Society for Mucopolysaccharide Diseases

ANNUAL REVIEW & ACCOUNTS 2011

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CHARITY INFORMATION

TRUSTEES

Sue Peach (Chair - appointed 24 June 2011)
Barry Wilson (Chair - resigned 24 June 2011)
Robert Devine (Vice-Chair)
Wilma Robins (Vice-Chair)
Judy Holroyd
Tim Summerton
Bob Stevens
Paul Moody
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Judith Evans

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TRUSTEES REPORT

The Trustees present their report and financial statements for the year ended 31st October 2011.

Welcome to the Society for Mucopolysaccharide Diseases Annual Report and Accounts for this year end. The past twelve months have again been a mixture of unexpected challenges combined with the rewards of being a highly regarded patient organisation in the rare disease sector and achieving and serving the public interest of the charity. With this in mind the Trustees are pleased to have kept the percentage of the Society's expenditure on governance and administration to 13%.

The MPS Society can be proud of the services it has delivered to those affected by these devastating diseases. The support offered within the individual advocacy service is necessarily wide-ranging with the aim of meeting the needs of a diverse group of over 1,200 affected children, adults and their families. The support needs vary considerably between the disease groups not least because haemopoietic stem cell transplant is the accepted treatment for babies diagnosed under 18 months with MPS I Hurler disease and enzyme replacement therapy (ERT) for MPS I Hurler Scheie and Scheie disease, MPS II Hunter attenuated form, as well as MPS VI Maroteaux Lamy and Fabry disease. At the other end of the spectrum for diseases, including the four types of MPS III Sanfilippo disease, the Mucolipidoses and the Oligosaccharide diseases, the only treatment is palliative.

During the year we have continued to support an increasing number of children and adults participating in the clinical trial Enzyme Replacement Therapy (ERT) Studies taking place at Manchester Children's Hospital, Birmingham Children's Hospital, Great Ormond Street Children's Hospital, the Royal Free Hospital, the National Hospital, Queen Elizabeth Hospital, and Salford Royal Hospital. In addition, the Society has supported children in clinical trial studies for intrathecal ERT for MPS II Hunter and MPS IIIA Sanfilippo disease. The MPS Society has been awarded responsibility by the relevant pharmaceutical companies to coordinate the Patient Access Scheme for these trials. This involves awareness of the recruitment programmes and organising all logistics and reimbursement for those participating. Weekly or fortnightly infusions of ERT have proved a lifeline to many of our members with MPS I, MPS II, MPS VI and Fabry Disease, and continuing drug development in this field is most important, no less so than the oral therapy also being clinically trialled for Fabry Disease.

Alongside treatment and clinical management goes support. Roald Dahl's Marvellous Children's Charity has continued to part fund our palliative care and bereavement advocacy worker who has made frequent home visits to those families caring for children and young adults dying from their diseases, often helping with care and end of life plans. Seven children and adults lost their lives to MPS, Fabry and related diseases during the year and a further 69 were newly diagnosed. In October families gathered in the Childhood Wood in Sherwood Pines, Nottingham, to plant oak saplings in memory of their children who had lost their lives to MPS and related diseases. They were joined by representatives of Nottingham County Council and the office of the Lord Lieutenant of Nottinghamshire.

Due to new therapies and excellent clinical care an increasing number of children are living into their twenties and beyond. In July 2010 the Henry Smith Charity awarded a grant to fund the appointment of a Transition Advocacy Worker and in September 2010 the Department of Health awarded a Quality Improvement, Development and Initiative Scheme (QIDIS) grant to develop a healthcare transition model for lysosomal storage diseases. Throughout the reporting period the Society has conducted consultations with the paediatric and adult specialist MPS Hospitals and patient, parent, carer stakeholders with the objective of developing a model of transition for Lysosomal Storage Diseases that may be transferrable to other clinical disciplines. The recommendations and model will be delivered to the Advisory Group on National Specialist Services in 2012.

The Society has had a presence in Northern Ireland since its beginning in 1982 establishing the twice yearly Belfast MPS clinic and providing individual advocacy from the mainland. In August 2010 with a two year grant the MPS Society and the Primary Immunodeficiency Association (PIA) established a joint All Ireland Advocacy Officer post based at City Hospital, Belfast. During 2010/11 the Society has provided an expanding advocacy to MPS, Fabry and related diseases patients throughout the Island of Ireland as well as developing close relationships with the three service specialist hospitals in Dublin and Belfast. In May 2011 the Advocacy Officer and CEO organised meet and share evenings for families in Dublin, Cork and Galway. A total of 14 families participated.

Support activities and events continue to be an important part of the MPS Calendar, and included a Sibling mid-week break to Windmill Hill, East Sussex. In June 2011 the Society hosted a weekend Conference on MPS, Fabry and related diseases, with over 50 professionals and 80 families from throughout the UK coming together at the Hilton Hotel, Northampton to learn from the scientists and clinicians and share experiences. Throughout the weekend the MPS children and vulnerable adults were cared for by a team of 70 volunteers. The Society has a record for arranging disease specific professional expert meetings and in July 2011 held an expert meeting on MPS III, Sanfilippo Disease. 26 international experts from 23 countries came together to share knowledge in all aspects of clinical management and research that may lead to new therapeutic approaches.

MPS, Fabry and related diseases are rare, making ongoing public awareness and communications critical to getting the MPS message out in the public domain and growing support amongst the general public. During this reporting period the MPS Fundraising Newsletter has continued to be developed. The Society's focus in this area for the coming year will be improving accessibility of the MPS website and social networking to increase awareness and introduce new fundraising streams. The Society was represented at the Rare Disease Day at Royal Holloway College, London and featured in the national press promoting the needs of people with rare diseases. On May 15 the Society organised awareness activities to support the global MPS Awareness Day. A new fundraising initiative is being developed and will be launched in April 2012.

Funding research, after providing support and advocacy for our members, continues to be the most important objective. For the past 15 years the MPS Society has been one of four partner charities benefiting from the proceeds of the Jeans for Genes Charity. These funds have allowed the Society to establish and grow the MPS Stem Cell Research Group at the University of Manchester, as well as supporting research projects at the University of Cambridge and Kings College London. The Society has to identify potential new income streams to fund research to replace the funds received from the Jeans for Genes Charity following the breakup of the partnership by the Jeans for Genes trademark holder in March 2011. The Society has been most fortunate in securing £400,000 to fund a clinical trial to establish safety and effectiveness of the oral preparation, Genistein, from a private donor. A further £850,000 is still required.

Mindful that the economic challenges ahead are likely to bite deep into the pockets of our supporters and the public at large, the MPS Trustees set a prudent budget prior to the beginning of this financial year that provided for all core costs, advocacy service and essential events, and allowed for additional support and research activities to take place as and when restricted funding for that purpose was achieved.

Most importantly of all, our unique and disease specific individual advocacy service has been able to reach out to so many of those affected by MPS, Fabry and related diseases, including parents, partners, siblings and extended family. This could not have been achieved without the commitment of dedicated staff, volunteers, our members, MPS supporters, MPS specialist doctors, nurses and scientists.

Sue Peach
Chair of Trustees
28 January 2012

MPS SOCIETY AIMS AND OBJECTIVES, STRUCTURE AND PUBLIC BENEFIT

Our aims and objectives

- to provide support to individuals affected by Mucopolysaccharide and related diseases, their families and the professionals who care for them
- to increase awareness of Mucopolysaccharide and related diseases amongst health, social care and education professionals
- to extend this to the community at large to secure improved local services and inclusion for affected children and adults and their families
- to raise funds for research into Mucopolysaccharide and related diseases that may lead to improved clinical care, quality or life and new therapies

In order to carry out these objectives:

- we research the needs of children and adults affected by MPS and related diseases
- we fund cutting-edge, peer-reviewed research that may lead to therapeutic options for those affected
- we campaign for change in government policy and practice
- we deliver services to those affected by MPS and related diseases
- we operate in the four countries of the United Kingdom and collaborate with MPS Societies throughout Europe and the rest of the world, to raise money and seek ways to grow income

To achieve these objects, we rely on:

- funding from voluntary donations, corporate donors, charitable trusts and statutory bodies
- support from the public who fundraise and take part in events organised by the MPS Society

Our structure

The Society was founded in 1983 and is registered with the Charity Commission as an unincorporated association, registered charity number 287034. On 2 August 2011 the Society incorporated a new company limited by guarantee formed with the name 'Society for Mucopolysaccharide Diseases'. The assets, liabilities and activities of the Society were transferred into this charitable company on 1 November 2011. The Trustees, principal officers and professional advisors of the Society are listed on the Charity Information page. It is governed by a board of 13 Trustees known as the Board of Directors which meets a minimum of five times during the year.

The day to day business of the Society is managed by the Chairman, Vice Chairmen and Treasurer and is governed by the Articles of Memorandum. There are no sub-committees. One trustee's first language is Welsh, another lives in Scotland and a third is affected by a related lysosomal storage disease. Geographically, the MPS Society provides a UK-wide support and individual advocacy service from MPS House based in Amersham in Buckinghamshire. Our 12 employees are central to providing the support, individual advocacy services and the communication of our cause, while our 5,000 supporters are vital to us achieving our goals and objectives.

Recruitment and appointment of Trustees

New trustees are elected by the membership and may hold office for six years before retiring. The Board of Trustees (Directors) may co-opt up to three trustees who must stand for election at the next Annual General Meeting.

Trustees' Induction and Training

New trustees are provided with information about the Society and its work, the role and responsibilities of trustees, and the composition and workings of Trustees. They are given copies of the Society's governing instrument, a folder of all the Society's policies and the minutes of the last five Board of Trustees meetings. Trustees are regularly provided with the latest information on employment and charity law through the provision of the monthly Governance magazine.

