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

*Care : Information : Research*

**Annual Report**

2008-2009
Within the pages of this annual report you will find a summary of our activities, plans and challenges. I hope you will feel the sense of a changing world and the continual need for the Group to respond to the challenges that this brings.

Many of these changes represent progress, and as such are both exciting and challenging; some reflect the passage of time and require new inputs, others the need to respond to the current situation for families within the economy and the NHS.

I hope through all that is happening you are able to identify the encouraging nature of the progress and the fact that because of the energy, resources and expertise that the Group is given, a difference is being made. Is it fast enough? Can it be sustained? Where will we be next year?

The questions remain.

Progress never seems fast enough, perhaps the more useful question is “Can we move it any faster?” We will certainly try. Many of you reading this report will help speed the move down the path. Please don’t under-estimate what a difference you can make and if you can help, please let us know. The door is open.

Best wishes

Jim Green

Chairman 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 by undertaking 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.

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, Dr Ed Wraith, Consultant Paediatrician and Director of the Willink Biochemical Genetics Unit.

At present, 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 level of care and support appropriate to their needs.

Support is provided in many different ways, including:

**Care:**
- The provision of a Central Support Office, staffed by the Executive Director Toni Mathieson and the Administration Officer, Sue Lowe. Support and information is provided to families and associated professionals in the form of a 24-hour help line, 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 Manchester Children’s Hospital Trust, with support from family fund raising, BBC Children in Need, The Foyle Foundation and The Roald Dahl Foundation.
- 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 and a Family Directory, resulting in the development of a strong mutual support system.

**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.
Support for Research

- The continuation of funding for the post of a Clinical Research Nurse, Elizabeth Jacklin, in collaboration with the Willink Biochemical Unit until 2010, to carry out aspects of clinical research that will improve the understanding of Niemann-Pick diseases.
- To encourage strong links 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 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 to facilitate research.

Highlights of 2008 / 2009:

- Successful re-design and launch of the NPDG (UK) Website
- Heightened interest and awareness of Niemann-Pick diseases following an increase in media attention.
- Launch of the Family News Bulletin, providing regular updates on practical issues, research topics and fundraising.
- Successful partnership with the Hope for Hollie Campaign to provide an e-forum, enabling the creation of a robust family support network.
- Collaboration with other patient groups representing those affected by Lysosomal Storage Disorders, to create a strong lobbying and action group for LSD patients and their families in the UK.
- Organisation of the first International meeting of Niemann-Pick patient groups, in collaboration with the National Niemann-Pick Disease Foundation in the USA.
- This year has also seen two encouraging developments in the treatment of Niemann-Pick diseases: Actelion Pharmaceuticals received European approval for use of ‘Zavesca’ in patients with Niemann-Pick Type C, and Genzyme announced the completion of the Phase 1 trial of rhASM in Niemann-Pick Type B patients.
Financial Information 2008/9

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 2008/9 our ‘Awareness Indicators’ have shown a steep rise in the uptake of our services, and also in the amount of information being sought regarding the Niemann-Pick diseases. As we are always seeking ways to expand and improve the services we provide, funding opportunities are continually being sought, to sustain current services and to allow for future growth.

The following charts 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. In 2008, the Group was appreciative of the support of The Foyle Foundation, who generously granted funds specifically to support three of the Charity’s core posts.

The NPDG (UK) would also like to thank The Roald Dahl Foundation for their generous offer of grant funding support in 2008/9. This is 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.

The Group would also like to acknowledge the support of The London Law Trust, who has provided a generous grant of £5,000, which has been awarded specifically to support the work of our Clinical Research Nurse in 2009.

In April 2009 the Group was delighted to learn that the Big Lottery Fund Awards for All scheme had agreed to award a grant to assist in the development and dissemination of information packs to families.

The past 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 2008/2009.
In early 2009, the Group held a Strategic review meeting, to determine the priorities and focus of the Charity in the next three to five years. It was clear that we now live in a totally different ‘Niemann-Pick world’; knowledge of this group of diseases is advancing, with potential therapies being trialled and research projects actively pursued. As the knowledge grows so too does our job - to share that understanding. Therefore, our plans for the future need to take these developments into account, to enable us to respond to the increasing amount of information; to provide continuity of support to the increased number of families we are now in contact with and to facilitate and support clinical and scientific research as much as we possibly can.

In June 2009, the Willink Biochemical Unit will move to a new location in Central Manchester, becoming part of the new Manchester Royal Children’s Hospital. Our Clinical Nurse Specialist and Research Nurse will assist families in making the transition to this new site.

In October 2009, we are hosting the first meeting for representatives of existing NP patient support organisations throughout the World. This is being jointly run in conjunction with the NNPDF (USA). For the first time we will have a forum at which the many issues we have in common can be discussed. We will also be investigating any possible opportunities there are to support and collaborate with each other in order to improve care, facilitate exchange of information and speed up research developments.

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:

- 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.
- 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.
- 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.
- 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 actively promote, support and, where appropriate, fund scientific research into the Niemann-Pick diseases, aiming to establish relationships with those who decide health care policy in the UK. Where appropriate, we will lobby for improvement in the standards and provision of clinical care for those affected by Niemann-Pick diseases:

- We will support the collation of information/data to facilitate research into this group of diseases, by means of the Clinical Research Nurse.
- We will consider ways of encouraging new initiatives; including the commissioning of further research into the causes and possible treatments of Niemann-Pick diseases.
- We will monitor the access to, and affordability of, treatments and therapies – advocating where necessary and appropriate on behalf of NPD patients.
- We will ensure the 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 Development Plan, a copy of which can be found on our website www.niemannpick.org.uk*
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.

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<tr>
<th>Chairman: Jim Green</th>
<th>Secretary: Bill Owen</th>
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<tr>
<td>(Scotland)</td>
<td>Bill is also our Research Co-ordinator</td>
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<td>(Wiltshire)</td>
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<td>Treasurer: Richard Brooks</td>
<td>Vice Chair: Richard Rogerson</td>
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<td>(Wiltshire)</td>
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<td>Trustees:</td>
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<td>Janice Brooks</td>
<td>This year the Board has also been</td>
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<td>Caroline MacDonald</td>
<td>assisted by Co-opted member</td>
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<td>Tom Brooks</td>
<td>Helen Carter of Milton Keynes.</td>
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<td>Roger Franklin</td>
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<td>David Roberts</td>
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<td>Coral Mclean</td>
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<td>Andrew Mclean</td>
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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 coordinate/support an aspect of our functions. New trustees are continually being sought, if you are interested please contact the Central Office.

“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

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