

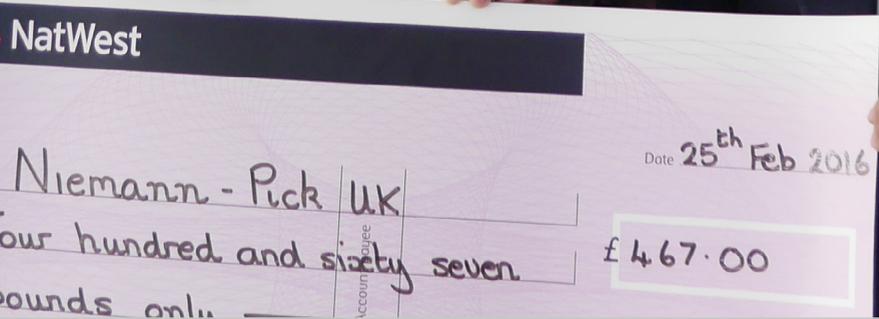
NPUK Annual Report 2015-2016



Niemann - Pick UK

Registered Charity No. 1144406

...AL Year 7: a safe and caring environment in which ... are helped to ach...



NPUK: Supporting those living with Niemann-Pick

Registered Charity Number: 1144406
Company limited by guarantee
Registered in England and Wales

Account No.
Sort Code

G.S.A.



Niemann - Pick UK

Registered Charity No. 1144406

Foreword4

About NPUK8

What is Niemann-Pick Disease?12

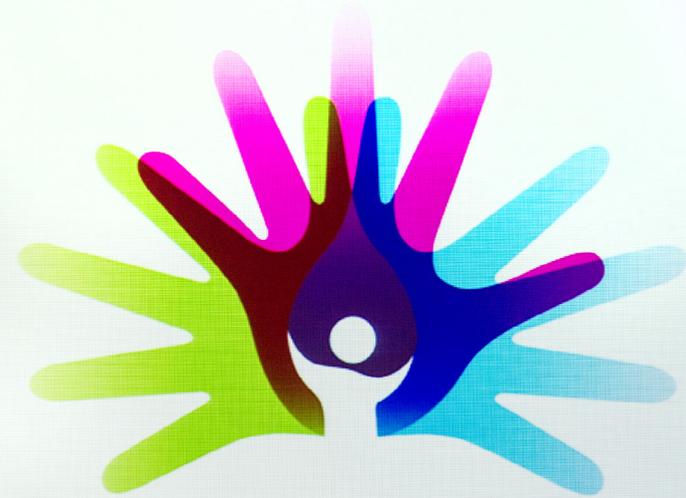
Our Impact18

Our Activities22

Financial Review 2015/2016.....38



Thank you



RARE DISEASE DAY.ORG

Chair's Foreword



David Roberts, who has served as Chair for Niemann-Pick UK for over five years - gives his overview of the charity's activities, plans, and challenges in this annual report.

This year's Annual Review celebrates the many ways in which NPUK has made a positive difference to those affected by Niemann-Pick diseases.

Our Clinical Nurse Specialist, Families Officer, and dedicated Central Office team continue to provide a high quality service, with their work continuing to have a tremendous impact on the Niemann-Pick community. Our achievements are described throughout this report and there are also many examples of how we fulfil our objective of providing much-needed advice, care and support to patients, families and the wider Niemann-Pick community.

In addition, our many passionate volunteers provide much-needed assistance at our Family Conference and in helping to deliver community activities.

Over the year we have fulfilled our key objectives of:

- Keeping patients and families informed about key developments in the field of Niemann-Pick diseases
- Facilitating clinical trials in the UK.
- Engaging with our community, including newly diagnosed patients and those considered to be harder to reach
- Redeveloping our brand and the external face of the Charity in order to assist in reaching a wider audience.

As a result of our work, we have been successful in obtaining a Big Lottery Reaching Communities Grant of £447,108, which will help us to deliver a new project in support of our growing community - "Shaping our Future Together".

The Big Lottery grant has also enabled us to commission an Organisational Strength Review and Training Needs Analysis for staff and trustees. The outcome from these exercises will feed into our next Strategic Review Meeting, due in March 2017. This meeting will review our work to date and set our objectives for the next three year period.

This year has also seen announcements confirming the start of clinical trials in the UK for both ASMD Niemann-Pick disease type B and Niemann-Pick type C, bringing much needed hope to our community. We are also aware of the impact of these trials on families and the many new challenges that they bring to our small, rare disease community and we have worked hard to ensure that families feel informed and have the ability to make an

informed decision about whether to participate. You can read more about these clinical trials later in this report. During the year, we have seen some changes in our Central Office staff and Board of Trustees;

Sue Woodhouse, our Information Officer and much valued team member left NPUK in March 2016 to develop her own business. The role of Information Officer will now be reviewed before we seek to recruit later in the year. In addition, we were joined by Louise Metcalfe whose role as Project Team Leader will ensure that we implement and evaluate our Big Lottery project in line with our commitments. The Board of Trustees was pleased to welcome Joella Melville in March 2016. Joella's experience in Health communications has strengthened the Board's skill profile and her experience in this field will help us focus on raising the profile of Niemann-Pick disease both in the UK and at an international level.

Facilitating research continues to be at the heart of all we do and during the year, thanks to the efforts of our dedicated fundraisers, we were able to provide grant funding in support of projects at the University of Oxford, Cardiff University and the University College London to further basic research into Niemann-Pick type C.

Our annual Interactive Workshop on Niemann-Pick Diseases for professionals and the Family Conference was a huge success once again. These events brought together patients, families, scientists, health care professionals and pharmaceutical companies from around the world and saw two young scientists receive the "Peter Carlton Jones Memorial Award" for their work in furthering the understanding of Niemann-Pick diseases.

Everybody involved was able to share their experience and stories during social time, building stronger networks and encouraging mutual support. Next year we will celebrate 25 years as a Patient Group and hope to build on this year's successes by making next year's celebration even more special.

NPUK continues to provide a voice for all those affected by Niemann-Pick diseases. At a strategic level, we continue to build on our strong relationship with the INPDA and the LSD Patient Collaborative and we have taken part in many high level meetings with national Rare Disease groups in order to influence national policy for the benefit of our all those affected by Niemann-Pick diseases. In addition, we continue our involvement with the Expert Advisory Group for Lysosomal Storage Disorders, which works to improve the clinical standards of care for those affected by these disorders.



