



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

Registered Charity No. 1061881

## **Annual Report 2004-2005**



*Lucy*

## Chairman's Foreword

This year has seen much happen in the world of Niemann-Pick Disease and the life of the NPDG (UK). The involvement for the first time of Niemann-Pick patients in a drug trial, the growth in knowledge and understanding about these diseases and the need for international collaboration, has meant that the NPDG (UK) has had to grow to match the role it is now required to undertake.

The work of our paid employees in developing the aims of the Group and the work of the trustees, parents and supporters, has meant a record breaking year in terms of activity and fund raising. The money raised has been put to work and has been used to fund a full-time Clinical Nurse Specialist who works throughout the country supporting and informing families and professionals. It has jointly funded research projects. It has been used to provide education and information materials and events. It has generally worked hard and achieved so much. We need to sustain this work now and develop it further.

Our drive next year, in order to take our aims forward, is to look to develop partnerships, here in this country with other support groups for lysosomal disorders, internationally with other Niemann-Pick organisations and, more generally, with scientists and clinicians.

All those who have worked so tirelessly throughout the year should look back on the year with great satisfaction and forward, with a renewed sense of direction and purpose in the knowledge that so much has still to be done.

*Jim Green*

## **Achieving our objectives 2004 – 2005**

The appointment of our National Development Manager, Tanya Collin-Histed, has enabled us to further our objectives, raise our profile in the field of rare diseases and be a support to families and professionals alike. Looking back over the last 12 months, it is clear to see how we have continued to further our objectives. The following is a short summary of the activities that we have undertaken throughout the year.

### **The Provision of a Support and Advice Service**

- The development of a central office for the group with a 24 hour contact line.
- Funding a Clinical Nurse Specialist for Niemann-Pick Disease, with support from BBC Children in Need, through the Manchester Children's Hospital Trust. Making a commitment to increase the hours to 37.5 hours a week in 2005.
- The appointment of an Administration Assistant to support the work in the NPDG (UK) office, with the support of the Alzheimer's Society.
- The production of the annual Family Directory to facilitate interaction between families.

### **The Dissemination and Sharing of Relevant Information**

- The development of the Website for the Group.
- The production of three newsletters annually with information on; Benefits, Research, Family Focus, Conference News, Fundraising and a News Page.
- Running a National Conference for families and professionals.
- The production of a new 'Introduction' leaflet and updating the 'Layman Guides' to NP A, B and C

### **The Provision of Support for a National Clinical Centre for NPD**

- The development of a baby clinic for NP C in Manchester.
- Providing continual support for clinic days for NP B and C families in Manchester.
- Funding a NP Clinical Nurse Specialist based in the Royal Manchester Children's Hospital.
- Supporting the clinical trial for a NPC therapy for adults and children at the Willink Biochemical Genetics unit in Manchester.

### **The Promotion of National and International Collaboration**

- Attending the NNPDF (National Niemann-Pick Disease Foundation) Board meetings and having representation on the NNPDF Board.
- Attending the NNPDF Annual Family Conference in the USA.
- Communication with other UK Patient Organisations on generic issues such as: Funding and Research.
- Maintaining active links with NPD Groups in Germany, Spain, and the USA and supporting families in other countries to develop support groups.
- Working in collaboration with Doctors in the USA on a joint research project for NPC.