Trustees Responsibilities Statement

The Charities Act 1993 requires the Trustees of the Charity to prepare financial statements for each financial year which give a true and fair view of the Charity's financial activities during the year and of its financial position at the end of the year. In preparing the financial statements the Trustees should follow best practice and:

- Select suitable accounting policies and apply them consistently;
- Make judgements and estimates that are reasonable and prudent;
- Follow applicable accounting standards and the Charities SORP, disclosing and explaining any departures in the financial statements; and
- Prepare the financial statements on the going concern basis unless it is inappropriate to assume that the Charity will continue in operation.

The Trustees are responsible for keeping accounting records which are such as to disclose, with reasonable accuracy, the financial position of the Charity at any time, and to enable them as Trustees to ensure that the financial statements comply with charity law. The Trustees are also responsible for safeguarding the Charity's assets and ensuring their proper application, and hence for taking reasonable steps for the prevention and detection of error, fraud and other irregularities.

The Trustees confirm that they have complied with the duty in Section 4 of the Charities Act 2006 to have due regard to the Charity Commission's general guidance on public benefit, "Charities and Public Benefit".

Risk Management

The Trustees have identified the level of risks to which the Society may be exposed and have established the implementation of a risk management strategy which comprises:

- quarterly reviews of the risks the Society may face
- systems and procedures to mitigate these risks
- implementation of procedures designed to report on and minimise any potential impact on the Society should any of these risks materialise

Public benefit

Over the year having due regard for the Charity Commission's guidance on public benefit, the trustees of the Society for Mucopolysaccharide Diseases have worked towards its charitable objectives in the following ways:

- providing advice and support to those affected by MPS and related diseases and/or their families to help them access the disability benefits to which they are entitled
- offering an individual advocacy service through home visits, face to face meetings, support at multi-agency meetings in writing, by telephone or email in areas of home adaptations, special educational needs, independent living and access to respite care
- through our dedicated Roald Dahl progressive neurological palliative and bereavement worker offering in-depth support to families caring for their child at the end stage of their disease, and preparing parents and siblings for loss
- arranging a UK-wide programme of events that included family days, a sibling weekend and a young MPS adult weekend
- organising a National Weekend Conference incorporating three symposia running in parallel on Mucopolysaccharide and related diseases
- recruiting, training and involving over 100 volunteers to provide a child and vulnerable adult care programme at the MPS Conference and event programme
- undertaking Quality of Life studies with the membership to inform the regulators and clinicians on the natural history of the MPS diseases and benefits from new therapies
- continuing to maintain the Childhood Wood at Sherwood Pines, Nottinghamshire facilitating the annual planting and Remembrance Day
- continuing to support the MPS Stem Cell Group at the University of Manchester with a programme grant, fund important research into the Blood Brain Barrier project at Kings College, London and by awarding pump priming research grants.
- Organise regional conferences and focus groups in Northern Ireland and Scotland.

Our Vision

A future where early diagnosis of a Mucopolysaccharide or related disease is routine, and new therapies are immediately available to prevent further multi-organ storage thus avoiding progressive physical disability, and in many diseases, neurodegeneration leading to early death, thereby allowing these children to grow up and achieve their full potential.

WHAT ARE MUCOPOLYSACCHARIDE AND RELATED DISEASES?

Mucopolysaccharide (MPS) and related diseases are rare diseases affecting one baby born every 8 days in the United Kingdom.

MPS and related diseases are a group of 24 rare metabolic diseases, referred to as MPS I-VII or more commonly by the name of the doctor who first described the condition: Hurler, Scheie, Hunter, Sanfilippo, Morquio, Maroteaux Lamy, and Sly.

Mucolipidoses and other storage diseases also covered by the Society are: ML I, ML II (I cell disease), ML III pseudo Hurler polydystrophy, ML IV, sialidosis, fucosidosis, mannosidosis, sialic acid storage disease, multiple sulphatase deficiency, aspartylglycosaminuria, Winchester, Metachromatic Leukodystrophy and Fabry disease.

Children born with a Mucopolysaccharide or related disease are unable to produce enzymes essential for the continual process of breaking down and replacing used materials. Babies may show no sign of the disease, but as more and more cells become damaged by the storage of used material, symptoms begin to appear. Sadly, these are progressive diseases which lead to an increase in problems as the years go by. Effects of the disease vary but are all associated with progressive physical disability. Over half these diseases cause degeneration of the brain resulting in severe learning difficulties and death in childhood.

HOW ARE THEY MANAGED CLINICALLY?

The Society continues to work collaboratively with the lead specialist centres in MPS and LSDs.

Until 2004 Mucopolysaccharide and related diseases were managed at a variety of District General Hospitals as well as recognised specialist centres in England. This resulted in a significant variation in quality of care and considerable “postcode prescribing” for new therapies. In 2004 the MPS Society initiated the process that led to the 50 lysosomal storage diseases (LSDs), which includes the 24 MPS and related diseases, being managed nationally in England by the National Specialist Commissioning Group (AGNSS). This designation changed almost overnight the patient’s right to be assessed and managed clinically at a recognised LSD specialist centre for children or adults and, where therapy is available to treat their disease, to be assessed against appropriate clinical guidelines.

The NCG LSD specialist centres are:
Paediatric
Great Ormond Street Hospital, London
Birmingham Children’s Hospital
Manchester Children’s Hospital

Adult
National Hospital, London
Royal Free Hospital, London
Birmingham University Hospital
Addenbrooke’s Hospital, Cambridge
Hope Hospital, Manchester

Wales, Scotland and Northern Ireland have designated specialist hospitals for managing metabolic diseases but MPS patients do not have the benefits of the AGNSS model and access to treatment in the same way as England. This does mean some of our members in these countries are denied treatment or have suffered delays even when they meet the AGNSS treatment guidelines.

The designated specialist hospitals are:
Wales - University Hospital of Wales, Cardiff
Scotland - Yorkhill Children’s Hospital, Glasgow
Northern Ireland - City Hospital, Belfast

CAN MPS AND RELATED DISEASES BE CURED?

There is no cure for any of the MPS and related diseases.

In 1980 Bone Marrow Transplant (BMT) was tried experimentally on a young boy with MPS I Hurler disease. Throughout the 1980s a small number of children diagnosed with MPS I Hurler, MPS II Hunter, MPS III Sanfilippo, MPS IVA Morquio, Fucosidosis and Mannosidosis were treated with a BMT. Over time it became clear that except in the case of MPS I Hurler, BMT does not offer any therapeutic benefit. Haemopoietic Stem Cell Transplant (HSCT) which includes BMT and Cord Blood Transplant is now the treatment of choice for children diagnosed with MPS I and having two mutations consistent with the MPS I Hurler phenotype. The outcome of HSCT is extremely variable usually only modifying the underlying disease. As a result many MPS I Hurler children who have undergone HSCT may have a degree of learning difficulty. An HSCT in these children does not eliminate corneal clouding or modify the bone disease. As a consequence, the children may continue to have serious spinal complications that require surgical intervention, as well as other joint problems, however it is the treatment of choice for babies diagnosed with MPS I Hurler under the age of 18 months. HSCT may also be considered in babies under the age of 6 months diagnosed with MPS II where the family phenotype suggests severe neurological involvement.

In the 1990s several pharmaceutical companies started to develop Enzyme Replacement Therapy (ERT) as a potential therapy for the LSDs. The first ERT was given an orphan drug licence to treat Gaucher disease in 1990. Over a decade passed before ERT became a reality, firstly for Fabry disease in 2001, followed by MPS I Hurler Scheie and Scheie disease in 2003, MPS VI Maroteaux Lamy in 2005 and MPS II in 2006. The serious limitation of all Enzyme Replacement Therapies is that it cannot cross the blood brain barrier and, therefore, in their present licensed forms are not a viable option for over half the MPS and related diseases associated with neuro-degeneration. Enzyme Replacement Therapy involves a regular infusion, either weekly or every two weeks, lasting up to five hours. Children and adults usually receive ERT through a Home Care Service closely monitored by their specialist LSD centre. The dose of ERT is based on the weight of the patient making younger children cheaper to treat. It is not unusual for the cost of ERT for a 16 year old to exceed £150,000 per annum!

RESEARCH AND TREATMENT

Clinical trials in MPS for new therapies

New therapies require years of pre-clinical work followed by clinical trials before they can be licensed. It can seem forever when you are the patient or the carer of a child or adult with an incurable and life-limiting disease. Currently, there is a UK multi-centre clinical trial for MPS IVA Morquio to determine the efficiency of Enzyme Replacement Therapy in this disease. The MPS Society has played a crucial role in ensuring that those affected by MPS IVA and their families are aware of the clinical trial, thereby ensuring equitable access. In August 2011 the first child in the world received Intrathecal Enzyme Replacement Therapy for MPS III, Sanfilippo disease at the Royal Manchester Children's Hospital as part of a phase I/II safety clinical trial for MPS IIIA. Since then a further five children have joined the study, and an Intrathecal Enzyme Replacement Therapy for MPS II is underway. The MPS Society is funded by the two pharmaceutical companies which produce the two enzymes to provide support to families participating in these clinical trials, including arranging accommodation and travel to the clinical trial centres.

Future treatments

Gene therapy holds the promise of a complete cure for lysosomal storage disorders like any other genetic disorder. Initial studies have shown that gene therapy is possible but there are a lot of hurdles still to be overcome. The problems that are to be solved are gene delivery to the target cells and the target tissue in the affected organs and sustainability. Concerns over safety have been aroused by recent experiences in apparently successful gene therapy cases where the recipient has gone on to develop leukaemia. For lysosomal storage disorders one of the approaches may be the introduction of viral vectors directly into the Central Nervous System (CNS). Another approach that is under consideration is to alter the haemopoietic stem cells from the patient to express the missing enzyme and returning the altered cells by BMT.