Looking ahead, we are well placed to influence and provide a patient voice to NHS England on the NICE process for the evaluation of Highly Specialised Technologies and the provision of new drugs for rare diseases.

We have achieved a great deal over the last year and I hope you enjoy reading some of the highlights in the pages that follow. However, our work could not be accomplished without the amazing support of our families and friends, volunteers and supporters who have so worked so hard to raise funds and provide help and support when needed. The Trustees remain extremely grateful for your contribution to our success as a Charity and we thank you for your ongoing support.

The future promises to be a challenging and exciting time for NPUK, our families, and the whole rare disease community.

Dave
David Roberts
 NPUK Chair

About NPUK

The most important people to us are the members of our community - and as such, the main aims and objectives of the charity are the same now as they have always been...

Our primary focus is to make a positive difference, in any way possible, to the lives of those affected by Niemann-Pick diseases. We do all that we can to relieve sickness and any distress which may arise for the affected individual and their family. We also advance the education and awareness of families, professionals, and the general public in all matters concerning the disease. By sticking to the core values upon which we established 25 years ago we have enjoyed tremendous success and helped an innumerable amount of people along the way.

In order to achieve the above objectives, we rely heavily on funding from voluntary donations, corporate donors, charitable trusts, statutory bodies, and last but not least, the fundraising support of our members, friends, and families.

Our Objectives:

- To make a positive difference to the lives of those affected by Niemann-Pick diseases (NPD)
- Relieve sickness and any distress which may arise from NPD
- To advance the education and awareness of families, professionals and the general public in all matters concerning the disease



Going forward, our goal is relatively simple: to ensure the continuation, and successful delivery, of the services which we are currently providing, whilst also seeking sustainable expansion.

This is why we want to continue in the same way; by providing care and support to those in need, whilst working to reduce the feeling of loneliness and isolation. We are fuelled by the success of our past, and positive about our collective future, together.

We aim to meet our objectives by:

- * The provision of a support and advice service for families and individuals and associated health and social care professionals
- *The collection, collation and dissemination of relevant information
- *The provision of support for national clinical centres of expertise for Niemann-Pick diseases
- *The promotion of relevant research
- *The promotion of national and international collaboration

The Structure of NPUK

Niemann-Pick UK (NPUK), is a charitable organisation offering support and information to families affected by Niemann-Pick disease, and to associated health professionals working in the field of NPD research.

Our charity was formed by a small group of parents back in 1991, and therefore 2016 marks 25 years since its creation - a landmark date which will be celebrated at our Annual Family Conference by both families and attending health professionals.

Since its creation NPUK has gone from strength to strength; in 1997 it was granted charitable status as an independent charity registered with the Charity Commission for England and Wales.

In 2015-2016, NPUK was governed by a Board of ten trustees under our Memorandum and Articles of Association who for the purposes of company law are also the Directors of the organisation. NPUK conforms to the requirements of both Companies House and the Charity Commission for England and Wales and has adopted the provisions of the Statement of Recommended Practice (SORP) 'Accounting and Reporting by Charities' issued in March 2005. In all activities, the Trustees show due regard to the Charity Commission's guidance on public benefit.

The Board is made up of people who have family members or friends directly affected by this group of diseases - and as a result they are passionate about the positive advancement of the charity, and the wellbeing of the families which the charity assists.

All of NPUK's trustees are volunteers, and each takes on responsibilities and duties within the Board to support a particular aspect of the charity's functions.

The Board has met on five occasions this year, with a number of additional teleconferences in between each event to ensure each member is kept up to date with the latest news and advances in the charity's operations. Such meetings and exchanges are necessary as the Board is responsible

for all the activities the charity undertakes, along with operational support and co-opted trustees.

Our three employees at the NPUK Central Office in Washington, Tyne & Wear, are fundamental to the day-to-day running of the charity; as they provide support and advice (by way of a 24 hour helpline and regular social media interaction), and raise much needed awareness of both Niemann-Pick disease and of the social and economic challenges faced by those affected.

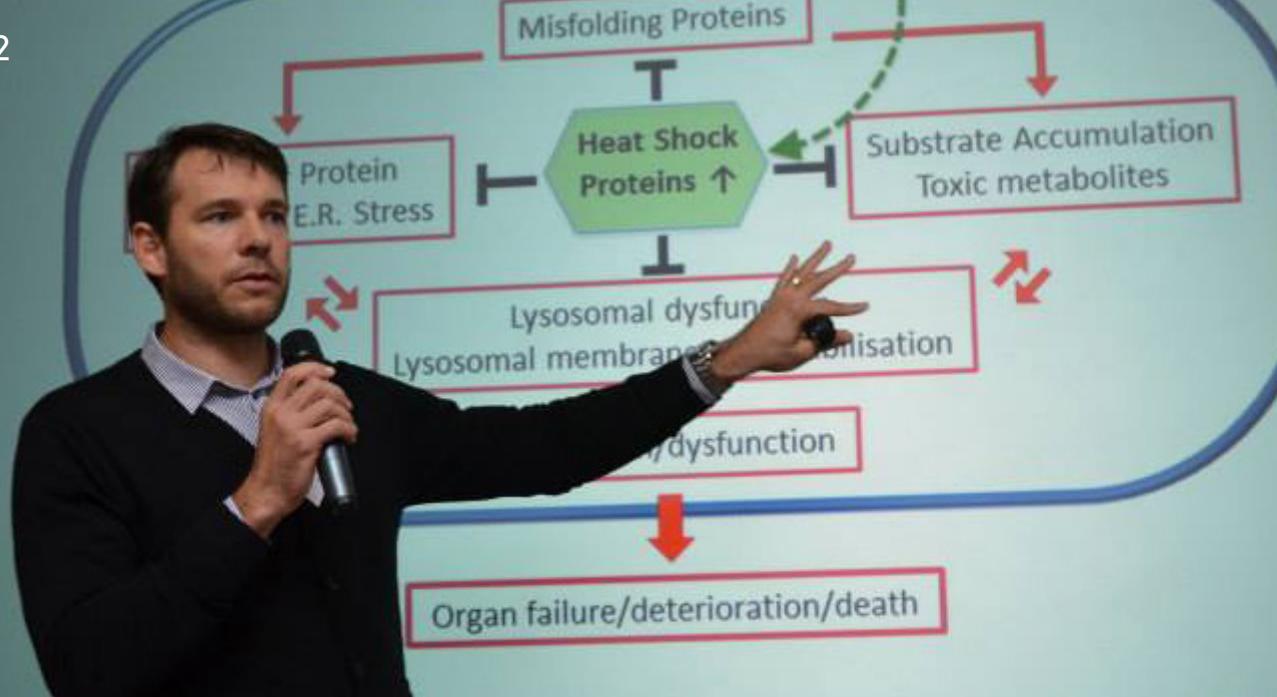
NPUK also employs a Clinical Nurse Specialist, Laura Bell and a Families Officer, Elizabeth Davenport. Their work is invaluable to our community as they offer a unique and dedicated service to those affected by Niemann-Pick disease both directly and indirectly. Also, thanks to the Big Lottery Fund, NPUK has been able to welcome Louise Metcalfe to our staff team in the role of Project Team Leader

