Society for Mucopolysaccharide and Related Diseases

National Registered Charity No. 287034

Summer Newsletter 2005

Mucopolysaccharide and Related Diseases are individually rare; cumulatively affecting 1:25,000 live births. One baby born every eight days will be diagnosed with an MPS or Related Disease. These multi-organ storage diseases cause progressive physical disability and, in many cases, severe degenerative mental deterioration resulting in death in childhood.

What is the Society for Mucopolysaccharide Diseases?

The Society for Mucopolysaccharide Diseases (the MPS Society) is a voluntary support group, founded in 1982, which represents from throughout the UK over 1200 children and adults suffering from MPS and Related Diseases, their families, carers and professionals. It is a registered charity entirely supported by voluntary donations and fundraising and is managed by the members themselves.

What are the aims of the MPS Society?

To act as a support network for those affected by MPS and Related Diseases

To bring about more public awareness of MPS and Related Diseases

To promote and support research into MPS and Related Diseases

How does the Society achieve these aims?

Advocacy Support
Provides help to individuals and families with disability benefits, housing and home adaptations, special educational needs, respite care, specialist equipment and palliative care plans

Telephone Helpline
Includes out of hours listening service

MPS Befriending Network
Puts individuals suffering from MPS and their families in touch with each other

Support to Individuals with MPS
Empowers individuals to gain independent living skills, healthcare support, further education, mobility and accessing their local community

Regional Clinics, Information Days & Conferences
Facilitates eleven regional MPS clinics throughout the UK and information days and conferences in Scotland and Northern Ireland

National & International Conferences
Holds annual conferences and offers individuals and families the opportunity to learn from professionals and each other

Sibling Workshops
Organises specialist activities for siblings who live with or have lived with a brother or sister suffering from an MPS or Related Disease

Information Resources
Publishes specialist disease booklets and other resources including a video

Quarterly Newsletter
Imparts information on disease management, research and members' news

Bereavement Support
Supports individual families bereaved through MPS and the opportunity to plant a tree in the Childhood Wood

Research & Treatment
Funds research that may lead to therapy and treatment for MPS and Related Diseases as well as furthering clinical management for affected children and adults

Cover photograph: Aisha Seedat (MPS IV) and Maryam Ahmed (MPS IV) with their siblings and childcare volunteers at the MPS Conference Weekend.

CARE TODAY HOPE TOMORROW

Newsletter

MPS Society

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Newsletter Deadlines

Autumn	1 Sep 2005
Winter	1 Dec 2005
Spring	1 Mar 2006
Summer	1 Jun 2006

Become a Friend of MPS

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CONTENTS

Welcome to the Summer MPS Newsletter. This is a bumper edition filled with a selection of interesting articles, news from around the world and, of course, stories and photographs from the MPS National Conference which was our biggest and best ever.

I hope you find the following pages informative, emotive, inspirational and motivating. As always, if you feel you would like to contribute - a story, a photograph or a drawing, please contact me at newsletter@mpssociety.co.uk.

Antonia Crofts
Editor

This MPS Newsletter has been funded with the kind support of a grant from the Roald Dahl Foundation.
www.roalddahlfoundation.org

CHIEF EXECUTIVE'S REPORT

Summer 2005

The last three months have flown past in a blaze of activity here at the MPS Society. So much happens so quickly it is hard to know where to start.

After months of planning and excitement the MPS National Conference Weekend was here. Eighty seven affected children and adults, their families and carers came together at the Northampton Hilton Hotel for a weekend of learning, sharing and fun. We were joined by nearly one hundred professionals from the fields of medicine, education and social care all keen to become more knowledgeable about MPS and the effect on those with these diseases and their families.

We were particularly pleased to welcome representatives of many of the Children's Hospices in the UK as well as the organisers of the newly established Spanish MPS Society, Jordi and Mercedes, whose own daughter, six year old Sofia, accompanied them to Northampton. Sofia, who has Sanfilippo Disease took part in the Children's Programme along with 114 other affected children and their brother and sisters. This was the biggest UK MPS Conference yet and, going by the comments and letters of thanks, the best yet.

During the Conference Weekend the Society's Annual General Meeting took place. We are delighted that Paul Sagoo, father of Nancy who lost her life to Mucolipidosis Type II was elected. Wilma Robins and Ann Green were also elected for a further three-year term. During the weekend three parents put themselves forward for co-option to the Management Committee so I hope in the near future there will be some new faces joining the Board of Trustees.

In June we said goodbye to Samantha Vaughan who had been with the Society for the past two years working on Advocacy and Events. Sam liked this area of work so much that she has moved on to work for an events management company and we wish her all the best for the future.

Fundraising, well what can I say? Over the past months our members, their families and friends have raised record sums for the Society. Between the seven London Marathon runners they raised £18,950. Then there was the Farnham Castle James Bond Ball looking to raise a record £30,000.

We send our deepest appreciation to everyone for all their support. It doesn't matter whether you bought one MPS awareness band or 100, raised £5 at a coffee morning or raised thousands in a sponsored event, your support is much appreciated and much needed to enable the MPS Society to continue its valuable work supporting over 1200 individuals and families and funding essential research into MPS, Fabry and other related lysosomal storage diseases.

Christine Lavery
Chief Executive

News from the Management Committee

The Society's Board of Trustees meet regularly. Here are the key issues discussed and agreed at their meetings held on 8-9 April and 3-4 June 2005.

Individual Advocacy

The Chief Executive informed Trustees that she and the Advocacy Events and Information Officer had travelled to Paris to carry out a risk assessment on the hotel and environs for the next Fabry patient meeting in October 2005. Trustees also considered the issues raised over a member living in Wales who had been denied access to Enzyme Replacement Therapy and suffered a life threatening adverse event due to the progressive nature of her disease. Trustees further considered the implications of this and other matters in relation to the continuing involvement of the Society and others at the Cardiff MPS Clinic.

MPS Education and Information Centre

In April 2005 Trustees were informed that suitable new premises had been identified for the Society to purchase and relocate to. The Chairman outlined the details of this building and explained the layout. There was a unanimous decision to acquire the building at a purchase price of £826,000 by securing a mortgage and using the Society's Jeans for Genes Capital Fund of £300,000 restricted for this purpose. The Trustees agreed that the new premises will provide MPS with a secure base from which to extend its support services to members, provide an educational role and support research.

Policies and Governance

The Trustees reviewed and agreed the following policies: Data Protection, Child Protection, Moral and Ethical, Moving and Handling, Care Plans, Funding Medical Research, Death of an MPS Sufferer, Working Away from the Office, Volunteer Carer's and Media Handling. The Trustees, following the completion of a self assessment checklist, highlighted the need for a remuneration committee and this was agreed.

Global Organisation for Lysosomal Storage Disorders

Trustees were informed that the Global Organisation for Lysosomal Storage Disorders has relocated to a new office and vacated the small office they occupied for the past year whilst GOLD established itself as an independent charity.

Jeans for Genes

Following the decision in February 2005 to invite the Chairman of Jeans for Genes to address Trustees at their meeting on 4 June Chris Holroyd, J4G Chairman, updated Trustees on new initiatives for the Jeans for Genes Campaign's 10th Anniversary year including a carol concert at St James' Church, Piccadilly on 16 December 2005. The Society will have 100 tickets to sell costing between £25 and £35. Bob Devine also advised Trustees that he wished to retire as a J4G Trustee and it was agreed unanimously that Ann Green will replace Bob in this capacity.

MPS Accounts for Year Ending 31 October 2004

The year end accounts were considered and approved by the Trustees and duly signed by the Chairman. A letter of representation from the Society to its auditors, McLintock and Partners of Chester, was agreed by Trustees.

Generating Funds

The Trustees approved the Finance Officer's fundraising report and acknowledged her considerable achievements in respect of individual fundraising. The commitment and achievements of the Human Resources and Information Officer in developing the fundraising newsletter were also recognised. It was agreed that due to staff absences and in view of the increased line management responsibilities of the Chief Executive that a Trust and Corporate Fundraiser be appointed for an initial three month period to complete existing applications and seek new funding opportunities.

International Symposium on MPS Diseases 2006

The Chief Executive informed Trustees that the International Symposium 28 June – 1 July 2006 is to be held at the Venice Lido in Italy. It was agreed that before the Society could decide on the feasibility of supporting MPS members to attend, particularly those wishing to take affected children, the Chief Executive should carry out a risk assessment and report back to Trustees.

MPS Research Grants

The Chief Executive advised Trustees that, as agreed, £60,000 from the 'Ollie G' Ball would fund the first year of the research Project 'Development of Gene Expression – Targeted Isoflavone Therapy (GET IT)' being carried out at the University of Gdansk. It was confirmed that letters have been sent to all successful research grant candidates. The Chief Executive also informed Trustees that she had consulted with several major research charities as well as the Association of Medical Research Charities in drawing up the terms of any future Intellectual Property Agreements. Cheryl Pitt attended the meeting on 3 June 2005 to present her proposal for the Society to fund three psychosocial research projects related to MPS and Fabry disease to be spread over a period of five years and commencing 1 April 2006. Following discussions on the projects and the peer review comments the Trustees agreed to fund these projects subject to sufficient income from Jeans for Genes over the coming five years.

Childhood Wood

Trustees approved the draft letter to be sent to all bereaved member families who have a tree in the Childhood Wood advising them of future plans for the management of the Wood.

Announcements

WILLIAM KEOWN TRUST AWARD WINNERS ARE AN EXAMPLE TO US ALL

Photo and article courtesy of Coleraine Times 2005: William Todd from Bushmills receives his Special Certificate of Merit from UTV's Kate Smith, President of the William Keown Trust.

Spare a thought for the five local winners at this year's William Keown Trust Awards for Personal Achievement at the Clandeboye Lodge Hotel recently. Julie Tagg (Coleraine), Margaret McNeilly (Kilrea), William Todd (Bushmills), Alex Watt (Kilrea) and Stephanie Smyth (Castlerock) were all rewarded by the Trust in their 25th anniversary.

The Trust recognises and rewards the accomplishments and courage shown by children and adults with disabilities. At the gala evening, the five courageous winners were presented their awards by William Keown Trust President, Kate Smith of UTV, and local entertainer Gene Fitzpatrick. It was a very special occasion for Julie, Margaret, William, Alex and Stephanie.

William has a progressive growth disorder that has led to a loss of much of his vision, hearing and mobility. He requires overnight oxygen treatment to help him breathe properly. The 24-year-old has never shown the slightest bit of self pity and is unfailingly cheerful and popular with his friends and the medical staff he comes in contact with. He has always shown concern to others especially those with MPS disorders.

William and his family are great supporters of the MPS Society and have helped with fundraising on several occasions. Manchester United is the greatest love in William's life and he can't wait for the start of the new season when he is certain the Red Devils will win the Treble again! William's story is told on page 7.

BBC SONGS OF PRAISE

On 22 September 2005 Songs of Praise will be celebrating the harvest festival and will feature Gordon and Ann Hill whose daughter Louise is 26 years of age and has Sanfilippo Disease. Through interviews with Olympic Triple Jumper, Jonathon Edwards, the progression of Louise's condition will be told.

New Members

Daniel Taylor and Alison Jarman have recently been in contact with the Society. Their daughter, Cody, has a diagnosis of Hurler Disease, MPS I. Cody is 16 months old. The family live in the North West.

Julie has recently been in contact with the Society. Julie has Fabry Disease. Julie is 30 years old and lives in the South West.

Valerie has recently been in contact with the Society. Her son, David, has Fabry Disease. David is 34 years old and lives in the North East.

Deaths

We wish to extend our deepest sympathies to the family and friends of:

Christopher Rees, who suffered from ML III, and who died in hospital on 25 April 2005.

Azeem Farooqui, who suffered from MPS IV, Morquio Disease and who died in hospital on 8 March 2005.

Emily Mazzara, who suffered from MPS I, Hurler Disease and who died in a residential home on 23 April 2005.

William's Story

My name is William Todd and I am 25 years old. I live in County Antrim about 50 miles from Belfast. I was diagnosed with Hurler Scheie Disease when I was three and a half years old. My Mum was concerned about me when I was a young child because my hearing was so poor and I had an unusually large tummy. The Doctor diagnosed an enlarged spleen and liver and this turned out to be Hurler Scheie Disease. I was able to attend Primary School and then went on to the local High School where I had many friends. I enjoy going out for meals, shopping, talking to people and I am a Manchester United Fan.

My medical tests started when I was three and a half years old and since then I have had 13 operations. These included vents inserted in my ears, pollops removed from my nose, a hamstring operation, eye operations, hernia operations, carpal tunnel operation on my hands and a neck fusion operation. I found going to hospitals for operations a nightmare as I hated needles. I had my neck fusion surgery in Manchester Children's Hospital where Dr Ed Wraith looked after me. My Mum and Dad came with me and they had support from the MPS Society.

When I was 21 years old, Dr Wraith told me about Enzyme Replacement Therapy. The hospital were running trials for the therapy and it might be possible for me to take part in the trials. I was quite poorly at that time and my tummy had become very large and uncomfortable. I went to Manchester for tests and was delighted when I was accepted for the trials. I couldn't wait to get started as my only relief from my sore tummy was to lie in my recliner chair.

There were some people on the Enzyme Replacement Therapy and others were on placebo. I was lucky to have received the treatment. I had to travel to Manchester every week from my home in Northern Ireland. I improved gradually over the next three months. Within ten weeks my tummy had reduced in size, I began to feel better, I was more comfortable and my mobility improved. My headaches disappeared, my appetite improved and my long distance sight has improved. I feel more confident now and am a much happier person.

My treatment in Manchester continued for the next two years and in 2003 the therapy came to the City Hospital in Belfast. This made life easier for Mum and me. I am now able to have my treatment in my local hospital since February 2005. I appreciate all the help and support I am given by my Mum, Dad and family. I am also grateful for the help I have received from the medical staff in all the hospitals I have attended. I cannot express my happiness since starting my Enzyme Replacement Therapy and the chance to live a relatively normal life. I would recommend this treatment to anyone who is able to have it as it has changed my life completely.

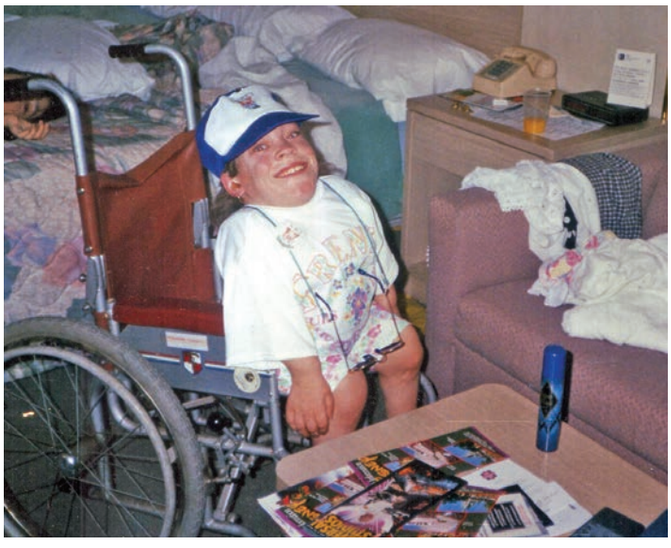
In remembrance... Sam Mullen (MPS IV), by her sister Larraine

In the last MPS newsletter my sister's name was on the remembrance page. I want to tell you a bit about Sam so you can maybe see someone in your mind and not just a name.

Sam was my twin sister (we are not identical). I remember growing up together wearing matching outfits walking around together hand in hand getting up to all sorts of mischief, as you do when you are growing up. We were always known as the twins.

Then when we got to 12 or 13 we started to be very different. We stopped wearing matching outfits (we still got matching outfits but we just made sure we wore them on different days!), we started going to different places but always came together to tell each other what we had done.

Sam got her own place in 1998. She was very happy being independent and she was a very stronged-willed person who knew what she wanted out of life and she made sure she got what she wanted. Sam enjoyed sci-fi programmes like Buffy and Star Trek. She even went to a Star Trek day at Hyde Park and had her photo taken while being beamed into outer space with Captain Kirk!



Sam enjoyed pop music too. I am sure she was Steps' biggest fan what with all the CDs she had of them. I will miss Sam very much and I will miss our little rows and togethers we had. The last thing I can say is I am very proud to have had Sam as my sister.

Introducing... Paul Sagoo

My name is Paul Sagoo. I am 34 years of age and live in Berkshire. I have been a co-opted Trustee for the Society for just over a year. I had taken a keen interest in the Society during the diagnosis of our late daughter, Nancy, who died of ML II over two years ago. This period indisputably proved the most trying in my life and is unlikely to be surpassed.

