the chronic, later-onset **Type B**, with **Type A/B** exhibiting features of both.

- **ASMD Type A** is the most severe form, primarily affecting the brain and major organs. It presents in infancy with rapid neurological decline and organ enlargement; most affected children sadly do not survive beyond early childhood.
- **ASMD Type B** is a chronic form that does not significantly affect the brain but impacts the spleen, liver, and lungs. Individuals may experience respiratory and liver complications but often live into adulthood.
- **ASMD Type A/B** includes individuals who show both visceral and neurological symptoms to varying degrees.

NPUK currently supports 13 children and 29 adults living with ASMD across the UK.

### Niemann-Pick disease type C (NPC),

NPC is a progressive, neurodegenerative disorder caused by changes in either the NPC1 (approximately 95% of cases) or NPC2 gene. These mutations impair the transport of cholesterol and other lipids within cells, leading to their accumulation in organs such as the liver, spleen, and brain. The condition is highly variable: symptoms, severity, and age of onset differ widely - even within the same family. Common features include difficulties with movement, speech, swallowing, cognition, and coordination, alongside liver or lung involvement.

NPUK currently supports 60 children and 72 adults affected by NPC.

Both ASMD and NPC are progressive, life-limiting genetic conditions that cause complex physical, neurological, and emotional challenges. Families often face long diagnostic journeys, limited treatment options, and barriers to accessing expert care. The rarity of these conditions can lead to isolation and a lack of understanding across health and social care systems.

### How we help,

We provide specialised support, advocacy, and information to ensure that affected individuals and their families do not face this journey alone. Our aim is to minimise the burden of living with Niemann-Pick, by providing practical advice, emotional support, and disease-specific information that helps to build community knowledge, confidence, and resilience. We are committed to helping every individual reach their full potential, 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 support and information services are also used by the health, social and education professionals involved in the care of patients and their families.

**Trustees Annual Report  
For the Year ended 30 April 2025****Review of Activities (Continued)****How we help (continued)**

Our strong governance, strategic and operational leadership ensure that the needs and voices of our community are 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 these diseases 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 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 (UK 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 regular volunteers (35) 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 having a family member diagnosed with a rare condition. The NPUK team has made an enormous difference to how we cope with this condition".*

The past year has presented many challenges for families affected by Niemann-Pick diseases, and this has led to a sustained high demand for the services we provide. The NHS and the Social Care sector has continued to struggle with their planned recovery programme, and this has had a profound impact on the ability of our families to access healthcare and statutory services.

## Trustees Annual Report For the Year ended 30 April 2025

### Achievements and Performance (Continued)

#### *Care and Support, (continued)*

These issues have been compounded by the escalating cost of living crisis and ongoing benefit reforms, which have made it harder for families to access crucial disability benefits. This financial strain adds another layer of stress to already vulnerable households and increasingly, those most in need are having to rely on siblings to act as carers. Furthermore, both newly diagnosed families and those experiencing the distress of disease progression are reporting increased difficulty in securing necessary medical support leading to heightened anxiety and unmet clinical needs.

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. Family and liaison with health and social care professionals have enabled families to access more timely support from local health and statutory services and to reduce anxiety and stress.

Our Clinical Nurse Specialist has provided individualised patient-centred care and support for newly diagnosed patients, and those experiencing disease progression. Liaising with patients, families, clinicians and healthcare providers, she has enabled access to vital and timely support. Helping to maintain patients' mobility, cognitive function, and physical and mental wellbeing. The Clinical Nurse Specialist has also provided tailored information sessions in schools for teaching staff and pupils and has given guidance on the disease, how to manage symptoms, feeding and other related aspects of Niemann-Pick experienced by children attending school.

The work of our Families Officer had a significant impact on the emotional wellbeing of patients and their families. In an increasingly tough 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.

An essential part of her role is organising clinic days for children and adults and advocating for access to Special Educational Needs and Disabilities support, Education Health and Care Plans and home adaptations.

This support has been essential to those who find it difficult to navigate these processes and to ensuring they achieved the desired outcome. Providing ongoing emotional support and a counselling service for newly diagnosed and bereaved families and organising family meetings, events and fun days throughout the year ensures that our families never feel alone and always have someone to turn to.

#### *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 three regional meetings, at places of interest in Rotherham (June), RHS Bridgewater, Salford (September) and Eastbourne (October). 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 with each other, to 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 place at St John's Hotel, Solihull (November) and offered an ideal opportunity for an early visit from Santa Claus. The party, as always, was a great 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 individual presents for all affected children and young adults.

#### *Interactive Workshop and Family Conference,*

Our Annual Family Conference and Interactive Workshop for professionals takes place every eighteen months and this year took place at Wyboston Lakes over a 3-day period from 4 to 6 April 2025. The whole event was attended by 213 professional and family delegates including a total of 80 community members from the UK (64); Germany (3); Spain (3); Belgium (2); Brazil (2); USA (3); South Africa (1); Canada (1) and Taiwan (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.

Professionals attending 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.

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**Trustees Annual Report  
For the Year ended 30 April 2025****Achievements and Performance (Continued)***- Interactive Workshop,*

The Interactive Workshop on Niemann-Pick Diseases takes place on the day before the Family Conference and is a platform for scientists, clinicians, researchers, and industry representatives to collaborate and share insights into advancing the understanding of Niemann-Pick diseases from basic research into the cellular mechanisms of Niemann-Pick diseases across the spectrum to the factors affecting the understanding and care of patients.

There were 5 workshops made up of 24 presentations including research updates, understanding and treating Niemann-Pick diseases, preclinical updates and advancing the care and understanding in Niemann-Pick diseases. Each workshop pulled together the latest developments and innovations in the key areas and gave hope and a strong commitment to improving the lives of people affected by Niemann-Pick diseases.

