



Niemann-Pick Disease Group (UK)

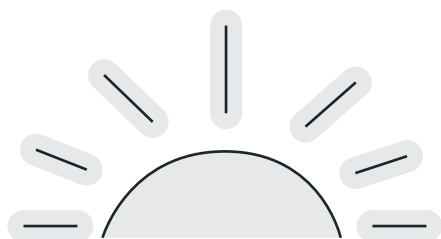
Providing effective support and making a positive difference to families affected by Niemann-Pick diseases through the provision of;

Care and Support - Information - Research



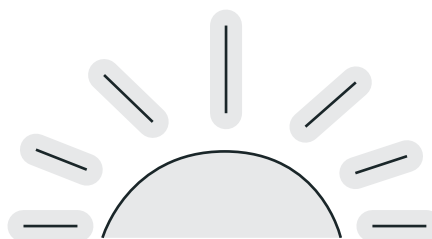
Making friends at the NPDG(UK) Annual Family Conference 2007

**Annual Report
2007-2008**



Niemann-Pick
DISEASE GROUP (UK)

Registered Charity No. 1061881



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Chairman's Foreword

Progress Means "Good" Problems

The introduction to last year's annual report was headed "*No false dawn, just a gradual increase in the Light!*" This year the light burns brighter. Although we do not yet have treatments for this group of diseases there has been a definite move towards transitional research, for instance, talk is more of patients and therapies as well as cells, biochemistry and science. Therapy trials are now underway for type B patients, the trial of a drug to "slow the pace" of progression in type C is now going through evaluation and the torturous route of regulation and approval. Patient information is being collected in a number of natural history studies and a number of new therapies are on the horizon. It's never fast enough, but progress is being made and we, the NPDG (UK), on behalf of the Niemann-Pick families, are doing everything we can to push it along.

As this report indicates it is not just in the fields of research that progress is being made. Because of the fantastic work of fund raisers everywhere we have been able to extend the work of family support, information and awareness raising. This has meant increased demands on our time and resources but this is a "good" problem to have. It arises because there is more information and more support we can provide. In fact our other "good" problem has been trying to keep up with the latest information – in this day of international developments and collaboration making sure we are aware of all that is happening is one of the greatest supports and challenges we can have. A much better problem to have than if there were no information and nothing happening. Good problems come when progress is being made – we are certainly feeling the pressure of good problems but are definitely not complaining.

At this year's Annual Family Conference the theme will be "Care Matters" – of course there is a double meaning to that, but in both meanings, there is relevance to the direction of our charity. Families, of course, deal with many care matters and it is the Charity's job to support them in any way we possibly can and because care matters we treat it very importantly in our priorities.

As many of you know, on the patient support side, Jackie Imrie, our Clinical Nurse Specialist, and Liz Jacklin, our Clinical Research Nurse, have been providing huge support to families. What some don't realise is that both Jackie's and Liz's posts, although operated by the NHS, are funded by the NPDG (UK) through the fund raising support received from families and grant giving bodies. We are continuing to further develop this unique service, thanks to the work of many different people. Toni Mathieson, our National Development Manager, and Sue Lowe, our Administration Officer, have been working really hard to ensure the development plan for the Group is taken forward and that the Group has a point of contact that is caring and effective.

Our volunteer Trustees are all working hard to support the development of the Group and this year have contributed greatly to the progress made. We still have many challenges ahead, as you will see from the report.

Tackling the problems be they bad or "good" ones is always easier if you work together. We hope to have more "good" ones to deal with in the year ahead.

Jim Green
Chairman NPDG (UK)

Achieving Our Objectives

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 therefrom, and to advance the education and awareness of families, professionals and the general public in all matters concerning the disease.

In 2007/8, the NPDG (UK) aimed to meet these objectives by undertaking a wide range of activities in the three key areas of **Care, Information and Research**. Our goal was to ensure the continuation and the successful delivery of the services we provide, whilst seeking sustainable expansion. The last twelve months have been a period of significant but positive change for the Group; following is an overview of our achievements during this time.

