



## **Niemann-Pick Disease Group (UK)**

*Providing effective support and making a positive difference to families affected by Niemann-Pick diseases through the provision of*

***Care and Support - Information - Research***



**Annual Report  
2011-2012**

## ***A Year of Change and Challenge***



This is my first Annual Report as Chairman of the Niemann- Pick Disease Group (UK). My interest in Niemann-Pick Disease started in 1993 when Caroline, my wife's niece was first diagnosed. Little was known about Niemann-Pick Disease in those days and it took 6 long years from the age of 12 before Caroline was diagnosed with Niemann-Pick Type C.

Sadly, Caroline died in 2005 soon after her 30th Birthday. During her lifetime, I had observed the disease progression and the difficulties of living and coping with a child affected by this disease. It was during 2005 that I attended my first NPDG (UK) Board Meeting and was moved by the tremendous amount of work being done by such a small Group.

On behalf of us all at the NPDG (UK), I would like to acknowledge the tremendous debt of gratitude that we owe to Jim Green, our out-going Chairman, for his guidance and support over the last 20 years. We are delighted that Jim will continue as a Trustee of the NPDG (UK) thus ensuring that we retain his knowledge, experience and world-wide network of contacts. Sharing knowledge and forming alliances is a core strength that we have developed as a Group, over many years, and we are grateful to Jim for his continuing support.

Throughout our Annual Report you will see evidence of the Group's performance against our key objectives. I am glad to report that we continue to make good progress in shaping the Group to meet the needs and expectations of all those we seek to support and that our activities and actions are in line with the outcomes of our 2011 Strategic Review:

- We have relocated our Central Office to rented accommodation in Washington, Tyne and Wear to improve our service to families and to meet the needs of the future. We are indebted to Toni Mathieson, our Executive Director, for the use of her home as a Central Office over the previous seven years.
- Our Clinical Nurse Specialist, Jackie Imrie, retired in December 2011 after 12 years of dedicated service. We have retained Jackie's services on a consultancy basis as we move through the process to recruit her successor.
- Elizabeth Davenport took on the new role of NPDG (UK) Families Officer in early 2011. We are now seeing the positive impact of her work to provide families with non-clinical care and support, information and advice.
- Following a consultation process, the Group underwent the process of Incorporation in late 2011, becoming a private company limited by guarantee and re-registering as a charity, with the same name. This was a milestone in our history, giving the Charity its own legal identity and creating the opportunity to review our policies and procedures.
- In late 2011 we commissioned the development of a new interactive website.

Whilst shaping the Group for the future, we are also focussed on the present day needs of the families and carers affected by Niemann-Pick diseases. Our Central Office team continue to provide up to date advice and information and operate the 24 hour help line to provide immediate support to families in need.

As interest in this group of diseases grows, there is an increasing need for us to facilitate research. We are committed to supporting projects at the University of Oxford and also at Cardiff University, bringing renewed hope of therapies that could change the lives of those affected by Niemann-Pick type C. We are also hopeful that the coming year will bring news of clinical trials for ASMD Niemann-Pick type B.

As we look forward to consolidating the achievements of the last year, we also need to bear in mind the needs of the future. These will be very much influenced by proposals for NHS and Welfare reform and as we move into our 21<sup>st</sup> year as a Charity, much of our work will be directed towards engaging with the appropriate regulatory bodies to ensure, as far as possible, that health and social care services for families affected by Niemann-Pick disease are sustained.



Our work both in the past and in the future could not be accomplished without the support of grant giving organisations and the many families, friends and supporters of NPDG (UK) who make donations and regularly give their time and effort to raise funds and awareness. I would like to acknowledge the huge debt of gratitude to each and every one – your continuing support makes a huge difference.

It is a privilege to be part of the NPDG (UK); I hope you enjoy reading this report and that it provides a sense of hope and encouragement. By working together, we can achieve our objectives and meet the challenges posed by these devastating diseases.