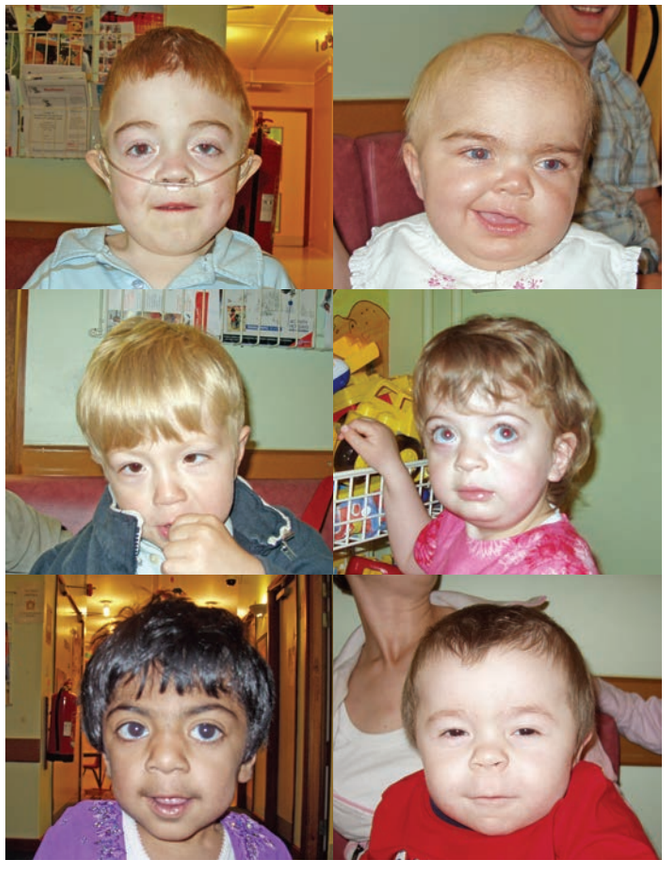
Being a Trustee has offered me the unique opportunity to converse openly with others on the Board of Trustees who have had similar experiences. This has been and continues to be of immense value and reassurance to me. It is a privilege for me to be amongst a group of people who are simply dedicated to upholding the reputation and long term stability of the Society. Of course, the Society's members, staff and volunteers play as equally an important role in the cause and direction of the Society. Moreover and much to my content, I am occasionally reminded that I am the youngest Trustee!

I work for the MoD in corporate affairs and strategy implementation. My job is challenging, exciting and requires me to deliver a variety of projects without recourse to more money and time. The request for extra resource is probably a somewhat familiar conception for most I would think operating in a comparable line of work! Prior to my current role, I worked in the engineering and retail sector.

I am currently doing a Masters in Business Administration, a Proof-reading course and an Events Management course. My wife, Gudiya, often states that I spend too much time working and not enough time accompanying her during her shopping trips!

In my limited spare time, I enjoy playing snooker and am a member of a local club. I also enjoy reading, going to the theatre and eating out. I have an insatiable appetite for exploring different restaurants! I have developed a system for establishing a scorecard which is primarily based on food, ambience and value for money. I am hoping to submit my critique to Harden's UK Restaurant Guide.

MPS Regional Clinics



BMT Clinic by Cheryl Pitt

It was a balmy old day in Manchester on 27 May 2005 for the BMT clinic! Though quite overcast outside the temperature in the Willink reached dizzy heights, and rendered some of you quite hot and bothered as you chased after small children and fought with nappies. As usual, the clinic ran very smoothly, and the biscuits and drinks didn't last long. We had some new faces to coo over and giggle with however, as this was the first clinic for some recently transplanted MPS I patients, and it was good to see all of them looking so happy and well.

It is likely that I will not be attending any more of the BMT clinics, as I have now finished data collection for the BMT project and will be busy with the latter stages of the research. I would therefore like to take this opportunity to thank all the MPS I BMT families for your generous support of our psychosocial research. As you know this is a very important area of research, which we could not have done without your participation. Thank you!

Thanks to Jean Mercer for organising the clinic and arranging refreshments. Thanks also to the Consultants: Dr Wraith, Mr Meadows, and Dr Wynn for their excellent work and a successful clinic. And a final thanks from the bottom of my heart to the Florence Nightingales for the painkillers!

Photos clockwise from top right: Melissa McKie, Rachel Rothwell, Tyler Green, Rubina Jalani, Oliver Gosling, Leighton Barker (all MPS I)

Birmingham MPS Clinic by Sophie Denham

As most of you already know, the Birmingham MPS clinic is now an outreach clinic due to the lack of space and facilities Birmingham Children's Hospital (BCH) could afford the clinic. The first outreach clinic was held on 30 March 2005 at Victoria School, Northfield, South Birmingham.

The clinic went exceptionally well and there was plenty of space including ample parking, which is definitely a bonus for all. The consulting room was extremely large with hoisting facilities and a bed. There was a room allocated to the MPS Society, which afforded us for the first time the opportunity for families to discuss issues in private and in confidence if required.

Verbal feedback at the clinic was positive with most families finding the clinic accessible and easy to find. The only negative point in being away from the hospital is that if there were tests needing to be done, most could not be done at the clinic and an additional appointment would have to be made at the hospital.

If was lovely to meet all of you who attended the clinic and it was wonderful to finally be able to re-establish these clinics for the Birmingham area. We would also like to extend our sincere thanks to all the BCH team, Dr Chakrapani, Dr Hendriksz, Joy Hardy and Louise Simmons and to Dr Ed Wraith for their commitment and dedication to these clinics.

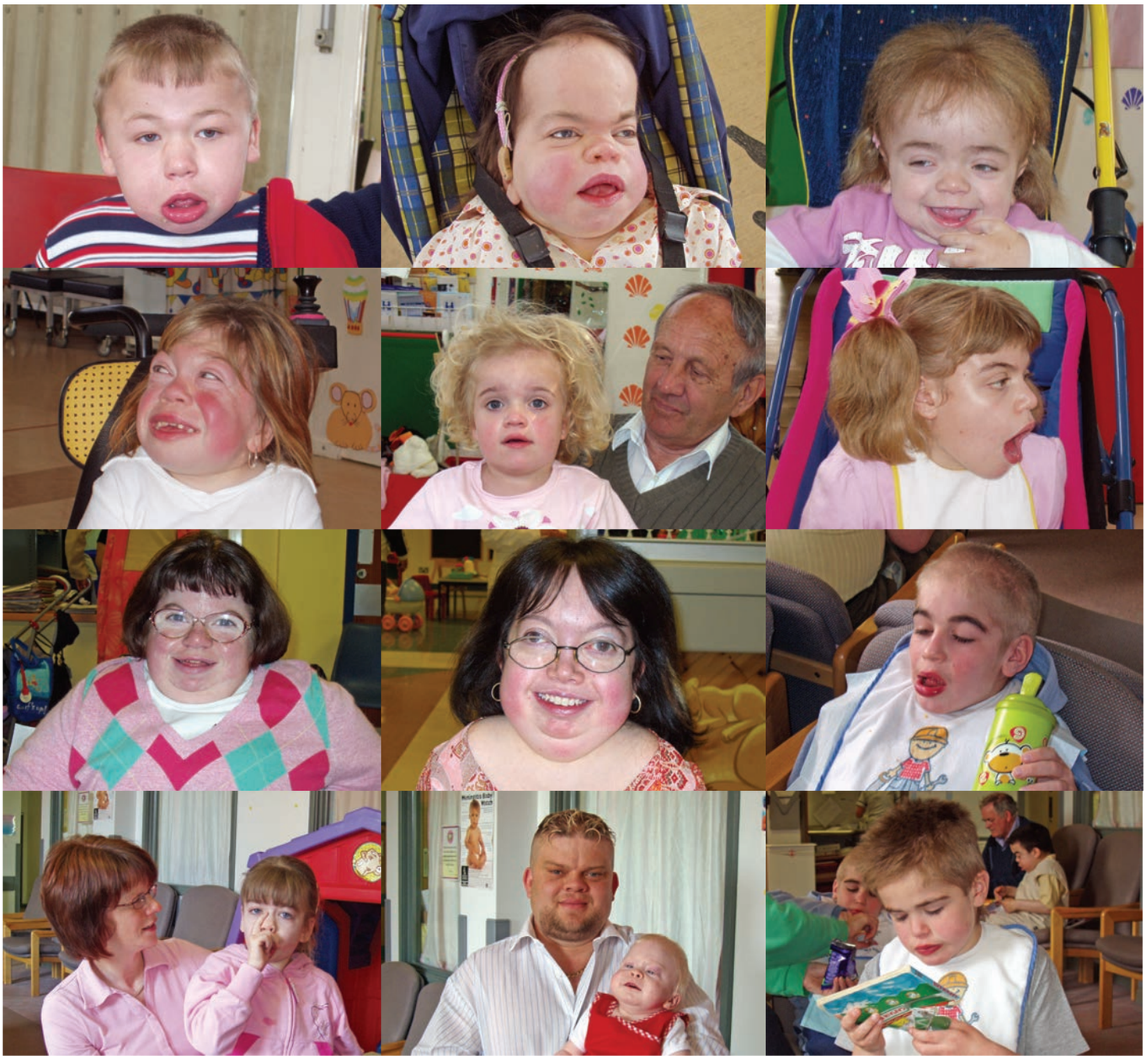
We hope to have a clinic again in October at the same venue and as mentioned in the last newsletter, all queries regarding this clinic need to go to Joy Hardy the clinical nurse specialist, who now co-ordinates the clinics and who will be liasing with individuals and families to secure appointments.

Members' photos clockwise from top right: Jebram Shoukhat (MPS IV), Jack Onion (MPS II), Shabana Shoukhat (MPS IV), Luke Chappel (MPS III), Alex Dearn (MPS I BMT)

Left to right: Louise Alderson, Dr Ed Wraith, Dr Chris Hendriksz, Joy Hardy, Louise Simmons, Dr Anupam Chakrapani

Clinics

Since the last newsletter, clinics have taken place in Bristol (twice), Northern Ireland and Cardiff. The Bristol clinics were held on 5 April 2005 and 7 June 2005 and seemed to run well, keeping to time. As ever, we are very grateful for the continued support afforded to us from Dr Phillip Jardine and Dierdre from Frenchay Hospital who assist us greatly with the co-ordination and administration of the clinics. The Cardiff Clinic was held on 13 April 2005 which necessitated another journey down the M4 to find the hospital with the daffodil painted on the side! This was an extremely busy clinic but I hope that everybody had the opportunity to speak to us if they wanted to. Once again thanks to Graham Shortland and Sue Crownshaw for the facilitation of this clinic. And finally, the flight to Northern Ireland was a pleasant one (no hold up with air traffic control this time!) and the clinic equally ran smoothly and to time. As ever the Irish hospitality was much appreciated and the new location of Antrim Area Hospital once again proved a success (any comments to the contrary would be welcome). Dr Fiona Stewart and Sandra, her secretary need to be thanked for their efforts in supporting us with this clinic, it is much appreciated. And to conclude, this article would not be complete without us extending our grateful thanks to Dr Ed Wraith whose willingness to travel to these clinics enable them to be the success that they are, without him, we would be lost, thank you!



Photos left to right along the rows, starting on the top row: Ben Wilton (MPS II), Daina Green (MPS I), Lauren Graver (MPS I), Aime Oliver (ML II), Alice Coombs (MPS III), Lorren Damen (MPS III), Helen Skidmore (MPS I), Katie Smithers (MPS I), Aaron Doherty (MPS III), Jade McAfee (MPS III), Bridget McDonagh (ML II), Dean Doherty (MPS III)

MPS National Conference 2005

Conference A

by Linda Norfolk

For all of you who were unable to attend the National MPS Conference this year, the programme for Conference A covered a variety of topics such as Carrier Testing for MPS Diseases; Clinical Management of MPS I and II with Neurological Involvement; Managing the Three Clinical Phases of MPS III; Strategies for Managing Challenging Behaviour in MPS; The CNS in MPS Disorders; Recognising Epilepsy in MPS Diseases; The Sleep Study and its Role in Management of Children with MPS Disorders; The Role of Education in an MPS Sufferer's Life and, finally MPS – support for the family "a hospice perspective". All of the presentations were very interesting and informative and there was a good mix of professional and personal perspectives focusing on issues related to Sanfilippo, Hurler and Hunter Disease & ML and Fucosidosis where there is progressive brain disease.

There were four presentations from parents of children with MPS, which were both amusing and moving at the same time, and certainly brought tears to my eyes, along with several other audience members, but which managed to be inspirational as well.

The day ran quite smoothly, but did overrun a little which didn't allow time for a question and answer session at the end, but all in all was very successful.

Conference B

by Cheryl Pitt

I hope you found the Conference B programme on Saturday to be helpful and informative. We had some superb presentations from professionals, patients, and parents of MPS children, which gave us great insight into various issues surrounding bone marrow transplant, orthopaedic implications of MPS diseases, physiotherapy, cultural & educational issues, and social and emotional development.

Three young women affected by MPS diseases, Joanna Wilson, Joanne Evans, and Naomi French, all gave fantastic accounts of their independent lives, from preparing for university, to life at university, to the decision to have orthopaedic surgery. Three parents of children with MPS diseases, Carla Pollock, Maria Murphy, and Bernie Drayne, gave wonderful accounts, from experiences of spinal surgery, to life after BMT, to a child's right to a good education.

Four orthopaedic surgeons provided us with easily digestible accounts of carpal tunnel syndrome, cervical instability and treatment options, the challenges of spinal surgery in patients with MPS diseases, and hip replacement therapy. They were Dr Deborah Eastwood, Mr Alakandy Lichith, Mr Brad Williamson, and Dr Tim Meadows. I hope you will agree that, although orthopaedic issues are complex and difficult to evaluate in terms of necessity and long-term efficacy, the speakers gave honest accounts of their experience in these areas, and addressed as many of your concerns as they could.

Dr Chris Hendriksz gave a very interesting talk about cultural issues and how these can challenge the delivery of a regional clinical MPS service, and Clare Cogan spoke about the pros and cons of direct payments. And finally, Dr Rob Wynn spoke about the evolution of bone marrow transplants and gave an account of where we are today with this treatment; and Cheryl Pitt gave a snapshot of some of the emotional and behavioural issues that may be pertinent in life following bone marrow transplant for those affected by MPS I Hurler disease.

We would like to extend our great thanks to all the speakers that gave presentations in Conference B, and to Dr Rob Wynn for chairing this session and keeping it so beautifully to time.

Conference C

by Clare Cogan

Conference C covered issues related to Fabry Disease and was attended by sufferers, professionals and those interested in learning more about Fabry Disease and its effects.

The meeting was chaired by Dr Atul Mehta, with Dr Patrick Deegan taking over the responsibility in the afternoon to allow Dr Mehta to give his presentation. The group for Conference C was relatively small compared to the other conference groups but this afforded plenty of scope for discussion and group participation bringing a sense of unity. There were a number of excellent varied presentations targeted at all levels such as looking at Biochemical diagnosis in Fabry Disease and understanding genetics, Fabry Disease in both adults and children, Inner ear in Fabry Disease, Strokes & TIA's, the role of the nurse and a family's perspective of ERT at home for their child.

Alistair Kent's presentation on Family Tracing in Genetic Disorders: Your Rights, generated a lot of discussion for both sufferers and professionals, over confidentiality and the responsibilities and dilemmas of professionals in respecting a patient's rights to confidentiality and their responsibility to inform other family members that they may also have Fabry Disease. I am sure many professionals have taken this information away with them to digest and consider.

Conference C in my opinion was very successful and I would like to say a very big thank you to all those who attended and especially to Dr Atul Mehta and Dr Patrick Deegan for chairing the meeting.

Clare Cogan's first MPS Conference

Well, what an experience! From weeks of planning and a few sleepless nights the conference has finally happened. I always thought 'initiations' into a job occurred within a first few weeks of starting, but I can safely say that this event was an important aspect of my learning curve. I can definitely say that I was working on overdrive, from wearing holes in the carpet to and from the crèche room, to liaising with Helen Patterson, our Volunteer Co-ordinator to ensure that our volunteers and children on the outing had a good time, addressing any issues as they arose. It was the most fantastic experience meeting all the families and children and finally being able to put faces to names! My favourite part of the weekend was waiting outside with all the parents waiting for the children to come back from the trips. It made all the hard work worthwhile when you saw the tired, but happy faces of the children and young people emerging from their coach after their trip out and one that I won't forget in a hurry. I am really pleased to hear that people had such a good time and we are already planning for 2007...

Siblings Workshop by Sophie Denham

On Friday afternoon 20 children and young adults attended the siblings workshop. The workshop was split into two groups, with the older children partaking in the 'pots of fun' before going swimming and the younger children being entertained by Mr Crackers before decorating the piece of pottery of their choice. All activities went down really well and there was a great variety of things to chose and decorate from Pots of Fun such as elephants, plates, cups, lizards, sea horses, ducks, trinket boxes and much more.

Mr Crackers was excellent and definitely kept the younger children entertained with his wit, magic tricks and balloon modelling. He even tried to engage the older children by offering to show them how to model their own balloons but this was not seen as being cool and it was the volunteers who were eager to take part in this, naming no names, Alex!

All the children and young adults seemed to have a good time and we would like to extend our thanks to Gillian Groom at Pots of Fun, Mr Crackers and all the volunteers for their time, dedication, fun and laughter. I hope all the siblings who attended the workshop enjoyed themselves as much as I did.

The Creche by Laura Brownie

This was the second MPS conference I have attended as a volunteer, and this time I was saved from my personal hell, riding roller coasters and getting my hair wet on log flumes, and was asked to work in the crèche instead.

Perfect I thought. After a heavy week at work, I can go and crash out on some nice comfy mattresses with the little 'uns and sleep all day. How wrong I was! Following a very late Friday night for the majority of our charges, these energetic toddlers kept us on our toes for 8 hours, with constant rounds of "Baa Baa Black Sheep", Tots TV on repeat and bracing "walks" around the hotel grounds.

But considering all us volunteers had a very early start after a late night, the energy of the kids was completely infectious, and we were soon rolling down hills, putting out "fires" and taking part in crazy food fights which left us all stinking of fish fingers.