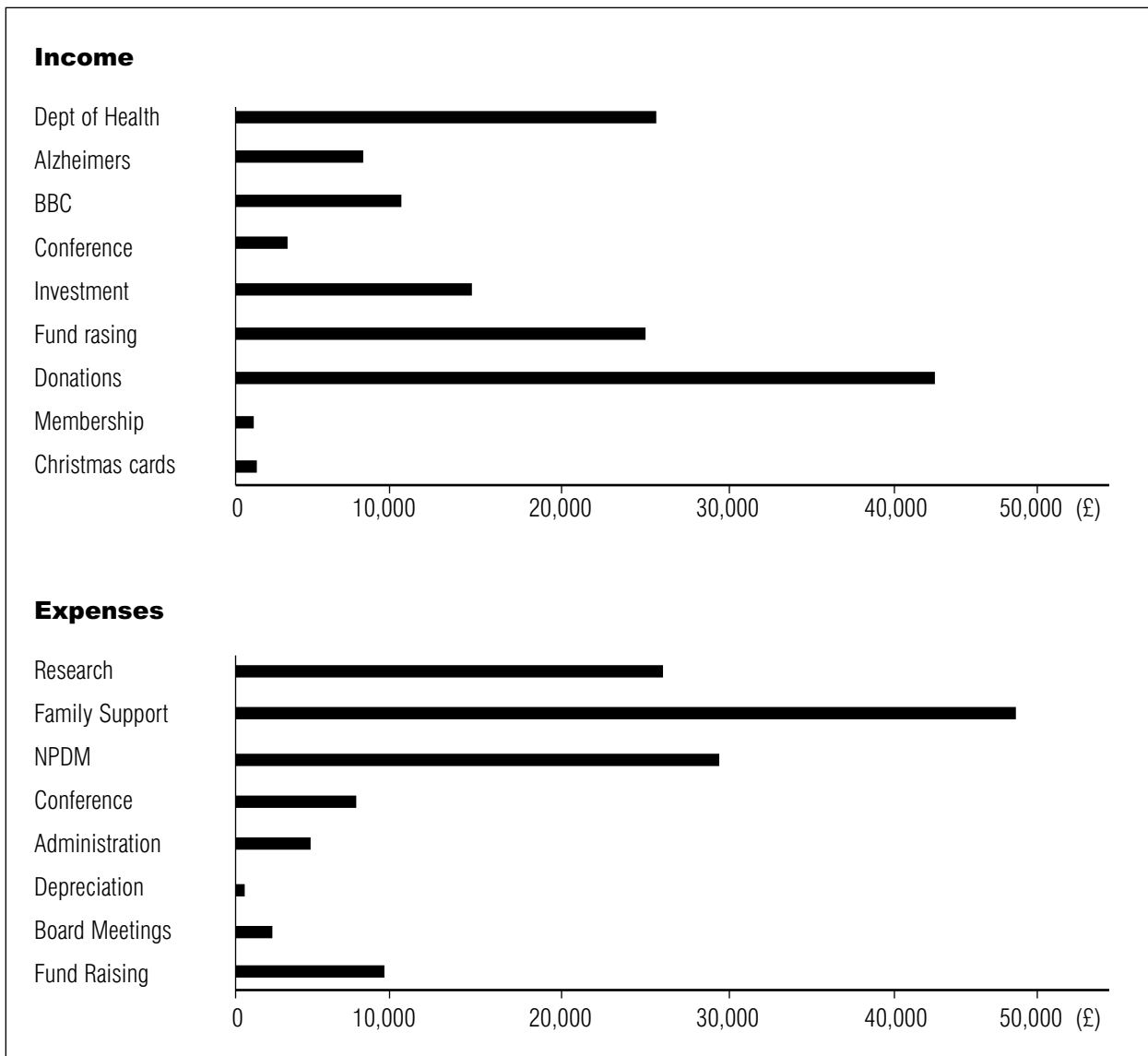
### **The Promotion of Relevant Research**

- Joint working with the USA on a collaborative research project for NPC.
- Close liaison with Pharmaceutical Companies regarding existing and possible future developments in Niemann-Pick Disease therapy.
- The Group's commitment to support the development of a Research post with the Willink Biochemical Genetics Unit, to carry out aspects of clinical research to further understand this Group of Diseases.
- The continued collaborative project with Cambridge University into the possibility of gene replacement therapy for NP-C.

## Enabling the Work of the Group.

The responsibility to ensure the forward movement of the Group rests on the shoulders of everyone – the Board of Trustees, Clinicians, families and their friends and the general membership of the Group. Alongside, the drug companies are taking their share of the responsibility to help in their forward work with clinical trials. Research is on going. Much is being done but there is yet far to go.

Financially the graphs below indicate the year's income and expenditure. A third of the salary cost of the Clinical Support Nurse has been provided by a Children in Need grant and a further third by investment income. From 2003 the newly appointed National Development Manager's post has been funded by finance from the Department of Health within a three year period of commitment. An education grant from the Alzheimer Society continues to support a newly appointed Administrative assistant who works alongside the NPDM.