Best Wishes

*D. Roberts*

Dave Roberts  
Chairman, NPDG (UK)

## ***About the NPDG (UK)***

***The main aims and objectives of the NPDG (UK) are; to make a positive difference to the lives of those affected by Niemann-Pick diseases (NPD), relieve sickness and any distress which may arise there from, and to advance the education and awareness of families, professionals and the general public in all matters concerning the disease.***

The NPDG (UK) aims to meet these objectives through:

- 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.

To achieve our objectives, we rely on funding from voluntary donations, corporate donors, charitable trusts and statutory bodies, plus the fundraising support of our members, friends and families. Our goal is to ensure the continuation, and the successful delivery, of the services we provide, whilst seeking sustainable expansion.

### ***Structure of the NPDG (UK)***

The Niemann-Pick Disease Group (UK) is a charitable organisation offering support and information to families affected by Niemann-Pick diseases and to associated professionals.

The organisation was formed by a small group of parents in 1991 and in 1997 was granted charitable status as an independent charity, registered with the Charity Commission for England and Wales.

In January 2012, whilst remaining a registered charity, the NPDG (UK) changed its unincorporated charitable status to a charitable company limited by guarantee registered in England and Wales, company number 07775835.



The NPDG (UK) is managed by a Board of Directors (or Trustees), primarily consisting of people who have family members or friends directly affected by this group of diseases, and governed by its Articles of Association. The NPDG (UK) conforms to the requirements of both Companies House and the Charity Commission for England and Wales.

The Board of Directors, all of whom are volunteers, meet at least four times a year, with a number of additional teleconferences. The Board is responsible for all of the activities the Group undertakes, with operational assistance from a number of Sub-Committees. The 2011/12 Board of Directors and professional advisors are listed at the end of this report.

Our staff team are central to achieving our objectives; providing support, information and advocacy services and raising much needed awareness of Niemann-Pick diseases plus the social and economic challenges faced by those affected.



- Since 1999, the Group has funded the salary of a full time Clinical Nurse Specialist, Jacqueline Imrie, providing expert care and practical advice, plus home visits whenever necessary. Genetic counselling and advocacy services are also provided. Jacqueline retired in December 2011, after 12 years of dedicated service. The process of recruiting her successor is now underway.

- Elizabeth Davenport took up the post of NPDG (UK) Families Officer in early strengthening our support service and providing non-clinical advice and information as needed.
- The Group's Executive Director, Toni Mathieson, and Information Officer, Sue Lowe, manage the NPDG (UK) Central Office, operating a 24 hour helpline and ensuring the smooth running of the Group's day to day activities.

Most importantly, the Group would not exist without the support of our members, friends and families; their efforts are vital in assisting the achievement of our aims and objectives.



# ***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.

There are two commonly recognised forms of the disease: Acid Sphingomyelinase Deficiency (ASMD) Niemann-Pick Disease Type A and Type B 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) Niemann-Pick Disease***

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 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, though it most usually affects children of school age. Life expectancy varies considerably and there are a variety of symptoms. These may include an enlarged spleen and 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, also 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 the Niemann-Pick diseases, although there is considerable research activity taking place around the world. Currently, those affected may benefit from palliative treatments - individual medication to treat the symptoms of the disease.

### ***Research and Treatment***

Over the years the NPDG(UK) 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. During these years the NPDG (UK) has played a key role in supporting and facilitating many research projects.



Recent advances in characterisation and understanding of the disease have led to a number of possible therapeutic targets being identified.

Our extensive clinical experience, built up through the existence of a clinical centre and a clinical nurse specialist, makes the UK very well placed to be a part of any future trials.

Enzyme replacement therapy is currently being investigated for NP-B and studies are looking at potential therapies that may control the neurological symptoms of NP-C.