*- Family Conference.*

51 speakers from 8 countries across the world presented, in parallel sessions, a number of informed topics reflecting the most up-to-date information available. These focused on several research topics including current trials and therapies for ASMD Niemann-Pick diseases and Niemann-Pick type C, plus combined sessions covering childhood dementia, mental health, developing emotional resilience and the International Niemann-Pick Disease Registry. Stories of hope and inspiration were also included and interactive sessions exploring impact, listening and learning were well attended.

The special 'Reflections' support group, which was set up specifically for those whose child or loved one have passed away was available throughout the conference. This safe and welcoming space, away from the main conference programme, is where bereaved families and individuals can meet together privately and talk freely about their memories, individual thoughts and feelings and provide support for each other.

Patients and families were also able to connect with the NPUK staff and trustees throughout the weekend, and feedback received from those attending the conference 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. This helped to ensure parents and carers had peace of mind when attending presentations and workshops, as well as being able to enjoy social time together.

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.

**Call to action.**

During the Interactive Workshop and the Family Conference, our CEO, highlighted NPUK's deep disappointment at the decision by the National Institute for Health and Care Excellence (NICE) not to recommend Xenpozyme (olipudase alfa) for routine NHS use in treating ASMD types A/B and B. At this event, NPUK took the opportunity to launch the 'Life Is Rare' Campaign to advocate for a change in this decision, and access to this groundbreaking enzyme replacement therapy.

Despite acknowledging the treatment's clinical effectiveness, NICE deemed its cost to be too high under the current framework for highly specialised medicines.

This decision denies patients access to a therapy that could significantly improve their quality of life and slow disease progression, leaving them with no option except complex and costly supportive care. This decision has had a devastating impact on the ASMD community in England, Wales and Northern Ireland, whilst patients in Scotland can access the drug.

The CEO called for collaboration among NICE, the NHS, policymakers, and pharmaceutical companies to find a sustainable solution that ensures patient access. She highlighted the need for flexibility in assessing rare disease therapies and urged all parties to engage in meaningful discussions.

A recent study showed life-changing effects of the treatment, and the decision has highlighted inequalities in patient access in the UK. The CEO assured families that NPUK remains committed to advocating for equitable access to Xenpozyme and will lobby for change and improvement to the NICE process and guidelines, that currently work against the approval of drugs for rare diseases.

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**Trustees Annual Report  
For the Year ended 30 April 2025****Communications and Information.**

*"NPUK is a massive community network which means I do not 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. These strong connections with shared experience, mutual support and empathy leads to long-lasting friendships and reduces loneliness and isolation.

Social media platforms and messaging services are our most active form of communication, and our presence is growing across all platforms. To gain maximum impact, 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. However, we took note of feedback that reported that the site was too complex and difficult to navigate and commencing in 2024 with our chosen partner, this work will be complete in the second quarter of 2025.

We have won multiple film awards and have developed a strong reputation for producing short films and campaigns that create awareness of issues affecting the Niemann-Pick and wider Rare Disease Community. We have witnessed the impact and benefits of using this form of communication to engage with the wider general public, advocacy groups and key stakeholders. We have built on this strong reputation to create powerful video testimonies to highlight the unmet needs within the Niemann-Pick Community and raise awareness of the urgent need for improved access to care, treatment and services for those affected. This includes a short film for the 'Life is Rare' Campaign, focussing on our ASMD community, and the profound impact of NICE's decision not to approve Xenpozyme for use as a routine treatment available on prescription through the NHS.

**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 to influence policy for the benefit of all those affected by rare diseases. and to strengthen our collaborative campaigns for better healthcare outcomes for the LSD and wider rare disease community

At an organisational level, we have built on our strong relationship with the UK Lysosomal Storage Disorder (LSD) Collaborative and, in consultation with each of the charities involved, have held formal discussions and taken legal advice about how we might more effectively utilise our combined resources to provide essential services and reduce overall costs. The outcomes from these discussions are now being considered by each of the member charities and NPUK will further debate the possibilities at a strategic review meeting in July 2025.

We continue to provide first-hand support to both the International Niemann-Pick Disease Alliance (INPDA) and the International Niemann-Pick Disease Registry (INPDR) in their global activities,

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.

The INPDA is a global network of non-profit organisations working in the field of Niemann-Pick disease (NPD). Founded in 2009, through the collaborative efforts of NPUK and the National Niemann-Pick Disease Foundation (USA), the INPDA now has 29 member groups in 21 countries:

Both the INPDA and the INPDR are independent charitable companies with NPUK representatives appointed as Board Members.

We have developed strong relationships with scientific institutions and pharmaceutical companies around the world to appropriately assist in facilitating research and clinical trials. This activity is key to developing new and improved treatments, diagnostic tools, and care strategies that will lead to better health outcomes and higher quality of life for our community.

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**Trustees Annual Report  
For the Year ended 30 April 2025****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 multiple scientific conferences and presented a poster at the WORLD symposium, as well as maintained 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.

NPUK, along with our community, has been involved in the development of Xenpozyme (olipudase alfa) for over 25 years. During this time patients and families have participated in multiple surveys and research studies, with some taking part in the clinical trials. These efforts helped to further our understanding of ASMD Niemann-Pick diseases and to support the successful development of this effective therapy.

It was, therefore, deeply disappointing that the National Institute for Health and Care Excellence (NICE) did not recommend Xenpozyme as a treatment for ASMD Niemann-Pick diseases type AB and type B (6 March 2024). This resulted in our scarce resources being redirected to support preparation of an appeal, which was upheld. However, despite acknowledging the clinical effectiveness of Xenpozyme, NICE concluded in its Final Guidance (2 April 2025) that the cost of the treatment is too high to justify NHS funding under its current framework for highly specialised medicines.

This prompts further involvement and effort from NPUK, including the designation of critical resources in taking forward the “Life is Rare” campaign to highlight the growing gap in access to innovative rare disease medicines (that are available in other countries) and the need for change in the NICE appraisal process.

In exciting news for NPC families, two drugs were recommended by the USA Food and Drug Administration (FDA) for the treatment of NPC. These will now progress through regulators in other countries, including the European Medicines Agency (EMA) and the Medicines and Healthcare products Regulatory Agency (MHRA) in the UK. This is positive and encouraging news for our community and we await the outcome of regulatory reviews and subsequent NICE appraisals.