*Admin and Management costs amounted to 6.58% of total expenditure or 6.50% of total income. The increase over last year is accounted for by the extra cost incurred in paying for an admin assistant to work alongside the NPDM.*

*This Report should always be read in conjunction with the Annual Independently Examined accounts for 2004/2005.*

## 2005 – 2006 and Beyond

### *Where do we go from here?*

We need to ensure that we can continue to sustain the work already underway. In order to be able to achieve this, the group will need to:

- raise enough money to be able to fund our activities and effectively distribute that money, to ensure 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.

We will need to ensure that we are securely positioned to embrace the developments 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 Disease is on the agenda.
- encouraging and developing close communication channels with Pharmaceutical Companies and Professionals and ensuring 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 to represent the needs of the families and all others associated with Niemann-Pick Disease.

Therefore this 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 fundraising and seek to raise as much money as we can to support the work of the group through fundraising activities and external funding bodies.

### *Risk Assessment*

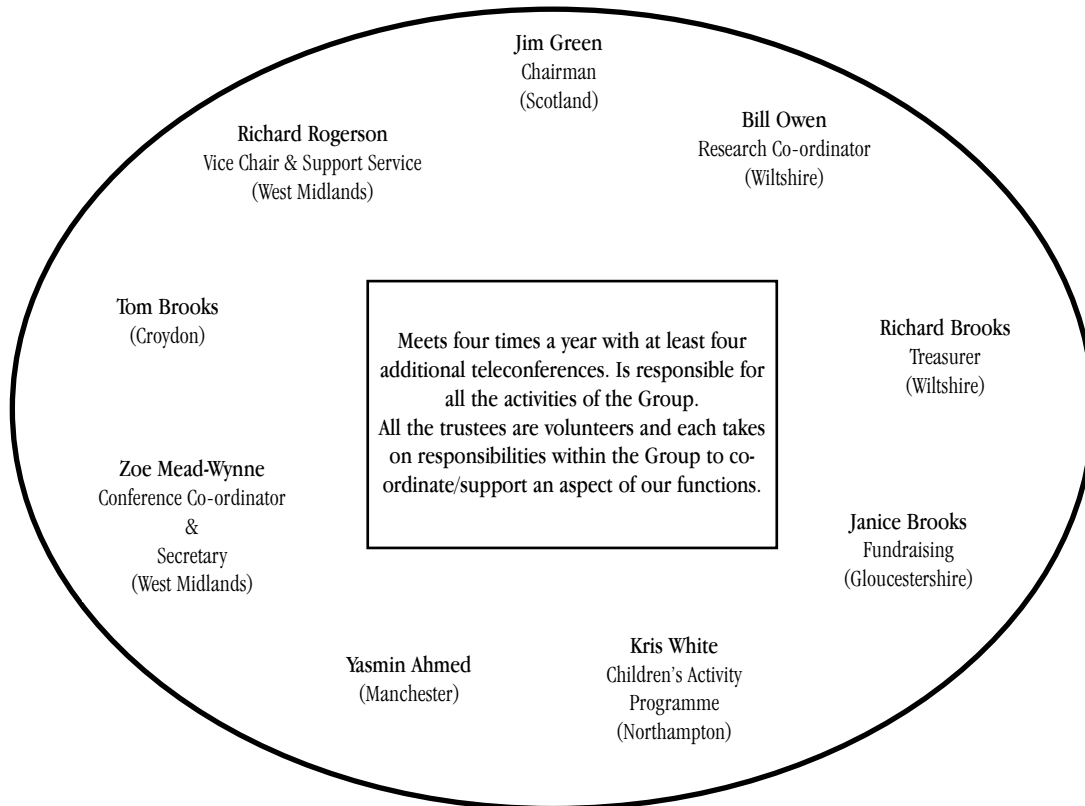
As part of the Charity Commission's procedures, each charity is required to undergo a risk assessment process within which it identifies the major risks which face the charity. This keeps us aware of the possible problems which lie ahead.

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

- To 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 fulfil 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 professionals, drug companies and the families.

## 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; Audley Harrison MBE, BSc

### MEDICAL ADVISOR

J.E.Wraith MB, ChB, FCRP, Willink Biochemical Genetics Unit, Royal Manchester Childrens Hospital

Registered Address:

98 Yarningale Road, Kings Heath, Birmingham, B14 6NB

Telephone: 01453 843665