## ***Risk Management***

The Board of Directors have considered and reviewed the possible risks to which the Group may be exposed, and have established the following risk management process:

- The development and implementation of a risk register
- Quarterly risk reviews
- Procedures to identify and mitigate risk

The major risks identified this financial year include:

- The retirement of the Clinical Nurse Specialist and the need to plan for succession
- Succession planning for Trustees and the development of skills
- Economic and social pressures which will adversely affect incoming resources and coincide with the ending of grant funded projects in 2012/2013
- NHS and Welfare Reforms which could adversely affect services for families affected by Niemann-Pick Diseases

Actions are being developed to address these risks and will be communicated when formalised through newsletters, bulletins and letters as appropriate.

## **Public Benefit**

The Trustees confirm that they have complied with the duty in section 4 of the Charities Act 2006 and when planning our activities for the year, the NPDG (UK) 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**:

### **Care and Support Services**

The NPDG (UK) 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.
- Continued to fund the post of Clinical Nurse Specialist for Niemann-Pick Diseases, with support from family fund raising, BBC Children in Need and The Big Lottery Fund.
- Offered UK wide networking and mutual support opportunities for families through the organisation of an Annual Family Conference, Clinic Days, a website e-forum, social networking sites and the production of a Family Directory.
- Further developed the role of NPDG (UK) Families Officer, offering an individual advocacy service, including home visits and assistance at clinic appointments and providing non-clinical advice, information and support.

*"The Families Officer handled all the paper work for my appeal. If she hadn't done this for me I would have given up as I didn't have the strength anymore"*

*"We had been trying to get an adapted house for some time. We felt as though all the other agencies involved were not listening or understanding us. Elizabeth then started to work with us. She talked to the team involved and stuck up for us. We now at last have a house!"*

- Involved volunteers in working with NP families through the Children and Young Persons' Activity Programme at our Annual Family Conference; ensuring they receive the training and support they need to fulfil this valuable role.



## **Information Services**

*The NPDG (UK) recognises the need to provide current, relevant, information and news regarding all aspects of the Niemann-Pick diseases in a timely manner. This year we have:*

- Continued to develop and distribute educational information on Niemann-Pick diseases to support families and professionals.
- Encouraged close working relationships with other Niemann-Pick and associated disease groups to share information, stimulate interest in, and further the knowledge and understanding of this group of diseases.



- Launched 'The Together Support Initiative'; a support programme specifically designed to meet the needs and challenges of patients with Niemann-Pick type C disease (NP-C) and their carers. This unique project was completed with the support of Actelion Pharmaceuticals.
- Organised an Annual Family Conference to encourage the sharing of knowledge and experience between families and professionals.
- Organised a Workshop specifically for health professionals providing the opportunity to share the latest information regarding clinical care and scientific advances.

## **Supporting and Influencing Research**

*Facilitating progress towards therapeutic interventions for all of these diseases is central to everything we do in the NPDG (UK) and has been for the past 20 years. This year we have:*

- Maintained links with clinicians, scientists and pharmaceutical companies in the development of possible therapies for Niemann-Pick diseases, through support for clinical trials for patients with ASMD Niemann-Pick Type B and Niemann-Pick Type C, both in the UK and in the USA.
- Actively supported and, where appropriate, funded scientific research into the Niemann-Pick diseases.
- Nurtured the interest of young NP researchers through 'The Peter Carlton Jones Memorial Award', granted in response to the submission of an original contribution to the scientific or public understanding of the Niemann-Pick diseases and/or their treatment or cure.
- Monitored scientific advances and effectively communicated news in a number of different formats – such as through our website, newsletter, social media and bulletins.

# ***Making a Difference: Highlights of 2011/2012***

## ***Annual Family Conference 2011***

*In September 2011* we held our 18<sup>th</sup> NPDG (UK) Annual Family Conference, over the course of three days, welcoming professional and family delegates from not just the UK but around the World. The theme, 'Keeping Pace with Progress' highlighted the increasing activity in the field of Niemann-Pick diseases that has brought renewed hope and encouragement to us all, along with the challenge of keeping up to date with the vast amount of information now available.