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 programme. Getting an accurate diagnosis, even when there is no treatment, provides the opportunity for people living with a rare disease and their families to plan their care and future decision making. However, this is a novel campaign, and we need to gain traction by achieving a wider collaboration to influence decision-makers.

**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”. In addition, 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.

This year, our part-time Fundraising Officer continued 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. In addition, they raise funds in lieu of birthdays, anniversaries and in memory of their loved ones.

NPUK also supported the 18th 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 success with the Tesco Blue Token Scheme providing grants for the care and support of local families living with Niemann-Pick diseases in the Birmingham areas. In addition, our strong connection with the Rotary Clubs of District 1060, centred on Birmingham and the West Midlands was further strengthened when a trustee and our fundraising officer attended their annual conference and gave a presentation about the work we do and how families benefit from the services we provide. We were delighted and extremely grateful to their District 1060 Governor for announcing NPUK as one of their charities of the year.

Our regular donors have also 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 support for the work we do.



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**Trustees Annual Report  
For the Year ended 30 April 2025****Fundraising. (continued)**

Having reviewed our Grant Application process, the trustees agreed the need to increase our capacity to write grant applications and during the year, we welcomed a Consultant Grant and Trusts Officer to our team. This helped to increase the number of applications to grant funding organisations which resulted in thirteen successful grant applications, ranging from £500 to £19,851 and totalling £82,681. This was an increase of £73,112 over the previous year.

**Impact.**

Our desired impact is to improve quality of life for UK patients and families, by supporting them in coping with, and minimising, the day-to-day challenges presented by Niemann-Pick diseases. During 2024/2025 we supported 76 children, 98 adult patients and 488 family members.

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 of better care, hope and quality of life for all those 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 describes by our community as a roller coaster of emotions. We are therefore developing our own combination of measures that accurately reflect the patient and family experience and the impact of our work. We are committed to making a meaningful difference, and to recording the impact of our work 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.
- 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 actions we take to 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.

Our Annual Conference is a terrific opportunity for our families and patients to tell us about the impact our work makes to their daily lives. 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 daily living, with some families 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, felt less anxious and better able to cope after accessing support, advice and guidance from our Clinical Nurse Specialist, particularly when they could not achieve a timely appointment to see their healthcare team
- 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.

**Trustees Annual Report  
For the Year ended 30 April 2025****Impact. (continued)**

- Patients and family members accessing our counselling and wellbeing support service reported improved levels of confidence, emotional resilience and mental wellbeing, and described positive change in their relationships and ability to communicate their feelings.
- Loneliness and isolation were reduced, through contact with our team and opportunities to attend the Family Conference and Christmas Party plus virtual and in-person regional events.
- Harder to reach groups have become more integrated into the Niemann-Pick community, feel more comfortable in accessing support services and participating in community events.
- Children and young adults affected by Niemann-Pick diseases have maintained fine motor and cognitive skills and participated 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 support from the NPUK community, involvement in our Reflections programme and access to counselling.
- Professionals tell us that access to our services helps patients and families to cope better 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 the Charity's 'objects'. All decisions are agreed collectively and in the best interest of the Charity.

- Looking forward, there remains a need to recruit a Treasurer. Although widely advertised through NPUK networks and other appropriate channels, we have not yet been able to fill this position. In the meantime, the previous Treasurer kindly continues in the role as an acting Treasurer.

Succession Planning for the post of Chief Executive Officer remains a risk to the future success of the organisation and limited financial resources have delayed the recruitment of an Executive Assistant. Trustees providing limited support to the Chief Executive Officer is no longer a viable proposition and this matter will be discussed at the Strategic Review Meeting in July 2025.

- A strong dependence on key NPUK members of staff with no spare capacity has increased the need to review our organisation structure and further strengthened the need to consider closer collaboration with the UK Lysosomal Storage Disease (LSD) Collaborative to make the most of common resources in order to improve the effectiveness and resilience of our organisation.

- At the Board Meeting in June 2024, the Trustees took the difficult decision not to renew the NHS contract for the provision of our NPUK Clinical Nurse Specialist (CNS). This was a strategic decision and was influenced by four key factors which are summarised below,

- A community consultation indicated a greater need for mental health and non-clinical support.
- The provision of a CNS service at each of the eight NHS Specialist Centres across the UK, a service which has emerged since our own was established.
- The end of a three-year BBC Children in Need grant (August 2022), and an inability to reapply due to changing eligibility criteria. This was very disappointing news and brought to an end 23 years of valued funding and support from the BBC Children in Need programme.
- Strategic decisions regarding succession planning and organisational structure could not be progressed due to financial constraints, and it was therefore agreed that continued funding of the CNS post was no longer sustainable at this time.

The Trustees considered that, since the formation of the Niemann-Pick Support Nurse role in 1999, the NHS had introduced five national adult and three paediatric "Specialist Lysosomal Storage Disease Centres". Each has an expert, multidisciplinary team delivering high-quality clinical care, psychological support and nutritional guidance for Niemann-Pick patients.

The provision of this service was a key factor in the Trustees decision making process, and the decision to end this supportive role - and a successful arrangement with the NHS - was not taken lightly.

The role of Clinical Nurse Specialist, has, through the dedication and commitment of two role-holders made a lasting and significant difference to the lives of multiple patients and families over many years. In addition, it created a strong foundation for mutual support and collaboration between NPUK and the NHS, that will continue into the future.

**Trustees Annual Report  
For the Year ended 30 April 2025****Governance (continued)**

We recognise that this change will require adjustment for the families we support, and we are committed to ensuring a positive and coordinated approach between our Families Officer and each of the Specialist Centres. Together, we will continue to advocate for patients and families to receive the appropriate level of ongoing advocacy and clinical support.