Small Molecule Therapy

Inaccessibility of the CNS because of the blood brain barrier and limitations of available forms of therapy, bone marrow transplantation and enzyme replacement therapy, has prompted scientists to think of alternative strategies.

The main attraction of drug therapy is that the small molecules can easily cross the blood brain barrier unlike the enzymes. The principle of using small molecules in lysosomal storage disorders is to keep the production of substrate so that it matches its impaired catabolic rate. This principle is variously named as substrate deprivation, substrate reduction or substrate inhibition.

The Society has funded pre-clinical studies to explore the viability of Genistein as a small molecule for the treatment of progressive CNS involvement by storage of GAGS in the brain in MPS III A, B and C. Scientists and clinicians at Manchester Children's Hospital and the MPS Stem Cell Group in Manchester are ready to recruit to a two year clinical trial. Applications for funding are being made to the Wellcome Trust and Medical Research Council, but clearly the success of the Society's new fundraising initiative to be launched in April 2012 will be critical.

CHILDREN AND ADULTS FIRST

Individual advocacy service

The MPS Society provides help to its members through an individual advocacy support service. The rarity of MPS and related diseases means affected individuals and their families may experience difficulties accessing adequate needs-led support and services in their local area. The MPS Society's advocacy team provides a support network promoting awareness and understanding of the diseases and works in partnership with members and their families, as well as developing professional relationships with local social care and health professionals.

Support following diagnosis & beyond

The Society provides support to individuals and families where a diagnosis of MPS has been made. Contact can be made by telephone, letter or registering details on the Society's website. On receipt of a new diagnosis, the Society will make contact and provide a membership pack which includes information about the Society and the support it provides, details on how to become a member, copies of the relevant disease booklet and copies of the MPS magazine. Each new member is offered a home visit and will be given information about the centres specialising in MPS and, specifically, the support the Society can provide.

At present the Society supports over 1,200 individuals and families affected by MPS and related diseases. The amount of support and contact varies greatly and is dependent on the needs of the individual and their family. This year the advocacy team provided support in a number of areas listed here. Although this list is not exhaustive due to the complex and continuing needs of our membership, it highlights the important role that advocacy still has in ensuring best practice and fair access to resources, support and information for all. This has become more apparent in the current climate where resource budgets have been cut, impacting on both the social and practical support offered.

- Telephone Helpline – the Society provides an active listening service, information and support; this includes an out of hours service
- Disability Benefits – in understanding the complexities and difficulties individuals and families have in completing Disability Living Allowance claim forms, the Society continues to provide help and support in completing these forms and, where needed, will take a representative role in appeals and tribunals
- Housing and equipment – the Society continues to take a major role in supporting and advocating appropriate housing and home adaptations to enable the needs of an individual with an MPS or related disease to be met; where requested, we can provide comprehensive and detailed housing reports based on individual need
- Education – the Society helps members to access appropriate education and adequate provision for its implementation; this is achieved through providing educational reports used to help inform and educate professionals, and in many instances, to inform if Statements of Special Educational Need are necessary; where requested, we also provide information days/ talks to schools and relevant professionals
- Respite Care – the Society continues to work closely with a number of respite providers and, where appropriate, can make individual referrals
- Independent Living/Transition – the Society provides advice, information and support through the transition from child to adult services; this could include access to independent living, learning to drive, further education, employment and relationships; the Society is currently undertaking a review of transition services for the Department of Health with a view to producing national guidelines
- MPS Careplans – the Society undertakes a comprehensive assessment of the issues which need to be addressed when caring and providing support to a specific individual diagnosed with an MPS or related disease, as well as other family members, through the writing of a careplan
- Befriender Service – the Society links individuals and families affected by MPS and related diseases for mutual benefit and support
- Social Care – the Society is seeing an increased demand for support in the social care area; this includes access to assessments and support of the provision of services

Access to MPS / LSD clinical management and therapy

The Society continues to work collaboratively with the lead specialist centres in MPS and LSDs. We embrace an equitable access to all specialist centres and continue to play an important role in promoting the Department of Health guidelines on Advisory Groups for National Specialised Services (AGNSS).

The MPS regional clinic programme involves centres including Manchester, Belfast, Birmingham, Bristol, Cardiff and Newcastle. The programme aims to enable individuals affected by MPS and related diseases to access a consultation with a tertiary centre consultant without having long distances to travel to the NCG (AGNSS) designated clinical centre of excellence. The clinics also aim to increase expertise in the regional centres on MPS and related diseases in the clinical management of those affected.

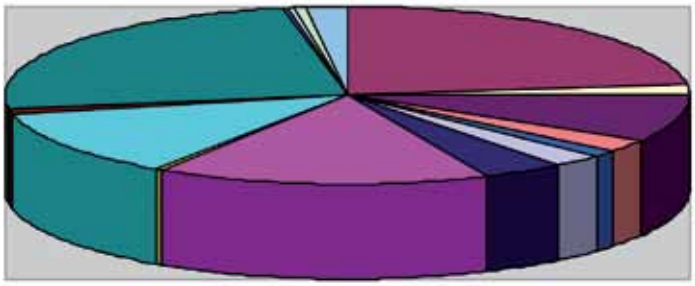
The MPS Society supports the regional clinic programme, and in some instances, arranges and co-ordinates dates of the annual programme and funding of the AGNSS centre doctors' travel and subsistence costs to the regional centres. Specific arrangements for each clinic and for organising appointments differ for each of the centres. At one, a member of the Society's advocacy support team meets the individuals and families face to face to offer individual advocacy support.

Palliative care, pre and post bereavement support

Services for children and young people with life-limiting conditions and who require palliative care vary from locality to locality. Although much has been done to educate service providers, there are areas which are still considerably lacking the knowledge, expertise and resources required to carry out the services needed.

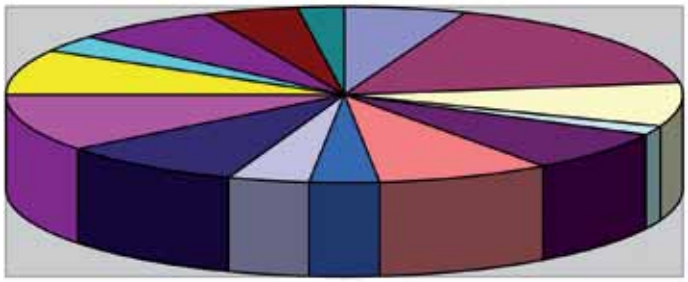
The Society continues to develop and review this service and is in the process of producing some fact sheets in this area of work.

Breakdown of Members Supported by Disease



- MLD
- Sanfilippo
- ML III
- MSD
- Morquio
- Maroteaux Lamy
- Mannosidosis
- ML II
- Hurler Scheie
- Hurler
- ML IV
- Hunter
- Geleo Physic Dysplasia
- Fucosidosis
- Fabry
- AGU
- Sly
- GM I Gangliosidosis
- Sialic Acid Disease
- Winchester
- Scheie

Areas of Support Provided by the MPS Advocacy Team



- Clinical Management
- New Members
- Disability Benefits
- MPS Individual Care Plans
- Work with Local Authorities & Other Organisations
- Palliative Care and Bereavement
- Transition
- Befriending Links
- Education
- Housing
- Grants for Individuals
- Clinical Trial Support
- Home Visits
- Professional Meetings
- Presentations/School Talks/Training

MPS SUPPORT EVENTS

National MPS Conference

24 - 26 June 2011

Every two years the Society holds its National conference in Northampton. This is a well-attended event with professionals and families attending from across the world. We hold simultaneous conferences with the most up to date pioneering information and news in the field of LSDs. While parents and professionals attend the conferences, over 70 volunteers provide care and support to over 100 children and young adults enrolled on the childcare programme. Activities include day trips to theme parks and local attractions, as well as providing a crèche facility for those children too young to go out or who are unable to go due to their complex medical condition.

"I'm just writing to thank you and everyone there for giving us such a great time at the conference last week. It really was enjoyable meeting up with all our MPS friends old and new and sharing experiences and finding solutions. We came back feeling energised, although tired, but we really did have fun too, we had so many laughs."

Sibling Activity Week

25 - 29 July 2011

Another successful activity week was organised at Windmill Hill, East Sussex for children who have a brother or sister with an MPS or a related disease, aged 7-15 years. These breaks away from home enable these siblings to have fun with youngsters in similar circumstances, meet new friends and have new experiences.

Every day there were five activities to keep the children occupied with things such as raft building, zip wire, abseiling, climbing, trapeze, Jacob's ladder and BMXs.

Here are some of the siblings' comments:

"I really enjoyed this week, especially when we had a water fight and we soaked our leaders. I would like to come again."

"I loved sibling week because I met new amazing friends and adults. Although some activities were challenging I really enjoyed abseiling and the giant swing. I hope to come to a sibling week again."

"I liked the BMXs and Giant Swing. I really would like to come to a sibling week next year."

Post-Christmas Blues Party Scotland

23 January 2011

Following a late funding donation to hold an event in Edinburgh, Scotland, we came up with the idea of holding a Post Christmas Blues party to bring a bit of light and laughter to the dreary month of January. This was to include the traditional Christmas fair, crackers and a special visit from Father Christmas himself. We also had a magician come to entertain us with his mixture of comedy and magic and he also made balloon models for each of the children to take home.

MPS Awareness Day 5th Anniversary celebrations

15 May 2011

Each year the Society celebrates International MPS Awareness Day on 15 May. This is a day devoted to raising awareness of MPS and related diseases. To recognise the fact that MPS Awareness Day has been celebrated for 5 years, the Society decided to hold a family day at Whipsnade Zoo. Families were invited to arrive at 10.30am to enjoy the zoo with a lunch being provided at 12.30pm for all families to come together to celebrate this event. During the lunch we had speeches from our Chairman and also one from a family. They both spoke about the significance of MPS Awareness Day and how the Society has supported them. After lunch had finished families were free to access the zoo again.