*"This was my first NPDG Meeting – amazing to see so many enthusiastic and engaged people committed to improving the lives of people affected by this disease."*

## ***2<sup>nd</sup> Interactive Workshop on Niemann-Pick Diseases***

Following on from last year's successful 'Interactive Workshop on Niemann-Pick Diseases' we hosted our second event exclusively for healthcare professionals prior to the start of the Annual Family Conference.

*"The Conference provides a vibrant venue for dialogue between patients, clinicians and scientists. Thank you – it is a privilege to attend."*

This event brought together scientists, researchers, clinicians and other health and social care professionals with an interest in this group of diseases. Delegates from the UK, Europe and the USA were given the opportunity to share current information regarding all aspects of Niemann-Pick diseases, including clinical management, research and potential therapies.

## ***Children and Young Person's Activity Programme***



The Children and Young Person's Activity Programme took a trip to Celtic Harmony and also had the opportunity to meet the Medikidz Cartoon Characters from the recently published educational NP-C comic book 'What's up with Lorraine?' The comic book, aimed at the teenage audience, uses the cartoon characters to explain NP-C in an exciting and non-threatening way.

*"It was fantastic for Medikidz to be able to attend the Conference and carry out acting activities and give a presentation with the children who thoroughly enjoyed it!"*

The Characters also led a theatrical, and very humorous, workshop before assisting the children in giving a short dramatisation of the comic book, which was very much enjoyed by all. Many thanks go to Alison Hitchins and her team of dedicated volunteers, without whom this programme would not be possible.

***With grateful thanks to our Conference sponsors, Actelion Pharmaceuticals UK, Actelion Global, Genzyme Corporation and Dr Hetti Davies.***



## ***The Big Lottery ‘Family Care and Interactive Support Service’***

The overall aim of the project, now in its second year, is to overcome isolation and improve the quality of life of families coping with Niemann-Pick diseases, through the use of interactive technology such as video cameras and media links, to improve access to advice and information from the NPDG (UK) Clinical Nurse Specialist (CNS), and their own health care team. In particular, the project helps those families who may feel isolated, or who are struggling with the challenges associated with Niemann-Pick disease.

***“Just tonight our daughter had one of her seizures, I was able to quickly grab the iPad and video her. The seizures have been getting worse despite increase in meds. I can now send this video to her consultant instead of trying to describe the seizures and we can talk about what might be happening, and what meds might be best. Also if school have any concerns about her condition they can video them and send this home.”***



The project enables families who would benefit from additional contact with the NPDG (UK) Clinical Nurse Specialist, or their own Healthcare team, to access advice and support in a timely and effective way – via secure video and media links.

This service does not take away the need for regular appointments or face to face meetings; it simply provides supplementary help and has increased the capacity of our own organisation to support more families.

The provision of interactive technology has enhanced the services we provide by assisting families facing disease progression and changing symptoms, enabling them to access immediate advice or to provide evidence of symptoms or behaviour that occur outside of clinic appointments.

***“Using Skype when I need advice from the NPDG (UK) Families Officer and Clinical Nurse Specialist is so useful and quick – especially as we live so far from them and home visits are not easily arranged”.***

The interactive media technologies have also been used to strengthen links between families affected by the disease, who are often geographically isolated and for whom travel can be extremely difficult.

### ***Information and Awareness***

The NPDG (UK) produces a wide range of informative materials and educational resources to assist families and professionals in dealing with all aspects of the Niemann-Pick diseases. These are regularly updated to reflect the changing needs of those we support.

During the year we have also distributed our regular Newsletter, “Niemann-Pick News” plus e-bulletins and a fundraising bulletin to our members and supporters free of charge.

Our Clinical Nurse Specialist, Executive Director and members of the Board of Directors have given talks and presentations at conferences and fundraising events across the UK, including educational talks to schools, hospitals and hospices.

## ***National and International Collaboration***

### ***International Niemann-Pick Disease Alliance***



The International Niemann-Pick Disease Alliance (INPDA) was formed in 2009 with a view to providing a forum where, through the exchange of information, experience and knowledge, progress could be accelerated.