- Following approval by the Trustees, endowment funds of £85,000 were transferred to 12-month fixed rate products with the Redwood Bank with a yield of 5.2%

**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 NPUK.

**Principal Risks.**

The key risks based on probability and impact are:

- Increasing competition for grants and pharmaceutical donations make long term planning, financial stability and service sustainability less predictable and harder to achieve.
- Key members of staff, under an increasing workload and work-related stress, will consider leaving the organisation if we do not provide additional support. This would cause a severe disruption to Business Continuity and the services we provide.
- Increasing competition and a reduction in grant support for small, national single-disease charities like our own, will affect our ability to secure grant and trust funding to support and sustain our services
- Further benefits reform, external economic and social pressures will increase the demand for our service and reduced levels of disposable income could reduce donations to the charity and make fundraising more difficult.
- Intentions to further reduce NHS service costs and the lack of reimbursement for rare disease medicines will lead to market withdrawal, reduced research activity and will increase the demand for NPUK Care and Support Services.

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

- Trustees will hold a Strategic Review Meeting in July 2025 to consider proposals to strengthen the organisation structure to ensure we have a sustainable charity that can successfully adapt to the identified risks and challenges of the future whilst continuing to achieve our objectives and goals.
- We will increase engagement with politicians and lobby policy makers through collaboration with the Specialised Health Care Alliance, Genetic Alliance UK and other umbrella organisations to influence rare disease policy and access to treatment.
- We will investigate ways to further strengthen our collaboration with the UK LSD Collaborative to achieve common objectives, widen the services we provide and leverage funds.
- We will maximise our influence with rare disease policy makers to advocate for a change in NICE evaluations and processes for rare diseases and lobby for reforms to the Highly Specialised Technologies (HST) Programme.
- We will strengthen our support and relationships with pharmaceutical companies and encourage their engagement with regulatory bodies in the UK to anticipate and overcome obstacles to reimbursement for rare disease drugs.

We will re-commit the resources necessary to develop a suitable (bespoke) impact measurement tool that can be integrated with our existing database and increase our ability to connect with our wider patients and stakeholders

**Plans for Future Periods.**

- We will hold a Strategic Review meeting in July 2025 to develop a three-year strategy to address succession planning, and to create a robust, resilient, and financially sustainable charity that has the necessary resources to provide the services necessary to support the patients and families we support.
- 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.
- We will further develop our services to support a growing number of adult patients with complex and/or mental health needs, and will work with our partners in the UK LSD Collaborative and other relevant organisations, to influence national policy and improve access to mental health services for the Niemann-Pick and wider LSD community.
- We will build on the “*Life is Rare*” campaign and lobby politicians and policymakers to promote equal access to medicines and therapies for rare disease patients and support changes to NICE and HTA appraisal processes.
- We will keep up to date with the latest research into Niemann-Pick diseases and focus on supporting the UK Newborn Screening Collaborative for the early detection of LSDs and Niemann-Pick diseases.
- We will collaborate with UK research and academic institutions and, where possible, provide small grants in support of relevant projects. 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.

**Trustees Annual Report  
For the Year ended 30 April 2025**

**Plans for Future Periods (Continued)**

- We will further strengthen our working relationship with the UK LSD Collaborative and explore ways to effectively utilise our combined resources, reduce overall costs and enable more patients to access and benefit from our combined services.
- We will continue to 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 in its role to collect worldwide data and information about the health and experience of Niemann-Pick patients.
- We will promote 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 Highly Specialised 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.

**FINANCIAL REPORT.**

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 for the foreseeable future.

**INCOME,**

Income for the financial year 2024/2025 was £479,696 (against £422,159 for the previous year) and a prior year adjusted budget of £477,401.

In more detail:

**Conference income and grants (£144,775)**

- Income for the Interactive Workshop and Family Conference was £144,775 against a budget of £112,500 (+£31,275). Of this amount, £134,329 was donated by pharmaceutical companies without any control or influence over the content of the programme or the selection of speakers.

**Restricted Voluntary donations (£1,000)**

Donations of £1,000 were received towards the Peter Carlton Jones memorial award.

**Unrestricted Voluntary donations (£144,014)**

*Unrestricted Voluntary Donations*

- Income from voluntary donations amounted to £43,437 against a budget of £35,000 (+£8,437).
- Unrestricted grant applications amounted to £42,800 against a budget of £50,624 (-£7,824).
- Additional grants from pharmaceutical companies amounted to £39,000 (£34,000 from Azafaros and £5,000 from Zevra). These were used to collect evidence of patient reported outcomes in support of establishing drug efficiency.
- The balance of funds was made up of Gift Aid totalling £7,719 and six other small amounts totalling £11,058.

**Restricted Income from charitable activities (£46,777)**

- Income from Charitable Activities amounted to £46,777 and was made up of donations to the William French Memorial Fund (£6,896) and two grants received from the National Lottery Community Fund totalling £39,881.

**Other Trading Activities (£138,980)**

*Fundraising,*

Fundraising events contributed £81,728 against a budget of £81,726. These included,

- A one-off Memories Ball; £18,595 against a budget of £10,000 (+£8,591),
- Family and Community events including 12 fundraisers and the Great North Run totalling £25,539 against a budget of £21,989 (+£3,550),
- The NPUK Annual Golf Day; £11,495 against a budget of £10,000 (+1,495),
- On-line giving platforms; £26,069 against a budget of £44,060 (-£17,962). This shortfall was almost entirely made up of a shortfall in JustGiving of £17,264 (£17,796 against a budget of £35,060),
- Receipts from the on-line shop totalling £30.

In the difficult financial landscape faced by many of our families, we are extremely grateful to all those who spent so much of their time in organising and completing fundraising events and making contributions through on-line platforms.

## Trustees Annual Report Financial Report (Continued)

### Other Trading Activities (continued)

#### *Third Party Reimbursements,*

During the year we received third party reimbursements totalling £57,252 in wage costs and overheads against a budget of £51,856 (+£5,396). This represents appropriate reimbursements for the hours spent by NPUK staff in supporting the following related organisations; INPDA £9,050; INPDR £25,532; and INPDR Gateway £22,670.