THE CHILDHOOD WOOD

In 1992, the Society was asked to propose an idea to commemorate children who had died from an MPS or related disease. Out of many ideas, the Childhood Wood was born.

The following year the MPS Society was given under licence an area of Sherwood Forest to create a wood of saplings which were cloned from the Great Oak.

In February 1993, 150 saplings from ancient Sherwood Oaks were planted by MPS families, supported by Sir Andrew Buchanan, Lord Lieutenant of Nottinghamshire, along with local MPs and representatives from Nottinghamshire County Council.

Since then, each of the oak trees planted annually celebrate the life of a child whose childhood was destroyed by these cruel, degenerative diseases. The trees which were planted in the early years are now branching out and forming a canopy.

Each year, further saplings have been planted by families and friends of children who have lost their lives to MPS or related diseases.

Over the years and working in collaboration with the Forestry Commission, the MPS Society has put in place memory boards and new pathways, all of which are accessible to visitors to the Childhood Wood. Amongst the pathways, there are wooden animals and picnic areas offering a peaceful setting for those who wish to reflect quietly before leaving the Childhood Wood.

The Childhood Wood Planting

Those families who lost a child in the previous 12 months were invited to plant a tree, in their memory, at the Childhood Wood planting day on 21 October 2011. Following lunch at Sherwood Pines Cafe, families and dignitaries gathered at the Wood to read the names of the children and adults being remembered and to listen to a reading of the poem ‘Remember’. Families and friends were then able to take their time to plant the sapling trees and release balloons in memory of loved ones.

ENGAGING WITH VOLUNTEERS

MPS Childcare Volunteers

The Society made use of over 80 childcare volunteers who generously gave their time to care for children and young adults affected by MPS or related diseases, and their siblings, at activities and events organised during the year. Recruiting volunteers to assist at our events and conferences provides parents and carers an opportunity to listen to presentations and learn more about MPS and related diseases and to have time to relax, while their children are looked after by our trained volunteers in a supported and happy environment.

We organise a programme of activities for all children, sufferers and their siblings, from babies to teenagers. Volunteers are assigned to a specific child or children and these may be children suffering from an MPS or related disease, or their healthy siblings. Children and their volunteers are put into groups according to age and well-being, and are supported by volunteer group leaders. New volunteers are always supported by an experienced volunteer. Volunteers are provided with care plans specific to the children in their care, along with any additional information or equipment necessary.

All volunteers are required to register with the Society and attend training prior to the event including a briefing on MPS and related diseases, childcare arrangements, details about the event, roles and responsibilities as well as appropriate Health and Safety guidance, Child Protection and when appropriate Moving and Handling training. Volunteers will also have undergone a Criminal Records Bureau check carried out through the MPS Society.

Hannah's Story

"I've been volunteering for the MPS society for a few years now and I love it.

One of my brothers (Dan) and my sister (Amy) both have Sanfilippo and when I was younger I really enjoyed coming along to the MPS conferences. Our family used to need quite a few volunteers and I have really happy memories of fun volunteers who gave us all such a great time.

As a volunteer now, seeing the younger MPS children and their siblings brings back happy memories of when Amy and Dan were little and needed fast runners to chase them! It feels really good to be able to give something back now. The weekends are always such good fun. What could be more fun than a hotel full of MPS kids running around and causing chaos!

It's great to meet some amazing families and spend the day with fantastic kids in the hotel or taking them out for the day to a theme park or zoo. It's definitely hard work and challenging at times and you need to be prepared to run fast and have hands that can be in four places at once!

Sometimes things don't always go to plan either… like when fire alarms go off and you have to evacuate all the children out of the hotel! But it's all great and by the end of the day a good night's sleep is always guaranteed!

It's satisfying too; I really enjoy getting to know the kids and their siblings over the weekend. Seeing them enjoy themselves whilst knowing that their parents are able to appreciate a bit of a break is great. It's nice to feel like you've really been a help and its really rewarding to have a parent say to you "Wow you can come again, she's gone straight to sleep she was so worn out!"

Being trusted to look after someone else's child for the day does feel like a big responsibility, but the weekends are always so well organised. The volunteers are like a big team and all work together and help each other out. It's been good coming back year after year, catching up with all the other volunteers and seeing the families that I've got to know before.

This last year it's been really nice to volunteer on the young adult weekend too. This was in Blackpool and was quite a bit different to the other weekends I'd volunteered on before. It was good to spend time talking and getting to know each of the young people and I was inspired by their positive attitude towards life, especially with the many challenges that they face and overcome every day.

I've really enjoyed volunteering at the MPS weekends, getting to meet some amazing people, experience some really interesting situations and have a great time! MPS weekends are definitely the most exhausting weekends of my year but also some of the most rewarding and fun ones too." **Hannah Donegani**

MPS Office Volunteers

The Society made use of three office volunteers to provide secretarial support, pack the quarterly MPS magazine and continue to develop an access database for maintaining epidemiological data on MPS diseases.

One of these volunteers has given half a day a week throughout the year to continue to develop and maintain the MPS Registry that provides valuable demographic and epidemiological data on MPS diseases. Over the past year the work has concentrated on updating and verifying data on all registrants entered with a diagnosis of MPS III and MPS IVA as well as producing reports to support presentations made by the Chief Executive and Senior Advocacy Officer.

MPS INFORMATION AND AWARENESS

MPS Publications

The Society is continually updating and developing our range of educational materials and information resources to reflect the changing needs of our membership. The Society has finalised a range of fact sheets on each of the MPS and related diseases. These are downloadable from the MPS website or available in printed form from the MPS office for a nominal charge.

In the last year we have published a factsheet for siblings of those affected by MPS and related diseases. We have also begun work on three new publications focussing on bereavement and end of life, living with restricted growth and transition.

We distributed our quarterly MPS magazine and fundraising newsletter to the entire membership free of charge.

During the year the advocacy team gave 12 school talks and information days in the South West, South East, North West and the Midlands. They were at the request of the education authorities, in conjunction with individual families, to provide strategies and understanding so the affected child's school care needs can be met.

MPS Awareness

In 2007 the MPS Society announced its first ever MPS Awareness Day as part of an international campaign to raise awareness of MPS and related diseases. On 15 May 2011 the Society was delighted to hold its fifth MPS Awareness Day. The Society's members and supporters helped us celebrate this day by organising a number of awareness events and activities throughout the Country and a family day was organised by the Society at Whipsnade Zoo to celebrate MPS Awareness Day and the start of our thirtieth anniversary year.

MPS Website

The development and management of the MPS website, www.mpssociety.co.uk, continues and attracts considerable positive feedback with the number of downloads and latest news being added to regularly.

We aim to have an online shop live in the next year to reflect an increasing demand for this type of shopping.

COLLABORATION

LSD Patient Organisation Collaboration

The patient organisations for those affected by LSDs work collaboratively with the umbrella of the 'LSD Patient Organisation Collaborative' to work and lobby on behalf of LSD patents and their families in the UK. The group is made up of representatives from the Association of Glycogen Storage Disease, Batten Disease Family Association, the Gaucher's Association, the Society for Mucopolysaccharide Diseases and the Niemann-Pick Disease Group UK. As part of this collaboration, the MPS Society has undertaken to jointly promote and share understanding of their diseases to advance standards of care and to enhance the well-being of those affected. The LSD Collaborative has now achieved the status of a recognised forum and has been invited to provide the expert patient perspective to AGNSS in respect of procurement of Enzyme Replacement Therapy and Home Care and the approval of new therapies coming to the market.

MPS International Network

We are members of the MPS International Network which is the official body of the patient associations representing MPS Societies at a European and global level. We are also members of the Fabry International Network the equivalent global body for Fabry disease. These bodies act as an independent forum to promote the well-being of those affected by MPS, Fabry and related lysosomal storage diseases through the best practices of clinical diagnosis, management and treatment. They promote public and professional awareness of MPS and related diseases, identify research priorities and potential collaborations where appropriate, working in partnership with the academic community and pharmaceutical industry. The MPS International Network meets at least annually and in 2011 had a one day meeting preceding the Taiwan and South East Asia MPS Society Conference.

INFLUENCING RESEARCH

The Society's overarching research objective is to 'Promote and Support Research into MPS and related diseases.'

Its purpose is to play a leading role with clinicians, scientists and academics in initiating and funding innovative research projects which may have a therapeutic benefit to those affected by MPS and related diseases.

Initially, the MPS Society funded one-off projects because it could not rely on regular funding. In 1995 the MPS Society entered into a partnership with the Jeans for Genes charity that generated regular income for research. As a result of this income, the Society undertook to develop an MPS Stem Cell Group at the University of Manchester with an initial programme grant of over £300,000. As you will read here, support to this group has grown and many research projects have been funded.

The Society's Research Strategy 'Making a difference 2005-2010' set out how this was to be achieved:

- promote and fund research into the causes and effect of these severely life-limiting diseases with particular emphasis on getting enzyme into the brain
- extend the psychological research programme identifying the impact of MPS and related disorders and publish the results
- continue to develop the MPS registry

Between 1985 and 2011 the Society has funded and participated in innovative projects that have advanced the knowledge of MPS and related diseases. Unfortunately, due to the decline in income for research, many important research projects remain unfunded.

In this reporting period the Society continues to support the following grants:

Prof Ed Wraith, Dr Rob Wynn and Dr Brian Bigger
MPS Stem Cell Research Group,
University of Manchester
Final year of a five year programme grant to establish the Stem Cell Therapy Group with funding for a Senior Research Fellow at the University of Manchester. The post holder, Dr Brian Bigger, is responsible for leading and developing the group in stem cell therapy for the Mucopolysaccharidoses.

