

Niemann-Pick Disease Group (UK)

Registered Charity No. 1061881

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

Care : Information : Research



Annual Report

2009-2010

A Year of Landmarks and Major Developments

Within the pages of this Annual Report you will find an overview of our activities, plans and challenges. What a busy year it has been! It has been a year of major developments in a changing Niemann-Pick Disease world and therefore for the Group as well. Major developments, like the first ever conference held at the National Institutes of Health in Washington DC to discuss a range of potential therapies for NPC; like the formation of an International Niemann Pick Disease Alliance to assist in the transfer of information and support; like the award of \$2.5 million government grant in the USA to speed up the development of therapeutic interventions in NPD. Developments for the Group too: – like the award of a Big Lottery Fund grant of over £172,000 over three years to support the development of our support service; like the decision to appoint a new “Families Officer”; like the inclusion of a professionals day at our Family Conference and the development of a range of new materials to support more effective diagnosis and understanding of NPD. I could go on, but won't – you'll find the rest in the report.

This change in environment and level of activity means that the Board is aware of the need to review and modify our plans to achieve maximum progress. I hope you will feel the sense of a changing world and the continual need the Group has to respond to that changing world.

This increase in activity has extended to our families and supporters as well. In spite of the country's economic situation the funds have continued to come in. Some amazing fund raising activities have taken place with spectacular events across the country – from Sky Dives to Glamorous Balls from coffee mornings to school cake sales – this is providing the life blood of our organisation. People are amazing!

The challenges lie ahead of us and the achievements lie behind us – many people deserve to share in these and the more that do, the quicker we will progress.

I hope you enjoy the report and I look forward to seeing or hearing from you throughout the year ahead.

Best wishes

Jim Green

Chairman NPDG (UK)

Can you help in any way?

If you can, please contact us by calling the helpline or writing to us.

The Board meets four times a year with at least four additional teleconferences. It is responsible for all the activities the Group undertakes. All the Trustees are volunteers and each takes on responsibilities within the Group to co-ordinate/support an aspect of our functions.

New trustees are continually being sought, if you are interested please contact the Central Office on 0191 415 0693, or email niemann-pick@zetnet.co.uk

“We can make a difference”

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 by:

- The provision of a support and advice service
- The collection, collation and dissemination of relevant information
- The provision of support for national clinical centres for NPD
- The promotion of relevant research
- The promotion of national and international collaboration

To achieve our objectives, the Group undertakes a wide range of activities in the three key areas of **Care and Support, Information and Research**. Our goal is to ensure the continuation, and the successful delivery, of the services we provide, whilst seeking sustainable expansion.

The Group was first formed in 1991 by parents Susan and Jim Green, following the diagnosis of Niemann-Pick Disease Type C in two of their three children, and was awarded charitable status in 1996, registered charity number 1061881

Since 1999, the Group has funded the salary of a full time Clinical Nurse Specialist, providing expert care and practical advice, plus home visits whenever necessary. Genetic counselling and advocacy services are also provided.

In 2006, the Group employed a Clinical Research Nurse to enable the collection of much needed data regarding clinical aspects of this group of diseases. The creation of this post has helped to improve understanding of Niemann-Pick diseases, encourage further research and assist in the development of therapies.

Both of these specialist nurses are based at the Manchester Royal Children's Hospital and work alongside the Medical Adviser to the Group, Consultant Paediatrician Professor Ed Wraith.

Today, the NPDG (UK) support over one hundred families affected by Niemann-Pick diseases, plus their extended families and the health and social care professionals involved in caring for them. In total, we provide support to almost seven hundred individuals. 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.



Support is provided in many different ways, including:

Through care:

- The provision of a Central Support Office, staffed by the Executive Director Toni Mathieson and the Information and Communication Officer, Sue Lowe. Support and information is provided to families and associated professionals in the form of a 24-hour help line, a website, educational literature, regular newsletters and bulletins.
- The Continuation of funding for the post of Clinical Nurse Specialist for Niemann-Pick Disease, Jackie Imrie, through the Central Manchester Foundation Trust, with support from family fund raising, BBC Children in Need, The Roald Dahl Foundation and The Big Lottery Fund.
- The provision and maintenance of a comprehensive website, providing information on all aspects of Niemann-Pick diseases, plus practical advice for daily living.
- The provision of networking opportunities for families who are geographically widely dispersed, through the organisation of an Annual Family Conference, Clinic Days, a website e-forum, social networking sites and a Family Directory, resulting in the development of a strong mutual support system.