#### *Investment income,*

Bank interest of £5,150 was received on deposits from cash held in the HSBC Business Money Manager account.

### EXPENDITURE.

Expenditure for the year was £476,175 against a budget of £480,888. This gave a marginal reduction in expenditure of £4,713 against the budget.

All budgets were tightly controlled but there were two adverse budget accounts:

- The costs of the Family Conference and the Interactive Workshop, over three days, was £127,342 against a budget of £115,580 (+£11,762). However, this budget overspend was entirely funded by unrestricted conference donations (£143,775).
  - The conference overspend was attributed to the costs of the venue plus facilities which were £8,469 over budget (£116,549 against a budget of £108,080 plus Speakers travel and expenses which were £3,705 over budget (£8,205 against a budget of £4,500). These discrepancies are explained by the budget being set prior to the conference programme being finalised.
- The total cost of research, excluding the William French Memorial Award, was £30,544 against a budget of £16,500. (£14,044 over budget). However, this budget overspend was entirely funded by incoming funds (£32,000).
  - The research overspend was caused by a non-forecast study to collect evidence of patient outcomes for a Health Technology Assessment (HTA) at a cost of £13,679 and non-forecast campaign in support of equal access to the treatment for ASMD patients at a cost of £7,200.

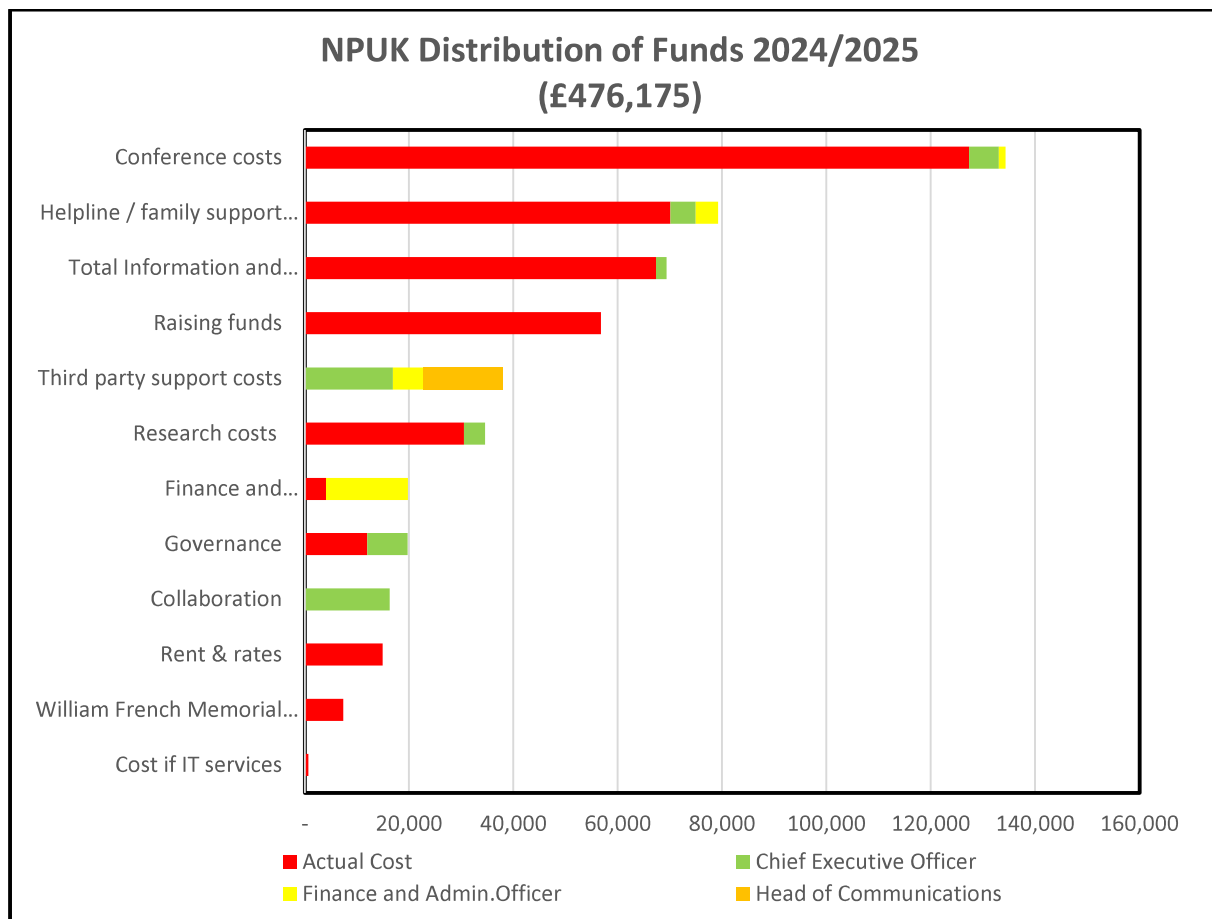
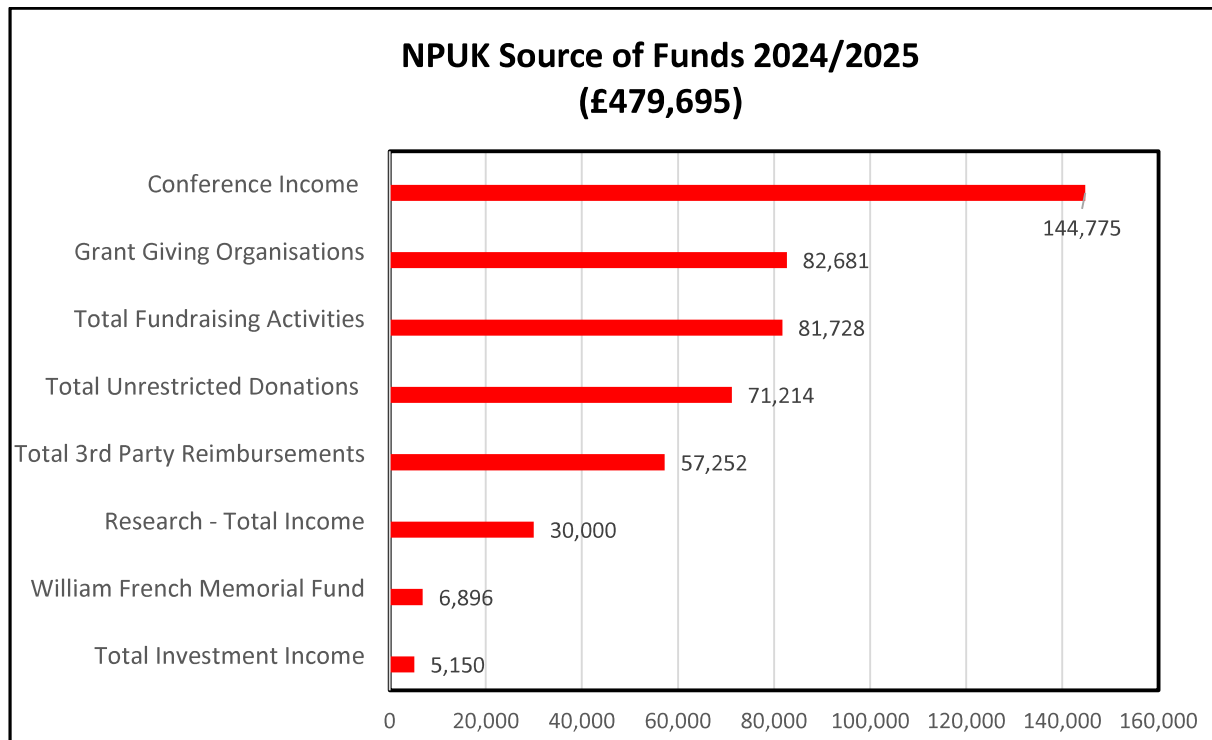
These overspends against budget were partially offset by small saving in other research activities and the HTA study was fully funded by the pharmaceutical company, Azafaros.