Dr Brian Bigger
MPS Stem Cell Research Group,
University of Manchester
Final year of a two year grant for improving enzyme secretion and tracking in lentiviral mediated stem cell gene therapy of MPS III

Dr Brian Bigger
MPS Stem Cell Research Group,
University of Manchester
One year funding to investigate Genistein and Synaptic Decline in MPS IIIB

Dr Brian Bigger
MPS Stem Cell Research Group,
University of Manchester
1 year grant to research Non Myeloblative Bone Marrow Transplantation for MPS I

James Blundell
University of Birmingham
Year one of a three year grant to fund a post graduate studentship to develop of sensitive and efficient methods for measuring cognitive abilities in the areas of memory, language, attention and motor control in children with Mucopolysaccharide and related diseases

Dr Chris Hendrikz
University of Birmingham
Contribution to grant by the LSD Patient Collaborative to the LSD Clinical Study Group

Dr Patrick Deegan
University of Cambridge
1 year grant to research Therapeutic targeting of lysosomal enzyme glycoforms in Hunter syndrome and Fabry disease

Dr Brian Bigger
University of Manchester
Grant to research 'The brain degeneration in Sanfilippo mice reversible after long term substrate reduction therapy'

Dr Brian Bigger
University of Manchester
Grant to research 'The effect of heparan sulphate on stem cell homing and engraftment in MPS I'

Dr David Begley
Kings College London
To study protein fusions in respect of the Blood Brain Barrier

POLICIES

Restricted Funds

The Society receives grants for activities which are considered to be restricted funds as such funds can only be used for the purpose for which the funds were given. Restricted funds held by MPS at 31 October 2011 represent unspent grants at the end of our financial year. This position has arisen as not all the grants run co-terminously with our financial year and so balances are taken forward into the following financial year.

Grant Making Policies

The Society's charitable grant making policies include offering support to children and individuals with MPS and their families and funding research. The Society allocates a small budget each year to provide financial assistance to families in genuine need for the purchase of essential equipment, and to access events organised by the Society.

There is a thorough application process for applying for MPS research grants. All but small grants are peer reviewed, and short-listed applicants may be required to present to the Management Committee.

Investment Policy

The Trustees have the power to invest surplus funds as appropriate under the constitution as they see fit. The Trustees have sold any investments in favour of holding all such funds in low-risk bank deposits. Consequently, the Society no longer holds any fixed asset investments, and bank deposits are classed as current assets as appropriate. The Society holds a small amount of Treasury Stock donated by a Charitable Trust. These Treasury Stocks are reviewed by the Trustees annually.

Reserves Policy

The primary aim of the Society's Reserves Policy remains that we will hold adequate funds, which may include any equity on the building MPS House, to maintain the long term sustainability of the Society's support and advocacy service, its research projects and to manage liquidity effects of short-term volatility in income. This policy is designed to ensure that the Society can:

- continue to meet its financial commitments
- deploy funds promptly, in a planned way and react to new opportunities
- balance sound investment, in a planned way and react to new opportunities
- not be forced into short-term decisions to the detriment of its long-term vision

The bulk of the Society's charitable expenditure is by way of provision of the Society's support and advocacy service and its commitment to research projects funded from the restricted income from grant-givers and fundraising donors. In respect of the support and advocacy services, which provides a lifeline to over 1,200 affected families, the Trustees wish to hold sufficient free reserves to ensure that, in the event of the worst case scenario, they could continue to meet the costs of the service for at least 6 months whilst action is taken to increase income and/or reduce other expenditure in an orderly and controlled manner.

Based on the budget for 2011/12 and having deducted restricted funds received for these essential care services in this financial year, the reserves required amounts to £420,000. Currently the MPS Society owns a building with an outstanding mortgage of £237,545 and the book value of MPS House is £725,120.

During the year the Trustees have reviewed the Reserves Policy and have re-examined MPS' requirements for free reserves in the light of the predominant risks to the organisations and its working capital requirements.
The three major risks are:

- the impact of the economic climate
- loss of reputation resulting in the loss of corporate and trust support
- loss of fundraising income due to the severity of MPS diseases and the demands on families and carers

Auditors

McLintocks have signified their willingness to continue in office and a resolution to re-appoint them will be proposed at the forthcoming Annual General Meeting.

Sue Peach
Chair of Trustees
28 January 2012

FINANCIAL REVIEW

As well as applications to grant giving bodies for special projects and advocacy support worker salaries, the Society relies heavily through the year on its members and supporters to raise unrestricted income by way of donations and fundraising. Fundraising receipts were up by £12,793. This may be attributed to the downloadable fundraising materials available on the MPS website and the support members are receiving in their fundraising efforts from the Communications Officer. In contrast, general donations and legacies are down by £84,417. This is primarily due to a one-off major legacy of £100,000 received in the previous financial year. Whilst compared with many charities the Society's income in these areas held up well, the Society still has the ongoing challenge of attracting unrestricted income to enable the Society to deliver its support and individual advocacy service.

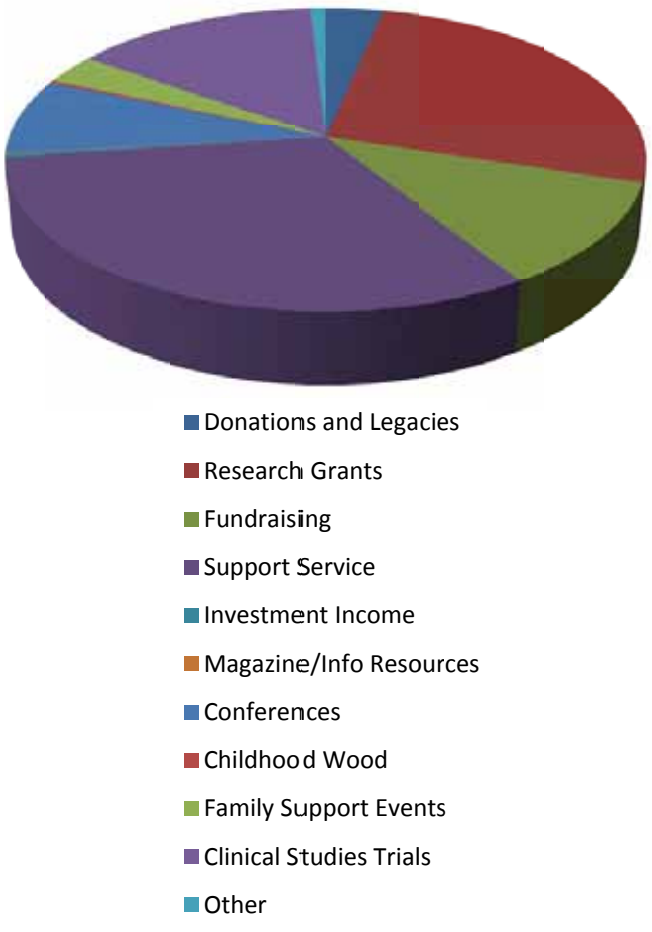
Overall income decreased by £494,623 to £1,319,561 compared to overall expenditure of £1,227,284.

The total charitable spend decreased by £79,369 to £1,023,870 in this financial year. This is accounted for by a decrease in income for research of £628,737 from the previous financial year. Research expenditure fell by £167,782 in this financial year. It should also be noted that the majority of grants awarded have not yet been drawn down. At the year end the Society has a surplus of £92,277.

The MPS Society provides a Patient Access Scheme for members participating in the MPSII, MPSIIIA and MPSIVA clinical trials. The MPS Society, under separate agreements with Biomarin Pharmaceutical Inc. and Shire Human Genetic Therapy, meet the costs of reimbursement of travel, accommodation, meals, additional educational and employment needs of patients participating, and their parent/carer(s). The Society meets these costs, which in this financial year accounted for £209,585, and is reimbursed in arrears by the respective companies.

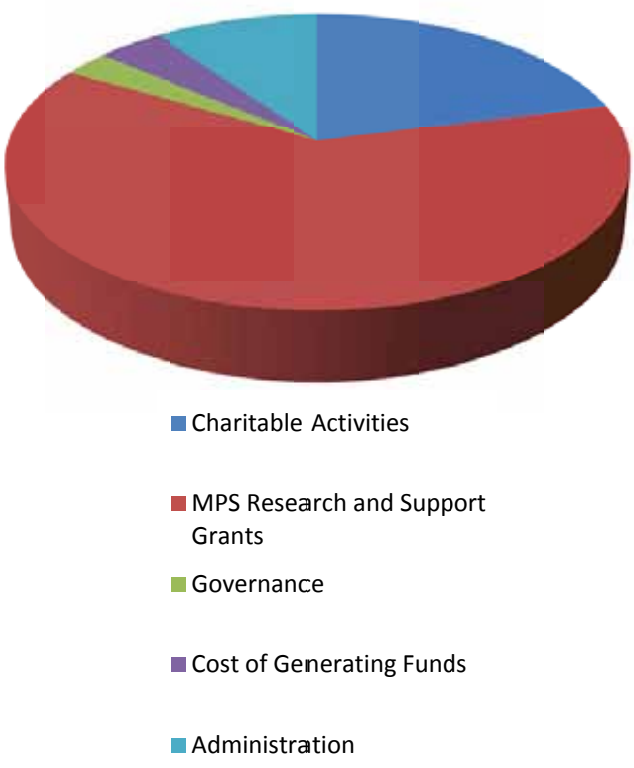
Where the money came from

The MPS Society's income was £1,319,561 and was generated in the following areas:



Where the money was spent

The MPS Society's expenditure was £1,227,284 and was spent in the following areas:



FUNDRAISING FOR THE MPS SOCIETY

We recognise and appreciate the enormous effort that goes into fundraising and would therefore like to convey our appreciation to all our members, donors and fundraisers for their support to the Society over the year. This will help us strive to meet the needs of the increasing number of MPS families and in finding a cure for these devastating diseases. Our supporters have held many events for us in the last twelve months. Look out for forthcoming events in the fundraising newsletter which is packed full of inspirational fundraising ideas. Don't forget to send in your stories and photographs which we can include in the newsletter to fire the imagination for others!