Our team are amazing, however it is important to mention that NPUK would not exist without the support of our members, friends, and families...their incredible efforts are vital in assisting the achievement of our aims and objectives.

“NPUK would not exist without the support of our members, friends, and families”

Risk Management

The Board of Trustees have considered and reviewed the possible risks to which the Charity may be exposed, and have established and implemented a risk management strategy, comprising a quarterly risk review and procedures to identify, mitigate and minimise the impact of risks to which the organisation may be exposed.



What is Niemann-Pick Disease?

Niemann-Pick diseases are a group of rare, inherited, metabolic conditions that can affect children and adults. These conditions are caused by specific genetic mutations and are pan-ethnic.

Commonly recognised forms of the disease are: Acid Sphingomyelinase Deficiency (ASMD) Niemann-Pick Disease Type A and Type B which represent opposite ends of 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. Niemann-Pick Type C is not caused by an enzyme deficiency, but the end result is the same; an accumulation of materials (cholesterol and other fatty acids) in the body's cells.

Acid Sphingomyelinase Deficiency (ASMD)

ASMD Niemann-Pick Types A & B, rather than being two separate 'types' of the disease, actually represent the opposite ends of a

spectrum of the same disease, both caused by a deficiency of the enzyme Acid Sphingomyelinase. Many variations exist within this spectrum, in terms of clinical symptoms and rate of progression.

ASMD Niemann-Pick Disease Type A (NP-A)

ASMD NP-A is a rapidly progressive neurological disease that usually reveals itself within the first few months of a baby's life. Symptoms vary, but may include early feeding difficulties, failure to thrive, and an abnormally large abdomen. Life expectancy rarely exceeds five years of age.

ASMD Niemann-Pick Disease Type B (NP-B)

In ASMD NP-B there is generally little if any neurological involvement. Symptoms can include an enlarged liver and spleen, delayed puberty, susceptibility to respiratory infections and increased stress on the heart. Most patients will survive into their teens and adulthood.

Niemann-Pick Disease Type C (NP-C)

The presentation of NP-C is very variable and the onset of symptoms may occur at any time from early infancy to adulthood. Life expectancy varies considerably and there are a variety of symptoms. These may include an enlarged spleen, liver, and in newborn babies there may be prolonged jaundice.

The disease is neurologically degenerative leading to progressive loss of motor skills and difficulty with walking. Speech can become slurred and swallowing problems may develop. Patients may experience sudden loss of muscle tone which can lead to falls, and epileptic seizures that are generally difficult to control.

A symptom that is particularly suggestive of NP-C is difficulty with upward and downward eye movement. In those young adults, where onset is later, psychological problems and dementia can be major symptoms.

Is there a Cure?

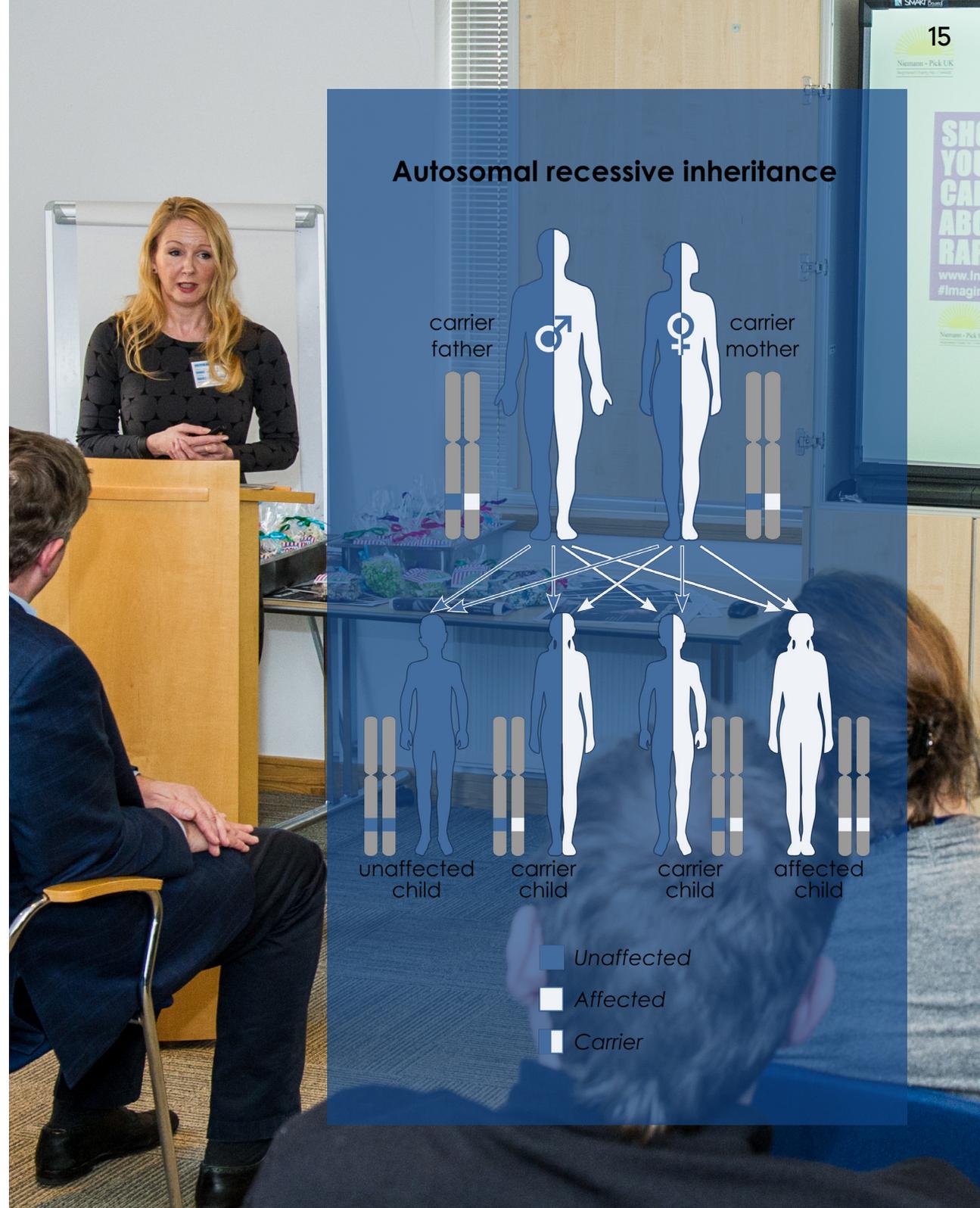
At present, there is no cure for Niemann-Pick disease, although there is considerable research activity taking place on a global scale. Currently, those affected may benefit from palliative treatments - individual medication to treat the symptoms of the disease.