Through the provision of information:

- The provision of an up to date information and advice service, as mentioned above.
- The continual development of educational information on Niemann-Pick diseases to support families and professionals.
- The encouragement of 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.
- Maintaining active links with international NPD Groups and supporting families in other countries to develop support groups.
- The provision of an Annual Family Conference to encourage the sharing of knowledge and experience between families and professionals.
- The dissemination of information regarding Niemann-Pick diseases at various national and international meetings.

Through support for research:

- To facilitate, support and where possible, carry out aspects of clinical research that will improve the understanding of Niemann-Pick diseases.
- To encourage links with clinicians, scientists and pharmaceutical companies in the development of possible therapies for Niemann-Pick diseases and to ensure the timely dissemination of information to families.
- Supporting clinical trials into potential therapies for patients with Niemann-Pick Type B and Type C, both in the UK and in the USA.
- To actively promote, support and, where appropriate, fund scientific research into the Niemann-Pick diseases.
- To support the collation of information/data on Niemann-Pick diseases in order to facilitate research into potential treatments and therapies.

Highlights of 2009 / 2010:

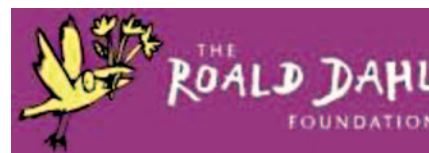
- The 16th NPDG (UK) Annual Family Conference attracted record attendance. Throughout the Conference, presentations by world renowned experts in the field of Niemann-Pick diseases provided an overview of the latest information regarding research, therapies and related care issues. Social time provided a valuable opportunity for families and professionals to meet, network and share their skills and experiences.
- Heightened interest and awareness of Niemann-Pick diseases and an increased workload for the Group has led to the creation of a new post – an ‘NPDG (UK) Information and Communications Officer’.
- In 2010, the Niemann-Pick Disease Group (UK) was delighted to learn our work is to be supported by a grant from the Big Lottery Fund Reaching Communities Programme over the next three years. The grant, for the amount of £172,601, has given us the opportunity to enhance our current support service with a new project, entitled the ‘Family Care and Interactive Support Service’. The overall aim of the project is to improve outcomes for families with Niemann-Pick diseases, through the use of interactive technology that will enhance access to support and information services.
- Our new look website now receives over 50,000 hits per month.
- Our partnership with the Hope for Hollie Campaign continues to provide a popular e-forum, facilitating a strong mutual support network.
- For the first time, the NPDG (UK) hosted an ‘Interactive Workshop on Niemann-Pick Diseases’ exclusively for health care professionals, featuring both clinical and scientific presentations and providing an opportunity for both UK and International professionals to share current information regarding all aspects of Niemann-Pick diseases, including clinical management, research and potential therapies.
- Our work with the LSD Patient Organisation Collaboration has 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 Gaucher’s Association, the Society for Mucopolysaccharide Diseases, Save Babies Though Screening Foundation - representing Krabbe Disease, and the Niemann-Pick Disease Group (UK). As part of this collaboration, the NPDG (UK) has undertaken to jointly promote and share understanding of their diseases to advance standards of care and to enhance the well-being of those affected.
- The first International meeting of Niemann-Pick patient groups, hosted by the NPDG (UK) in collaboration with the National Niemann-Pick Disease Foundation in the USA, took place in October 2009, leading to the creation of the ‘International Niemann-Pick Disease Alliance’. The priorities of the Alliance are to provide mutual support, to develop the availability of information and to stimulate research into this group of diseases.



Financial Information 2009/10

The NPDG (UK) has developed a process to monitor and evaluate our services in an effort to increase our ability to make a difference. In 2009/10 our 'Awareness Indicators' have shown that the uptake of our services continues to rise, and that there has been increased demand for information regarding the Niemann-Pick diseases. Therefore, we continue to seek ways to sustain and improve our current services, and to allow for future growth.

In 2009/10, the Group have been thankful for the support of the following grant giving bodies:



In 2010, the NPDG (UK) successfully applied to the **Big Lottery Fund Reaching Communities Programme**, securing £172,601 over three years, giving us the opportunity to enhance our current support service with a new project, entitled the 'Family Care and Interactive Support Service'. We would like to thank the Big Lottery Fund for choosing to support our organisation over the coming years.

grateful for the continuing support of **Children in Need**, who provide a grant to meet a third of the salary cost of the Clinical Nurse Specialist.