Here are stories from some of our fundraisers:

MPS Awareness at Work
"I have worked at Veolia water for 10 years and have a daughter called Tillie Mae who was diagnosed with MPS III in April 2010. I felt like my world had ended, my poor little girl. That was before I contacted the MPS Society which offered us great support through our difficult time. There is no cure for my little girl and all the other children that have MPS III but money and research is in full force. When my manager asked me what she could do to help, I replied help me get the awareness out there and let's raise lots of money.

We set about getting great raffle prizes including a personal shopping experience day, theatre tickets, hotel night stays etc. We also asked friends to come in for the day and set up a tombola stall, Virgin Vie stalls, Bodyshop stall and beauty treatments and of course a yummy cake stall. The day went very well with lots of people asking questions and saying "Oh I've never heard of this condition." So, one thing we achieved was the awareness and a bonus of raising £830. Big thank you to the Veolia Water staff."
Michala Mawdsley

Charity Skydive
Alex Spina took part in a charity skydive and raised £812.80 for the MPS Society on Justgiving.com. On his Justgiving.com page, Alex writes: *"At 7 months my cousin was diagnosed with Hurler's syndrome - a rare genetic, metabolic disorder.*

Due to a malfunction of enzymes, carbohydrates build up in the bones, joints, organs and brain, ultimately affecting appearance, mobility, mental development and organ function. So I decided to take part in the Great Warwick Jump (a charity skydive) to raise money for the MPS Society in the UK, which funds leading research and provides support to families affected by the disease."
Alex Spina

Hat Trick Charity Fundraising climaxes with a 'Walk on Hot Coals'
Lily O'Hara and Steve Hwozdyk walked over Hot Coals, twice, at Old Trafford Cricket Ground on Saturday 16th April to raise money and awareness for MPS. Family and friends from as near and far as America and Canada, as well as Rochdale raised £1,165.88 including Gift Aid to watch and cheer Lily and Steve on whilst they 'hot stepped' across the coals. Both Lily and Steve said it was a great adrenalin rush, but emotional at the same time, knowing they were doing the 'Firewalk' for MPS and Sam.

Lily and Steve completed the 'Firewalk' for their nephew, Sam Jamil who appeared on the cover of the winter edition of the MPS magazine.

Sam suffers from Mucolipidosis III. Sam's mum, Shirley, has also managed to raise £310.00 for the charity by practising 'The Bowen Technique' on her case studies. Shirley had heard that the Bowen Technique was very good for helping with pain relief. In an attempt to help Sam deal with his pain, and strengthen his muscles, Shirley decided to train in Bowen Technique. Her case studies kindly gave donations to the MPS Society, as thanks for being able to experience the many benefits that Bowen can bring, including increased muscle strength, helping digestive and respiratory problems, and general pain amongst many other benefits.

Sam also enjoys Bowen, as he says 'It helps him kick the football without it hurting him as much.' To achieve the hat trick, family friend, Caroline Fogarty managed to get colleagues of the Royal Bank of Scotland to assign £500 from their payroll charity-giving scheme to the MPS, as featured in the Spring 2011 edition of the MPS Fundraising Magazine.

The Fundraising total so far is £1,975.88 including gift aid.

Corporate Giving

Our biggest group of corporate supporters are the pharmaceutical companies which are working in the fields of research and therapeutic treatments for MPS and related diseases. We value their contributions but are careful to maintain our independent position and acceptance of a donation does not imply endorsement by the MPS Society of the company's products.

We also have a number of businesses and companies which fundraise regularly for us because a member of staff has a relative with MPS, or knows someone who is affected. Their efforts on our behalf are much appreciated.

We are keen to engage with any companies which are interested in working with us in the following ways: payroll giving, matched funding of staff fundraising efforts, magazine sponsorship, 'in kind' donations of goods or services, and recruitment of volunteers.

Give As You Earn

Contributions by way of regular direct debit have increased and Give As You Earn through employers' schemes have risen enabling the Society to plan for the future.

Department of Health Third Sector Investment Programme

In April 2009, the MPS Society was awarded a three year grant by the Department of Health under its Third Sector Investment Programme 'Innovation, excellence and service development' fund. The grant was awarded to establish national standards of care and guidelines on clinical management of the 17 lysosomal storage diseases for which there is no treatment. The resulting 'best practice model' will be made widely available to other organisations providing services to patients and families with rare diseases.

In March 2010 the Society reported its first year's work which resulted in draft clinical and social guidelines for children and adults diagnosed with Sanfilippo disease. These guidelines have now been peer reviewed and the second year's work to develop clinical and social guidelines for the Mucolipidoses is nearly complete.

Relationship with the Pharmaceutical Industry

Since the development and approval of the first Enzyme Replacement Therapy for lysosomal storage diseases by Genzyme there has been a rapid growth and interest in the development of new therapies for MPS diseases. Although the number of diseases that currently benefit from treatments is very small, this progress is very much welcomed by the Society and its members. There are now at least six pharmaceutical companies actively involved at pre-clinical and clinical trial stages to develop treatments for other MPS disorders. As a result the MPS Society works independently but collectively with the pharmaceutical industry to promote the MPS diseases, facilitate clinical trials and ensure that new therapies reach the patients.

CORPORATE SUPPORTERS & FUNDERS

We make carefully targeted applications to suitable trusts and foundations for specific areas of our work and understand the desire of grant-makers to see exactly where their money is going and what it is achieving. This might be to give a group of young carers a respite holiday, provide support to patients and their families at regional MPS clinics or offer bereavement support. Grants for medical research are sought whenever we need funds for our ground-breaking programme of clinical studies which are leading to new treatments for MPS and related diseases. We also welcome contributions towards the core costs of providing our nationwide advocacy service and are particularly keen to establish long-term relationships with trusts and foundations whose aims and mission are a close match with ours.

We welcome visits and discussions to explore the different ways in which grants can help us improve the lives of individuals with MPS and related diseases, and the families who care for them.

The MPS Society would like to thank all MPS members, families and subscribers, corporate donors and charitable trusts for their invaluable support this year. Some of our benefactors are included here:

Greggs Foundation
Andy and Jenny Hardy
The Towersey Morris Men
The Bown Family
The Pearson Family
Kingscote Preparatory School
Bank of America
Carrington Fox
Stratstone of Mayfair – LandRover
The Drayne Family
The Souter Charitable Trust
Anne Cotton and Heather Gordon
Royal Bank of Scotland
Marina Foster and Friends
Asda Eastbourne
Veolia Water
Scottish Congregational Women's Fellowship
Baily Thomas
Biomarin Europe
Constance Green
DHSS – England
The Eveson Charitable Trust
Genzyme Therapeutics
Leather Sellers Company Charitable Fund
Roald Dahl's Marvellous Children's Charity
Shire Pharmaceuticals
The Henry Smith Charity
The Shauna Gosling Trust
The Sheldon Trust

TIMELINE OF THE MPS SOCIETY

1982
The MPS Society is founded. The first meeting of the MPS Management Committee is held on 13 October. The first MPS newsletter is dispatched on 31 October to 70 families and 12 hospitals.

1983
The Society is granted trusteeship and registration as a UK national charity in May. The first 40 founding member families come together for the first weekend conference in Birmingham in September.

1984
The Society produces its first formal annual report and statement of accounts.

1985
The MPS Society first starts funding basic research. The MPS Society facilitates the first cervical infusion for Morquio disease in England using the halo method and bone grafts from the legs. The surgery is carried out at University College Hospital involving British and American doctors. The MPS Society receives a grant to establish the first MPS registry collecting incidence data and epidemiological data for the UK and Europe.

1986
Whilst the newsletter continues to be produced by a member family in a domestic environment, the first tentative embrace of new technology is made by the production of the newsletter on a home computer linked to the printer purchased by the Society in 1985.

1987
At the Society's annual family weekend conference at the Posthouse Hotel, Heathrow, bloods from over 50 MPS II Hunter families were taken to enable research being conducted by Prof John Hopwood from the Women's and Children's Hospital, Adelaide, Australia to be carried out.

1988
The MPS Society announces four year funding of Dr Ed Wraith's post as Consultant Paediatrician specialising in MPS at Royal Manchester Children's Hospital (RMCH). In May the first MPS family day in the Republic of Ireland is held.

1989
The first disease booklets are published by the MPS Society. Prof John Hopwood isolates the gene for MPS II Hunter disease making the path clear for potential treatment.

1990
The first international symposium on MPS diseases is hosted by the MPS Society at the University of Manchester. It brings together over 150 families and 300 professionals to share experiences from all parts of the world.

1992
The Society celebrates its tenth anniversary year with a Reception at the House of Commons in June.

1993
Inaugural planting takes place of 150 saplings from the ancient Sherwood Oak planted by MPS families. The Rt Hon Michael Howard, then Secretary of State for the Environment, plants the first oak in memory of Simon Lavery. Christine Lavery is appointed by Trustees to the post of Chief Executive.

1995
The number of newly diagnosed families seeking support from the MPS Society doubles.

1997
Following three year funding to the RMCH and Christie Hospital Manchester the first gene therapy trial for MPS I Hurler disease is carried out at RMCH.

1998
The MPS Office moves to Woodside Road, Amersham from Hill Avenue. In December the Rt Hon Gordon Brown MP as Chancellor of the Exchequer hosts a children's party at No. 11 Downing Street.

1999
Significant research in ERT has led to clinical trials for Fabry and MPS I disease. The MPS Society receives its largest grant yet to fund the MPS advocacy service for the coming three years.

2000
The MPS Society celebrates its 18th birthday.

2001
Enzyme Replacement Therapy is approved in Europe for Fabry and the Fabry Patient Support Group is amalgamated with the MPS Society.