- The total cost of all Fundraising Activities was £56,802 (24.11% of voluntary and grant income) against a budget of £62,544. This includes,
  - £16,169 (10.57% of voluntary income) for the cost of events and merchandising.
  - £10,485 (6.86% of voluntary income) for the Fundraising Officer and associated costs.
  - £30,150 (36.47% of grant income) for the consultant grants officer associated costs.

The cost of 'events and merchandising' reflects the increasingly high cost of staging grand events balanced against ticket prices and the high relative cost of raising grant applications reflects the cost of researching and submitting a total of 13 successful applications with a value of £81,681 plus 81 applications, pending a decision from the grant funders, with a total value £730,351 and 644 applications which were unsuccessful.

**Trustees Annual Report  
For the Year ended 30 April 2025**

**Charts.**



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**Trustees Annual Report  
For the Year ended 30 April 2025****Charts (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.

**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 to fund the operation of the Charity within a reasonable period of receipt, 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 Finance Subcommittee 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 2025 to April 2026, takes into account:

- The risk of raising insufficient incoming funds during the financial year 2025/2026, 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 additional risk of reduced grant opportunities resulting from a growing trend by large grant giving organisations towards funding umbrella organisations in favour of smaller national charities.

**Level of Reserves.**

The Trustees have reviewed the Reserves Policy and have considered the key objectives and commitments for the period 2025/2026. 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 2025/2026 are, therefore:

- To address a possible reduction in the incoming funds from pharmaceutical companies (£20,000).
- To address the risks associated with a reduced rate of success in obtaining grant funding (£30,000).
- To hold sufficient funds (4 weeks) to manage salaries and cash flow for the day-to-day operation of the Charity (£33,063).

The total desired free reserves are therefore £83,063 which is equivalent to 4 weeks of planned expenditure. This is considered the minimum level of reserves necessary to manage the identified risks, wage costs and cash flow.

**Designated Funds.**

- There are no commitments that require designated funds to be set aside.

**Free Reserves.**

The level of free reserves at the end of April 2025 was £65,614. This means a shortfall in free reserves of £17,449 to meet the desired level of £83,063. The Trustees therefore approved the transfer of a £10,000 fixed interest bond to be transferred from the endowment to the operations fund, on maturity in August 2025, should it become necessary to do so.

The Trustees will take action to secure the necessary levels of income and mitigate the risks identified to ensure that the charity safeguards against financial uncertainty.

**Endowment.**

NPUK has an expendable endowment that can be used, if necessary, to assist in financing of the family care and support service provided to families affected by Niemann-Pick diseases. In addition, the Trustees have the discretionary power to transfer the endowment to Operation Funds or Restricted Funds should it become necessary to do so.

The value of the endowment on 30 April 2025, stood at £205,233 and is held as cash at the HSBC bank (£120,233) and as 12-month fixed term bonds with the Redwood Bank (£85,000).

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**Trustees Annual Report  
For the Year ended 30 April 2025****Endowment (Continued).**

If, at any time, the Charity is forced to close, the 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, Head of Communications, Social Media Assistant, Families Officer and Fundraising Officer. (The Group also paid the full employment costs of a Clinical Nurse Specialist who is employed by the Salford Royal NHS Foundation Trust up to the end of September 2025). 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.



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Trustees Annual Report  
For the Year ended 30 April 2025

**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,

*Helen Carter*

H. Carter  
Trustee

5 November 2025



J. Melville  
Trustee

5 November 2025

I report on the accounts of the company for the year ended 30 April 2025 which are set out on pages 18 to 27.