Of course we did get a few children missing mummy and daddy, one girl got so distraught that she vomited down Claire's back during a screaming fit, but the vast array of toys, books and videos seemed to distract them (until the nappies needed changing of course!)

What more can I say? All the volunteers were fantastic, and it was a pleasure to spend the weekend with you, and as for the kids – lets just say I found it pretty difficult to give them back on Sunday!

Dear Christine

I felt I had to write to thank you and all the team for such a brilliant conference. The whole weekend was so well organised and very enjoyable and informative. It was lovely to meet so many families and other professionals, also to hear about the current and future therapies.

Many thanks for your hard work.

From Mary Bradshaw (care team member at Little Bridge House Hospice)

The Society would like to thank the following people for their contributions which have been received in many ways, time, support and money towards the MPS conference:

Hilton Hotel Northampton
Ollie G Ball
Chiltern District Council
Moose International
Bartle Family Charitable Trust
Amersham Round Table
Mr Crackers from Busy Bee Entertainments
Gulliver's Land in Milton Keynes
Drayton Manor Park, Northampton
Mega Bowl, Sixfields Leisure Centre
Alec Head Coaches
J & L Coaches
Pots of Gold
Barbara Bonner Walter
Bernie Hephrun

To the MPS Society

Thank you for looking after us this weekend. We really enjoyed ourselves. Isaac loved the days out and the way you organised the carers was very thoughtful. This was our first conference in the UK and was indeed an eye-opener! Everything was planned out so well and precise. Thankyou! We were made to feel very welcome and normal. We are sorry about Isaac's escape on Sunday morning and we didn't plan it, honestly!

Thanks for everything and love to you all.

Andy, Kate and Isaac Hall (MPS II)

Dear MPS Staff

Well done for such a successful conference. All your hard work was much appreciated. We had a fun (and tiring) weekend and look forward to the next time.

All the best from James and Mel Wood

Dear All at MPS

Just a note to thank you all for the great weekend at the Northampton conference this year. I think a good time was had by all that went. We will all have come back home with more knowledge of the conditions talked about and will have added some more people to our list of friends! We had a great gala dinner on Saturday which ended at about 5am for a few! The non-stop supply of food provided by the hotel was fantastic. The weather was good for the children's outings which they thoroughly enjoyed. A big thank you goes to the volunteers who looked after the children, without them we would not have been able to do what we could. Daisy is still talking about Ramina! The teenagers had a great night bowling and they made friends and exchanged email addresses etc.

It was lovely to be able to mix and chat with the doctors socially about the children and everyday normal things, also meeting new assistants and researchers as well. Harrison didn't jump in the fish pond this year which I was pleased about!

We all left looking forward to meeting up again at Alton Towers next year. The group photo (shown below) was taken on Sunday as most were recovering from hangovers! Once again, thank you to the MPS Society for the support you give to all the MPS families. Thankyou!

Madeleine Luckham
Harrison King's mummy (Harrison (MPS III) is pictured on the left)

MPS Conference 2005

Here are a selection of photos from the childrens' outings over the weekend to Drayton Manor Park and Gulliver's Land

Off to University and Facing Independence

Jo Wilson, MPS I, was invited to talk at the MPS Conference. Here is a transcript of her speech.

Good morning and first can I thank the MPS Society for inviting me to speak at your conference. I'm here today, to talk to you all, about going to university and facing independence. Actually the heading up there should say 'I hope I am off to University'. My exams are now all over but I sadly have to wait for the results with some trepidation.

As any parent, carer, or sibling of an MPS child/person will know, the life of anybody with MPS is one where you get to know the inside of several hospitals intimately, it seems one spends half one's life in them. Apart from the regular check ups, it's operations for hernias, carpal tunnel, osteotomy, to name but a few, plus the aftermath of recovery from them. I almost forgot ERT treatment for some lucky ones, for about five hours each week. Actually I didn't forget, let me say how extremely grateful and privileged I am to be receiving ERT, it's certainly changed my life. However, it's not surprising therefore that there isn't a lot of time left for school, especially if you are aiming for higher education. But in spite of this time out of school or restriction in our lives, one or two of us are fortunate enough to get to University.

Up until very recently, I have always relied on my Mum and Dad to sort out my medical situations, help me through my operations and remember my appointments, to get me there on time. But now that I drive, I have started to go for my weekly infusions and appointments on my own. As if that wasn't scary enough, should I get to university, I will have to do it in Southampton, a new and for me, uncharted territory.

In fact when I was preparing for this talk, the realisation of what was to come started to dawn on me, from a normal routine of: Wake myself up in the morning. Make my own breakfast. Make my bed, well that may not happen! Check the schedule (that's if I've actually managed to prepare one) to see if there are any hospital appointments etc for the day. Prepare any tablets such as premed for any hospital visits. Try and fit in some lectures. Make tea. Do some revision, well perhaps! Work out my finances, that is, can I afford to go to the student bar this evening? Go to bed.

To the questions facing a person with MPS, about to live on their own for the first time, such as: How will I cope with managing hospital appointments and university study and a part time job? How will I cope with new doctors, who may never have heard of MPS before, or know very little about it? Although, thanks to ERT, I can now wash and dry my own hair or do my shirt buttons up, but how will I cope with having to face everyday living on my own, still with limiting factors of MPS? How will I cope with silly little things like my wrists being too weak to open food jars? Or cope with fiddly, rather hard tasks with my hands, such as sewing? How will I cope with the physical exhaustion of an energetic university life? All these questions that cannot be answered really until the time comes!

It's now of course you realise just how much you rely on your parents, brothers, sisters, friends etc to get through a day. I know all young people of my age go through a similar process, but most of them are strong and healthy with lots of energy and don't spend a large proportion of their lives waiting in hospitals.

Assuming that the PCT will still be funding my ERT, I've had to ensure that moving away from my local area to Southampton University, that there will be a hospital and doctors and facilities such as bed space, that will cater for my treatment. I can't just assume, with such a rare condition and treatment, that these will be available for me.

Facing independence for the first time is a scary task for any young person in today's society, however, when you add to that having to cope and manage with a condition such as MPS, it makes the whole situation a lot more daunting. There's always the worry of being alone in a new city, being surrounded by new people, who don't really know you or understand about your condition, and worrying about any problems or medical issues that may arise, and for a short while you're completely on your own.

I'm lucky, I can see that my Mum and Dad have been preparing me all my life for this forthcoming independence. Mum has passed on a lot of her cooking skills and her life's experiences about just how to survive out there as a young woman. Dad has taught me how to drive and how that I am as good as anybody else out there and therefore not to be intimidated by anybody. Most of all, they both showed me that you can enjoy life no matter what your starting point as long as you're honest and caring; and "listen to your consultants once in a while". And my Babcia (that's Polish for Grandma), over the past twelve months, has gathered together an impressive array of essentials, pots, pans, kettle, iron, sheets, pillow cases, cutlery to name but a few, all ready for me when I go off into that big wide world out there.

Some people may say that I am unlucky to have MPS; I say how lucky I am to have such a large circle of friends that I have made because of my MPS. It is through these friends and of course my family that hopefully if I should get the right grades and go to university I will have achieved one of my major ambitions in life and one that ultimately will enable me to give a little back to the community that has given me so much. That is why I am hoping to go to university this year to study medicine. So, from a very happy almost medical student, to all the scientists, doctors, nurses, MPS Society, Mum, Dad and anybody else that has helped to get me this far, thank you.

I'm at University

Jo Evans, MPS IV, was invited to talk at the MPS National Conference. Here is a transcript of her speech.

There was never any question about me going to university. Even from the age of 3, I can remember being driven past Glasgow University and announcing "I'm going to go there" but that might have been because it looked like a fairytale castle rather than the idea of it being a prestigious university at that point! (And every Glasgow student is incandescent with rage at the fact that the building wasn't chosen to be 'Hogwarts' in the Harry Potter films – purely because we all want to run amok with cloaks and broomsticks rather then work!).

The University of Glasgow is the second oldest university in Scotland after St. Andrew's and dates from the middle of the fifteenth century, although the current campus dates from the seventeenth century (and yes, we do have ghosts!). Today it has almost 16,000 undergraduate and 4,000 postgraduate students with 5,700 staff. Most of the university's 100 departments are found on the Gilmorehill campus, centred on Sir George Gilbert Scott's neo-Gothic main building. Its spire, added by his son John Oldrid Scott, is a landmark across Glasgow. This campus has more listed buildings than any other and reflects a vast range of styles. Pearce Lodge and the Lion and Unicorn Staircase are relics of the old University, moved stone by stone to the new site. The circular Reading Room is an (accessible!) listed building from the 1930s while the Library, Boyd Orr and Adam Smith buildings reflect post-war fashions in public building design. The new Wolfson Medical School was opened in 2003. The Veterinary School is located three miles away at the Garscube Campus which is also home to the new outdoor sports facilities.

I fell in love with Glasgow as soon as I set foot in it. The ancient surroundings certainly give it an atmosphere, and the Special Needs Department there is first class. I chose not to go to the Open Day but to do a personal visit there instead, just so I'd have a member of staff to show me around (although the access is pretty good, you do sometimes have to take different routes to get to places). Also, as a disabled student, there are other things to consider such as DSA exam requirements. I am living at home at the moment so I didn't have to worry about accommodation but that is another thing that would be discussed at pre-entry. It was also arranged for me to meet with representatives from the subjects I was interested in studying. (Here I should explain that Scottish universities are unique in that all first and second years study three subjects, and then decide what they want to take to Honours in their third year).

I was offered a conditional place to study at Glasgow, and from that moment on I was determined to go there. It's one of the top universities in Scotland and nothing looks better on a CV than an 'ancient' university, as St Andrew's, Glasgow, Aberdeen and Edinburgh are collectively known. I attended the Applicants' Day in March 2004, coming back with a bag full of leaflets – nothing like having a distraction when you've got 2 Highers and an Advanced Higher to study for! But after that day, I had a better idea of what opportunities the University of Glasgow could offer me, and what other subjects were available to me.

I arranged all the access-type things before Fresher's Week started so I'd be organised before the rush of term time started. I was eligible to apply for DSA, which enabled me to have taxi allowance. This really allows me the freedom to do what I want, when I want, and if I didn't have it I think my university experience would be very different. With the rest of this grant I'd ordered a laptop, a mini-disc recorder (to record lectures, not that the lecturers are too keen on it, they think you're going to abuse their copyright and sell the recordings off at exam time) as well as keeping some back for paper, discs, etc. The equipment didn't arrive until November, by which time I'd worked out how to cope without it (but we're not telling anyone that, because I want to keep it). I'm okay with taking notes in lectures, despite my weak wrists. Of course, one of the first things I learnt is that students routinely miss lectures to do more interesting and disreputable activities!

Back to Fresher's Week. Not too sure on how much I should actually say here because (a) my parents are in the room, and (b) I'd hate to spoil the surprise for Jo! But let's just say that the rumours of how med students enjoy themselves are very accurate! What I will say is that because Glasgow has two unions (the QM used to be the womens' union) there is great competition between the two and the freshers actually get a double dosage! For Fresher's Week only, you are allowed in free ('free' meaning you bought your £30 Fresher's Pass!) at both the unions, afterwards you have to choose one to join. I wrote off the GUU right from the start, as it is housed in a listed building, full of stairs with only one lift that doesn't even allow access to every level. The QMU, although less attractive on the outside (it was built in the 1960s) is much better for access (and I know all the drunken medical students are very glad of the ramp when they are too hammered to manage the stairs).

Orientation week is about the more serious side of University – by that, I mean the academic side! All first years matriculate and see their Adviser of Studies to sort out their curriculum for the first year. As I said before, three subjects are compulsory and this often means most people get a shock when it is announced to them that they have to choose two other subjects and they usually need a little bit of help. Most of the clubs and societies have 'introductory meetings' at orientation so you can decide which ones you really want to join. (During Fresher's Fayre, most students have raging hangovers and will sign up for anything, especially as freebies are involved!)

Clubs and societies hold an equal importance to academia, although academic work is always put first. University is as much about having fun, meeting new people and learning how to interact socially as it is about getting a degree. And I've taken full advantage of as much of this social interaction as possible! However, I would add that I have passed all my first year exams, unlike some of my medic friends!

Life with Sanfilippo

Fer Pidden was invited to talk at the MPS National Conference about her daughter Natalie, MPS III. Here is the transcript of her speech.

Hi, I am Fer Pidden and my daughter Natalie has Sanfilippo A disease. To talk about life with Sanfilippo, I feel I ought to start from the beginning and try to give you a glimpse of our lives for the last 24$^{1/2}$ years. It will also be a brief life story of our gorgeous and precious daughter.

On 1 October 1980, we became the proud parents to a baby girl, our first born, Natalie Elif, declared healthy and normal at Cambridge Hospital maternity unit. Everything seemed fine. All the routine check-ups went well apart from one instance when a doctor at the St. Ives clinic was concerned about the size of Natalie's head. She referred her to a paediatrician in Cambridge. The paediatrician told me that she was just a big baby, and that her body would catch up with her head. The worry over the size of her head somehow stayed at the back of my mind. Apart from that, we had a few carefree years enjoying her and making plans for her future.

In the summer of 1983 we had moved to Westbury in Wiltshire, and in September Natalie started going to the local play school and the mother and toddler group. I had become increasingly concerned about her speech not coming along as it should, and the way she was behaving compared to other children around her. My husband had become quite worried about me worrying about our daughter and we went to see our GP. Glue ear was mentioned as a possible cause for the delay in her speech, but her appearance had triggered some textbook information in our GP's memory and he referred us to a paediatrician in RUH in Bath. The lengthy tests and examinations and repeated tests due to errors at the lab, took nearly 6 months. During this time I was at my wits end with worry. I could sense that something was terribly wrong with her, but no one would tell us anything until they had the test results back.

The diagnosis were disclosed to us on 4 June, 1984 when she was 3$^{1/2}$, 21 years ago. We were called to our surgery and were ushered to a side room where we met a doctor we had never seen before. He told us the unpronounceable name of this incomprehensibly devastating disease which meant nothing to us. Gargoylism was also mentioned. As Natalie sat on my husband's knee, we were told of the prognosis that she would not be living beyond 10 to 12 years of age. Then we were left to leave the surgery and find our way home with tears streaming down our faces. I do not know to this date how my husband managed to drive home.

We were devastated and shattered, as our world has collapsed and lives changed completely with this news.

I was unable to eat or sleep for days, and thought that there was no point in carrying on. It would be best to spare Natalie from the future that was waiting for her; I should end her life somehow and hang myself in the garden.

Weeks later the health visitor appeared. She was asking me whether I was ready to know more about this disease, because there was a Society that I could contact. I was furious that no one had bothered to tell us about this straight away. I immediately rang the MPS Society and it felt as if a life-line was thrown to me. We were not alone and there was so many things to ask about. We attended the 2nd MPS Conference in Harrogate. It was heart-breaking to see the other Sanfilippo children in their advanced stages. Still, there was hope and there were things we might do. Bone marrow transplant possibility gave us hope. After several visits and stays at RUH, we managed to get Natalie referred to Great Ormond Street Hospital. There we found a wealth of information and help. Long term grommets were put in her ears.

Life was endless waiting rooms and consultations with specialists while I was trying to contain a child who was getting increasingly hyperactive and unstoppable. Sleepless nights were never ending. We visited Westminster Hospital for bone marrow transplantation. We were desperate for donors. A sibling would have been perfect. I became pregnant. Unfortunately, tests revealed an affected child and a termination followed. It was a double blow; not only were we not able to have a donor, we were also deprived of another child. It was at this time that we were told that bone marrow transplant was not suitable for her because of the blood/brain barrier.

Natalie started going to the local playgroup for children with learning difficulties. In the meantime, things got from bad to worse with the introduction of an incompetent social worker who could not even arrange suitable respite care for her. She thought that her condition could get better with help and perhaps parental neglect had contributed to it. We were told back-handedly that Natalie was put

on a special list to help us! I realized something was wrong and demanded to see our records. This was refused by social services. Our solicitor got involved. This was like rubbing salt into a wound. Finally after a lot of fighting, Natalie's name was removed from the 'at risk register' and I refused to see any social worker and health visitor, banning them from my home for a very long time.

From the shambles of tests, the way the diagnosis was told to us, to the behaviour of the social services, our case has been given as an example of how things should not be done. Thankfully things have changed since then and we have the MPS Society as a watch dog.

Natalie started going to the local school for children with learning difficulties. We found and organised respite care for her through word of mouth, and things looked a bit better.

This pretty little hurricane could do a runner with such speed and spontaneity, as well as having eyes at the back of her head.

At one time she had managed to undo her straps in her buggy and did a disappearing act in our civic hall where I was looking at the wool on sale. That is the only time I called 999 in panic and desperation. I could visualize her under a car. Luckily someone had found her wondering around the supermarket car park and handed her to the police.

Natalie could reach with extreme dexterity any object behind her and sweep surfaces off their contents with one swing of her hand. She would give you an innocent quizzical look wondering why you looked so upset and stressed out. She would chew any thing she could lay her hands on, including clothes and her own hands. We had to devise methods of splints, etc. to keep her hands out of her mouth.

Sleep was something that accidentally happened in my life and I became a master of cat naps. One room was allocated for Natalie to run in while being made safe with soft sofas and a gate. Nappies had to be accepted as part of life after a period of denial. A special high chair was used when feeding her. A special high board was made to fit at the side of her bed to stop her falling out while she was twisting and turning in bed.