Care

- The continuing provision of a Central Support office for the Group with a 24 hour help line number, staffed by the National Development Manager, Toni Mathieson and Administration Officer, Sue Lowe.
- Continuation of funding for the post of Clinical Nurse Specialist for Niemann-Pick Disease, through the Manchester Children's Hospital Trust, with support from family fund raising, BBC Children in Need, The Foyle Foundation and The Roald Dahl Foundation.
- Enabling families to attend Clinic Days for those affected by Niemann-Pick Type B and Type C, held at the Willink Biochemical Genetics Unit in Manchester. Continual development of the Clinic Days to include access to professional support and counselling services.
- The provision of networking opportunities for families who are geographically widely dispersed, through the organisation of an Annual Family Conference, a website e-forum and a Family Directory, resulting in the development of a strong mutual support system.
- The on-going evaluation of the services we provide, through an annual survey of our members and use of an external auditor, to ensure the continual development and improvement of these services.

Information

- The provision of an up to date information and advice service, by means of the National Development Manager, annual newsletters, information leaflets, telephone help line and website.
- 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 development of a new website for the Group, making it more user friendly and accessible.
- The provision of talks and presentations at various national and international meetings.
- Facilitation of an International Liaison Group for NP disease.

Research

- The continuation of funding for the post of a Clinical Research Nurse in collaboration with the Willink Biochemical Unit for a further two years, to carry out aspects of clinical research to improve the understanding of Niemann-Pick diseases, help with patient support and assist in therapy development.
- To encourage close liaison with pharmaceutical companies and clinicians 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 at the Willink Biochemical Genetics unit in Manchester and in the USA.
- To actively promote, support and, where appropriate, fund scientific research into the Niemann-Pick diseases.
- To support and facilitate research through the collation of information/data on Niemann-Pick diseases.



Enabling the Work of the Group

The NPDG (UK) are continually looking for ways to expand and improve the services we provide to ensure that, in the future, we are better able to address the presenting needs of those affected and their families. Over the previous two years, we have developed a process to monitor and evaluate our services in an effort to increase our ability to make a difference. The feedback we have received from those using our services has been invaluable and will assist us in ensuring our organisation meets high standards in the key areas of Care and Support, Information and Research. In 2007/8 we have successfully implemented a Trustee Appraisal process and continue to offer annual employee appraisals, enabling us to ascertain that the Group operates in an effective, responsible and accountable manner.

To ensure the forward movement of the NPDG (UK), the Board of Trustees is continually seeking further funding to sustain and improve current services and to allow the development and implementation of new ones.

The charts opposite indicate the year's income and expenditure. The Group is once again 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. This year, the Group successfully secured grant funding from The Foyle Foundation, specifically to support the posts of Clinical Nurse Specialist, National Development Manager and Administration Assistant. This is an important contribution to the funding of the above posts and the support of The Foyle Foundation is greatly appreciated.