Research and Treatment

Over the years NPUK has built a strong working knowledge in the field of NP research by developing and sustaining robust relationships with key figures and organisations across the world. Due to NPUK's standing within the research community the charity is kept up to date with research that is taking place, its status, and where it is happening. Since its creation, NPUK has played a key role in supporting and facilitating many research projects.

NPUK desires to forever be on the forefront of any and all advances in the NPD arena - this tenacity has led to the development of extensive clinical experience, built up by our passionate staff, community members, and the existence of highly specialised clinical centres and a wclinical nurse specialist: all of which makes the UK very well placed to be a part of any future trials.

For further information on research please turn to page 24.



Expert Care

We continue to support and work collaboratively with the designated highly specialised centres caring for those affected by Niemann-Pick Disease and other Lysosomal Storage Disorders (LSDs).

In England, LSD patients have had access to a nationally managed specialised service since 2004, providing equitable access to expert clinical care and treatment which can make a true difference. The centres are as follows:

*Paediatric Highly Specialised LSD Centres:

- Great Ormond Street Hospital, London
- Birmingham Children's Hospital
- Manchester Children's Hospital

*Adult Highly Specialised LSD Centres:

- National Hospital for Neurology and Neurosurgery, London
- Royal Free Hospital London
- Queen Elizabeth Hospital, Birmingham
- Addenbrooke's Hospital, Cambridge
- Salford Royal Foundation Hospital, Manchester

Patients in Wales, Scotland, and Northern Ireland also have designated specialist hospitals providing care for LSD patients:

- Wales: University Hospital of Wales, Cardiff
- Scotland: Yorkhill Children's Hospital, Glasgow
- Northern Ireland: City Hospital, Belfast

We provide a unique care and support service for those affected by Niemann-Pick diseases and their families.

We are the only UK charity offering care, support, information, and advice to the whole Niemann-Pick community including patients

parents, carers, extended family, health and social care professionals, science and research academia. Our service is closely monitored to ensure that each activity is of value and benefit to the community we support, our team consists of:

Elizabeth Davenport, NPUK Families Officer, provides an individual non-clinical advice and information service for affected individuals and their families, plus counselling and support following bereavement.

Laura Bell, our Clinical Nurse Specialist, provides those affected and their families with expert care and practical advice within the clinic setting or at home.

Our NPUK Central Office Team, Chief Executive, Toni Mathieson, Finance and Administration Officer, Christine Jopling, and Big Lottery Project Leader, Louise Metcalfe, support the day to day management of our Central Office, operating our 24 hour helpline and ensuring the smooth running of all of our activities.



NPUK: Our Impact

A breakdown
of how we have
progressed, and the
impact of our work
in 2015/2016

Communication with our community

Our dedicated 24 hour helpline handled 1,284 calls over the year. This service means that our community can always count on us!



Fundraising and awareness

£85,713 has been raised through extensive fundraising campaigns and the collective effort of our volunteers, families, and friends.



Increased interaction with scientists and health professionals

The NPUK Annual Family Conference saw a record number of scientists and health professionals in attendance sharing the latest research and clinical updates for our community. In addition, our Clinical Nurse specialist handled 2,025 contacts from professionals over the 12 month period.



Care, support and interaction

As our community grows, so does the necessity for care and support. We now have more employed staff than ever before, all who are dedicated to helping our community as much as possible. Events such as our Annual Family Conference, Christmas Party, and regional get-togethers throughout the year provide fantastic opportunities for our community to connect and communicate.



Making a Difference

A little more our impact, and how we help ...



Public Benefit

The Trustees confirm that they have complied with the duty in section 4 of the Charities Act 2006 when planning our activities for the year, NPUK Trustees carefully consider the Charity Commission's guidelines on Public Benefit. Through our work, we aim to ensure that each individual affected by, or connected to, this disease, is able to access the best possible care, support and information appropriate to their needs. In working towards our charitable aims, the Group undertakes a wide range of activities in the three key areas of Care and Support, Information and Research.

Unique Care and Support Services

NPUK provides a unique care and support service to families affected by the Niemann-Pick diseases. The service has been developed over a number of years and is closely monitored to ensure that each activity is of value and benefit to the community we support.

This year we have;

- Provided support and information to families and associated professionals in the form of a 24-hour help line, a comprehensive website, educational literature, regular newsletters and bulletins - with over 1,000 information packs being distributed throughout the year.
- Continued to fund the post of a dedicated Clinical Nurse Specialist for Niemann-Pick Diseases, with support from family fund raising, BBC Children in Need, and the Big Lottery Fund - this post attracted over 70 enquiries per month, all of which were dealt with positively.
- Offered UK wide networking and mutual support opportunities for families through the organisation of an Annual Family Conference, Clinic Days, a website, social networking sites and the production of a Family Directory.