During these years of chaos, time had gone by without us realising our wish to have another child. When we had given up hope and I had sold all my baby clothes in aid of MPS, finally I was pregnant again. This in itself was a miracle at the time! I was so superstitious that I did not even tell my husband the news until I had my chorion biopsy results well after three months of pregnancy.

Our son Anthony arrived almost seven years after Natalie to a great welcome and jubilation. His arrival was even heralded in the local press! Now we had the chance of bringing up a normal child. That child who had brought us so much joy is now a great young man by my side helping me with the IT side of my speech. He has been a great help with his sister. I could not have managed to lift her up without him.

Natalie, bit by bit lost the skills she learned. Speech went. She became unable to walk unaided and eventually totally buggy bound. We had endless episodes of ear infections and colds. Epileptic seizures started. She became unable to sit unsupported. Feeding difficulties followed. Natalie had a gastrostomy tube put in her stomach after her 18th birthday at Bristol Children's Hospital. Some weeks afterwards the stoma got infected and I was convinced that this was it! Since then Natalie has been admitted to RUH with chest infection, gastrostomy tube coming out, two false alarms and pneumonia. Last December just before Christmas she was there again. This time it was acute pancreatitis as a result of a rare side effect of Epilim. We were almost saying our farewells when she got better Christmas Eve. She was in hospital nearly a month and longer at home. My fighting girl pulled through again. I think she likes to keep us on our toes!

School finished at 19. Now Natalie goes to a residential home for six young adults as part of her day care and respite care. She is taken from home 8.30am and brought back home 4.15pm. We have a carer in the morning and in the afternoon helping me with her. We had also used children's hospices Little Bridge House and Naomi House in the past, and we are hoping to use the Young Adults Hospice Douglas House in Oxford.

We have managed to get an extension built for Natalie five years ago and it made a tremendous difference to the quality of our lives. There are hoists over her bed and bath. When she is with us in the lounge, we put a little bed on the floor for her. She becomes uncomfortable sitting in her special chair for a length of time. Recently we have a mobile hoist for lifting her off from this bed. Part of the house has become like a hospital ward with all the equipment and the medical supplies!

We have come a long way in the 21 years; devastation, hopelessness, despair, denial, anger, going to unbelievable lengths and trying anything and everything in the hope of helping her, fighting for her welfare with hand and tooth, becoming a professional parent and a learning to be assertive (all the agencies that deal with us learned to have respect for me!), organising funerals in my head were all part of the process.

We will never be able to accept it, but over the years we have learned to live with it. From the fast lane we have reached quiet tracks which wind through laboriously and slowly.

We have learned to accept the unexpected, be prepared for everything, never take a good day for granted and most of all to enjoy every precious moment of this beautiful child's remaining time with us.

I have stopped worrying about tomorrow. I am trying to live today to the full with the mindfulness for tomorrow. My only wish now is that I shall be here to see her life through and that she is free of pain and suffering. I know I shall somehow know the right thing to do when the time comes. I am very grateful that she looks peaceful and is quiet now and we are still rewarded with her warm smiles now and then.

Every time I come to an MPS conference, my heart sinks and feels for the newly diagnosed families. Please have hope and trust life. There is life after diagnosis even though it is not the one you have planned for. Take heart from us. Enjoy your very special children. ■

Bereaved Programme at the Childhood Wood

As part of the MPS Conference Programme, an outing to the Childhood Wood was organised for bereaved parents. Robin Lavery tells us more...

Children's outings? Easy peasy. As a first for MPS family conferences, the Staff Team organised visits to Rufford Abbey and the Childhood Wood for fourteen bereaved parents and grandparents. A great day was had by everyone, but there were some quirky moments which stretched my grey cells more than at any time since I retired from the civil service.

The highlight was what has now become the traditional short and simple ceremony at the Wood. Vivienne Culley read out the names of the particular children we were remembering that day – the children and grandchildren of those on the outing: Thomas Birch, Robert Culley, Gethin Robins, Faye Rowe, Simon Lavery and Jeremy Papworth. Viv also asked everyone to remember all those children and young adults who have lost their lives to MPS and related diseases.

Then Wilma Robins, in her melodious Welsh voice, read Christina Rosetti's poem "Remember." Its power and the sensitivity which Wilma brings to it, never fails to bring a tear or two to everyone's eyes, even to those whose child died many years ago. Nevertheless, the Childhood Wood is a happy place, and a joy to visit. The Wood is becoming more mature and increasingly is a powerful reminder of the children and young adults we celebrate there.

After a 1.5 hour coach drive from the Northampton Hilton, the morning got off to a leisurely coffee break, sitting in the sunshine at Rufford Abbey. Then we were bounced into a serious bit of anatomical observation: metal statues of two naked men pulling at the separate ends of a thick rope, by Robert Bryce Moore, called "Mea Culpa." Well, everyone knew where to look or to avoid gazing, if you see what I mean. Unless you are up to explaining the facts of life, don't take your young children there: as the statues are much bigger than real life there is a lot to explain.

After lunch at the nearby Rose Cottage our coach took us to Sherwood Pines. Individuals chose their favourite walk to our Wood (about 10 minutes). For some reason I decided to take the long way round (ie. I took a wrong path). With young parents-in-law (how young? – I am 61) I power-walked for 20 minutes, not realising that there was a group of five behind me losing touch. We were eventually reunited at the Childhood Wood where Viv and Wilma got down to business.

On the way home our coach broke down with a seriously leaking radiator. Martin, our cheerful driver, managed to pull off the M6 and we parked relatively safely on a slip road leading off the motorway at junction 22. Martin then tried to fix the problem, with Ken Ballard, Peter Robins and Gordon Rowe lending useful/useless advice, whilst others looked for a decent place to have a wee or a smoke. Martin also tried by mobile 'phone to summon up another coach for our rescue. As I had once been stranded for hours in a broken coach, I set in train Plan B which was to ring the hotel and ask for three drivers to be put on standby to come up by car and rescue us. I did this almost as soon as we realised the coach couldn't go on, and before potential drivers had had time to prop up the hotel bar. Were we glad to see Ann Green, Dave Peach and Will Cogan? Not half we were, and for Gina's co-ordination back at base.

I guess the Office will want to try this event again. If so, the coach should be loaded with nappy buckets and a case of Stella Artois, and some port and lemon. In the event of a breakdown, some long range electric wheelchairs should be loaded too, provided everyone is clear how they fold and unfold.

Photo bottom left: Peter and Wilma Robins, Andrew Culley; Photo top right: Vivienne Culley and Ken Ballard

Dear MPS Society

Just a few words to say thank you for the weekend. As you all know, when the conference was approaching I was having doubts about coming as it was my first conference since losing Thomas. When we arrived at the hotel I had butterflies in my stomach and didn't think I could go in, but there were only a few families already there at the time which I think helped and the staff were very good so I started to feel at ease.

It was lovely to see families that we hadn't seen for a while and nice to catch up with some of the professionals. Especially being able to wind one up by wearing my Arsenal shirt! It was also good seeing volunteers who used to take Thomas on the trips. They all do such a good job.

I am so glad that I went to the conference, being able to speak about my son and also see other boys like Connor and Craig. I am looking forward to the next one and would like to thank you all for the support you give to us.

Linda and Sharon (mother of Thomas, MPS II)

Childhood Wood

The Childhood Wood is now 12 years old and for those who have been fortunate to pay a visit over the past year or so, you will have seen that many of the trees planted in the early years are branching out.

The type of oak tree planted does not grow taller than 8-9 feet before forming a canopy, and it is now almost impossible to identify some of the trees planted in memory of loved ones who have lost their lives to MPS and related diseases. With this in mind and working on the advice of the Forestry Commission, the Society has laid a new pathway through the Childhood Wood and over time wooden woodland animals and a picnic area will be introduced.

With the growth of the Childhood Wood, these new features and the need to uphold the ecological and environmental ethos of the Wood and its surrounding area, the individual plaques will be replaced in the near future by a new information and remembrance board that will be physically accessible to all visitors to the Childhood Wood.

Work on the Childhood Wood is underway and we expect it to be completed by the late Autumn when the next planting takes place.

If you are planning a visit to the Childhood Wood in the near future please contact us if you would like more information.
01494 434156

Why volunteering to help at the MPS National Conference has (probably) changed my life

By Paul Tilston

As a somewhat hard-bitten and slightly cynical professional sales person, it generally takes a lot to move me towards genuine expressions of enthusiasm for anything that isn't directly related to paying off my mortgage. So when I was volunteered by a powerful combination of my wife and MPS Outings Co-ordinator Helen Patterson (and anyone who has met Helen for more than two minutes will know I have chosen the word "powerful" carefully) to help at the MPS National Conference, I have to confess I did so with a mixture of quiet resignation and trepidation at what might lie in store for me over the weekend.

When I then heard that my main tasks would involve looking after some of the MPS-affected children themselves along with their more mobile siblings, sweat was in serious danger of overwhelming my brow. As my wife will tell you, I have found that looking after my own five little cherubs, two of whom suffer with mild autistic conditions, has proven on occasion to be "something of a challenge" as a professional psychologist might euphemistically phrase it. How on earth was I going to cope with a bunch of kids I didn't know, some of whom were suffering from a medical condition I'd never met before and didn't understand, as well as a few potentially lively teenagers?

After an extremely well-run and informative 2-hour training and briefing session in the leafy Buckinghamshire village of Chalfont St. Peter (best known as the current home town of Mr. and Mrs. Ozzy Osbourne) for which particular thanks have to go to Helen, Clare Cogan and Sophie Denham, some of my initial fears were allayed, although I did wonder when exactly I would find the time to read through the vast reams of background material they decided to dump on me.

Inevitably the morning of Friday July 1st eventually arrived, and having gleaned what I could from a few hurried minutes over the previous two weeks looking through the contents of my Volunteer Pack on the Chiltern Line train between London Marylebone and Amersham, I lugged my backpack up the hill to the MPS Society HQ (given it happens to be at the top of my road, I suppose I was destined to have something to do with it eventually). Straight away I was greeted with an enthusiastic "Hello Paul" from the Chief Executive, Christine Lavery, herself, and that really set the tone for the whole weekend. In business, if the CEO of a company comes to speak to a humble part-time worker of his or her own free will, and gets that person's name right first time, you can be fairly certain they're actively involved and interested in every aspect of how that organisation functions, including the welfare of the people they work with. I knew from that moment on that this was going to be a good experience.

Having taken the opportunity to show off my muscular prowess in front of a dozen or so very presentable young ladies by loading countless heavy boxes and two televisions onto the coach, much to our driver Charlie's obvious irritation, I eventually boarded for the 90-minute run up to Northampton. Again the friendliness and openness of the MPS staff greatly impressed me as we chatted during the journey. It was obvious that Volunteers like me weren't there just to make up the numbers or do the "less important" jobs - we were clearly seen as a vital part of the smooth running of the weekend.

Once we'd arrived and offloaded at the Hilton hotel itself, and after lunch, a cup of tea and a ridiculously complicated diversion to the nearby Holiday Inn Express to offload a number of the Volunteers' own luggage, I was deployed for my first real task: looking after a handful of the teenage siblings during "Pots of Fun" and swimming. Now I have to say that I personally wouldn't have thought that painting a few china cups, plates and animal figures could capture the attention of an energetic 15-year old boy, but I very quickly learned that these youngsters seem to have an added sense of the importance of accommodating and respecting the needs of others, as they worked quietly and diligently and were beautifully behaved. Each of them spoke with genuine affection about their MPS-affected siblings, and if there can be justifiably be said to be any benefits to the prospect of witnessing the debilitating effects of mucopolysaccharide diseases at first hand, then this must surely be one of the main ones.

Later in the evening, I met the Obaidi family for the first time for whom I was to have a special responsibility over the weekend, including 15-year old Marwan, a wheelchair bound Morquio sufferer. I was warmly greeted by all 5 family members, and quickly built up a rapport with Marwan through time-honoured topics of male-bonding topics of conversation such as football and mobile phones. It was very clear that this lad had a sharp mind and a wicked sense of humour, and was pretty determined not to let his disabilities get in the way of him having a good time!

Saturday morning saw us up and out very early to Drayton Manor Theme Park complete with big dippers and a 360 degree revolving beast of a ride rather threateningly named

"Maelstrom" and the beginning of the part of the weekend I had been dreading the most. How was I going to keep an 18-year old young man and an excitable10-year old girl entertained, as well as ensuring that we gave Marwan all the fun he was looking for without exacerbating his injuries? (like many Morquio sufferers he has recently undergone a spine fusion operation). I needn't have worried. Mustafa was clearly protective of his younger brother - almost too protective for an 18-year-old - (aren't they supposed to be irresponsible at that age?) and also very well used to dealing with his brother's urge to test his disabilities to the limit, and little Malak was always respectful of the need to make allowances for her sibling. Everyone had a great time.

Saturday evening took us to the Megabowl up by the Sixfields football stadium, home of Northampton Town, for tenpin bowling. Here the challenge was to keep a handle on any boisterous behaviour and watch out for tantrums and the like. Again, no concern was necessary. Everyone behaved superbly, and in my group, the eldest and most able-bodied players, namely Mustafa and myself, were soundly beaten by Marwan and his 14-year old friend, fellow Morquio sufferer Sam Wheeler.

Sunday morning saw us venture just up the road to Gulliver's Land Theme Park. In truth this was pitched more at the younger children, and at first it looked like I would struggle to find anything that held Marwan's interest for very long. Eventually, though, we found a rollercoaster which were just right for Marwan. Anything with too steep a descent would have put an unacceptable strain on his back and thrown him too close to the guard rail at the front of our car, accentuating his breathing difficulties. This ride had just the right combination of thrills and manageability - so much so that Marwan nagged me incessantly to repeat the experience. After 4 times round, I decided that enough was enough, and after a couple of rides in a boat which swung 180 degrees up in the air, we made our way giddily back to the coach, where we were duly delivered back to the hotel for the final lunch of the Conference and the inevitable "Goodbye".

Marwan (MPS IV)

So why the starry-eyed description of this weekend as a life-changing experience? Well there are far too many individual impressions for me too reveal here, let alone try and place them in a logical and coherent format. These range from the verbal tennis match I had with 15 year-old SF sufferer Zara Watson on the coach back from Drayton Park, which mainly consisted of one person saying "I'll sort you out" and the other one responding "No, I'll sort you out" (I think she was flirting with me) to listening to 18 year-old Joanna Wilson, herself an MPS sufferer and the daughter of the Society's Chairman Barry, revealing her long-term future plans and helping out as a fully-fledged Volunteer. All I can say is I learned more about teamwork, humility, love and hope in a little over 48 hours than I have done in a long while previously.

Thank you to everyone who allowed me to share in this experience, and I think you can safely say I'll be delighted to be invited back again. Now what's this I hear about Venice...?

May we express to you our extreme thanks for the superb weekend that we experienced at the 2005 MPS Society conference. It was our second conference; the first we didn't bring Jasmin and just came for the day, but this time Jasmin joined us for the complete weekend.

The event was excellent. Massive congratulations to you on your organisation. We gained a lot from the professional lectures and shed plenty of tears during the family speeches. A 'huge' well done to all of the terribly brave parents who shared their lives of worries and woes with us all. It was a great relief not to having to be self-conscious of Jasmin running riot and yelling, as being in the company of other MPS families we were all in the same boat. It was a real community atmosphere.

The thought of Jasmin going on trips and having child-care unknown to us was a concern, but this was immediately reassured on the sensibly arranged meeting Friday evening with our lovely carer Elizabeth Bartlett, who did a marvellous job entertaining Jasmin and actually managed to get her to sleep Saturday evening! Thank you Elizabeth.

The Hilton Hotel did an exceptional job in all aspects over the weekend, which was tremendously busy for them. After 12 years as a Conference & Events Manager myself (Maria), I can only applaud their efficiency and 'un-renowned to hotels' high staffing levels. The catering was excellent, especially the varied food for the children - it was great to see traditional cuisine as appose to 'kids meals' i.e. burgers and chips.

The gala dinner was super, our winning on the raffle obviously a bonus! It's just a shame that the dancing didn't start until 10.30pm leaving us just 30 minutes before we had to return to our children at 11pm. Maybe next time they'd consider staying until mid-night so that we could all enjoy a boogie! We came away richer in knowledge and friendships and thank you again for this.

Maria, Dave & Jasmin Heap (MPS III)

Hoists and lifting equipment

For most people, getting from Point A to Point B doesn't present too many intractable problems, even if the two points are thousands of miles apart and the process requires a number of lifting operations, from the escalators and lifts at the airport, to the aircraft itself.

On a more mundane but no less important level, the process of getting from the bed, say, to a wheelchair, can present us with a range of lifting options as well as the potential to get it wrong. And it isn't just the welfare of the person who may require assistance to move from one spot to another that we need to consider. Lifting, moving and handling can result in back pain or other injuries for the professional or personal assistant, especially if they've been doing it for years.

The Carers Act 1996 allows 'carers' or personal assistants to request an assessment of their needs from the social services. It is no longer considered reasonable for people to risk injury to themselves or to develop chronic back problems through assisting others. This makes sense, but it does mean that sometimes there is a bit of confusion as to who can lift what and when. It is therefore advisable that anyone who is likely to find themselves in a situation where they might be required to do some lifting and moving is aware of their physical as well as legal limitations.

