



Niemann-Pick Disease Group (UK)

Registered Charity No. 1061881

Providing effective support and making a difference to families through

Care : Information : Research

Annual Report

2006-2007



Rachael, NP-C 7/06/83 - 16/02/06

Chairman's Foreword

"No false dawn, just a gradual increase in the Light!"

Our work this year has focused on refining our activities and evaluating what we are doing to see if we are "Making a Difference" and to see if we can "Make a Greater Difference". We have asked you what you think by way of a questionnaire, we are looking at our performance over a range of different aspects and, above all, we are trying hard to maximise our use of resources. We are collectively looking to the future and hence it seems only natural that our conference this year (June 23rd & 24th) has the theme "Focus on the Future".

Why is it that we can now begin to do something (look to the future) which, for many of those affected by these diseases, has not so far been possible? Because, relatively speaking, there is so much happening now that wasn't happening before that provides us with the light of hope. No false dawns just a gradual increase in the light.

At this year's conference you will hear of the first trial of enzyme replacement therapy for NP type B. This is the first time, for NP type B, that something with the potential to treat the disease has been trialled. News too of the trials and investigations into drugs that will hopefully make a difference to those with NP type C.

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 Group through the fund raising support received from families and grant giving bodies. We are looking to continue 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 Assistant, 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. Hopefully, many who read this report will feel able to support our journey in what ever way they can.

Making a difference has never been easy but it can and does happen when people work together.

Jim Green

Achieving our objectives 2006 – 2007

NPDG (UK) aims to meet its objectives by undertaking a wide range of activities in the three key areas of Care, Information and Research. Following is an overview of our achievements over the last year.

Care

- The provision of a Central Office for the Group with a 24 hour help line number, staffed by the National Development Manager and Administration Assistant.
- Continuation of funding for the post of Clinical Nurse Specialist for Niemann-Pick Disease, with support from BBC Children in Need, through the Manchester Children's Hospital Trust.
- Further development and support for Clinic Days for NP B and C families held at the Willink Biochemical Genetics Unit in Manchester. Enabling families to attend and have access to professional support and counselling services.
- Supporting the clinical trial into a potential therapy for adults and paediatric patients with NP C at the Willink Biochemical Genetics unit in Manchester.
- The creation of opportunities to enable families to develop mutual support networks, through the provision of Niemann-Pick Clinics, the Family Directory and an Annual Family Conference.
- The evaluation of the service we provide, through an annual survey of our members and use of an external auditor, to ensure the continual development and improvement of this service.

Information

- The provision of up to date information and advice by means of the National Development Manager, annual newsletters, information leaflets, telephone help lines and the website.
- The development of educational information on Niemann-Pick Diseases to support families and professionals, such as Care Manuals and a 'Frequently asked Questions' leaflet.
- 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 the disease.
- Maintaining active links with NPD Groups in Germany, France, Spain, and the USA and supporting families in other countries to develop support groups.
- The provision of an Annual Family Conference, to encourage the sharing of information between families, researchers and clinicians.
- The development of a new website for the Group.
- The provision of talks and presentations at various national and international meetings.
- Facilitation of an International Liaison Group for NP disease.

