



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

Registered Charity No. 1061881

Providing effective support and making a difference to families through

*Care : Information : Research*

## **Annual Report** **2005-2006**



Brandon Perrin

## Chairman's Foreword

The year past has seen the reshaping of our Charity. The changes in the understanding of the diseases; the need for more involvement with trials of new drugs; the need for more networking and collaboration with other groups involved with similar diseases has seen a need to rethink and restructure our activities.

Central in our thinking is that we are primarily a support group **for families** – everything we do has to be designed to meet our triple aims of providing support for families through *Care, Information and Research*. Doing this effectively and efficiently remains the priority of the Board of Trustees.

We have seen a change of some staff this year and although we miss both Tanya, our first National Development Manager, and our clerical assistant Michelle, it has provided us with an opportunity to bring new perspectives and skills to the Group in the form of Toni Mathieson (NDM) and Susan Lowe (clerical support).

They have been joined by a Clinical Research Nurse, Liz Jacklyn, who has been employed initially on a two year contract to carry out much needed clinical studies to support the increased knowledge now available from the scientists. Liz works alongside Jackie Imrie, our Clinical Nurse Specialist, who continues to provide much needed support and advice to families and professionals.

Jackie's knowledge and expertise are much in demand and the need for all employees to raise awareness as well as understanding amongst the public and decision makers becomes an increasing priority. We must keep our eye on the approaching new horizons, potential therapeutic developments are coming closer and we must do everything possible to make sure we are well positioned for our arrival at that new land. We also need to do everything we can to make the journey towards these shores as fast as possible. Sadly for a number of children this year, their journey proved too exhausting but they have all given such a great deal to those who travel with them.

Fund raising and donation of time and skills are what helps to keep us moving – the more we raise, the more we can do. Our thanks to all who gave so generously this year of their time, money and resources to help us along the way.

This annual report I hope gives you a feel for what has been achieved and what still has to be done.

*Jim Green*

## **Achieving our objectives 2005 – 2006**

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 and help line number.
- Funding a Clinical Nurse Specialist for Niemann-Pick Disease with support from BBC Children in Need through the Manchester Children's Hospital Trust. We have increased the hours to 37.5 hours a week (full time) in 2005/06.
- The appointment of an administration assistant to support the work of the NPDG (UK) office.
- The production of the annual family directory to facilitate interaction between families affected by this disease.

### **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 provision of talks and presentations at various national and international meetings
- Hosting the first meeting to discuss establishing an international registry for NPD under the auspices of GOLD (Global Organisation for Lysosomal Disorders)

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

- The development of an adult 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 NP C therapy for adults and paediatric patients at the Willink Biochemical Genetics unit in Manchester.

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

- Attending the NNPFD (National Niemann-Pick Disease Foundation) Board meetings and having representation on the NNPFD Board.
- Attending the NNPFD 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, France, Spain, and the USA and supporting families in other countries to develop support groups.
- Funding in collaboration with NNPFD (USA) a research project for NP C.
- Working to establish an International Liaison Group for NP disease

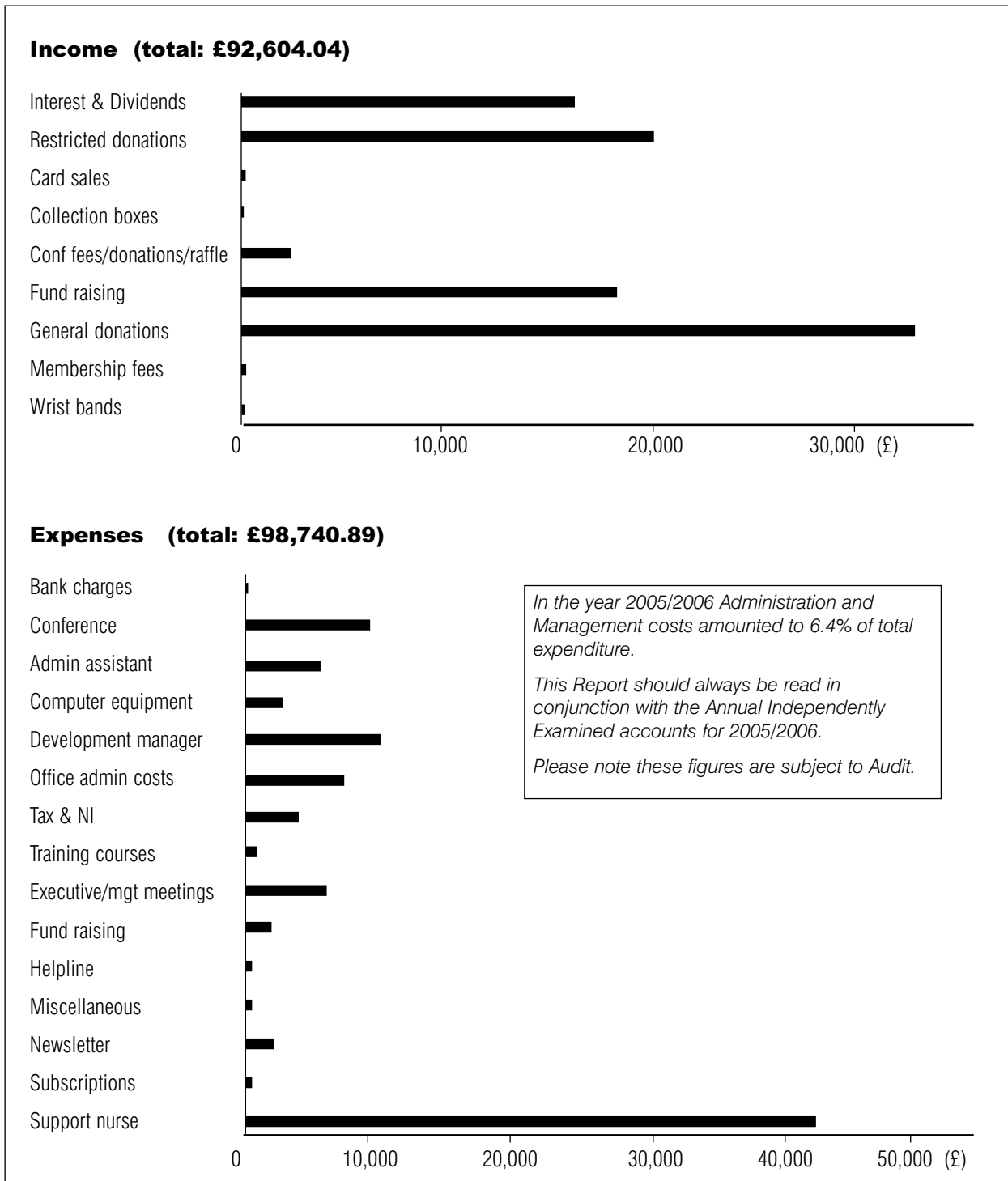
### **The Promotion of Relevant Research**