Our Activities

2015/2016 has been a year full of productivity and vitality for NPUK and our fantastic community. Through our continued collective efforts we have been able to achieve so much this year - and our collective future looks even more positive than ever!

Here are just a few of the activities and events we have offered this year:

* Supporting and Influencing Research

Over the years the NPUK has built a strong working knowledge in the field of NP research. By developing and sustaining robust relationships with key figures and organisations across the world, the Group is kept up to date with research that is taking place, its status and where it is happening.

* NPUK Annual Family Conference

The NPUK Annual Family Conference 2015 was held in September at Wyboston Lakes, Bedfordshire and once again offered the opportunity to hear the latest developments from around the world regarding therapies and clinical trials for Niemann-Pick disease. Many of our speakers travel great distances to be with us year after year and are well aware of the difficulties in conveying the science, as well as in living with the challenges of Niemann-Pick disease. Although their presentations can be complex, it brings hope of a brighter future knowing that potential therapies are constantly being sought.

* Care & Support Service

Through our team of skilled staff, we have carried on providing an individual care, support, and advocacy service for those affected by Niemann-Pick disease, including individuals, families, and associated health and social care professionals.

Our service is flexible and offers a wide range of support on a needs led basis. Our Clinical Nurse Specialist and Families Officer provide a unique support service to those affected, with the aim of ensuring that each family or individual receives the level of support they require to meet their needs. Due to the rarity of these conditions, many health and social care professionals have little or no knowledge or experience to assist them in caring for affected individuals, therefore our expert support service is much-needed and appreciated at both a local and national level.

* Info & Awareness

We recognise the need to provide current, relevant, information and news regarding all aspects of Niemann-Pick disease. In order to facilitate this we produce a wide range of informative materials and educational resources to assist families and professionals in dealing with all aspects of Niemann-Pick disease. These materials include our newsletter, website, social media channels, and much more! We ensure they are regularly updated to reflect the changing needs of those we support.

Supporting Research, Raising Awareness

For almost 25 years, facilitating progress towards therapeutic interventions for NPD has been central to everything we do as a charity.

With increased research activity and additional clinical trials on the horizon for ASMD Niemann-Pick type B and Niemann-Pick type C, this year, our efforts have largely been focused on providing clear and accurate information to our community.

In addition, there has been a greater need for collaborative working to ensure the patient voice is heard and can in turn influence progress.

Our Board of Trustees, the majority of whom have family members or friends directly affected by NPD, understand the urgency to find effective therapies and the fact that progress never seems fast enough.

By working together through the relationships and networks we foster and by sharing knowledge and expertise we believe progress will be even faster.

In 2015/2016 we have continued to:

- * Actively support and, where appropriate, fund scientific research into Niemann-Pick disease. This year, we have supported studies at the University of Oxford, Cardiff University, and the University College London.
- * Maintain links with clinicians, scientists, and the pharmaceutical industry in the development of potential therapies for NPD both in the UK, and the USA.
- * Attend relevant scientific and research events around the world, including but not limited to; Brains for Brain, the British Paediatric Neurology Association Annual Conference, and the Society for the Study of Inborn Errors of the Metabolism (SSIEM) annual event.
- * Monitor scientific advances and effectively communicate news in a number of different format - through our website, newsletter, social media, e-bulletins and of course face to face at our Annual Family Conference.

The Peter Carlton Jones Memorial Award:

We do everything we can to nurture the interest of young researchers, as they truly are the future. One way in which we enthuse young scientists is through our annual 'Peter Carlton Jones Memorial Award', which is presented to an individual engaged in either research, teaching, treatment, or care within the public or private sectors in the United Kingdom.

The award, of up to £1,000 is granted in response to the submission of a research project which provides an original contribution to the scientific or public understanding of the Niemann-Pick diseases and/or their treatment or cure.



The International Niemann-Pick Disease Registry (INPDR)

The International Niemann-Pick Disease Registry (INPDR) is an EU funded project to establish a global registry for Niemann-Pick diseases.

Why is the INPDR needed?

A registry is an important tool for collecting and recording information about a disease, such as the effect it has on a patient and how it progresses. At the moment registries are typically held by pharmaceutical companies to investigate a single drug. This can lead to several registries for the same disease, and more often than not, limited access to the data they hold.

The INPDR is a disease registry owned by patient groups and clinicians involved in the care of Niemann-Pick disease patients.

It will enable progress by allowing authorised access to anonymised clinical data, helping to identify and recruit patients to clinical trials, coordinate research efforts and improve patient outcomes globally.

The INPDR is owned by the International Niemann-Pick Disease Alliance and is being developed and coordinated by University Hospital's Birmingham NHS Foundation Trust (UHB) in the UK, with 11 Associate Partners across 7 EU countries (including NPUK) and 17 international collaborating partners in the USA, Canada, Italy, France, Estonia, Netherlands, Australia, Brazil, and Argentina.

This 2m project commenced in April 2013 and is co-funded by the EU Health Programme and the partner organisations. Niemann-Pick UK is leading Work Package 2, delivering the dissemination of the project.

This much-needed project is truly a collaboration between clinicians, scientists, and patient associations across the world, with the collective aim of improving care and treatment for Niemann-Pick patients worldwide.

“The INPDR will generate a highly-needed public resource that unites diverse international attempts to better understand the biology and clinical picture of Niemann-Pick diseases. The research community may learn how to better diagnose the disease and why some patients are doing better than others. For the individual patient, the information therein might speed up inclusion into clinical trials.”

Dr. Heiko Runz, MD





NPUK Annual Family Conference 2015

Every year we hold the NPUK Family Conference and Interactive Workshop...and each year it keeps on getting bigger and better than the last. This year was no exception!

In the beautiful picturesque setting of Wyboston Lakes, Bedfordshire our community, which includes those affected by Niemann-Pick disease directly, were able to meet together with health professionals who are carrying out research in the NPD field. Such a meeting is a unique experience for families and scientists alike, which is why so many people look forward to our Conference!

There were, as always, a number of activities and opportunities for every single member of our community, no matter their age.

Everything from tombolas, to interactive workshops, research updates to theme park visits - this is exactly what makes our Annual Family Conference such a treasured and widely loved event!

The separate 'Children and Young Person's Activity Programme' is especially helpful for the parents in attendance as it gives the opportunity for mothers, fathers, and carers to know that their children are in safe hands whilst they take part in workshops, and hear talks and presentations from the most respected health professionals in the NPD field.