There is a raft of legislation and guidance around lifting, moving and handling that all employers should be aware of: Manual Handling Operations Regulations, Management of Health and Safety at Work, etc. They suggest that employees should not undertake any manual handling operations that could lead to risk of injury, or that if they do, they should use the appropriate equipment.

There are obviously cost implications here. Mechanical or electric/battery powered hoists are not cheap, but then neither is the cost to an employer of staff off with back injury. Keen students of day time television will be familiar with companies that are always on the lookout for people that may have injured themselves at work, so a correct approach and policy to manual handling could save an employer a great deal of money in litigation.

As a rule of thumb, in the domestic or healthcare environment, manual lifting of people by staff or carers should be a last resort (Disability Information Trust: Equipment for Disabled People). Ideally and as far as possible, people should move or transfer themselves, whether totally independently or with the aid of an assistant or a piece of equipment, like a hoist, transfer board, or standing aid. Of course, not everyone will have the option to move themselves about on their own. Where someone is likely to be assisting, the following points should be considered including the weight and size of the person to be lifted, their strength and balance and whether they are able to bear weight and pain and other medical considerations that may be relevant.

Where a hoist is to be used, there are a few specific considerations: Get the right hoist! There are so many available, all offering different features. It is important to make sure everyone who is likely to use the hoist is sufficiently trained, including knowledge of the sling. The hoist, as well as all training, must be acceptable to the user.

Beyond these general points, bear in mind also the following questions: Is the hoist for a specific task, like getting from wheelchair to toilet? Is the hoist for independent use? Only overhead hoists are appropriate in this case. How high/low does the hoist need to go? Will it be used often or infrequently? If it is for the home, do you have adequate room and storage? Will the hoist move easily across the floor surface? Will the design of the hoist allow it to go under beds, chairs, etc? Can it be easily charged? Can it be easily taken out of the home on holiday or elsewhere?

Photographs and article, written by Chris Goddard, have been reproduced courtesy of Ability Link Magazine (sales@ability-link.com)

The standard mobile hoist comes in two basic varieties, the small one and the big one! In the former the maximum lifting capacity is around 25 stone, whereas the larger hoists will lift up to 40 stone. These latter generally are for use in hospitals and residential homes where there is more space to move around and store such large items.

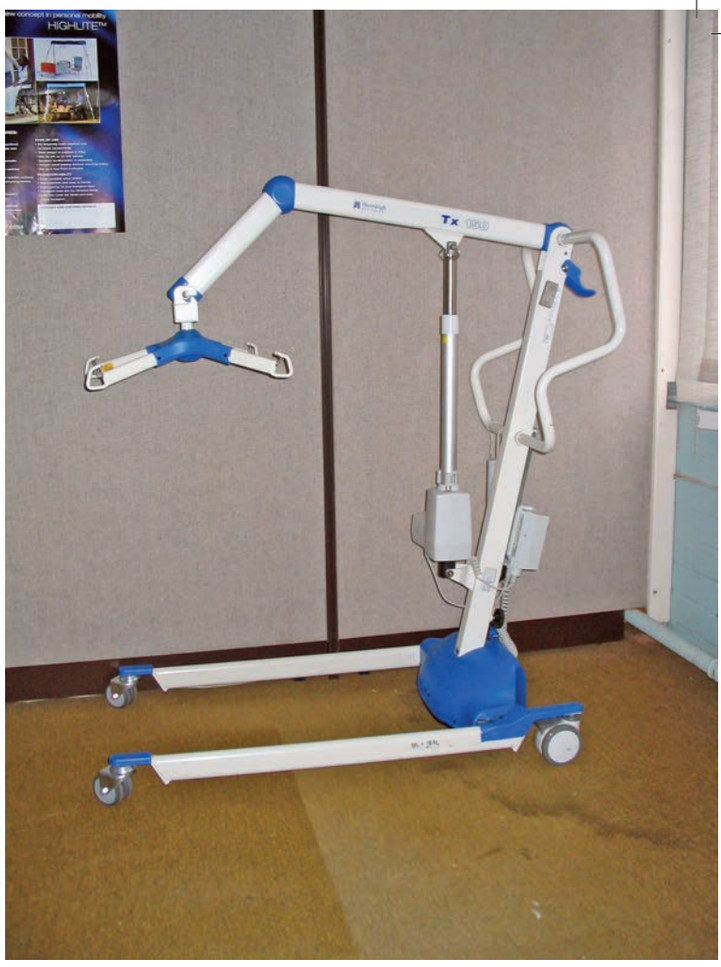
The standing/toileting hoists are designed for people who can bear a certain amount of weight through their legs. With the user in the semi-standing position, a PA, family member or other healthcare professional can more easily adjust the clothing. The sling goes under the arms and round the back, so is not ideal if the user has pain in that area, or perhaps a skin condition that chaffing and rubbing could exacerbate. Seat hoists are primarily to assist with transfer to the bath or, in some cases where the seat has a commode aperture, to the toilet, and are operated by a PA or healthcare worker, they cannot be used independently. They don't use a sling, so don't offer quite so much support, but for the specific purpose of bathing they do tend to be quicker and easier to use.

Overhead tracking hoists can be operated by the user or a personal assistant. Many today come with remote, infrared controls, and with turntables and junction points, it is possible for the user to travel long distances throughout the house and from room to room quite independently. The main advantage of such a hoist is that it takes up very little room and is relatively easy to use, although it is still as important to make sure the correct sling is used and that it is fitted properly.

There are 'A' frame gantry hoists on the market, like the free standing gantry hoist from Likorall, which are a good option if it isn't possible to make structural changes to the building. Moving them around the house, though, is not always a straightforward procedure, so although they are portable, they'll tend to stay put in one room or hospital ward.

A hoist is only as good as the sling you use with it, and it almost goes without saying that you should use only the sling recommended by the hoist manufacturer. There are slings that can be used with different hoists in the same range, but generally you should not swap them around, as they might not fit properly and could therefore result in injury.

The main type of sling are hammock slings, toileting slings and quick fit, divided leg slings. Hammock slings are single pieces of fabric offering full body support. Some are available with commode aperture, although these are not always easy to position precisely. The principle disadvantage of the hammock sling is that it isn't that easy to put on or take off because the person essentially needs to be rolled onto it.



The design of toileting slings allows easy access to clothing and to the toilet; though they don't offer full support so won't be suitable for some users.

Quick fit, divided leg slings provide support for each limb and can be put on or taken off without too much trouble, often by the user. They offer a choice of leg positions, and with the leg bands crossed over, it is very difficult to fall out of them.

As with all pieces of equipment, there are many points to consider before going out and acquiring a hoist. Remember to discuss with a healthcare advisor the availability of grants, or if the hoist can be supplied through social services. Put the hoist thoroughly through its paces, testing it in all the situations you're likely to use it. It is probably a good idea to have your health worker revisit after a few weeks to make sure the hoist is being used properly, the slings are correctly fitted and that your lifting and moving needs are being adequately and comfortably met.

To find out which hoist will suit you, contact your local Disabled Living Centre for impartial advice and information on the huge range of aids available. ■

Manufacturers and Suppliers

Westholme Ltd 01422 260011 **www.westholme.co.uk**
Invacare Ltd 01656 753200 **www.invacare.co.uk**
Huntleigh Healthcare Ltd 01582 745700 **www.huntleigh-healthcare.co.uk**
Dolphin Mobility 01276 856060 **www.dolphinlifts.co.uk**

Wheelchair Passenger Vehicles

There are many disabled people who choose or wish to travel as a passenger. It may be simply that they do not wish to learn to drive, but often it will be due to the nature or extent of their disability.

VW Caravelle with electric lift added to side door
Photo courtesy of Invatravel Conversions

Many can transfer to the seat of a standard vehicle, especially when aids such as swivel seats are fitted. However, for those wheelchair users who find getting in and out of the car really difficult the solution may be a wheelchair passenger vehicle, or WPV. These are also called wheelchair accessible vehicles, or WAVs.

They are designed to carry the disabled passenger seated in their wheelchair in the vehicle. Often thought of as being just for adult passengers, they are increasingly being used for children, where they are either too big to be safely lifted by a parent, or need very specialised seating, which may be easier to achieve as part of their wheelchair.

Access is usually through the rear doors via a ramp. The ramps are usually manually operated, but should be constructed so that they are easy to lower and unfold. A top hinged door has the advantage of providing some extra cover in bad weather, but may be difficult in a garage or similar place where height is limited. Sometimes access is via a side door and may utilise a powered lift. Remember that whatever type of access you choose, you do need ample space where you park to use the ramp or lift and manoeuvre the wheelchair.

Some WPVs have lowering suspension making the ramp less steep or shorter, but this does add to the cost and complexity. Some vehicles are fitted with a motorised winch to pull the wheelchair up the ramp, but many users will find this unnecessary, especially if the wheelchair is motorised or the pusher is fit.

The wheelchair user needs to be firmly strapped in and the wheelchair firmly secured. Without this they could, in a serious accident, fly through the front windscreen killing the driver and other passengers as they do so.

Key points to consider when choosing a WPV are as follows: Is the system for loading the wheelchair suitable for the users? Is the open door height and the internal height sufficient for the wheelchair user to sit comfortably? Can the wheelchair user enjoy a reasonable view ahead and to the sides from their seated position? Will you be able to load and unload the vehicle safely in the places you regularly use, such as home and work? Are there any height restrictions where you need to use the vehicle, which are lower than the roof of the vehicle? Do you need to choose automatic transmission or are all your drivers licensed and happy to use a manual gearbox?

The design of WPVs has improved enormously in recent years. This has been helped by better standards within the industry, but is also due to the introduction of multi-passenger vehicles (MPVs) and boxy shapes, such as the Renault Kangoo and Citroen Berlingo.

There is an enormous range of models to choose from. At the upper end of the range come vehicles such as the Chrysler Grand Voyager Stow'n'Go that has recently become available converted by Gowrings Mobility.

Ensuring your vehicle is safe

There is little regulation over the conversion of vehicles for disabled people. As the creation of a WPV usually requires major structural changes, it is vitally important that the finished vehicle is at least as strong as the original vehicle that was designed by the manufacturer and crash tested.

Customers using the Motability contract hire scheme can be pretty certain that the workmanship is satisfactory. Motability has an excellent technical team and regularly checks the engineering standards of conversions done for them.

For customers purchasing without using Motability it is less easy and it is unlikely that you would want to employ a consultant engineer to check the work before delivery.

Special Edition Renault Kangoo
Photo courtesy of Gowrings Mobility

Citroen Berlingo
Photo courtesy of Gowrings Mobility

One way of ensuring the vehicle's safety is to use a company that is a member of WAVCA. The Wheelchair Accessible Vehicle Converters' Association, or WAVCA, was set up by five key companies in the industry, with the aim of ensuring converted vehicles are structurally safe and suitable for the disabled passenger.

Companies who are members of WAVCA have to meet criteria that include providing a 3 year warranty, properly tested seats, seatbelts and wheelchair restraints, a commitment to customer service, and a consultative sales approach rather than hard-sell. However, clearly not all companies are members of WAVCA, but does this mean that other converted vehicles are less safe?

Certainly not, according to Dave Reid of Invatravel Conversions, who have been adapting the Volkswagen Caravelle people carrier for 21 years. He explains, 'We convert vehicles without structurally altering the vehicle. We are not cutting floors out or anything like that, as the majority of WAVCA members are doing.'

In fact, the vast majority of vehicle converting companies are not members of WAVCA. Dave's main reason for not getting involved is that he feels WAVCA is geared more towards vehicles that have had their interiors adapted. He explains, 'That is not a route we have been down, they are doing different things to us.'

A major benefit of WAVCA is that the vehicles are tested, which puts a safety system in place. However, Dave is quick to point out that all of Invatravel's conversions are approved by Volkswagen, who manufacture the original vehicle, and original vehicle structure is not altered by the adaptations. 'We are bolting items to the vehicle, rather than cutting holes in it,'says Dave, 'A lift can be bolted to the underside of the Caravelle if it is a side lift, or on the floor inside the rear tail for rear access.'

Because the Caravelle comes out of a factory as a luxurious people carrier, all that Invatravel need to do is take a seat out to make room for the wheelchair user. This purpose built people carrier is definitely at the luxurious end of the converted vehicle spectrum and as a result, does not come cheap. Prices are typically in the range of £25-32,000, but can be up to £40,000 if extras are added. ■

This article, written by Douglas Campbell, has been reproduced courtesy of Ability Link Magazine (sales@ability-link.com)

Points to remember

Remember that the vehicle manufacturer may invalidate the original warranty if the conversion work is unapproved. So, do make sure that your supplier guarantees that the original warranty is unaffected. Do also check that you can arrange routine maintenance locally and that they will supply spare parts on demand.

Any WPV supplier should be happy to arrange a demonstration at your home without any charge or obligation. Make sure you take this opportunity to thoroughly check that the vehicle on offer meets your needs. If you choose a different make or model insist on having a demonstration of that model before ordering. It could be a very expensive mistake if you get it wrong.

If cost is an issue, as it so often can be, it is worth knowing that the prices for WPVs on the Motability contract hire scheme have fallen dramatically and are now much more affordable. Advance rentals on the more basic vehicles on a five year contract are now well under £1,000. The company is also able to make grants available to some customers requiring more expensive vehicles.

WAVCA members are as follows:

Brotherwood Automobility	01935 872 603
Constables	01323 767 574
Gowrings Mobility	0845 608 8020
Lewis Reed	0845 345 0127
Widnes Car Centre	0151 420 2000

Remember that good advice is vital before buying any vehicle adaptation or aid. If a substantial amount is being spent it must make sense to get that advice before placing the order. Mobility Centres are located in many parts of the UK to offer advice that is totally independent of commercial suppliers. For details of local Mobility Centres contact the Forum of Mobility Centres on 08700 434700 or go to www.mobility-centres.org.uk

For general advice on vehicles and disabled people contact
The Disabled Drivers Motor Club
01832 734724
www.ddmc.org.uk

The Disabled Drivers Association
0870 770 3333
www.dda.org.uk

A rear lift added to a VW Caravelle
Photo courtesy of Invatravel Conversions

Determined David drives in style


David Oulton (MPS II)

David Oulton, from Liverpool, was presented with the key to his specially adapted Motability Scheme car by members of staff from Royal & SunAlliance Motability on Friday 27 May 2005, providing him with increased independence and opportunities. David is representative of many people they help with their ongoing programme of fundraising events for Motability's Charitable Grants fund. The presentation took place at Royal & SunAlliance, New Hall Place, Old Hall Street, Liverpool.

17 year old David has Hunters Syndrome, a progressive genetic disorder, affecting his growth and mobility. David has always been determined to drive, and he decided to use his government-funded mobility allowance to obtain a brand new Citroen C3 through the Motability Scheme. "As I am 4'3", the car needed to be adapted because my feet couldn't reach the pedals," said David. "So I applied for financial help through the Charitable Grants fund and push pull hand controls were fitted to solve the problem, as well as an integral switch to make the indicators, horn and lights easier to use. Motability even helped with the cost of my driving lessons.

"It's a great feeling having the freedom to drive myself rather than being reliant on my dad or my sister for a lift all the time. Now I can get to the hospital easily for appointments, visit friends and take my mum out. There's plenty of room for my electric scooter too when I need it. My nan had a Motability car when she was alive, that's how we first heard about the Scheme. Then my parents applied on my behalf when I was younger, with my dad as the driver. This is our fifth Motability car but now I'm in the driving seat!"

Ian Currie, Director of Royal & SunAlliance Motability, commented: "Our staff and our suppliers have been extremely energetic raising funds through a variety of events, one of which is our annual Charity Ball. Over the last two years the total has risen to £31,500.00! This is a wonderful result and I am delighted to see how it will help people like David."

Don Brereton CB, Director of Motability said: "Fundraising is an essential part of the Motability Scheme, without it we would not be able to help the vast number of people that we do. I am very grateful to the staff at Royal & SunAlliance Motability for their marvellous support and pleased that they have been able to meet David, as this illustrates how our customers can remain mobile and lead a full and active life."

Press release and photos appear courtesy of Motability
Left to right: Ian Currie, Director of Royal & Sun Alliance Motability, David Oulton, Don Brereton CB, Director of Motability

Motability
The leading car scheme for disabled people

Motability is the UK's leading car scheme for disabled people. Since it was set up on the initiative of the government in 1978, it has provided millions of people with affordable, convenient, trouble-free motoring through the provision of a new car. Powered wheelchairs and scooters can also be financed through the Scheme. Motability's car and wheelchair schemes currently provide freedom and independence to over 400,000 disabled people and their families, across the UK.

The Scheme is directed and overseen by Motability, a national charity that also raises funds and provides financial help to customers who would otherwise be unable to join the Scheme.

How the Motability Scheme works

As a unique and successful collaboration of the public and private sectors, the Motability Scheme enables disabled people to use their government-funded mobility allowances to obtain a new car, powered wheelchair or scooter through contract hire and hire purchase schemes.

By far the most popular option, chosen by over 97% of customers is the contract hire of a new car, which offers worry free motoring at affordable prices. Customers can choose a brand new car of their choice from over 20 manufacturers, on a three or five-year lease. Full insurance, breakdown cover, servicing, tyre and windscreen replacement are all included in a single monthly payment.

Many cars are available simply by customers transferring their allowance to Motability for the period of the agreement. However, on larger or more expensive models, an additional payment (known as an advance payment) may be required. Competitive hire purchase schemes, over one to three years, are also available to purchase a new or used car, powered wheelchair or scooter.