Research

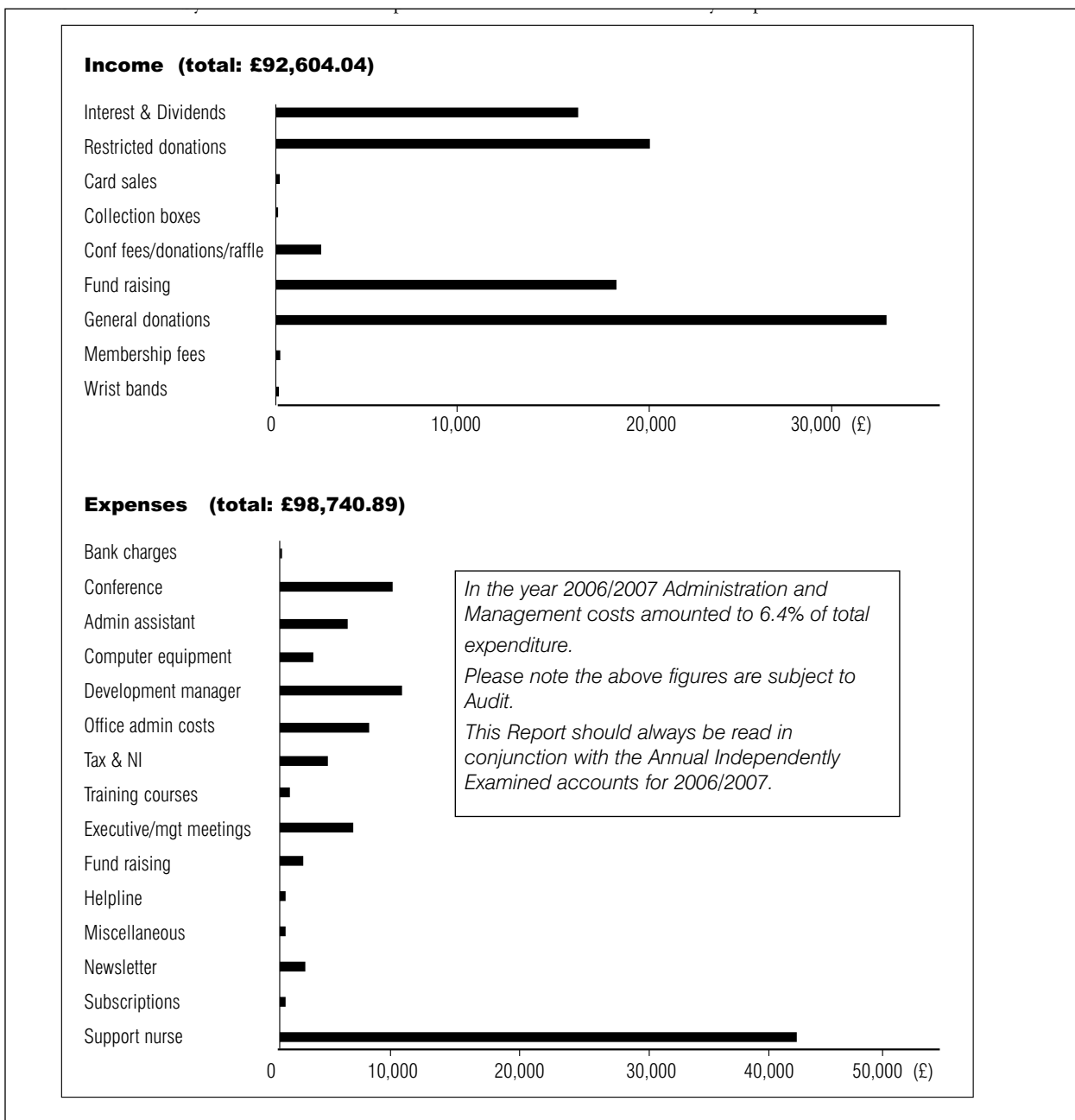
- Close liaison with pharmaceutical companies and clinicians in the development of possible therapies for NP diseases and ensuring the dissemination of information to families.
- The continuation of funding for the post of a Clinical Research Nurse in collaboration with the Willink Biochemical Unit, to carry out aspects of clinical research to improve understanding of this group of diseases, help with patient support and assist in therapy development.
- To actively promote, support and where appropriate, fund scientific research into the Niemann-Pick Diseases.
- To support the collation of information/data on NPD to facilitate research

Enabling the Work of the Group.

NPDG (UK) aims to meet high standards in how we, as an organisation work, both in the development and motivation of our staff and volunteers to the way in which we respond to the diverse needs of those who use the services we provide. During the past year we have taken steps to evaluate those services and to ensure that in the future, we are better able to address the presenting needs of those affected and their families. We have also started developing a process of capacity building to try to increase our ability to make a difference.

The responsibility to ensure the forward movement of the Group rests on the shoulders of everyone - from the Board of Trustees to the general membership of the Group. Alongside, pharmaceutical companies, scientists and clinicians are taking their share of the responsibility in their work with clinical trials.

Financially, the graphs below indicate the year's income and expenditure. Once more the Group is grateful for the support of Children in Need, who provide a grant to meet a third of the salary cost of the Clinical Nurse Specialist. The Group is actively seeking further funding to sustain and improve our current services and to allow the development and implementation of new ones.



2006 – 2007 and Beyond

Where do we go from here?

During the course of this year, with the assistance of an external consultant and the involvement of our families and members, the Group has undertaken an evaluation of the service it provides. The views and experiences of those we aim to support will form the basis of new approaches to the three key areas of our work – care, information and research. We hope this exercise will assist us in achieving our aims and objectives in the future.

What outcomes do we want to achieve in the coming year? How will we achieve them?

To ensure the continuation of the work already underway. To achieve this, the Group will:

- Raise enough funds from both formal and informal sources and effectively distribute those funds to ensure the continuation of our activities and the furtherance of the Group's objectives.
- Actively recruit and support volunteers to work on the Board of Trustees, developing their strengths and skills to carry out specific areas of the Group's work.

To ensure that we are securely positioned to embrace the challenges which lie ahead of the Group by:

- Facilitating and nurturing collaboration with other patient organisations, umbrella groups and the Government to ensure that Niemann-Pick Diseases are on the agenda.
- Encouraging and developing close communication channels with Pharmaceutical Companies and Professionals to ensure that current and future clinical trials and possible therapies are supported and encouraged.
- Developing transparent policies for Fundraising, Employment and Reserves.
- Developing a strong operational base for the Group through the central office.
- Being flexible in order to be able to respond to the ever changing environment in order to represent the needs of our families and all others associated with this group of diseases.