'We would like to say a huge "THANK YOU" to our wonderful volunteers who contributed to the success of our Annual Family Conference this past year.

The big-hearted bunch enriched the social aspect of the weekend with tombolas, raffles, and craft stalls - and most importantly they kept the children and young adults entertained with endless fun, games, and a trip to Wicksteed Park. Thanks to their vital help, parents were able to participate in the main Conference sessions to learn of recent advances in the field of Niemann-Pick diseases and share their experiences with professionals and families.

We genuinely could not run the conference successfully without their help and we owe a huge debt of gratitude to them for giving up their precious time for our collective benefit - THANK YOU from all at Niemann-Pick UK. As ever, we look forward to the next conference with our community!'

*Christine Jopling
Finance & Administration Officer*

Our Activities: National and International Collaboration

Through collaboration with other organisations and groups we are able to make larger and more meaningful strides towards our collective goals.

INPDA

The International Niemann-Pick Disease Alliance (INPDA) was formed in 2009 and provides a forum for the exchange of information, experience and knowledge, with the aim of accelerating progress. The INPDA brings together non-profit Niemann-Pick support organisations from around the world. Members include: Argentina, Australia, Brazil, Canada, China, France, Germany, Italy, the Netherlands, Norway, Spain, Switzerland, USA and the United Kingdom.

The Alliance provides a network that enhances the transfer of information, maximizes the use of resources and demonstrates that collectively, Niemann-Pick diseases are not as rare as was once thought. Further information can be found at www.inpda.org

The UK LSD Collaborative

Lysosomal storage disorders (LSDs) are a group of approximately 40 rare inherited metabolic disorders that result from defects in lysosomal function, including the Niemann-Pick diseases.

The UK LSD Collaborative consists of representatives from the Association of Glycogen Storage Diseases, Batten Disease Family Association, the Gauchers Association, the Society for Mucopolysaccharide Diseases, Save Babies Through Screening Foundation - representing Krabbe Disease, The Cure & Action for Tay-Sachs Foundation (The CATS Foundation), and Niemann-Pick UK. The Collaborative has undertaken to jointly promote and share understanding of LSDs, to advance standards of care and to enhance the well-being of those affected.

Students 4 Rare Diseases

'3.5 million people in the UK suffer from a rare disease (such as Niemann-Pick disease), 75% of which are children and often these diseases are life threatening.'

- students4rarediseases.org



Students4rarediseases aims to bring clinicians and patients together to increase awareness of rare diseases. One way in which they are able to achieve this is by promoting the benefits of early diagnosis to future doctors - if they are aware of rare conditions and are able to recognise their related symptoms, then they will be able to improve care and treatment for patients.

For these reasons, and countless more, The LSD Collaborative, of which Niemann-Pick UK is a founding member, is pleased to support students4rarediseases.



Source: <http://www3.nd.edu/>

Fundraising

Here is a look-back on some of our fundraising heroes from the past year, and all of their amazing selfless work. However, it is only a small taste of the many people who have helped us over the year - there are a lot more and we are thankful for every single one of them!



* It's great to have warriors on our side in the fight against Niemann-Pick disease! The Niemann-Pick Warriors raised over £1000 for NPUK as a result of competing in a Dragon Boat Race at Hetton Lyons Country Park on Saturday 5th September.



* NPUK fundraising went down-under when the fantastic Helen Roberts, took part in the gruelling Sydney Marathon in September, raising awareness and funds for NPUK in the process!

* Thanks to the Blue Moon Harmony Choir for their kind donation of £1,760, raised at their Autumn Contrasts Concert in November. The choir entertained an audience of 200 people in the stunning surroundings of All Saints' Church, Cheltenham.



* 'Fit for Sam', which attracted generous matched-funding from Santander, raised an INCREDIBLE £8090.28! This was the culmination of a lot of hard work and effort - so we want to thank each and every person who was involved!



* In March generous customers of Morrison's at Doxford Park in Sunderland, donated a whopping £407.81 to NPUK on a winter weekend!



* Sing Rock choir in Stevenage dedicated a performance to NPUK raising an amazing £385 in support of our work. The concert was well attended, and enjoyed by all!



* Huge thanks goes to the fantastic teams who endured 'Le Tour de Force' bike ride and Tough Mudder challenges on our behalf. With matched funding they were able to raise a combined total of over £7,000!





* The Vitality British 10K London, an event which is sure to test even the hardest of souls, was swept aside by our wonderful Niemann-Pick UK team of fundraisers (*pictured above*) back in July, 2015. They each had personal reasons for running, and each and every one of them made us extremely proud through their fantastic attitude and dedication.

* "MELFEST", held in July 2015, was a rock festival which truly made a difference for NPUK! This event featured a range of brilliant bands who all gave their time and talent voluntarily. Thank you to everyone who attended - you helped raise £600 for Niemann-Pick UK - you truly do rock!

Huge thank you to all of our fantastic fundraisers, your unbelievable efforts are truly appreciated!



DONATE NOW WITH
JustGiving™

There are many ways in which to get involved and make a true difference in the fight against Niemann-Pick disease...one of which is by supporting NPUK with donations:

The large majority of our fundraisers use the above global online social platforms for donations - either 'Virgin Money Giving', or 'Just Giving'. By setting up individual pages attributed to their specific fundraising event on either of these sites, they are able to easily advertise their fantastic efforts to their friends on social media who can then do their part by donating towards the worthy cause.

If you live in the UK you can donate to NPUK easily from your mobile phone - simple text: NPUK02 followed by the amount you'd like to donate to 70070. For example e.g. "NPUK02 £5" to 70070 (UK ONLY).

If you prefer you can always make a donation to NPUK by cheque, made payable to 'Niemann-Pick UK' and sent to: NPUK Central Office, Suite 2, Vermont House, Concord, Washington, Tyne & Wear, NE37 2SQ.

We rely heavily on funding from voluntary donations, so every little truly helps.

How to Donate and Make a Difference!