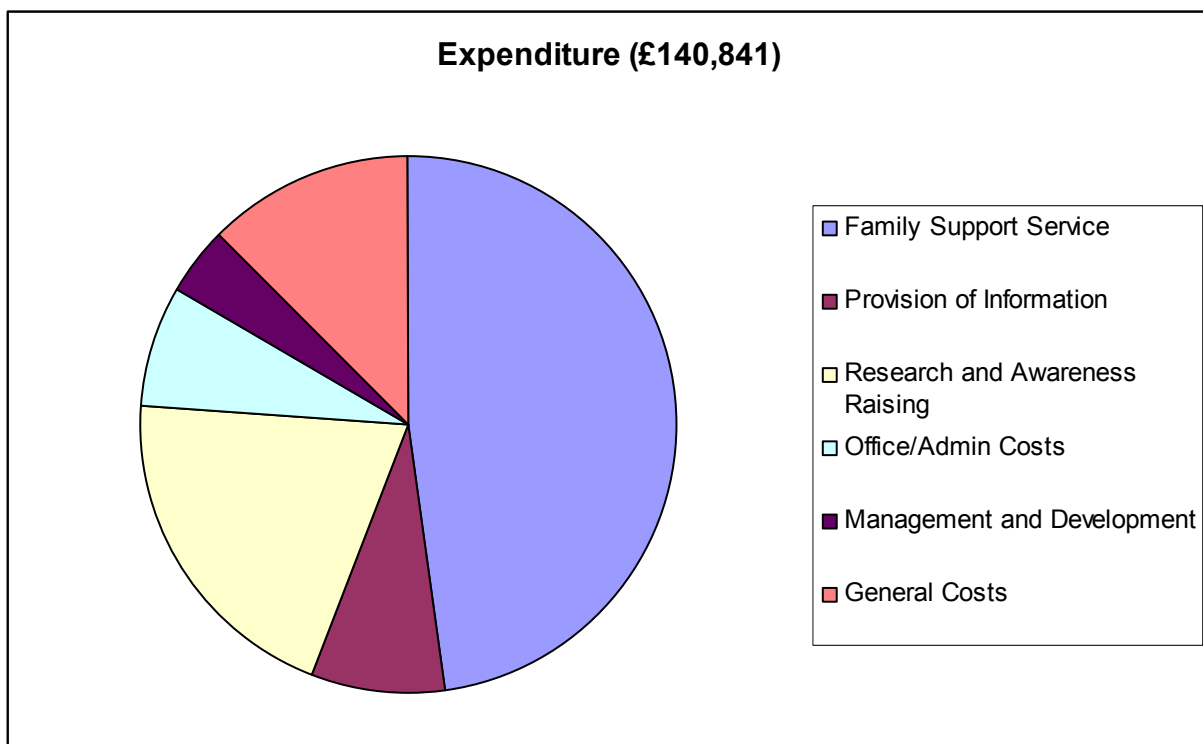
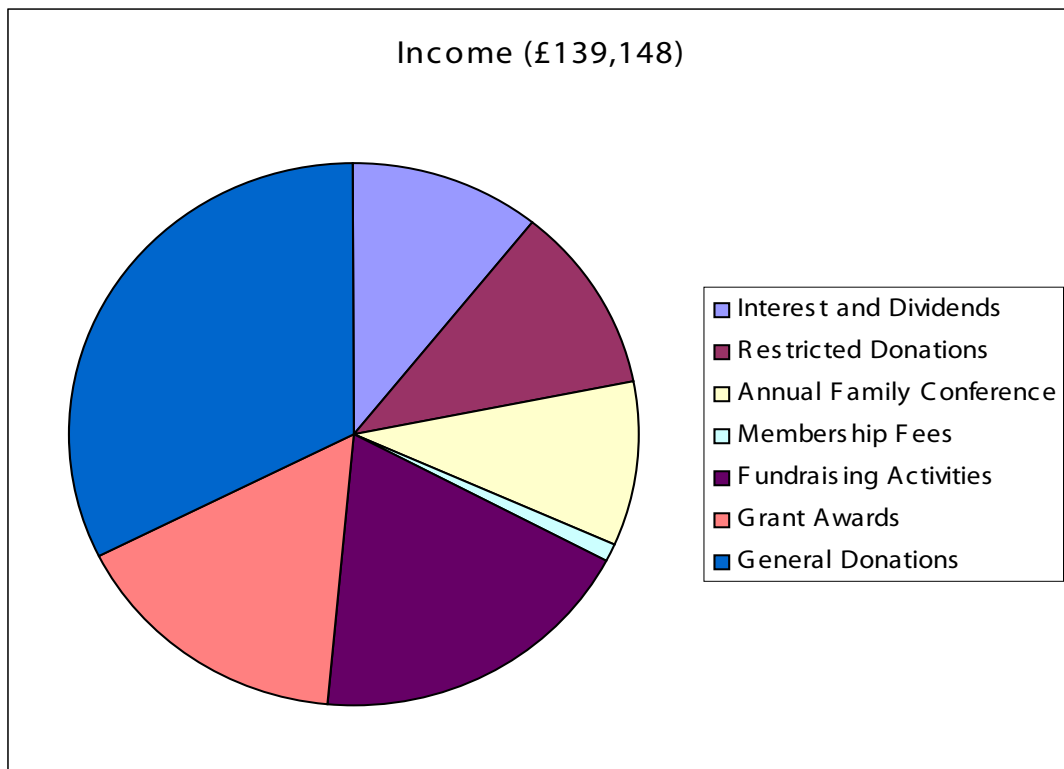
The NPDG (UK) is also grateful to The Roald Dahl Foundation for their generous offer of grant funding support in 2008/9. This will be the second time that the Group has received grant funding from The Roald Dahl Foundation and we are very appreciative of their continued support. The grant is specifically to support the travel costs of our Clinical Nurse Specialist, totalling £5000 over two years.