Therefore, in the coming year we will need to:

- Continually raise the profile and develop the Group through the work of the Central Office.
- Actively seek new trustees from the current membership and outside organisations to ensure continuity and development.
- Work in collaboration with other Patient Organisations and umbrella groups on generic issues.
- Encourage and seek to raise as much money as we can to support the work of the Group, through fundraising activities and external funding bodies.
- Develop continuous monitoring of our effectiveness through a system of "awareness indicators"

Risk Assessment

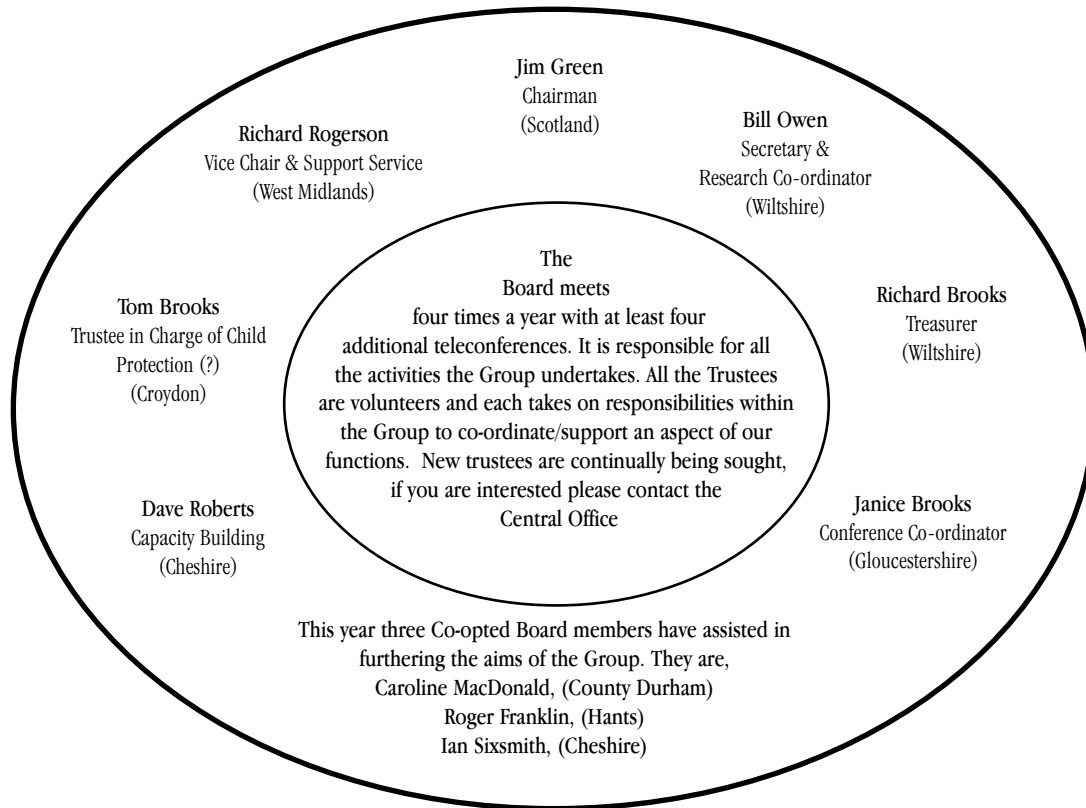
- As part of the Charity Commission's procedures, each charity is required to undergo a risk assessment process within which it identifies the major risks which face the charity. This keeps us aware of the possible problems which lie ahead.

Our risk assessment reveals the following main concerns regarding the future of the Group and achievement of its aims.

- 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, whilst continuing to manage the Group and meet the needs of the families 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.
- A number of Grants that have supported specific areas of work are coming to an end and it will be imperative that other funding sources are identified and successfully applied for.
- The need to fulfill our responsibilities as an employer and requirements of other legislative obligations i.e. Data Protection, Charity Commission put an increased demand on resources.
- With possible drug therapies and clinical trials in the pipeline the Group will face the challenge of an increased workload through information collation and dissemination, the need for liaison with families, professionals and pharmaceutical companies.

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.



You will have seen from this annual report that as a group we have so much to do.

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 Revd Dominic Walker OGS, Bishop of Monmouth; Dora Bryan OBE MA;
Sir Robin Catford KCVO CBE; The Rt Hon The Lord Bassam of Brighton; Professor Martin N Rosser MA MD FRCP;
Guy Johnston; Nicholas Mathias ARAM

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