2002
Christine Lavery is awarded the MBE for services to metabolic diseases. The MPS Society jointly hosts with the French patient association, VML, the International Symposium on Mucopolysaccharide Diseases in Paris, France.

2003
The Society celebrates the start of its 21st birthday year with a tea party at the House of Commons. Enzyme Replacement Therapy for MPS I Hurler Scheie and Scheie disease is launched.

2004
The MPS celebrates the close of its 21st anniversary celebrations with a Tea Party at Downing Street. Lysosomal storage disorders including all MPS and related diseases receive National Specialist Commissioning designation by the Minister of Health. No more “post-code prescribing” or legal challenges in England.

2005
Friends of MPS is launched. Launch of Naglazyme ERT for MPS VI. The MPS Society awards its largest programme grant in its history, £308,000, to fund a research fellow at the University of Manchester to develop the MPS Stem Cell Group working towards therapeutic outcomes for MPS.

2006
The MPS Society moves into MPS House. Elaprase ERT for MPS II is launched.

2007
The MPS Society celebrates its 25th anniversary. The first MPS Awareness Day is launched on 15 May. The Society achieves NHS funded ERT for three children in Scotland.

2008
The MPS Society hosted a 2 day international expert meeting on MPS IVA, Morquio disease.

2009
A Phase I/II clinical trial for ERT in MPS IVA disease is started at three specialist centres in England. The MPS Society becomes a charity registered in Scotland.

2010
A Phase I/II Intrathecal Enzyme replacement Therapy Clinical Trial is started at the Royal Manchester Children’s Hospital. The MPS Society hosted a 2 day international expert meeting on MPS III, Sanfilippo disease. An extension clinical trial with patients from the MPS IVA Phase I/II MPS IVA Clinical Trial started in September 2010.

2011
A Phase I/II intrathecal Enzyme replacement Therapy Clinical trial was started at the Birmingham Children’s Hospital. The MPS IVA phase III/IV continues with adults over the age of 18 years being admitted.

INDEPENDENT AUDITOR'S REPORT TO THE TRUSTEES OF THE MPS SOCIETY

We have audited the financial statements of The Society for Mucopolysaccharide Diseases for the year ended 31 October 2011 which comprise the Statement of Financial Activities, the Balance Sheet and the related notes. The financial reporting framework that has been applied in their preparation is applicable law and United Kingdom Accounting Standards (United kingdom Generally Accepted Accounting Practice).

This report is made solely to the charity's trustees, as a body, in accordance with Sections 43 and 44 of the Charities Act 1993 and regulations made under Section 44 of the Charities and Trustee Investment (Scotland) Act 2005. Our audit work has been undertaken so that we might state to the charity's trustees those matters we are required to state to them in an auditor's report and for no other purpose. To the fullest extent permitted by law, we do not accept or assume responsibility to anyone other than the charity and the charity's trustees as a body, for our audit work, for this report, or for the opinions we have formed.

Respective Responsibilities of Trustees and Auditor

As explained more fully in the Trustees' Responsibilities Statement set out on page 6, the trustees are responsible for the preparation of financial statements which give a true and fair view.

We have been appointed as auditor under section 43 of the Charities Act 1993, section 44(1)(c) of the Charities and Trustee Investment (Scotland) Act 2005 and regulation 8 of the Charities Accounts (Scotland) Regulations 2006 (as amended) and report in accordance with regulations of these Acts. Our responsibility is to audit and express and opinion on the financial statements in accordance with applicable law and International Standards on Auditing (UK and Ireland), those standards require us to comply with the Auditing practices Board's (APB's) Ethical Standards for Auditors.

Scope of the Audit of the Financial Statements

An audit involves obtaining evidence about the amounts and disclosures in the financial statements sufficient to give reasonable assurance that the financial statements are free from material misstatement, whether caused by fraud or error. This includes an assessment of: whether the accounting policies are appropriate to the charity's circumstances and have been consistently applied and adequately disclosed; the reasonableness of significant accounting estimates made by the trustees; and the overall presentation of the financial statements. In addition, we read all the financial and non-financial information in the annual report to identify material inconsistencies with the audited financial statements. If we become aware of any apparent material misstatements or inconsistencies we consider the implication for our report.

Opinion on Financial Statements

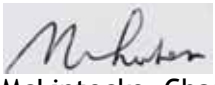
In our opinion the financial statements:

- give a true and fair view of the charity's affairs as at 31 October 2011, and of its incoming resources and application of resources for the year then ended;
- have been properly prepared in accordance with United Kingdom Generally Accepted Accounting Practice; and
- have been prepared in accordance with the requirements of the Charities Act 1993, the Charities and Trustee Investment (Scotland) Act 2005 and regulation 8 of the Charities Accounts (Scotland) Regulations 2006 (as amended).

Matters on which we are required to Report by Exception

We have nothing to report in respect of the following matters where the Charities Act 1993 requires us to report to you if, in our opinion:

- the information given in the Trustees' Annual Report is inconsistent in any material respect with the financial statements; or
- sufficient accounting records have not been kept; or
- the financial statements are not in agreement with the accounting records and returns; or
- we have not received all the information and explanations we require for our audit.



McLintocks, Chartered Accountants and Statutory Auditors, 2 Hilliards Court, Chester Business Park CHESTER CH4 9PX

28 January 2012

McLintocks is eligible to act as an auditor in terms of section 1212 of the Companies Act 2006

STATEMENT OF FINANCIAL ACTIVITIES

Incorporating an income and expenditure account for the year ended 31 October 2011

INCOMING RESOURCES	Notes	Unrestricted funds £	Restricted funds £	Totals 2011 £	Totals 2010 £
Incoming resources from generated funds					
Voluntary income					
Donations		44,150	-	44,150	28,567
Legacies		-	-	-	100,000
Jeans for Genes appeal		-	239,420	239,420	108,010
		44,150	239,420	283,570	236,577
Activities for generating funds		127,953	21,121	149,074	136,281
Investment income		4,428	-	4,428	1,151
Incoming resources from charitable activities					
Grants and research funds receivable	4		529,188	529,188	1,121,350
Clinical studies trial support		-	189,495	189,495	112,702
Conferences		-	106,492	106,492	165,930
Childhood Wood		-	3,744	3,744	2,208
Family support of special events		-	41,286	41,286	20,726
Resource materials and magazine		6,928	-	6,928	11,196
		6,928	870,205	877,133	1,434,112
Other incoming resources		5,356	-	5,356	6,063
Total incoming resources		188,815	1,130,746	1,319,561	1,814,184
RESOURCES EXPENDED					
Costs of generating funds					
Costs of generating voluntary income					
Jeans for Genes appeal		-	-	-	9,066
Fundraising trading: costs of goods sold & other costs		48,994	-	48,994	60,387
Charitable activities	5	20,851	1,003,019	1,023,870	1,103,239
Governance costs	6	33,431	-	33,431	15,399
Other resources expended	7	120,989	-	120,989	104,135
Total resources expended		224,265	1,003,019	1,227,284	1,292,226
NET INCOMING/(OUTGOING) RESOURCES		(35,450)	127,727	92,277	521,958
Total funds brought forward		598,399	957,963	1,556,362	1,034,404
TOTAL FUNDS CARRIED FORWARD		562,949	1,085,690	1,648,639	1,556,362

All amounts above are derived from continuing operations and the Society has no recognised gains or losses other than those passing through the Statement of Financial Activities.

BALANCE SHEET AT 31 OCTOBER 2011

	Note	2011 £	2010 £
FIXED ASSETS			
Tangible Assets for use by the Society	8	771,197	788,708
Investment assets	9	6,100	0
		777,297	788,708
CURRENT ASSETS			
Debtors	10	-	35,000
Short term deposits	11	1,158,038	1,114,609
Cash at bank and in hand		4,959	1,870
		1,162,997	1,151,479
CREDITORS - Amounts falling due within one year	12	94,110	143,332
NET CURRENT ASSETS		1,068,887	1,008,147
TOTAL ASSETS LESS CREDITORS DUE WITHIN ONE YEAR		1,846,184	1,796,855
CREDITORS - Amounts falling due after one year	13	197,545	240,493
NET ASSETS		1,648,639	1,556,362
FUNDS OF THE CHARITY			
Unrestricted funds - general funds	15	325,404	112,881
Unrestricted funds - designated capital fund	15	237,545	485,518
Restricted funds	16	1,085,690	957,963
		1,648,639	1,556,362

Approved on behalf of the Trustees on **28 January 2012**

Notes to the Financial Statements for the year ended 31 October 2011

1. ACCOUNTING POLICIES

Basis of Accounting
The financial statements have been prepared under the Charities Act 1993 and in accordance with applicable accounting standards and the Statement of Recommended Practice on Accounting and Reporting by Charities: the Charities SORP 2005 (Revised May 2008). The financial statements have been prepared under the historical cost convention as modified by the adoption of market value for investments held as fixed assets.

The following is a statement of the principal accounting policies adopted in the preparation of the accounts, which have been consistently applied.

Voluntary Income
Voluntary income, which is received by way of restricted and unrestricted donations, gifts, legacies and special fund raising events, is included in the SOFA in full as soon as it is received.

Grants Receivable and Payable
Grants receivable and payable are included in the SOFA in the year in which they relate. Grants received relating to future projects are dealt with as grants received in advance and are carried forward as a creditor in the balance sheet at the year end.

Jeans for Genes
In the five year agreement signed by the four partner charities in March 2006 at least 60% of the funds received must be spent on genetic research, with the remaining funds being used to support those suffering from genetic diseases. The majority of the research grants awarded by MPS are of three or more years in duration and paid in arrears; as a consequence the Society has considerable reserves allocated to Jeans for Genes research grants for which the monies have not yet been drawn down.