Who can apply?

The Scheme is available to anyone who is receiving one of the following benefits, and has at least 12 months award length remaining when they apply: Higher Rate Mobility Component of the Disability Living Allowance or War Pensioners' Mobility Supplement.

A parent or carer can apply on behalf of a child aged three or older and non-drivers can apply for a car as a passenger. Once their application has been accepted, the customer pays all, or part, of their allowance to Motability for the duration of the contract hire or hire purchase agreement.

Getting on the road

Cars are supplied through a network of over 3,500 Motability dealerships across the UK. There is also a national network of accredited retailers of powered wheelchairs and scooters.

Adaptations to cars

For most Motability customers, a standard production car is suitable for their needs, but special adaptations are needed for around 10% of customers, to enable them to drive safely, or travel in comfort as a passenger.

Providing additional financial help

Motability also awards various grants to help those customers who need additional financial help to obtain the mobility solution they need. These grants go towards a range of essential vehicle adaptations and conversions, the advance payment towards a chosen car and to assist in the funding of driving lessons.

As well as administering government-funded grants, Motability makes its own charitable grants. Motability's fundraising team exists to help meet this demand and raises money in a variety of ways, with the help of individual supporters, corporate support and events, trusts and appeals and employee fundraising initiatives.

For further information about the Motability Scheme, please telephone 0845 456 4566, or visit the Motability website at **www.motability.co.uk**

MPS Regional Clinic Dates 2005

Birmingham
Wednesday 26 October

Bristol
Tuesday 22 November

Cardiff
To be confirmed

Northern Ireland
Thursday 17 November

The Royal Free

Lysosomal Storage Disorders Unit

History

The Lysosomal Storage Disorders Unit (LSDU) at the Royal Free Hospital is a designated specialist centre treating patients with Anderson–Fabry and Gaucher's Disease. The clinical service to patients with Gaucher's Disease was initiated in the department over 25 years ago. Only supportive therapy was available at that time but when enzyme replacement therapy became licensed in 1990, a dedicated clinical service began on the haematology day ward. Seven years later, the Royal Free Hospital was designated a national specialist centre for Gaucher's disease by the Department of Health National Specialist Commissioning Advisory Group. 2005 - This year has seen the designation of the centre for all lysosomal storage disorders for which therapy is currently available and our move from the haematology day ward to a dedicated clinical area specifically designed for our patients.

Clinical Care

The past year has been rewarding and exciting for all of us, everyone involved in the Royal Free LSDU. We have welcomed new members of staff to reinforce the multidisciplinary team that provides medical care and support for a growing number of patients. This currently includes over 100 patients with Anderson-Fabry disease and 70 patients with Gaucher's Disease. We provide genetic counselling, diagnostic services, clinical assessment of patients and ongoing supportive care for both those requiring treatment and those in whom observation is currently appropriate.

All patients attending our unit are seen by a physician (Dr Mehta or Dr Hughes) and specialist nurses within the LSDU. We currently have a team of five clinical nurse specialists trained in the care of patients with lysosomal storage disorders including individuals with specific expertise in genetic counselling, paediatrics and women's health. After their initial assessment patients may also be seen by other specialists appropriate for their individual case. We have built an experienced team including neurologists (Dr Ginsberg), radiologists (Dr Berger), dermatologists (Dr Orteu), pain management (Dr Ordman), gastroenterologists (Dr Keshav), renal physicians (Dr Burns), opthamologists (Miss Davey) and audiologists (Dr Cadge) all of whom have expertise in the ways in which lysosomal storage disorders affect their speciality.

We are particularly pleased to have the expert assistance of the cardiologists at the London Heart Hospital (Dr Elliott and Dr Shah) in the assessment of our patients and our

newest member Dr Agatha Pachnis, a paediatrician, who will assist in the assessment of adolescents and in their transition from paediatric to adult services. We aim to provide a personal service individualised to the needs of each patient and recognise that each patient's experience of their condition will be very different.

For those requiring more information or support we provide a telephone helpline line for patients, medical professionals and members of the public requiring advice and operate a website (http://freenet/Lysosomal/index.htm), which can be accessed both by clinicians and by patients.

Therapy

At the Royal Free LSDU, patients with type I Gaucher's Disease receive ERT with Imiglucerase (Cerezyme®; Genzyme)., which is a recombinant form of human glucocerebrosidase. Patients with Anderson–Fabry Disease are offered ERT with Agalsidase alfa (Replagal®; TKT 5S) or Agalsidase beta (Fabrazyme®; Genzyme). Almost all of the patients receive their ERT at home. Patients are initially trained to self-administer ERT in the LSDU by clinical nurse specialists. Home infusion nurses are available to continue patient training at home, as well as to deliver enzyme preparations and to provide on-call support should patients experience problems with ERT or SRT at home.

Our specialist nurses also provide telephone help and consultations for minor problems which may occur between visits to the centre. Although some patients are virtually independent of the LSDU, most patients visit us every 3–6 months. Less than half of all our patients live in and around the London area, and some travel from as far away as Northern Ireland, Wales and Scotland to attend approximately every 6–12 months.

Decreasing the rate of synthesis of the lysosomal storage compound, so-called substrate reduction therapy (SRT), is a new alternative to ERT. The prototype SRT, Miglustat (Zavesca®; Actelion) is an oral treatment for patients with mild to moderate type I Gaucher's Disease who are unsuitable for enzyme therapy, and has been shown to produce improvements in spleen and liver volumes and other key clinical parameters of disease. It is used in our unit to treat patients in whom venous access had become impaired with vascular deterioration, those with needle phobia, or patients who need to travel regularly. Home support is also offered to patients receiving oral medication.

Research

The LSDU research activity includes both clinical trials, and our laboratory research programme. Our laboratory team has developed a number of cell culture systems designed to explore the mechanisms underlying the disease processes in lysosomal storage disorders.

Our research efforts together aim to extend our understanding of the pathophysiology of Gaucher and Anderson–Fabry Disease and the way in which these rare diseases may be treated. We participate in a number of database studies which are designed to improve our knowledge of the wide range of problems that patients with these conditions may experience. We are also currently conducting clinical trials looking into the best doses and/or dose intervals for treatment of both Anderson-Fabry and Gaucher's disease. We are also collaborating with Drs B Bax and M Bain at St George's Hospital to explore new mechanisms for delivering enzyme by encapsulating it within red cells. This natural mechanism of delivery should mean that the enzyme persists longer within the patients and is directly targeted to the cells where it is most needed. Other ongoing projects included observational studies of the incidence of neurological and bone-related complications of type 1 Gaucher's Disease.

We earnestly believe that improved patient care will be derived from an evidence-based approach to our practice and regularly audit our clinical protocols against patient satisfaction and treatment outcome data. We are always pleased to have suggestions and comments from our patients and try to organise patient meetings at least once a year. All of our team actively participate in meetings organised by patient associations and we also encourage our patients to join these meetings. This year a group of our patients with Fabry Disease travelled to Rome to participate in a meeting of the Fabry patients from all over Europe.

Conclusion

At the Royal Free LSDU, we hope to continue providing and improving care and support for patients with lysosomal storage disorders. Through our research we hope to contribute to the further understanding of the problems associated with these conditions and to contribute to the development and evaluation of new treatments for these debilitating conditions. ■

Capital Doctor of the Year Award is won by Dr Atul Mehta

Dr Atul Mehta, who leads the NSCAG Lysosomal Storage Disease Centre at the Royal Free Hospital in London, has won the prestigious Capital Doctor of the Year Award which was presented to him at a ceremony at the Royal College of Physicians on 5 February 2005. He received the award in recognition of his pro-active work in haematology and rare diseases including Fabry Disease.

International Working Party on MPS Diseases

Frambu, Norway by Clare Cogan

Norway hosted this meeting on 19-22 May 2005 which saw representatives from 13 different countries come together with one common interest, MPS.

Each country gave their own presentation on their Society and what they have achieved and lots of opportunities were afforded to us to share ideas and experiences.

Representatives from the three pharmaceutical companies, Biomarin, TKT and Genzyme also joined the group and gave presentations themselves, 'from a Company Perspective'.

All the discussions took place in the National Centre for Rare Disorders and Disabilities, Frambu, Norway. Topics covered included a review of the research for MPS Diseases, discussions around ERT, the role of Children's Hospices and medical aspects of palliative care. The working party also incorporated small group workshops led by myself, Barbara Wedehase and Christine Lavery on Advocacy, Communicating with the Media and Governance and Managing your MPS Society.

The meeting afforded the opportunity to get to know each country's representative on a more informal basis through the Norwegians' excellent hospitality and organised trips to the Viking Museum, and a trip up the Norwegian Fjords. I don't think I have ever been so cold.

During the course of the meeting the Working Party decided to change its name to the International MPS Network which it was agreed conveyed the purpose of the group and its work, a mission statement is a job for the future!

It was clear that by the end of the three days, people had formed strong bonds with people across the world and I felt privileged to be part of such a dynamic and forward thinking group of people.

New protocol from the University of Minnesota

Scientific protocol for treating children with severe MPS I with a combination of stem cell transplantation and enzyme replacement therapy at the University of Minnesota

The University of Minnesota has been a pioneer in the treatment of children with severe MPS I, also called Hurler syndrome, and other storage disorders using stem cell transplantation (i.e. bone marrow or cord blood transplantation).

To date, approximately 100 transplants have been performed at the University of Minnesota for Hurler syndrome alone. We are committed to the comprehensive and long term care of these children, including evaluation and treatment of issues unique to these children before and after transplantation. Our team has extensive and long-standing experience in the assessment and treatment of complications of Hurler syndrome, including problems of the eyes, ears, lungs, heart, bones and joints, as well as neurological difficulties. The University of Minnesota constantly strives to improve results and outcomes following stem cell transplantation. The most recent such innovation at Minnesota (for children with Hurler syndrome) is the development of a scientific protocol for the combined use of enzyme replacement therapy and stem cell transplantation. The basis of this combination therapy is as follows:

Hurler syndrome is the most severe form of MPS I and is characterised by progressive mental regression, heart and airway disease as well as bone deformities; death typically results in the first 10 years of life.

Laronidase (trade name Aldurazyme) is the commercial preparation of the enzyme that is deficient in Hurler syndrome. Laronidase enzyme replacement therapy (ERT) does not directly benefit the brain in children with Hurler syndrome, and hence does not represent a satisfactory therapy in itself. However, Laronidase can provide other valuable benefits; in particular it clears airway obstruction and improves lung function.

Stem cell transplantation is the only proven therapy that can prevent the progressive mental retardation and give hope for long-term survival. However, stem cell transplantation is not free of risks and complications. Children with Hurler syndrome are particularly prone to get lung and airway related complications during transplantation.

Based on these observations, we believe that treating with ERT for 12 weeks before and 8 weeks after stem cell transplantation will decrease complications associated with stem cell transplantation, thereby improving outcomes and survival in children with Hurler syndrome undergoing transplantation. The University of Minnesota has developed a protocol (the first of its kind) to systematically administer this therapy in a uniform manner as well as to study the results of this combination therapy as compared to stem cell transplantation alone.

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2005 MPS GRANTS TO RESEARCH

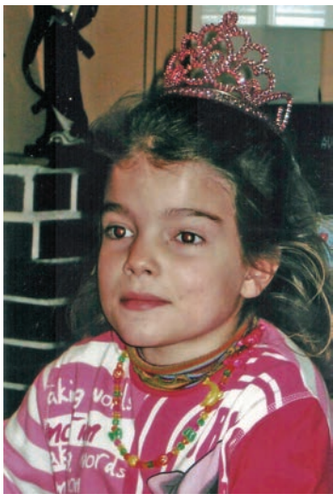
Applied Stem Cell Biology of MPS and other inherited metabolic diseases

Lead Investigator	Dr Rob Wynn	Royal Manchester Children's Hospital
Co-Investigator	Dr Ed Wraith	Royal Manchester Children's Hospital

In March 2004 the MPS Society approved funding for a lectureship to lead the work in cellular therapy of MPS at the University of Manchester. This is a five year position and is about to be re-advertised. The person appointed will be expected to develop their own research portfolio as well as collaborating with the lead investigator and co-investigator by virtue of their related interests. In March 2005 the MPS Society agreed to support the development of this stem cell group by funding a research technician over a period of three years enabling the group to work more quickly and consolidate their position.

Grant Awarded for lectureship	£273,003 over 5 years
Grant Awarded for research technician	£93,625 over 3 years

The Spanish Society for Sanfilippo Disease



We are writing to tell you a little bit about our organisation. But, firstly we would like to thank Christine Lavery and the UK MPS Society for their support to the Spanish Society for Sanfilippo Disease and today the Spanish Society for MPS and Related Diseases.

The Society

We are parents of Sophie. Sophie has Sanfilippo Disease and she is 7 years old. Sophie was diagnosed with MPS III when she was 5 years old and at that time we founded the Spanish Society for Sanfilippo Disease as there was no other organisation around providing support to those affected by this disease. The Society was founded in August 2003 and since this time, we have fought hard as parents looking for a treatment for our daughter's disease.

The objectives of our organisation include: Support the scientific investigation of the causes, development and treatments of Sanfilippo Disease; Provide advice, support and information to the families of those affected by this disease; Promotion of seminars, conferences and general publicity to enable the exchange of information between medical and scientific professionals and the patients; Promotion of scientific scholarships and prizes. These objectives are conveyed using many different media including radio, press, TV, stands at fairs. The most important objective is to collect funds for research. Since the Society was founded, we have collaborated with many people to receive donations, to become members, help us with fundraising etc.

Our family

Sophie is our beautiful princess. We call her 'La Bella' because she likes a lot of the Disney movies such as Beauty and the Beast, Mickey, Donald, Tigger and Winnie the Pooh.

Sophie's health is good but she uses nappies. She can't feed herself and needs our help 24 hours a day. She walks OK but often gets tired and needs a buggy. She eats gluten free foods and drinks soya milk. We give her melatonin at night with her dinner as it is very good for her hyperactivity and sleeping.

We live in a small village and our house is like a farm. We have six chickens. They have lots of freedom in the garden along with four ducks. Sophie runs around after them and this makes her happy. There are also two cats.

On 2 December 2004 our baby Jordi was born. Sophie wants to play with him and he is a great support for us.

2005 Sanfilippo Grants to Research

The Spanish Society for Sanfilippo Disease has opened the research of Sanfilippo Disease in Spain in Barcelona by the Group of Human Molecular Genetics at the University of Barcelona. This is conducted by the doctors Daniel Grinberg and Lluisa Vilageliu with the collaboration of Doctors Amparo Chabas and M. Josep Coll of Lisosomals IIIs of Clinic Biochemistry Institut.

Analysis of implicated genes in the heparan sulfat synthesis for development of a possible therapeutic strategy with the use of RNA cells of interference from the Sanfilippo Disease A.
Grant Awarded 6,000 Euros over 1 year

Involvement of the Blood Brain Barrier in MPS III A & B
Professor Bryan Winchester, Institute of Child Health, London and Dr David Begley of King's College, London
Grant awarded 9,000 Euros over 1 year

Direct Delivery of therapeutic lysosomal enzyme to correct CNS pathology in Sanfilippo Disease
Dr John Hopwood, Professor and Head Biochemical Genetics Services and Lysosomal Diseases Research Unit Department of Genetic Medicine, Women's and Children's Hospital of Adelaide, North Australia.
Grant awarded 9,000 Euros over 1 year
1. Establish the optimal protocol (dose frequency) for intra-CM injections of rhNS in MPS IIIA mice and determine the clinical efficacy and safety
2. Determine whether the various neurodegenerative and behavioural changes in MPS IIIA mice can be reversed, delayed or prevented by intra-CM injection
3. Improve understanding of the molecular mechanisms of MPS IIIA pathology to aid the identification of further therapeutic approaches to this disease.
4. Justify newborn screening for MPS IIIA and other LSD with potential CNS involvement, given efficacy and safety of CM-delivered therapeutic enzyme.

FABRY DISEASE SUFFERERS

RALLY AT QUEEN'S PARK

TORONTO – Patients suffering from a rare and fatal genetic disorder that ravages vital internal organs said Thursday the Ontario government will leave them to die if it does not immediately start paying for treatment they believe would save their lives.

Fabry disease sufferer Darren Nesbit pleaded with Health Minister George Smitherman to approve funding of enzyme replacement therapy. 'I'm not ready to leave this world yet,' the Samia, Ont., man wrote in a heart-wrenching letter he handed to the minister on Thursday.

But Smitherman told him the earliest he and Ontario's 30 other estimated Fabry patients can expect a decision would be in September, when provincial health ministers meet with the federal government to discuss drug funding. 'We think it's incredibly important that the federal government stand in its place and play a role in helping to offer appropriate funding for these orphan drug challenges,' Smitherman said. However, he reiterated expert opinion advising against public funding of such treatment which includes the drug Fabrazyme, citing a lack of scientific evidence that it works.

The latest recommendation came last month from an independent panel called the Common Drug Review, which provides advice to federal and provincial governments. But Nesbit, who is believed to be the first in Canada to undergo experimental trials of the drug, has no such doubts. 'Whether you believe in the science or not, I will bet my life on Fabrazyme before I bet my life on anything else,' said Nesbit, who has felt crippling pain in his hands and feet since ceasing treatment about two months ago. I'll die knowing that it works'.