The NPDG (UK) would also like to thank **The Roald Dahl Foundation** for their continued support in 2009. They generously provided the sum of £2500 to specifically support the travel costs of our Clinical Nurse Specialist.

provided a generous grant of £5,000, specifically to support the work of our Clinical Research Nurse. We would like to acknowledge this award and thank The London Law Trust for their support.

The past year has also seen an upward turn in the amount of fundraising activities organised by our members, friends and families. We recognise, and are very grateful to, all those who have given their time and effort in support of our work.

The Group is once again In 2009, The London Law Trust

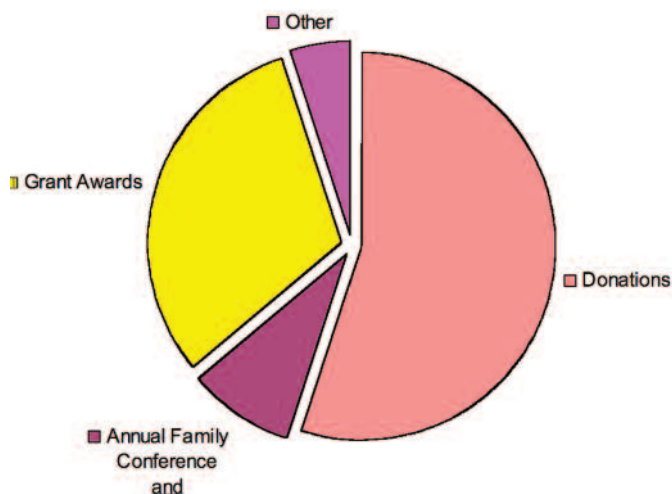
The following charts indicate the year's income and expenditure.

Please note the displayed figures are subject to Audit.

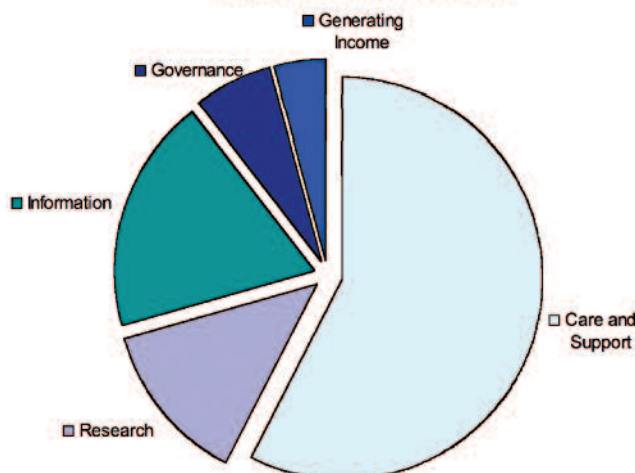
This Report should always be read in conjunction with the Annual Independently Examined accounts for 2009/2010.

In the year 2009/2010 Administration and Management costs amounted to 12.50% of total expenditure.

Income 2009/10 - £202,409



Expenditure 2009/10 - £176,965



Knowledge, understanding and interest in the Niemann-Pick diseases continues to advance; in order for the NPDG (UK) to respond effectively to increasing demands for services and information, our future plans must take this into account.

Most important is the need to ensure continuity of support for affected families and the health care professionals involved in their care. With potential therapies being trialled and research projects actively pursued within the field of Niemann-Pick diseases, there is a need for us to facilitate and support clinical and scientific research as much as we can, and to report findings to our members in a timely manner.

Our Objectives for the Coming Year

In order to respond to the ever changing environment and to represent the needs of our families and all others associated with this group of diseases, the NPDG (UK) will need to be flexible in its approach and continue to provide a strong operational base through the work of the Central Office:

Care and Support

We will monitor and evaluate our Family Support Service; seeking to develop new/improved ways of providing this service, and will involve our families/members in this process to ensure their needs are recognised.

Providing Information

We will continue to provide considered and co-ordinated information and advice on all aspects of Niemann-Pick diseases; by means of the NPDG (UK) Central Office and Clinical Nurse Specialist, newsletters, educational resources, information leaflets, telephone help lines, genetic counselling services and a website.

Collaborative Working

We will strengthen and encourage close working relationships with other relevant organisations, in order to facilitate the sharing of information and the effective use of resources. Through collaborative working, we will enable the development of a stronger voice with which to influence national policy, stimulate interest in, and further the knowledge and understanding of, this group of diseases.