The INPDA brings together non-profit NPD support organisations from around the world. There are organisations now in Argentina, Australia, Brazil, Canada, France, Germany, Italy, Netherlands, Poland, Spain, USA and the United Kingdom.

The purpose of establishing the Alliance was to provide a network that would enhance the transfer of information, maximise the use of resources and demonstrate that collectively, NP diseases are not as rare as was once thought. It is hoped that by sharing with each other, progress will be enhanced and duplication of effort will be minimised.

In 2011, the INPDA held their second face to face meeting, which was hosted by the Fundación Niemann Pick de España in Madrid. The meeting entitled “Progress Together” provided the opportunity to enhance networks, raise issues and most importantly discuss ways in which progress towards therapies can be enhanced.

Further information can be found at [www.inpda.org](http://www.inpda.org)

### ***LSD Patient Organisation Collaboration***



Lysosomal storage disorders (LSDs) are a group of approximately 40 rare inherited metabolic disorders that result from defects in lysosomal function.

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All lysosomal storage disorders share a common pathogenesis: a genetic defect in a specific lysosomal enzyme, receptor target, activator protein, membrane protein, or transporter, leading to accumulation of substrates in cell lysosomes.

As a member of the LSD Patient Organisation Collaborative, we have created a strong lobbying and action group for LSD patients and their families in the UK. The Group is made up 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, and the Niemann-Pick Disease Group (UK).

## ***Influencing Research***

The NPDG (UK) Board of Trustees is made up of people who have family members or friends directly affected by this group of diseases. We understand the urgency to find effective therapies and the fact that progress never seems to be fast enough.

We actively encourage Niemann-Pick disease research through the relationships we foster and the networks we contribute to. By sharing knowledge and expertise and by working together, we believe progress will be even faster.

The NPDG (UK) aims to maximise the benefits of funding, by working in partnership with like-minded organisations in pursuit of a better understanding and a cure for Niemann-Pick disease.

### ***Grants to the University of Oxford***

#### **Effects of cyclodextrin on a morphometric biomarker in the NPC1 mouse**

This six month pilot study aimed to see if it was possible to replicate the observed changes in NPC patients in the NPC1 mouse model and then to use this model to study the mechanisms underlying potentially adverse events in CD treated patient B-cells.



### ***Grants to Cardiff University***

#### **Uncovering the mechanisms by which miglustat mediates benefit in NPC patients; discovery of novel therapeutic targets.**

Funding granted to enable the completion of research work into the belief that stimulation of acid sphingomyelinase activity could clear the remaining sphingomyelin and cholesterol storage in NP-C cells.

**The Peter Carlton Jones Memorial Award** is granted annually 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. In Autumn 2011, the Award was presented to Benjamin Stewart, a medical student at the University of Oxford. Working in Professor Fran Platt's lab in the Department of Pharmacology, University of Oxford, under the supervision of Dr. Emyr Lloyd-Evans, Benjamin investigated the cellular pathogenesis of Niemann-Pick disease type C2 and has recently embarked on a clinical study at the John Radcliffe Hospital in Oxford.

***With potential therapies*** being trialled and research projects actively pursued within the field of Niemann-Pick diseases, there is a need for the NPDG (UK) to facilitate and support clinical and scientific research as much as we can, and to report the findings to our members. Through our activities this year, and our support for clinical centres of excellence here in the UK, we are encouraging the development and progress of such clinical trials and working to implement effective support systems for families wishing to participate.

# ***Our Objectives for the Coming Year***

## ***Care and Support***

- To continue the process of monitoring and evaluating our Care and Support Service, involving families in this process to ensure their needs are recognized.
- To provide support for families affected by welfare reform.
- To lobby appropriately to highlight the need for the continuation of effective, expert services for NP families within the NHS.
- To recruit a replacement Clinical Nurse Specialist, working in conjunction with the NHS.