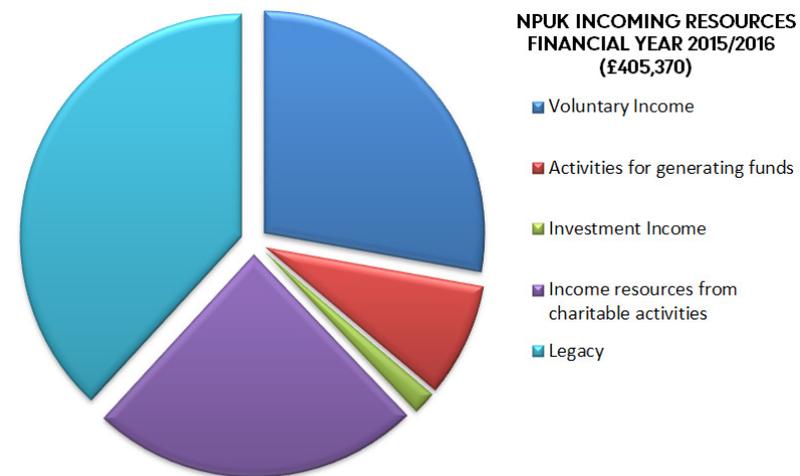
Financial Review:

2015/2016



Below is a brief analysis of the money NPUK has raised, and the money it has spent - which has allowed the charity to continue their support of those affected by Niemann-Pick disease.

(The following review should be read in conjunction with the Report and Financial Statements for the Year Ended 30 April 2016)



The total income for 2015/2016 was £405,370, of which £154,560 was kindly donated as an unrestricted legacy. The underlying income was therefore £250,810. This was slightly better than the previous year (£246,919) and £4,297 better than the budget.

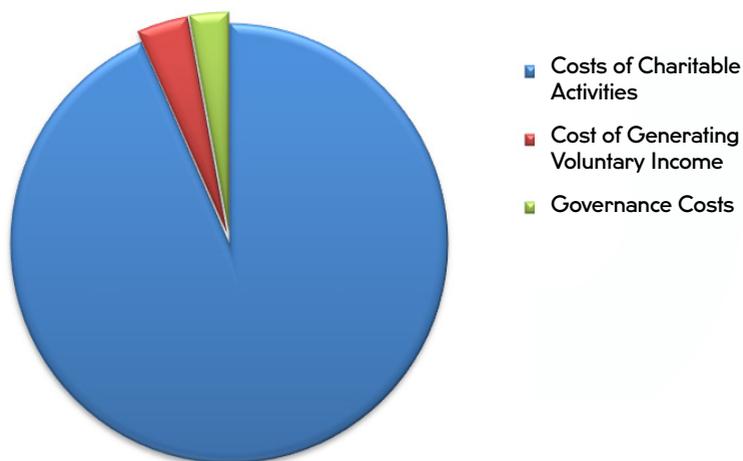
£115,375 of the total income was raised by donations and fundraising activities through the generosity of families, friends and the wider Niemann-Pick community and included £29,154 donated by pharmaceutical companies as unrestricted grants. Without this support, we would not be able to provide the core services which so many families have come to rely on.

In addition, £97,460 was raised through grant giving organisations including a Big Lottery Grant of £55,589. This was towards a care and support project "Shaping our Future Together" and was part of a 5 year grant totalling £447,108.

We are very grateful to all our grant providers for helping to provide financial stability for our care and support service. Income from Gift Aid (£5,542) and interest the endowment fund (£6,945) was spent on the care and support service and the interest received from the Endowment was equivalent to 3.13% of the total.

Total Income, including the legacy, exceeded expenditure by £136,148 and a proportion of this sum will be allocated to NPUK reserves. Independent Financial advice will be taken about investing medium term reserves to gain a better return than the current bank account rate of interest of 0.03%.

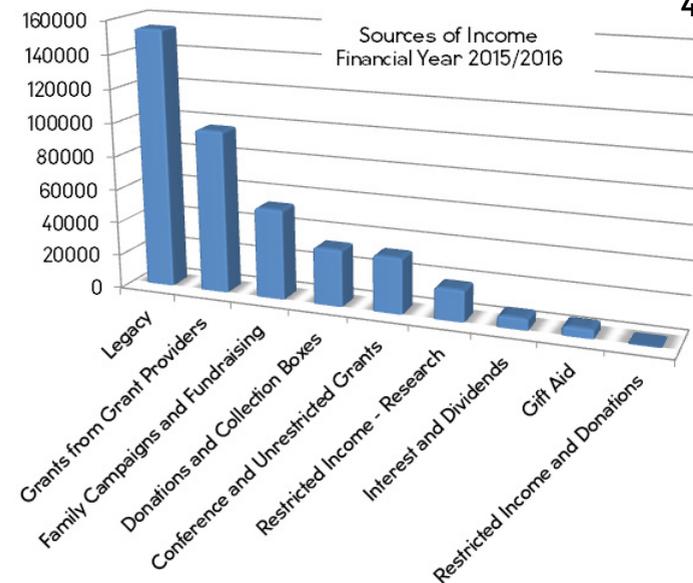
**NPUK RESOURCES EXPENDED
FINANCIAL YEAR 2015/2016
(£269,222)**



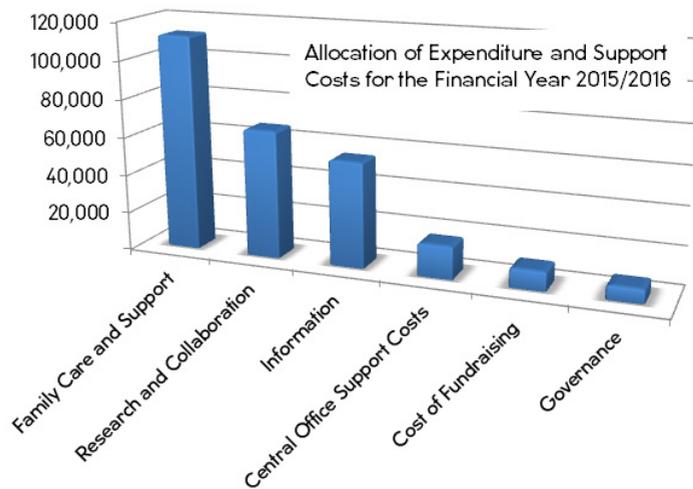
Total expenditure for 2015/2016 was £269,222. This was £14,677 higher than the previous year but better than budget by £3,798. The increase in expenditure was, in part, attributed to the planning and implementation of the Big Lottery project (£9,552). In addition, the conference costs shown in the Financial Statement appear to be £13,265 higher than the previous year. This was due to a change in accounting practice and included £10,107 for the Professionals' Interactive Workshop, which in 2014/2015 was allocated to Research.