#### 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.

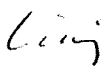
#### Basis of independent examiner's report

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

5/11/2025

**Statement of Financial Activities**  
**(Incorporating Income and Expenditure Account)**  
**For the year ended 30 April 2025**

	Notes	Unrestricted Funds £	Restricted Fund £	Endowment Fund £	Total Funds £	As Restated 2024 £
<b>Income and endowments from:</b>						
Donations and legacies	3	287,789	1,000	-	288,789	182,936
Charitable activities	4	-	46,777	-	46,777	109,626
Other trading activities	5	138,980	-	-	138,980	121,041
Investments	6	5,150	-	-	5,150	8,556
<b>Total income</b>		<u>431,919</u>	<u>47,777</u>	<u>-</u>	<u>479,696</u>	<u>422,159</u>
<b>Expenditure on:</b>						
Raising funds		56,802	-	-	56,802	43,367
Charitable activities	7	396,167	23,206	-	419,373	412,792
<b>Total expenditure</b>		<u>452,969</u>	<u>23,206</u>	<u>-</u>	<u>476,175</u>	<u>456,159</u>
<b>Net income / expenditure before gains on investments</b>		(21,050)	24,571	-	3,521	(34,000)
Net gains / (losses) on investments		-	-	433	433	2,867
<b>Net movement in funds</b>		<u>(21,050)</u>	<u>24,571</u>	<u>433</u>	<u>3,954</u>	<u>(31,133)</u>
<b>Reconciliation of funds:</b>						
Total funds brought forward		<u>86,664</u>	<u>103,807</u>	<u>204,800</u>	<u>395,271</u>	<u>426,404</u>
<b>Total funds carried forward</b>	15	<u>65,614</u>	<u>128,378</u>	<u>205,233</u>	<u>399,225</u>	<u>395,271</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 2025**

	Notes	2025 £	As restated 2024 £
<b>Fixed Assets</b>			
Tangible fixed assets	8	-	-
Investments	9	-	204,800
		<u>-</u>	<u>204,800</u>
<b>Current Assets</b>			
Debtors	10	57,056	56,311
Bank & cash		358,349	160,244
		<u>415,405</u>	<u>216,555</u>
<b>Creditors</b>			
Amounts falling due within one year	11	16,180	26,084
<b>Net Assets</b>		<u>399,225</u>	<u>395,271</u>
<b>Funds</b>	15		
Operations fund		65,614	86,664
Designated fund		-	-
Unrestricted funds		<u>65,614</u>	<u>86,664</u>
Endowment fund		205,233	204,800
Restricted funds		128,378	103,807
		<u>399,225</u>	<u>395,271</u>

The charitable company is entitled to exemption from audit under Section 477 of the Companies Act 2006 for the year ended 30 April 2025.

The members have not required the charitable company to obtain audit of its financial statements for the year ended 30 April 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 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 18 to 27 were approved by and signed on behalf of the board of directors on 5 November 2025.

*Helen Carter*



H. Carter, Trustee

J. Melville, Trustee

**Notes to the Financial Statements  
For the Year ended 30 April 2025****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 2025 (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 2025 (continued)**

	2025 £	As restated 2024 £
<b>3. Income from donations &amp; legacies</b>		
<i>General fund - unrestricted</i>		
Conference income and grants	143,775	99,531
Voluntary donations	144,014	76,363
<i>Restricted fund</i>		
Voluntary donations	1,000	7,042
	<u>288,789</u>	<u>182,936</u>

Included above were unrestricted grants received from pharmaceutical companies:

Sanofi Genzyme, Cyclo Therapeutics, Zevra Denmark, Azafaros BV, Intrabio Inc.

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.

**4. Income from charitable activities**

<i>Restricted fund</i>		
Hollie Foundation	-	7,569
William French Memorial Awards	6,896	102,057
National Lottery Community Fund	39,881	-
	<u>46,777</u>	<u>61,122</u>

**5. Income from other trading activities**

<i>General fund - unrestricted</i>		
Fundraising events	81,728	65,926
Recovery of costs charged to related parties (note 18)	57,252	55,115
	<u>138,980</u>	<u>121,041</u>

**6. Investment income**

<i>General fund - unrestricted</i>		
Bank interest	5,150	2,547
Dividend income	-	6,009
	<u>5,150</u>	<u>8,556</u>

**Note to the Financial Statements**  
**For the Year ended 30 April 2025 (continued)**

	2025 £	2024 £
<b>7. Expenditure on charitable activities</b>		
Helpline / family support / travel	70,090	106,277
Information - social media & publications	67,318	54,605
Conference costs	127,342	100,477
Research costs	30,544	32,448
William French Memorial Award	7,406	1,250
Central office wages	84,845	80,658
Upkeep computer	724	1,719
Rent & rates	14,987	14,531
Other administration costs	4,110	4,321
Insurance & subscription	2,899	2,725
Bank charges	152	146
Executive & management meetings	5,744	11,199
Strategic review meeting	-	-
Management & staff meetings	829	168
Independent examiner's fee	2,382	2,268
	<u>419,373</u>	<u>412,792</u>
Charged to - <i>General fund - unrestricted</i>	396,167	397,931
<i>- Restricted fund</i>	<u>23,206</u>	<u>14,861</u>
<b>8. Tangible assets</b>		
	<b>Equipment</b>	
	<b>£</b>	
<b>Cost</b>		
At 30 April 2024 and 2025	<u>4,158</u>	
<b>Depreciation</b>		
At 30 April 2024 and 2025	<u>4,158</u>	
<b>Net Book Value</b>		
At 30 April 2024 and 2025	<u>-</u>	
<b>9. Fixed asset investments</b>	<b>2025</b>	<b>2024</b>
	<b>£</b>	<b>£</b>
Balance brought forward	204,800	201,933
Increase in value	433	2,867
Transferred out	<u>(205,233)</u>	<u>-</u>
Market value at 30 April 2025	<u>-</u>	<u>204,800</u>
Historical cost of investments held at 30 April 2025	<u>-</u>	<u>220,000</u>
Investments at fair value comprise:	<b>2025</b>	<b>2024</b>
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 2025 (continued)**

	2025 £	As restated 2024 £
<b>10. Debtors</b>		
Prepayments	1,587	5,789
Amounts due from INPDA (note 18)	1,302	127
Amounts due from INPDR (note 18)	450	-
Other debtors	53,717	50,395
	<u>57,056</u>	<u>56,311</u>

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

Other tax & social security	2,276	2,124
Pensions	552	-
Accruals and deferred income	13,352	23,961
	<u>16,180</u>	<u>26,085</u>

**12. Employee information**

The average monthly number of employees was:	6	6
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	£	£
<b>Staff Costs</b>		
Salaries	129,967	133,237
Social security costs	14,900	13,812
Pension costs	2,841	2,805
	<u>147,708</u>	<u>149,854</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 £87,893 (2024: £83,492).