Restricted and Unrestricted Funds
The financial statements distinguish between restricted and unrestricted funds. The former is received from donors and is subject to restrictions on the purposes for which they may be used. Unrestricted funds are those where there are no externally imposed restrictions. They include those funds freely available to the charity for expenditure or appropriation to reserves for internally designated purposes, such as research programmes.

Donations in Kind
The MPS Society appreciates the level of active support it receives from volunteers who spend a considerable amount of time supporting our objectives, fund raising and administering our activities. Without this voluntary support the MPS Society would incur considerable additional expense. It is not considered practicable for the purposes of these financial statements to put a monetary value on this support; the Trustees can but offer their heartfelt gratitude to all its volunteers and supporters. Gifts and intangible income that can be quantified are recognised in the SOFA.

Investment Income
Investment income comprises building society and bank interest receivable. This is included in the income and expenditure account in the year in which it is received.

Expenditure
Expenditure is included in the SOFA on an accruals basis. Expenditure on discretionary research programmes is charged to the SOFA in the year in which the expenditure is incurred. All items containing the Society's logo, which are bought for resale, are written off in the year in which they are purchased. Costs of special events organised by the Society are included in the expenditure section of the income and expenditure account; donations and fund raising receipts relating directly to the staging of such events are included in the income section. 75% of all office overheads are allocated to charitable expenditure to allow for full cost recovery on all direct support costs.

Operating Leases
Rents payable under operating leases are charged to the SOFA on a straight-line basis over the period of the lease.

Pension Scheme
The Society contributes 5% of gross salary to individual employees' pension schemes. Contributions payable to individual pension schemes are charged to the SOFA in the period to which they relate.

Fixed Assets
Expenditure on fixed assets costing over £250 is capitalised. Expenditure on property and property improvement is depreciated on a straight-line basis over 50 years. Expenditure on computers and office furniture is depreciated on a straight-line basis over 3 years. Investment assets are included at market value. Investment assets are valued at open market value at the date of donation and subsequently revalued to open market value at least every 5 years.

Taxation
The Society as a registered charity is not considered to be liable to taxation. VAT incurred is not recoverable and is included in relevant expenditure.

1. ACCOUNTING POLICIES continued

Reserves Policy
The trustees have agreed to maintain unrestricted and undesignated funds at a level that ensures at least one future year's running costs can be sustained.

2. NET INCOMING RESOURCES	**2011 £**	**2010 £**
Net incoming resources for the year is stated after charging:		
Depreciation	17,511	19,380
Auditors remuneration	2,340	1,939
Trustees reimbursed expenses	12,073	10,352
Operating lease rentals	1,792	1,792

All Trustees are reimbursed for their expenses which are necessarily incurred in attending management committee meetings and in performing their duties as Trustees. The Chief Executive of the Society approves the reimbursement of such expenses.

3. STAFF COSTS AND NUMBERS	**2011 £**	**2010 £**
Wages and salaries	350,419	249,150
National Insurance	35,592	27,714
Pension scheme	13,590	10,130
	399,601	286,994

No trustee or related person received any remuneration from the Society.
No employee earned £60,000 pa or more.
The average monthly number of employees during the year, calculated on the basis of full time equivalents, was as follows:

	2011 No	**2010 No**
Director	1	1
Administration	1	1
Advice, information and support services	10	8
	12	10

4. GRANTS AND RESEARCH FUNDS RECEIVABLE

	Unrestricted funds £	Restricted funds £	Total 2011 £	Total 2010 £
Amicus Therapeutics	-	1,910	1,910	1,394
Bailey Thomas	-	10,000	10,000	-
Bernard Sunley Charitable Foundation	-	-	-	10,000
Biomarin Europe	-	38,000	38,000	17,000
Constance Green	-	5,000	5,000	-
DHSS – Northern Ireland	-	1,976	1,976	4,052
DHSS – England	-	50,000	50,000	48,960
Eveson Charitable Trust	-	15,000	15,000	15,000
Genzyme Therapeutics	-	11,250	11,250	15,100
Gill Turner	-	-	-	1,000
Hobson Charity Limited	-	-	-	20,000
Leathersellers Company Charitable Fund	-	3,000	3,000	3,000
Lloyds TSB	-	4,600	4,600	-
MPS research projects	-	-	-	836,485
Advisory Group for National Specialised Services (AGNSS)	-	30,150	30,150	-
Norah Ballah Foundation	-	90,000	90,000	-
Overseas Co-operation Grants	-	3,538	3,538	13,074
Roald Dahl's Marvellous Children's Charity	-	19,335	19,335	28,005
Sir Jules Thorn Charitable Trust	-	600	600	600
Shire Pharmaceuticals Limited	-	140,500	140,500	47,000
Souter Charitable Trust	-	1,000	1,000	-
The Henry Smith Charity	-	30,000	30,000	30,000
The Shauna Gosling Trust	-	59,476	59,476	30,000
The Sheldon Trust	-	5,000	5,000	-
Training grants	-	-	-	180
Walter Guinness Charitable Trust	-	-	-	500
Other	-	8,853	8,853	-
	-	529,188	529,188	1,121,350

5. CHARITABLE ACTIVITIES EXPENDITURE

	Unrestricted funds £	Restricted funds £	Total 2011 £	Total 2010 £
Research and support grants	-	258,023	258,023	425,805
Jeans for Genes joint venture loan	-	-	-	37,500
Clinical studies trial costs	-	209,585	209,585	109,836
Conferences	-	87,884	87,884	180,337
Family support special events	-	40,412	40,412	3,485
Overseas co-operation	-	13,823	13,823	9,664
Grants and donations	4,264	-	4,264	1,178
Childhood Wood	-	6,135	6,135	14,025
Magazine and resource materials	16,587	-	16,587	8,038
Support services	-	348,774	348,774	220,010
Office support costs	-	38,183	38,183	91,372
Training	-	-	-	1,889
MPS Financial Assistance scheme	-	200	200	100
	20,851	1,003,019	1,023,870	1,103,239

6. GOVERNANCE COSTS	Unrestricted funds £	Restricted funds £	Total 2011 £	Total 2010 £
Professional fees	21,358	-	21,358	5,047
Management committee expenses	12,073	-	12,073	10,352
	33,431	-	33,431	15,399

7. OTHER RESOURCES EXPENDED	Unrestricted funds £	Restricted funds £	Total 2011 £	Total 2010 £
Depreciation	17,510	-	17,510	19,380
Mortgage interest and bank charges	6,127	-	6,127	5,624
Office running costs	42,433	-	42,433	22,524
Payroll costs	45,834	-	45,834	50,452
Recruitment and advertising	9,085	-	9,085	6,155
	120,989	-	120,989	104,135

8. TANGIBLE FIXED ASSETS

	Freehold property	Property improvements	Computers and equipment	Office furniture	TOTAL
COST					
At 1 November 2010	824,000	51,524	143,042	20,606	1,039,172
Additions	-	-	-	-	-
At 31 October 2011	824,000	51,524	143,042	20,606	1,039,172
DEPRECIATION					
At 1 November 2010	82,400	4,416	143,042	20,606	250,464
Charge for year	16,480	1,031	-	-	17,511
At 31 October 2011	98,880	5,447	143,042	20,606	267,975
NET BOOK VALUE					
At 31 October 2011	725,120	46,077	-	-	771,197
At 31 October 2010	741,600	47,108	-	-	788,708

All fixed assets are held for charitable purposes.

9. INVESTMENT ASSETS

	£
Carrying value (market value)	
Additions to investments at market value	6,100
At 31 October 2011	6,100

Investment assets comprise donated jewellery.

10. DEBTORS

	2011 £	2010 £
Jeans for Genes appeal	-	35,000
	-	35,000

11. SHORT TERM DEPOSITS

	2011 £	2010 £
Cash on bank deposit	1,133,038	1,089,609
Treasury 4% consolidated stock	25,000	25,000
	1,158,038	1,114,609

12. CREDITORS - Amounts falling due within one year

	2011 £	2010 £
Bank loans and overdraft	64,303	135,426
Trade creditors	899	-
Other creditors	1,637	-
Accruals	27,271	7,906
	94,110	143,332

13. CREDITORS – Amounts falling due after one year

	2011 £	2010 £
Bank Loans and overdraft	197,545	240,493

Bank loans comprise of primarily a mortgage on the premises at Repton Place purchased on 16 December 2005. The mortgage represents approximately 29% of the property cost and is secured on the property.

14. OPERATING LEASE COMMITMENTS

The following operating lease payments are committed to be paid within one year:

	2011 £	2010 £
Expiring:		
Within one year	458	-
Between one and five years	-	1,832
	458	1,832

15. UNRESTRICTED FUNDS

	2011 £	2010 £
General Funds		
At 1 November	112,881	23,529
Net (outgoing)/incoming resources	(35,450)	89,352
Transfers	247,973	-
At 31 October	325,404	112,881
Designated funds - capital projects		
At 1 November	485,518	485,518
Transfers	(247,973)	-
At 31 October	237,545	485,518
	562,949	598,399

16. RESTRICTED FUNDS

	2011 £	2010 £
Jeans for Genes appeal	298,510	167,033
Other grants and donations	787,180	790,930
	1,085,690	957,963

Restricted funds comprise unexpended balances of donations, bequests and grants received from donors which are subject to restrictions on the purposes for which they may be used.

17. ANALYSIS OF NET ASSETS BETWEEN FUNDS

	Tangible fixed assets and investments £	Short term deposits £	Other net current assets £	Long-term liabilities £	Total £
RESTRICTED FUNDS					
Jeans for Genes appeal		298,510			298,510
Other grants and donations	6,100	859,528	(78,448)	-	787,180
	6,100	1,158,038	(78,448)	-	1,085,690
UNRESTRICTED FUNDS	771,197	-	(10,703)	(197,545)	562,949
	777,297	1,158,038	(89,151)	(197,545)	1,648,639