- Close liaison with Pharmaceutical Companies regarding existing and possible future developments in Niemann-Pick Disease.
- The appointment of a research nurse in collaboration with the Willink Biochemical Unit to carry out aspects of clinical research to further understand this Group of Diseases.
- The funding of a research project in Oxford into possible therapeutic responses to 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 still a lot to be done.

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.



## 2006 – 2007 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 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 the families and all others associated with the group of diseases.

**Therefore next 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.

### **Risk Assessment**

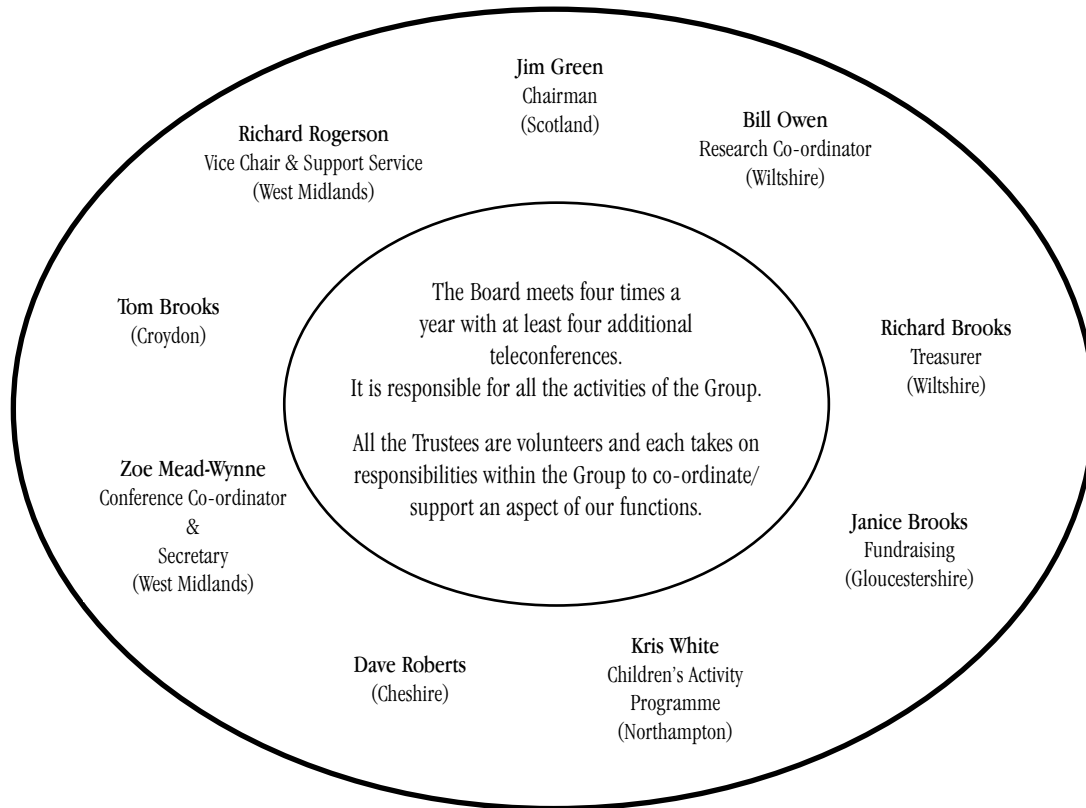
As part of the Charity Commission's procedures, each charity is required to undergo a risk assessment process within which it identifies the major risks which face the charity. This keeps us aware of the possible problems which lie ahead.

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 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

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