Nesbit, diagnosed 10 years ago, said he believes cost is the real reason the provincial government won't approve funding for the treatment. It costs $300,000 annually for a single patient. 'I said,' What's my life worth to you?'

Australia, Japan, the United States and some countries in Europe pay for enzyme replacement therapy. 'Every genetic doctor around the world is laughing at Canada right now. They think we are a joke because we won't pay for this,' Nesbit said.

Genzyme Canada, which produces Fabrazyme, recently stopped providing the drug to patients for free. It wants provincial governments to start picking up the tab because the drug was approved for use last year by Health Canada. But ministry officials say that approval only ensures the drug's safety to enter the market place and doesn't test its clinical efficacy.

In Fabry patients, an enzyme deficiency causes fats to accumulate in blood vessels, leading to damage of internal organs such as the heart and the kidneys. It also causes acute pain, often in the hands and feet. It is estimated between 150 and 300 Canadians have been diagnosed with Fabry Disease.

This article, written by Tara Brautigam, has been taken from Canadian Press, Thursday, June 02, 2005.

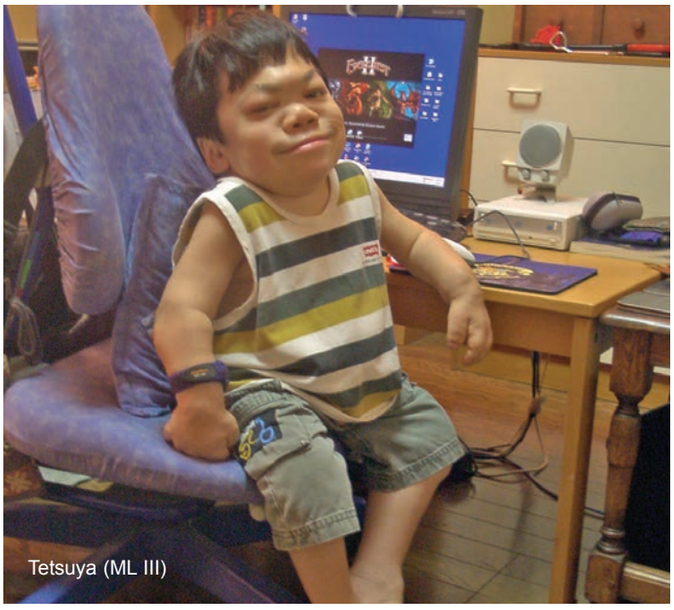
Elizabeth Herridge updates us on the Morquio Children of Chennai

Thank you to everyone who donated money towards the family of Manikandan, the little boy I have 'adopted' with Morquio in Chennai. He lives in a Cheshire Home there but his mother, grandmother, sisters and extended family lost their homes when the tsunami hit the beaches of Chennai in South India. They lived in straw huts on the beach and lost absolutely everything. It has been quite a long haul to find them an acceptable alternative.

We raised something like one thousand five hundred pounds from various sources and this, with other donations, has bought Manikandan's mother (it will be in her name and in Manikandan's name) a 900 sq foot plot of land about a kilometre in from the beach close to the main coast road. Because other donations have been added, it will be possible to construct a simple cottage. Much of the materials for it will be second hand and building materials will be donated with luck and a lot of pressure!

However, this will be wonderful for Manikandan's mother because she was living illegally on the beach, despite paying a high rent to do so. It will also ensure that any further tsunamis will not reach them. Progress is slow but positive. Meanwhile Manikandan charms everyone who meets him, is doing well at school and I am sure is now looking forward to spending some holidays in his new country home! I wish I was there with him! And he would, I am sure, want to say thank you himself.

Tetsuya Richard Motomura, ML III


Tetsuya (ML III)

My Name is Tetsuya Richard Motomura. I'm 21 now but was born with MLIII (Mucolipidosis III) enzyme deficiency disease that affects body growth in many ways. I will tell you what I had to live with and how I feel about it having this condition. Life is frustrating sometimes especially for me, being small and physically challenged.

When I go outside I can't walk far without getting tired. As a result I need to ride a wheel chair. This is sometimes irritating to me as I can't go out on my own. I would really like to walk down town and do my thing as young adults do. At home I walk around from room to room from one seat to another. All the chairs I have must be the right height. Normally I stay in my room. All my furniture is the right level for me or made so I can climb up to sit high. It seems I'm living in the parallel world in a smaller dimension. I'm happy when someone my own age is living in the same dimension as I am from LPA (Little People of America) and MPS (Mucopolyssacharidosis) Societies that cover similar enzyme deficiency diseases. They are organisations that help people like me and make it possible for people like me to get together. It makes me feel that there are more people like me who share the same obstacles as I face everyday. Also there is a website called Penguin Café. This website was set up for MLIII people around the world to pass information and encouragements to other members with the same conditions.

I have a difficulty moving my fingers and I'm only waist high to an average person, therefore I have a hard time reaching, opening things and switching things on. I can't put my foot down flat so it is very tiring to stand in one place for a long time. So when I'm at home I need help to do my daily routines like going to the men's room, getting a drink and taking a bath. I sometimes wish I could be able to do things on my own. I get frustrated and angry sometimes that I was born with this condition and wish I could be a fully able person. I have dreams to go to college and study sound engineering and computer studies. I am so determined to study them that I feel stubborn.

Another frustrating aspect of this condition is that I'm very small for my age group so some people on the street look at me but think of me as a baby because of my size. They don't know that I don't like being a called a baby. They sometimes don't believe that I'm come of age and I can't help being small. I'm glad and happy that I have many friends who see me as who I am and share my frustrations. They helped me a lot with my school work and making my young life enjoyable. My two brothers are very helpful, trusting, kind to me and loving too. I wonder where I would be now without them. I must thank my mother and father for raising me up to who I am now today. I had frustrating times and very happy times so far.

I have a talent as a story teller and I'm now learning how to write them down. I'm also learning computer the hard way and being taught basic things. I thought that school was a waste of time but now I realize if I didn't go I would not have learnt as much as I know now or achieved what I have accomplished. I will finish high school then think what I will do. I passed the first Amateur radio license test in 11th grade but I can't do it in Japan. I will study again once I have finished high school. For my pastimes I love reading, playing games plus computer games. I also love watching interesting programs on TV; nature, technology and space, plus other interesting topics. I will improve writing books and see if I can sell them.

Even people like me can improve their talents and do things we enjoy. We just have to work a little harder to fit into society. We are just miniature average people.

Editor's Note: Tetsuya's mother, Sally, adds that because of her husband's work, the family have made six international moves between Tokyo, New York and Europe in Tetsuya's lifetime. Each time the family have been faced with the hurdles of finding suitable educational and medical facilities for Tetsuya. But, through it all, Tetsuya is a great little guy, optimistic and gregarious. He has come so much further than we ever imagined possible.


Tetsuya, Kazuma, Jun, Yuki and Sally Motomura

CHRISTOPHE FLIES TO MANCHESTER

Hello everybody. My name is Christophe. I'm 21. I live in Tarascon du Rhone in France and I have Hunter Syndrome. Here is my story...

In March 2002, in consultation with Doctor Nathalie Guffon, I learnt that in the United States they had found a treatment for my illness, something I had been expecting for three years. In October 2003, a phonecall from Nathalie Guffon told me that the treatment was in Europe and did I want to take part. I can't tell you how happy I was! After tests in Lyon, I was very anxious to know if I would be accepted. It was to have gone to Germany but then ended up in England.

In December 2003, I went to Manchester with my parents which was for me the beginning of a big adventure as I had never been to England before and was very worried because neither I nor my parents speak English.

There I met Dr Ed Wraith, a long haired giant, nurse Jane Roberts, Sue our interpreter, Arnaud and his mum and sister as well as a little Italian, Antino and his parents who were there for the same reason as me. After undergoing tests which were quite difficult for me, we went back to France. It was hard there because I had to wait and see if I could participate in the double-blind tests. Normally the reply would come at the beginning of the year, but I had the best Christmas of my life when on 23 December, I received an e-mail telling me I would be off to Manchester again on 1 February 2004 to stay until the 5th to undergo more tests and sign the consent forms for a year's treatment.

On 12 February 2004, a day I will never forget, my first infusion took place at Addenbrooke's Hospital, Cambridge, then doing the journey each week and staying at a hotel for two nights. But then mum asked if we could live here as travelling was very tiring for me. So since 12 March, I am English by adoption. I live with mum in a lovely flat opposite the river Cam, and two nights a week Arnaud and his mother come and sleep at our place so as not to have to go to a hotel. (Arnaud is the other French boy who has the treatment with me).

Cambridge is a magnificent city with super colleges and huge parks. Life is very different from ours including the food (lots of sandwiches). The weather is very damp with not much sunshine, but luckily, people are very nice to us except for the weird taxi drivers.

So, on 12 February I met some really nice nurses (there's Daisy who speaks French), Janet our interpreter who's very kind (she explains a lot of things from English) and Doctor Uma, very sporty. And in my everyday life I've met some people that I like very much and who are very kind to me and mum.

The treatment is doing me a lot of good and I feel and can see that I'm very different. Even my family and the doctors have seen a change; I don't think I've been on the placebo. Luckily! It's been very hard being far from my family and friends. Some days I'm sad, but my mother is there to perk me up.

From 3 to 9 February 2005, I went back to Manchester for more tests and to sign another consent form for two years until March 2007. The 10 February 2005 was a big day when I got the real treatment (there's no more placebo, fab!). I'm living a marvellous adventure, I feel like a knight in the Crusades with the treatment as my grail.

I just want to say to all who are ill not to lose hope, you have to believe and have confidence, because soon everybody will have the treatment; I've always believed in people around me and in particular, in my favourite doctor, Nathalie Guffon, whom I adore.

I believe in the Force
And may the Force be with you all.

Translated from the French by Janet Erskine. This article was written for the charity A TIR-D'AILE who help to realise childrens' dreams.

Christophe Bonnet's letter of hope to TKT, Cambridge, Massachusetts, USA

My name is Christophe Bonnet. I live in Tarascon in Bouches-du-Rhône in France and I suffer from Hunter's syndrome. I'm writing to you to thank you for this great discovery which is going to make me live normally. Thanks to you my best Christmas present for 2003 has been to know that I was going to have the therapy in Cambridge, England. Since April, I feel different and I'm getting better and better. I'm very pleased with my results but I think mostly about the children to come who will be able to be treated from a very young age and won't have to go through what I've been through. My parents and my family share in my happiness. Now my biggest dream would be to meet you to thank you but mostly to be able to visit the laboratory where my cure was discovered. Translated from the French by Janet Erskine.

BioMarin Announces FDA Approval for Naglazyme

First Specific Therapy Approved for the Treatment of MPS VI

Novato, CA, June 1, 2005: BioMarin Pharmaceutical Inc. (Nasdaq and SWX: BMRN) announced today that the U.S. Food and Drug Administration (FDA) has granted marketing approval for Naglazyme™ (galsulfase), the first specific therapy approved for the treatment of MPS VI. As the first drug ever approved for MPS VI, Naglazyme has been granted orphan drug status in the United States, which confers seven years of market exclusivity. BioMarin plans to launch Naglazyme in the United States in approximately 30 days.

Naglazyme is indicated for patients with MPS VI. Naglazyme has been shown to improve walking and stair-climbing capacity. As post-marketing clinical commitments, BioMarin has agreed with the FDA to evaluate the effect of Naglazyme treatment on skeletal dysplasia in patients under the age of 1 and to maintain a clinical surveillance program to monitor patients on commercial therapy; no extension study of Phase 3 patients was required.

Clinical trials have demonstrated that Naglazyme provides clinically important benefits for MPS VI patients, specifically, improved endurance as demonstrated by the 12-minute walk test and 3-minute stair climb. Naglazyme reduced the excess carbohydrates (glycosaminoglycans, or 'GAGs') that are excreted in the urine of patients with MPS VI, an indication of enzymatic bioactivity.

'I have observed the positive effect that enzyme replacement therapy with Naglazyme can have on MPS VI patients, and I am very pleased that it will soon be made commercially available to those who need it,' stated Paul Harmatz, M.D., Associate Director of the Pediatric Clinical Research Center at Children's Hospital & Research Center at Oakland, California, and Principal Investigator of the Phase 3 clinical trial of Naglazyme. 'With Naglazyme now approved, physicians, for the first time, have a therapeutic to treat the underlying cause of MPS VI, increasing their ability to provide better care for MPS VI patients with this life-threatening disease.'

'The approval of Naglazyme is a significant milestone for those whose life has been affected by MPS VI and for BioMarin,' stated Jean-Jacques Bienaime, Chief Executive Officer of BioMarin. 'The disease burden of MPS VI is enormous for patients, families and physicians. Naglazyme holds a very real possibility for making MPS VI a more manageable disease.' Mr. Bienaime continued, 'BioMarin developed Naglazyme on its own and now, with our U.S.-based sales force in place, we are ready to bring it to market. Our efforts to identify individuals with MPS VI in the years leading up to this day have positioned us to rapidly get patients on therapy come product launch. I would like to thank the individuals with MPS VI and their families and physicians as well as BioMarin employees for their years of hard work and dedication toward making Naglazyme for MPS VI a reality.'

An application to market Naglazyme is currently pending in the European Union. BioMarin expects to receive an opinion from the European Commission in the fourth quarter of 2005, and if positive, final approval in early 2006.

About MPS VI

MPS VI (also known as Maroteaux-Lamy Disease) is a debilitating, life-threatening genetic disease caused by a deficiency of the enzyme N-acetylgalactosamine 4-sulfatase. This enzyme deficiency leads to the accumulation of certain complex carbohydrates, glycosaminoglycans (GAGs), in the lysosomes, giving rise to progressive cellular, tissue and organ system dysfunction. An estimated 1,100 individuals in the developed world have MPS VI. The majority of individuals with MPS VI die from disease-related complications between childhood and early adulthood. Additional information can be found at www.mpsvi.com

Phase 3 Clinical Trial & Extension Study Results

BioMarin completed a 24-week, Phase 3, multi-center, double-blind, placebo-controlled trial involving 39 patients. Patients were randomized on a one-to-one basis into a Naglazyme treatment group or a placebo control group and received a weekly intravenous infusion of either 1.0 mg/kg of Naglazyme or placebo solution. During the 24-week period, 19 patients received weekly intravenous infusions of Naglazyme and 20 patients received weekly placebo infusions. One patient in the placebo group dropped out of the trial for reasons unrelated to treatment. All 38 patients who completed the trial elected to receive Naglazyme in an ongoing open-label extension study.

Efficacy Data

After 24 weeks of treatment, patients receiving Naglazyme demonstrated a statistically significant improvement ($p=0.025$) in endurance compared to patients receiving placebo as measured by the change relative to baseline in the distance walked in 12 minutes. The Naglazyme-treated group showed greater mean increase in distance walked in 12 minutes compared to the placebo group. The model-derived mean difference measured as a change from baseline between patients receiving Naglazyme and patients receiving placebo after 24 weeks was 92 ± 40 meters.

Following an additional 24 weeks of treatment with Naglazyme in the extension study, for a total of 48 weeks, patients demonstrated further improvement in endurance as measured by the change in distance walked in 12 minutes, relative to baseline. From week 24 to week 48, patients receiving Naglazyme since week one of the trial improved their mean walk distance an additional 36 ± 97meters.

After 24 weeks of treatment, patients receiving Naglazyme demonstrated an improvement (p=0.053) in stair-climbing ability compared to patients receiving placebo as measured by the change relative to baseline in the number of stairs climbed per minute. The Naglazyme-treated group showed greater mean increase in the rate of stairs climbed in three minutes compared to the placebo group. The model-derived mean difference measured as a change from baseline between patients receiving Naglazyme and patients receiving placebo after 24 weeks was 5.7 ± 2.9 stairs per minute.

Following an additional 24 weeks of treatment with Naglazyme in the extension study, from week 24 to week 48, patients receiving Naglazyme since week one of the trial improved their mean number of stairs climbed per minute by an additional 3 ± 7 stairs. After 24 weeks of treatment, patients receiving Naglazyme experienced a statistically significant reduction ($p<0.001$) of GAGs excreted in the urine, compared to patients receiving placebo. The average urinary GAG reduction in patients receiving Naglazyme after 24 weeks was 75.5 percent. This initial reduction in urinary GAG levels was maintained following an additional 24 weeks of treatment in the extension study.

While in the extension study, patients who receive placebo solution during the initial 24-week trial demonstrated an improvement in endurance following 24 weeks of treatment with Naglazyme as measured by the change in distance walked in 12 minutes, relative to baseline. From week 24 to week 48, the original placebo group demonstrated a mean increase of 65 meters relative to week 24 values. These patients also demonstrated an average improvement in stair-climbing ability as measured by stairs climbed in three minutes, relative to baseline, of 5.7 stairs per minute following 24 weeks of treatment with Naglazyme. Additionally, patients initially receiving placebo demonstrated a reduction in urinary GAG levels following 24 weeks of treatment with Naglazyme comparable to that observed for those treated in the initial 24-week, double-blind portion of the trial.

Safety Data

Data from the Phase 3 clinical trial and extension study indicate that Naglazyme was generally safe. The most common adverse events observed in clinical trials in Naglazyme-treated patients were headache, fever, arthralgia, vomiting, upper respiratory infections, abdominal pain, diarrhea, ear pain, cough, and otitis media. Over 95 percent of the infusion-related adverse events were considered mild or moderate and were easily managed. Infusion-related adverse events commonly included fever, chills/rigors, headache, rash, and mild to moderate urticaria. Severe reactions included angioneurotic edema, hypotension, dyspnea, bronchospasm, respiratory distress, apnea, and urticaria. No patients discontinued Naglazyme infusions for adverse events and all patients that completed the double-blind portion of the trial continue to receive weekly infusions of Naglazyme.