Strategic Governance

We will develop succession planning strategies and promote the recruitment of new Trustees to ensure the Board has the necessary human resources to pursue and implement the functions of the Group.

We will further develop our fundraising strategy; looking for ways to inspire new supporters, whilst retaining more of our existing ones. We will identify potential grant funding opportunities and focus our efforts on creating successful applications.

Supporting and Influencing Research

In light of advances in research and understanding of Niemann-Pick diseases, and in response to the rapidly changing situation, we will carry out a full review of our research priorities. These will include

Supporting the collation of information/data to facilitate research into this group of diseases,

Considering ways of encouraging new initiatives; including the commissioning of further research into the causes and possible treatments of Niemann-Pick diseases.

Monitoring the access to, and affordability of, treatments and therapies – advocating where necessary and appropriate on behalf of NPD patients.

Endeavouring to provide timely dissemination of information to our families/members and other interested parties.

Full details of our objectives and how we intend to achieve them can be found in our new Development Plan, which will be available on our website, www.niemannpick.org.uk after updating for the year ahead.



When planning our activities for the year, the NPDG (UK) Trustees have considered the Charity Commission's guidance on public benefit.

Reserves Policy

The primary aim of the NPDG (UK)'s Reserves Policy is that we will hold adequate funds to maintain the long term sustainability of the Group's support and advocacy service, and to manage short-term volatility in income of liquidity.

During the year the Trustees have reviewed the Reserves Policy and have re-examined the Group's requirements for free reserves in the light of the predominant risks to the organisation and its working capital requirements.

The three major risks are:

- the impact of the economic climate
- loss of grant support
- loss of fundraising income due to the severity of Niemann-Pick diseases and the demands on families and carers

Based on the budget for 2009/2010 and having deducted restricted funds received for essential care and support services in this financial year, the reserves required amounts to £250,000.

Bankers

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

Auditors

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

Risk Assessment

Each year, the NPDG (UK) undertakes a risk assessment process within which it identifies the major risks which face the Charity. This keeps us aware of the possible problems which lie ahead. The Board of Trustees have considered and reviewed the risks to which the Group is exposed and have established procedures to manage those risks. Our risk assessment reveals the following main concerns regarding the future of the Group and achievement of its aims and objectives.

A robust succession planning strategy is needed to address the replacement of a number of volunteer Trustees on the Board, to ensure the continuation of the Group's ability to fulfill its aims and objectives, and also to meet our statutory duties.

It will be imperative to implement a strong financial policy to assist the Group in raising funds to support core functions. Efforts will be increased in identifying possible sources of funding and in making successful applications.

The Group will need to remain aware of the increased demand on our resources from an upsurge in use of our services, contact with families and professionals plus the need to fulfill our responsibilities as an employer and requirements of other legislative obligations i.e. Data Protection, Charity Commission.

With possible drug therapies and further clinical trials in the pipeline, the Group will face the challenge of an additional workload through information collation and dissemination. There will also be an increased need for timely liaison with families, professionals and industry.

Board of Trustees

The Group is managed by the Board of Trustees within the context of an approved Constitution. All trustees and office bearers are elected at the AGM.

Chairman: Jim Green (Scotland)

Secretary: Bill Owen (Wiltshire)

Bill is also our Research Co-ordinator

Treasurer: Richard Brooks (Wiltshire)

Vice Chair: Richard Rogerson (West Midlands)

Trustees:

Janice Brooks Tom Brooks
David Roberts Coral Mclean
Andrew Mclean Helen Carter

This year the Board has also been assisted by Co-opted members William Evans (Leeds), Prof. Frances Platt (Oxford), Dennis Evans (London) and Sue and David French (Buckinghamshire)

Patrons

The Rt. Hon. The Earl Cairns, CVO, CBE; The Rt. Reverend Dominic Walker OGS,
Bishop of Monmouth; Dora Bryan OBE, MA;
The Rt. Hon. The Lord Bassam of Brighton;
Professor Martin N Rossor, MA, MD, FRCP; Guy Johnston;
Nicholas Mathias ARAM, Denise Nolan, Marie T. Vanier M.D. PH.D.

Medical Advisor

J.E.Wraith MB, ChB, FCRP, Willink Biochemical Genetics Unit, Royal Manchester Children's Hospital

NPDG (UK)

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