## ***Providing Information***

- To continue providing up to date and accurate information on all aspects of Niemann-Pick Disease; by means of the NPDG (UK) Central Office and Clinical Nurse Specialist, educational resources, newsletters/e-bulletins, the website and telephone help line.
- To develop and improve our website, provide current content, direct news feeds and a discussion forum.
- To involve families in the production and of the 'Practical Guide to ASMD N-P Type B'

## ***Research***

- To promote Niemann-Pick disease research through the relationships we foster and the networks we attend.
- To support and encourage Niemann-Pick research by making grants available to laboratories working on this group of diseases.
- To position ourselves in support of clinical trials and to help families prepare for trials when the clinical conditions and funding becomes available.



## Enabling the Work of the Group

We continue to seek ways to sustain and improve our current services and allow for future growth. In 2011/2012 we have been thankful for the support of the following grant giving organisations:



The **Big Lottery Reaching Communities Programme** continues to support our 'Family Care and Interactive Support Service.' The Service is capable of adapting to family needs thanks to the Big Lottery Fund, who have supported the Group in making use of changing technology and therefore enabled a wider range of families to feel supported.

The Group is once again grateful for the continuing support of **Children in Need** who provide a grant to meet one third of the cost of salary for our Clinical Nurse Specialist.

We would also like to acknowledge the support of **Mazars**, one of Europe's largest accounting firms, for their generous donation of £8,200 for the publishing and printing of newsletters and strategic review documents.



## Financial Review

The net assets at the end of the financial year April 2012 were £320,954 – An increase of £2,087 over the previous year.

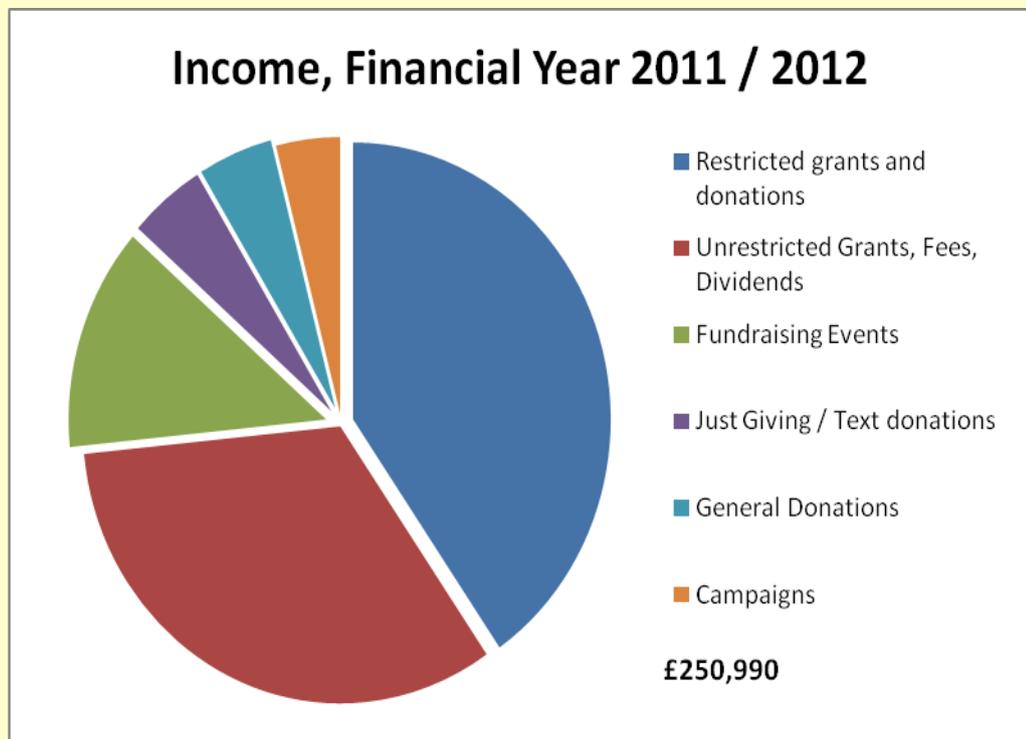
Of this amount, £210,435 is held as an endowment for the purpose of protecting the family care and support service, £9,238 is held as restricted funds to be spent over the year 2012/2013 and £101,281 is held as unrestricted funds.