A full breakdown of income and expenditure is shown on the charts on page 41.

The bulk of our income is raised through volunteer fund raising and donations, so we always ensure that we allocate the largest majority of our funds towards our 'Care and Support Services'; meaning that the funds are going directly to those affected by NPD.



Some of our lowest percentage costs are incurred by fundraising costs and governance. This is in contrast to some larger charities who may sometimes distribute their costs with a less positive share.



Net Assets at the end of the Financial Year were £397,681 of which £220,042 was invested in an endowment. This endowment 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. In addition, and in line with the Charity Commission guidance CC19 (January 2016) the NPUK Trustees moved that, should the Charity be 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. In addition, £63,783 was held as restricted funds and £113,856 was held in bank accounts to cover reserves (£86,000) and designated funds (£27,000).

Our Objectives: Future Plans!

Each and every year we hope to build on and improve our performance. Our aims and objectives are designed to ensure we push ahead to new heights that will ultimately amount to a better all around care and support service for those affected by Niemann-Pick disease.

This year our objectives are in three categories, and are as follows:

Care and Support

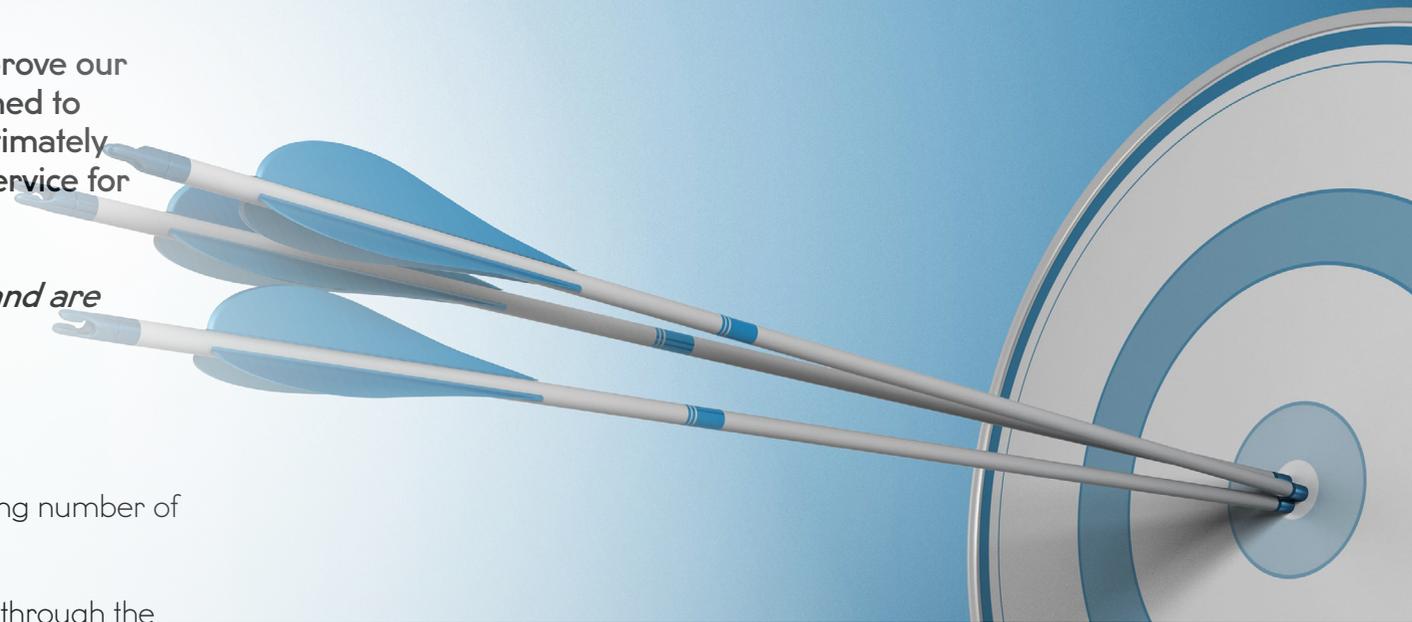
- To further develop our services to support an increasing number of newly diagnosed adults
- To continue to meet the needs of our existing families through the provision of our care and support service
- To further develop our support services for harder to reach groups, such as siblings, and the BME community

Research

- To promote Niemann-Pick disease research through the relationships we foster and the networks we attend
- To fully support the International Niemann-Pick Disease Registry project (INPDR)
- To encourage and facilitate clinical trials as appropriate
- To underpin our support for research into NPD through the provision of grant funding

Providing Information

- To complete the production of a new-look website along with a redefined social media and communication strategy
- To further research and develop information for the BME community, siblings, and those newly diagnosed
- To provide accurate and helpful information about clinical trials enabling families and individuals to make choices
- To continue providing up to date and accurate information on all aspects of Niemann-Pick Disease; by means of the NPUK Central Office and Clinical Nurse Specialist, educational resources, newsletters/e-bulletins, website and telephone help-line





Niemann-Pick UK

Reference & Administration

Registered Charity Number:
England and Wales: 1144406
Scotland: SCO45407

Company Limited by Guarantee:
Registered in England and Wales No: 07775853
Company Secretary: Mrs. Toni Mathieson

NPUK Registered Address:
Suite 2, Vermont House, Concord,
Washington, Tyne and Wear, NE37 2SQ

Auditors:

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

Bankers:

HSBC, Grays Branch
53 High Street, Grays,
Essex, RM17 6NH

Charity Trustees/Board of Directors

David Roberts (Chair)	William Evans
David Holton (Treasurer)	Prof. Frances Platt (Co-opted)
Bill Owen	Stefanie Rymcza (Co-opted)
Jim Green	Miriam Evans (Co-opted)
Janice Brooks	
Helen Carter	
Richard Rogerson	

Medical Advisor:

Dr. Simon Jones
Consultant in Paediatric Inherited Metabolic Disease,
St. Mary's Hospital, Manchester Academic Health Sciences
Centre, Central Manchester University Hospitals NHS Trust

CONTACT:

info@npuk.org
0191 415 0693

 @NiemannPickUK

 @NiemannPickUK