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

Please note the displayed figures are subject to Audit.

This Report should always be read in conjunction with the Annual Independently Examined accounts for 2007/2008.

In the year 2007/2008 Administration costs amounted to 8.33 % of total expenditure.



Plans for the Future

During the course of this year we have focused on refining our activities and evaluating our work to see if we are “Making a Difference” and to see if we can “Make a Greater Difference”. We have looked at our performance over a range of different aspects and have tried to maximise our use of resources. As always, the views and experiences of those we aim to support form the basis of new approaches to the three key areas of our work – care, information and research. Relatively speaking, there is so much happening now that wasn’t happening before, and this provides us with hope. We are collectively looking to the future – something which, for many of those affected by these diseases, has so far not been possible. As the knowledge grows so too does our job - to share that understanding.

Our objectives for the coming year are:

To ensure the continuation of the work already underway.

The Group will actively seek to raise funds from both formal and informal sources to ensure the continuation of current activities and to enable the furtherance of the Group’s objectives. The Board of Trustees, staff and volunteers will be encouraged to maximize their skills and experience through on-going training and development. New Trustees and volunteers will receive the support required to enable them to fulfill their role within the Group. We will continue to monitor and evaluate our performance to improve the quality of our service delivery, management and support operations.

To ensure the Group is securely positioned to embrace the challenges which lie ahead.

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. Collaboration with other patient organisations, umbrella groups and the Government will be encouraged and nurtured, to ensure that Niemann-Pick diseases are on the agenda. The Group will continue to develop communication channels between pharmaceutical companies and medical professionals to facilitate current and future clinical trials and possible therapies, ensuring they are supported and encouraged and the results disseminated in a timely manner.

Therefore, in the coming year we will need to:

- Raise the profile of The NPDG (UK) through the work of the Central Office.
- Actively seek new trustees and volunteers from our current membership and outside organisations to ensure continuity and the forward movement of the Group.
- Involve our members and those we aim to support in the development of new and innovative services to address their presenting needs.
- Refresh our strategic thinking and future plans, starting with a review meeting to be held in early 2009.
- Continue to work in collaboration with other Patient Organisations and umbrella groups on generic issues and to developing a collective voice with which to influence policy and service provision.
- Actively seek to raise funds to support the work of the Group, through fundraising activities and external funding bodies.
- Further develop our monitoring and evaluation tools to ensure the efficacy and high standard of the services we provide.

Risk Assessment

As part of the Charity Commission's procedures, each charity is required to undergo 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.