Because the Charity became a private company limited by guarantee in September 2012, the Financial Statement for 2011/2012 is split between the periods May 2011 to December 2011 for the original charity and September 2011 to April 2012 for the new limited company. Both sets of accounts are available to view on line at [www.niemann-pick.org.uk](http://www.niemann-pick.org.uk) or by post from the NPDG (UK) Central Office.

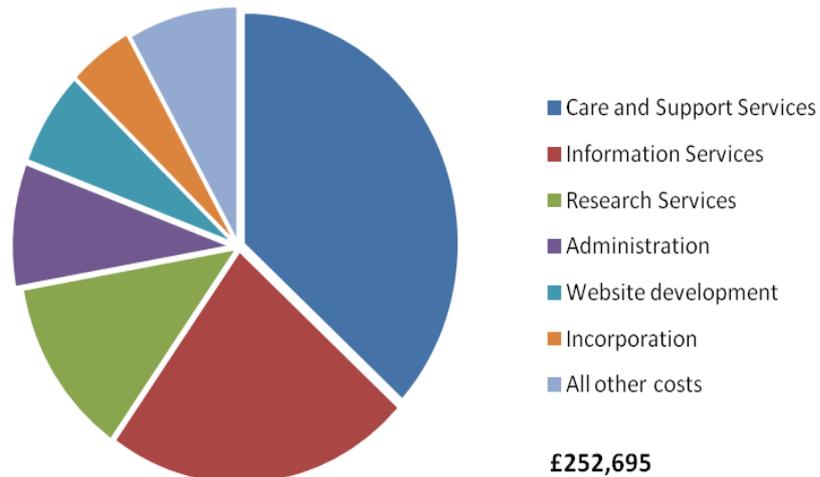
Over the total year 2011/2012, the operating income was £236,734 and the operating expense was slightly higher at £252,695. Bank interest, dividends and an increase in the value of the endowment fund made up this deficit to give a broad break even performance for the year.

The following charts indicate the year's income and expenditure:

**Please note:** *the figures shown are for the full year and have been amalgamated from the Financial Statements for the old charity and the new company and are subject to audit. This report should always be read in conjunction with the Independently Examined accounts*



## Expenditure, Financial Year 2011 / 2012



*Administration, Management and Governance costs for the year 2011/2012 amounted to 10.49% of total income. This excludes Incorporation which was a one-off cost funded by a grant.*

The level of funding and reserves is reviewed at each Board Meeting and decisions about future commitments are made against the need to fulfill our aims and objectives, our understanding of the economic climate and the prevailing risks to income.

Our Care and Support Service is protected by the Endowment Fund.

Of the unrestricted funds, £50,000 has been designated for research and £27,000 has been for designated for other commitments and to cover an estimated shortfall in funding. This leaves reserves of £24,281 that are freely available to spend on the charity's purposes.

### Restricted Funds

The NPDG (UK) is in receipt of grants and donations that are “restricted” and can only be used in a specific way or for a specific purpose.

The total amount received during the financial year 2011 / 2012 was £85,259.25 and the NPDG (UK) confirms that these funds are used only for the purpose for which the funds were given.

Further details are contained in the Financial Statement for the year ending 2011 / 2012 and any under spend is carried forward as restricted funds into the following year.