**13. Operating Leases**

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

	2025 £	As restated 2024 £
<i>Expiry date</i>		
Not later than one year	1,800	10,800
Later than one and not later than five years	-	1,800
	<u>1,800</u>	<u>12,600</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 £2,841 (2024: £2,805).

**Note to the Financial Statements**  
**For the Year ended 30 April 2025 (continued)**

**15. Statement of Funds**

<b>2024-2025</b>	<b>Balance 30/4/2024 £</b>	<b>Income £</b>	<b>Expenditure £</b>	<b>Transfer £</b>	<b>Gains / (Losses) £</b>	<b>Balance 30/4/2025 £</b>
General fund	86,664	431,919	452,969	-	-	65,614
Designated fund	-	-	-	-	-	-
<b>Total unrestricted funds</b>	<b>86,664</b>	<b>431,919</b>	<b>452,969</b>	<b>-</b>	<b>-</b>	<b>65,614</b>
<b>Endowment fund</b>	<b>204,800</b>	<b>-</b>	<b>-</b>	<b>-</b>	<b>433</b>	<b>205,233</b>
<b>Restricted funds</b>						
Stewardship Fund	-	-	-	-	-	-
Hollie Foundation	-	-	-	-	-	-
Lesley Carlton Jones	2,000	1,000	1,500	-	-	1,500
Hospital Saturday Fund	1,000	-	1,000	-	-	-
William French Memorial Award	100,807	6,896	7,406	-	-	100,297
NLC Fund 1	-	19,981	8,325	-	-	11,656
NLC Fund 2	-	19,900	4,975	-	-	14,925
<b>Total restricted funds</b>	<b>103,807</b>	<b>47,777</b>	<b>23,206</b>	<b>-</b>	<b>-</b>	<b>128,378</b>
<b>Total funds</b>	<b>395,271</b>	<b>479,696</b>	<b>476,175</b>	<b>-</b>	<b>-</b>	<b>399,225</b>

**Comparative Statement of Funds**

<b>2023-2024 (as restated)</b>	<b>Balance 30/4/2023 £</b>	<b>Income £</b>	<b>Expenditure £</b>	<b>Transfer £</b>	<b>Gains / (Losses) £</b>	<b>Balance 30/4/2024 £</b>
General fund	210,305	305,491	441,298	12,166	-	86,664
Designated fund	12,166	-	-	(12,166)	-	-
<b>Total unrestricted funds</b>	<b>222,471</b>	<b>305,491</b>	<b>441,298</b>	<b>-</b>	<b>-</b>	<b>86,664</b>
<b>Endowment fund</b>	<b>201,933</b>	<b>-</b>	<b>-</b>	<b>-</b>	<b>2,867</b>	<b>204,800</b>
<b>Restricted funds</b>						
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	-	102,057	1,250	-	-	100,807
<b>Total restricted funds</b>	<b>2,000</b>	<b>116,668</b>	<b>14,861</b>	<b>-</b>	<b>-</b>	<b>103,807</b>
<b>Total funds</b>	<b>426,404</b>	<b>422,159</b>	<b>456,159</b>	<b>-</b>	<b>2,867</b>	<b>395,271</b>

**Note to the Financial Statements**  
**For the Year ended 30 April 2025 (continued)**

**15. Statement of Funds continued**

**Fund descriptions**

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

There are no commitments that require **designated funds** to be set aside.

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:

Stewardship fund	Research
Hollie Foundation	Funding of Family Officer
Lesley Carlton Jones	Peter Carlton Jones award
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
NLC Fund 1	Families in Wales and Family Support
NLC Fund 2	Connected care

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

	Unrestricted Funds £	Restricted Funds £	Endowment Funds £	Total Funds £
<b>2025</b>				
Current assets	287,028	128,378	-	415,406
Current liabilities	(16,181)	-	-	(16,180)
Net assets at 30 April 2025	270,847	139,378	-	399,225
<b>2024 (as restated)</b>				
Fixed asset investments	-	-	204,800	204,800
Current assets	112,748	103,807	-	216,555
Current liabilities	(26,084)	-	-	(26,084)
Net assets at 30 April 2024	86,664	103,807	204,800	395,271

**Note to the Financial Statements**  
**For the Year ended 30 April 2025 (continued)**

**17. Trustees**

No remuneration was paid to Trustees during the financial period. During the year 11 trustees (2024: 10) were reimbursed travel and administration expenses in pursuit of charitable objectives and this amounted to £4,714 (2024: £6,317).

**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:

	<b>2025</b>	<b>2024</b>
	<b>£</b>	<b>£</b>
With INPDA		
Wages	7,978	4,681
Other costs	1,071	1,404
Reimbursement of research costs paid	-	(7,911)
Reimbursement of PhD study received	-	1,665
Sum due to NPUK (note 10)	1,302	127
With INPDR		
Wages	21,622	14,608
Other costs	3,910	3,240
Reimbursement of research costs paid	-	(24)
Reimbursement of PhD study received	-	2,500
Sum due to NPUK	450	-
With INPDR Gateway Ltd		
Wages	20,052	31,182
Other costs	2,618	-

**19. Prior Year Adjustment**

During the year it was identified that funds received in May 2024 should have been included in the previous year's accounts based on the date that the grant was advised to Niemann-Pick UK. Accordingly, the 2024 figures have been re-stated to include £48,504 grant as follows:

	<b>As restated</b>	<b>Original</b>
	<b>£</b>	<b>£</b>
Income from charitable activities	109,626	61,122
Other debtors	50,395	1,891
Restricted funds	103,807	55,303