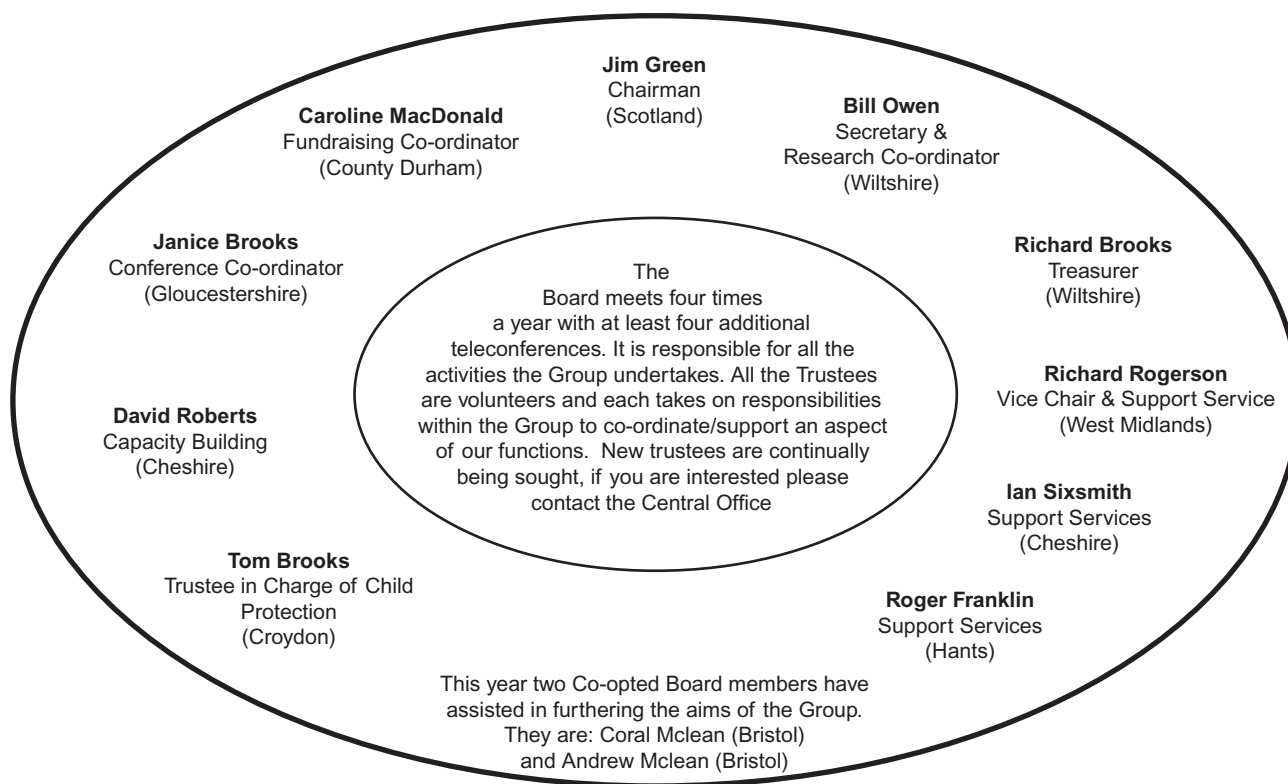
The Board of Trustees have considered and reviewed the risks to which the Group is exposed and have established procedures to manage those risks. Our risk assessment reveals the following main concerns regarding the future of the Group and achievement of its aims and objectives.

- Address the replacement of a number of volunteer Trustees on the Board who are needed to carry out the functions of the Group in the furtherance of its objectives, to ensure the Group have robust support structures and are able to meet the needs of those we support and our statutory duties.
- The threat of not raising enough funds through the Group's general funding activities to support the core functions of the Group, it will therefore be imperative that other funding sources are identified and successfully applied for.
- To meet 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 clinical trials in the pipeline the Group will face the challenge of an additional workload through information collation and dissemination; there will be an increased need for timely liaison with families, professionals and pharmaceutical companies.



The NPDG (UK) Board of Trustees

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



Can you help in any way?

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

“We can make a difference”

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.

Medical Advisor

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

NPDG (UK)

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