# Fundraising and the NPDG (UK)

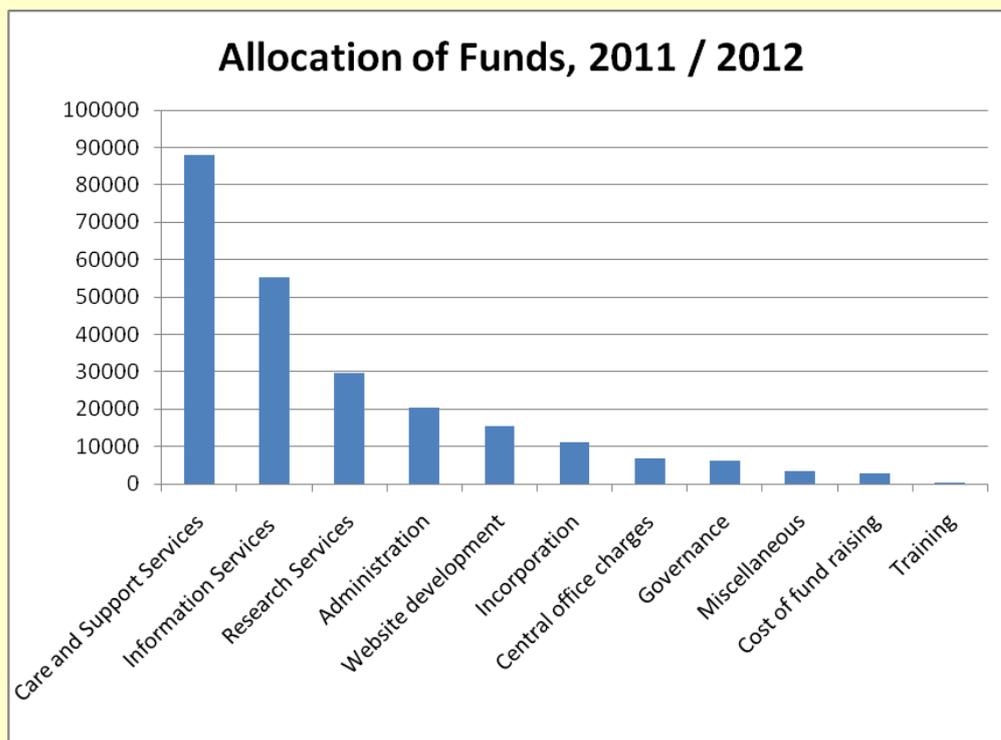
## The Efforts of our Families, Friends and Supporters

We are extremely grateful to all those who support of our work. The Group would not be able to meet the needs and expectations of families affected by this group of diseases without donations and funds raised by the activities of our members, families and friends.

Except for those donations that were restricted for use, all general donations and money raised by families during the financial year, 2011/2012, was allocated to our Care and Support Services and Research Services.

We recognize the tremendous effort that goes into organising fund raising events and, at a time of increasing economic uncertainty, it is important we develop funding streams that are capable of sustaining the future of the Group.

The following chart shows how the Group uses funds raised to meet its aims and objectives and demonstrates the need, and the importance, of your support and that of our grant funders, as being vital to the continued funding of our support services



As a Charity, we are reliant on fundraising and grants to provide the support and services we offer to families and professionals. Whilst we will strive to reduce our dependence on fundraising, it is vital that we maintain the existing level of income for the benefit of our families.

The NPDG (UK) is a company limited by guarantee and a registered charity governed by the Charity Commission for England and Wales. As such, it is required to meet specific criteria in regard to the management of all funds.

**Charity Trustees / Board of Directors**

David Roberts (**Chairman**)

Richard Brooks (**Treasurer**)

Bill Owen

Helen Carter

Prof. Frances Platt (Co-opted)

Jim Green

Janice Brooks

Richard Rogerson

William Evans

**Registered Charity Number:**

Prior to September 2011 – 1061881

After September 2011 – 1144406

**Secretary:**

Toni Mathieson

**Company Limited by Guarantee Registered in England and Wales No: 07775835**

**Medical Advisor**

Prof J.E.Wraith MB, ChB, FCRP

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Website: [www.niemannpick.org.uk](http://www.niemannpick.org.uk)

**Bankers**

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**Auditors**

James Anderson & Co,

Chartered Accountants,

Pentland Estate, Straiton,

Edinburgh, EH20 9QH

**Patrons**

The Rt. Hon. The Earl Cairns, CVO, CBE; The Rt. Reverend Dominic Walker OGS,  
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