Nearly all patients developed antibodies as a result of treatment, but the level of the immune response did not correlate with adverse events or impact the improvements experienced in endurance. Evaluation of airway patency should be considered prior to the initiation of treatment. Consideration to delay Naglazyme infusion should be given when treating patients who present with an acute febrile or respiratory illness.

About BioMarin

BioMarin develops and commercializes innovative biopharmaceuticals for serious diseases and medical conditions. The company's product portfolio is comprised of three approved products and multiple product and preclinical product candidates. Approved products include Naglazyme™ (galsulfase) for mucopolysaccharidosis VI (MPS VI), a product wholly developed and commercialized by BioMarin, Aldurazyme® (laronidase) for MPS I, and Orapred® (prednisolone sodium phosphate oral solution) for severe asthma. Investigational product candidates include Phenoptin™ (sapropterin hydrochloride), a Phase 3 product candidate for the treatment of phenylketonuria (PKU). For additional information, please visit www.BMRN.com.

Forward-Looking Statement

This press release contains forward-looking statements about the business prospects of BioMarin Pharmaceutical Inc., including, without limitation, statements about: the development and commercialisation of Naglazyme; expectations related to post-marketing commitments for Naglazyme; and actions by regulatory authorities. These forward-looking statements are predictions and involve risks and uncertainties such that actual results may differ materially from these statements. These risks and uncertainties include, among others: possible delays in launching Naglazyme in the United States and slow market penetration following launch; the content and timing of decisions by the European Commission and other regulatory authorities concerning Naglazyme; issues or complications associated with post-marketing commitments; and those factors detailed in BioMarin's filings with the Securities and Exchange Commission, including, without limitation, the factors contained under the caption 'Factors That May Affect Future Results' in BioMarin's 2004 Annual Report on Form 10-K and the factors contained in BioMarin's reports on Forms 10-Q and 8-K. Stockholders are urged not to place undue reliance on forward-looking statements, which speak only as of the date hereof. BioMarin is under no obligation, and expressly disclaims any obligation, to update or alter any forward-looking statement, whether as a result of new information, future events or otherwise. Aldurazyme® is a registered trademark of BioMarin/Genzyme LLC. Orapred® is a registered trademark of Medicis Pediatrics, Inc. and is used under license. ■

TKT Reports Positive Top-Line Results of Hunter Syndrome Pivotal Trial

Cambridge, MA, June 20, 2005 - Transkaryotic Therapies, Inc. (Nasdaq: TKTX) today announced positive top-line results from the company's pivotal Phase III clinical trial evaluating its investigational human enzyme replacement therapy, iduronate-2-sulfatase (I2S), for the treatment of patients with Hunter syndrome. Hunter syndrome, also known as MPS II, is a rare, life-threatening genetic disorder with no available treatment.

In the trial, patients who received 0.5 mg/kg of I2S on a weekly basis showed a statistically significant improvement in the primary efficacy endpoint (p=0.0049) compared to patients receiving placebo. Based on these results, TKT expects to file for regulatory approval of I2S in both the United States and Europe in the fourth quarter of 2005.

About I2S and Hunter Syndrome

I2S is a human iduronate-2-sulfatase produced by genetic engineering technology intended for long-term treatment of Hunter syndrome. TKT's I2S replaces an enzyme that is deficient in patients with Hunter syndrome, and therefore could potentially either stop or ameliorate the clinical manifestations of the disease. TKT's I2S product has been designated an orphan drug in both the United States and the European Union. There is currently no effective therapy for Hunter syndrome.

Hunter syndrome is a hereditary disorder characterised by the body's inability to produce the enzyme iduronate-2-sulfatase, which is essential in the continuous process of replacing and breaking down glycosaminoglycans (GAG). As a result, GAG remains stored in cells in the body causing progressive damage. The symptoms of Hunter syndrome are usually not visible at birth, but usually start to become noticeable after the first year of life. Often the first symptoms may include hernias, frequent ear infections, runny noses, and abnormal facial appearance.

As the disease progresses, a variety of symptoms appear including, enlarged liver and spleen, heart failure, decreased endurance, obstructive and restrictive airway disease, sleep apnea, joint stiffness, and, in some cases, central nervous system involvement. If central nervous system involvement exists, the life expectancy for patients with Hunter syndrome is typically 10-15 years of age, however, some patients can survive into the fifth or sixth decade of life. TKT believes there are approximately 2,000 patients worldwide afflicted with Hunter syndrome in countries where reimbursement may be possible. Additional information about Hunter syndrome is available online at http://www.hunterpatients.com

The primary efficacy endpoint of the trial, also referred to as the AIM study ('Assessment of I2S in MPS II') was a composite endpoint of two clinical measures previously used to assess clinical benefit in MPS disorders, forced vital capacity and six-minute walk test. The mean improvement from baseline to week 53 in percent predicted forced vital capacity was 3.4% in patients receiving I2S compared to 0.8% in patients receiving placebo. The mean increase from baseline to week 53 in the distance walked by patients receiving I2S was 44 meters as compared to 7 meters in the placebo group.

Joseph Muenzer, M.D., Ph.D., of the University of North Carolina at Chapel Hill, an internationally recognised leader in the diagnosis and treatment of MPS disorders and the lead investigator of the AIM study said, 'These findings are very encouraging for the medical and patient communities as we believe enzyme replacement therapy can bring new hope for patients and families addressing many of the symptoms associated with Hunter syndrome.'

Treatment with I2S was generally well-tolerated by patients in the trial. The most common adverse events observed were associated with the clinical manifestations of Hunter syndrome. Of the adverse events considered possibly related to I2S, infusion-related reactions were the most common and were generally mild. No patient withdrew from the trial due to an adverse event considered related to I2S.

We are extremely excited about the outcome of the study. In addition, we are very thankful to all the patients and their families who participated in this one year trial. Their commitment to this program was instrumental in generating the data which we believe will support regulatory approval of I2S,' said Kip Martha, M.D., Senior Vice President and Chief Medical Officer of TKT. TKT expects full data will be presented at a medical meeting in the autumn of 2005.

Trial Design

The AIM study was a Phase III double-blind, placebo-controlled clinical trial conducted at nine sites around the world, including the United States, the United Kingdom, Germany and Brazil. The primary goal of the study was to evaluate the safety and efficacy of 0.5 mg/kg of I2S administered weekly compared to placebo. Additionally, the trial evaluated 0.5 mg/kg of I2S every other week compared to placebo. Ninety-six patients with Hunter syndrome were randomised to one of three groups with each patient receiving a total of 52 infusions of either I2S, I2S alternating weekly with placebo, or placebo. Of the 96 who enrolled, 94 completed the study and they all elected to participate in the open-label extension study of I2S at a dose of 0.5 mg/kg weekly.

About TKT

Transkaryotic Therapies, Inc. is a biopharmaceutical company primarily focused on researching, developing and commercialising treatments for rare diseases caused by protein deficiencies. Within this focus, the company markets Replagal, an enzyme replacement therapy for Fabry disease, and is developing treatments for Hunter syndrome and Gaucher disease. In addition to its focus on rare diseases, TKT intends to commercialise Dynepo, its Gene-Activated® erythropoietin product for anemia related to kidney disease, in the European Union. TKT was founded in 1988 and is headquartered in Cambridge, Massachusetts, with additional operations in Europe, Canada and South America. Additional information about TKT is available on the company's website at http://www.tktx.com.

Forward-Looking Statements

This press release contains forward-looking statements including statements regarding TKT's development of I2S, as well as statements containing the words "believes," "anticipates," "plans," "expects," "estimates," "intends," "should," "could," "will," "may," and similar expressions. There are a number of important factors that could cause the company's actual results to differ materially from those indicated by such forward-looking statements, including: whether TKT will be able to complete and file applications for the marketing approval of I2S in the timeframes it anticipates; whether the FDA, the EMEA and equivalent regulatory authorities will view the data related to I2S in the same manner as TKT; whether such regulatory agencies will ask for additional information about I2S, including the manufacturing processes for I2S; whether such regulatory agencies will require additional clinical testing of I2S prior to approving I2S for commercial sale; whether such regulatory agencies will grant marketing approval for I2S on a timeline consistent with TKT's expectations, or at all; whether I2S will achieve the commercial success anticipated by TKT; whether the results of future clinical trials will be consistent with the results of earlier clinical trials of I2S and warrant further clinical trials or submission of applications for regulatory approval for such products to the FDA, the EMEA and equivalent regulatory authorities; whether TKT will be able to complete the manufacturing development necessary to satisfy regulatory requirements on a timeline consistent with TKT's expectations or at all, including with respect to repairing damage done to TKT's Alewife manufacturing facility where I2S is currently manufactured, and to manufacture sufficient quantities of TKT's products to satisfy both clinical trial requirements and commercial demand, or to manufacture material at all, if approved; the availability and extent of coverage from third party payors and the timing and receipt of reimbursement approvals for I2S; whether competing products will reduce any market opportunity that may exist; and other factors set forth under the caption "Certain Factors That May Affect Future Results" in the company's quarterly report on Form 10-Q for the quarter ending March 31, 2005, which is on file with the Securities and Exchange Commission and which factors are incorporated herein by reference. While the company may elect to update forward-looking statements at some point in the future, the company specifically disclaims any obligation to do so, even if its expectations change.

Gene-Activated® is a registered trademark and Replagal(tm) is a trademark of Transkaryotic Therapies, Inc. Dynepo(tm) is a trademark of Sanofi-Aventis SA. ■

SHIRE PHARMACEUTICALS BUYS TKT

Shire Pharmaceuticals of Basingstoke, Hampshire bought Transkaryotic Therapies (TKT) for £840 million in cash in May 2005. TKT develops drugs for Fabry Disease, MPS II and other rare genetic conditions. TKT has one drug on the market in Europe, Replagal for Fabry Disease, with sales of £40 million in 2004. I2S for Hunter Disease is in Phase III trials and the market for this drug is estimated at £170 million. G-GCB for Gaucher Disease is in the Phase I/II trials and Dynepo for kidney disease related anaemia launches in Europe in 2006 and potentially TKT's biggest product, targeting a potential £1.6 billion market although the US rights belong to Sanofi-Aventis.

Analysts fear that Shire Pharmaceuticals has paid too much for TKT and will not boost earnings until 2008. However, Shire's Chief Executive, Matthew Emmens, claims that TKT is a 'very strong strategic business fit for Shire'. From the MPS Society's perspective, and its members, it is business as usual. Whilst in recent months we have said goodbye to most of the Swedish Managing Directors of TKT 5s on the ground in the UK Sheila Bone, Area Director UK and Ireland, is still there and has been joined by Theresa Heggie, General Manager Europe.

FOCUS on EDUCATION

Special Educational Needs Law and Practice is now established, the current framework having first been introduced as far back as 1983.

Broadly, if a child has a learning difficulty or disability which means that they may require provision which is additional to, or different from, that available in ordinary local schools in their area (for example if they may need more help than the school can provide, therapy input beyond that which the school can provide or, very occasionally, a specialist school), the child concerned will be the subject of a formal procedure known as a statutory assessment.

Such an assessment can be commenced by a request from a parent or school and, sometimes, local authorities will commence an assessment themselves, upon receiving information from other agencies (such as the health authority). An assessment is a formal procedure known as a statutory assessment. Such an assessment is a formal procedure involving the obtaining of reports and information from a number of professionals and individuals, including the children's parents, school, an LEA education psychologist, health authority doctor and sometimes other professionals such as a speech and language therapist, occupational therapist or physiotherapist.

Once all of this evidence has been obtained, the local authority then decides whether or not the child requires additional or different help and, if so, will produce a draft, or proposed, statement of special educational needs. This is a formal document which is meant to set out all of the child's educational needs, together with the provision required to meet those needs.

Statements must be laid out in a particular form and there is, now, a fair amount of law setting out exactly what a statement should contain. Generally, statements should particularise all of the provision that a child requires to meet their needs and, if it is determined that the child requires extra adult support, ordinarily, that support should be quantified (in terms of numbers of hours/sessions per week/term, duration of the sessions, who is to deliver the sessions and whether the sessions should be on a one to one or small group basis). In addition, when the final version of the statement is produced, it will name the school that the child will attend.

Often, there are disputes between parents and local education authorities about this process, including whether or not an assessment should be undertaken, whether a statement should be produced and as to the contents of the statement – including the support that the LEA have decided is necessary and which school the child should attend. In each of those circumstances, a parent has a right of appeal to the Special Educational Needs and Disability Tribunal. However, sometimes there are disputes which cannot be remedied by appeal to a Tribunal; for example where there is no dispute about a statement but where the local education authority simply does not arrange the provision set out in the statement. Then, other 'remedies' may be necessary, including possible high court action.

Separately, since September 2002, schools, local education authorities and colleges have had obligations not to discriminate against disabled children. These provisions are separate from the special educational needs framework described above and, broadly, disabled children now have a right not to be treated less favourably for a reason relating to their disability without justification, and also have a right to have reasonable adjustments made to policies, practices and procedures, so as to remove any substantial disadvantage that they may otherwise face at school. If schools, LEAs or colleges breach these provisions, again, a parent may bring a complaint to the Special Educational Needs and Disability Tribunal.

There are a number of sources for further advice and assistance. The Government has produced a useful special educational needs Code of Practice which is available from DfES Publications, PO Box 5050, Sherwood Park, Annersely, Nottinghamshire, NG15 0DJ, telephone 0845 6022 260, email dfes@prologue.uk.com. In addition, the Disability Rights Commission is a useful source of guidance on disability discrimination law and they also produce a Code of Practice on disability discrimination for schools. Information is available from their website www.drc-gb.org or you can obtain copies of the Code by post from DRC Helpline, Freepost MID02164, Stratford-Upon-Avon, CV37 9BR, telephone 08457 622 633. Finally, there are a number of voluntary organisations which help families of children with special educational needs and disabilities, who are in dispute with their local education authorities and schools. In particular, IPSEA (the Independent Panel for Special Education Advice) is a very helpful organisation and they can be contacted on 0800 018 4016 (England and Wales), 0131 665 4396 (Scotland) or 0232 705 654 (Northern Ireland). Not many lawyers know about education law and if you do wish to instruct a solicitor, do ensure that s/he is a specialist.

This article appears courtesy of David Ruebain. David Ruebain is a solicitor specialising in education and disability law at Levenes Solicitors in London. Contact 020 8881 7777 or email druebain@levenes.co.uk

Inaccessible schools and the Disability Discrimination Act

The impact of the Disability Discrimination Act (DDA)

Three sections of the DDA apply to the school as follows:

Part IV – Education

The SEN and Disability Act 2001 extends Part IV of the DDA which now requires that you do not discriminate against disabled people in their access to education by imposing a planning duty. As a result the school is required to prepare an Accessibility Plan to cover the following areas: Increasing access to the school curriculum for disabled pupils; Improving the delivery of written information to disabled pupils; Improving physical access to the school environment. In turn, the Local Authority is obliged to formulate an Accessibility Strategy to cover all Local Authority schools which will relate to each school's Accessibility Plan.

Part III – Service Provision

Any area of a school that is visited by members of the public is covered under this section of the Act. Under this section, the Local Authority is obliged to make reasonable adjustments to these areas to make them accessible to members of the public.

Part II – Employment

As an employer the Local Authority cannot discriminate against disabled people in terms of employment, which may involve making reasonable adjustments to policies, practices or procedures or physical alterations to premises.

The MPS Society would like to hear from members who are experiencing difficulties in achieving appropriate special educational needs for their children particularly in relation to the DDA.

Please contact 01494 434156
or email advocacy@mpssociety.co.uk

Facilities grant hope as Wales ends means test

Disability groups in England have welcomed the Welsh Assembly's decision to scrap means testing for the families of disabled children who require adaptations to their home and called for similar action to ditch the measure in England.

Families applying for the Disabled Facilities Grant (DFG) were previously assessed on the income of both parents, but the abolishment of means testing could mean that the families of about 250 disabled children in Wales will receive grants to cover the full cost of adaptations they need.

The Assembly pays £33.5m in such grants every year to 5,000 people. Caroline Gordon, representing a coalition of the UK's leading housing, disability and children's charities said 'We are delighted with the decision in Wales and just hope that the review taking place in Westminster comes to the same conclusion'.

'There have been similar reviews in Wales and Northern Ireland, which have both concluded that abolishing means testing for children is the most logical way of helping disabled families, and we hope the same can happen to help families of disabled children here'. A review of the DFG in England is being undertaken by the Office of the Deputy Prime Minister and is set to deliver its findings in the next few weeks.

The Ellenor Hospice at Home

Ellenor Hospice at Home is a registered charity established in 1985. The aim of the organisation is to provide hospice care for patients of all ages living with cancer or other life-threatening illnesses, together with the support of their family and friends. The majority of this work takes place in the patient's home. The Ellenor works with the medical and nursing staff employed in the community and hospital to facilitate the provision of hospice care in the local community.

Every year the Ellenor helps more than 2000 people and provides a range of much needed services in North-West Kent and the London Borough of Bexley. A specially trained team of professionals and volunteers provide many services including palliative care consultants and nurse specialists, specialist care for children with life-threatening illnesses, counselling and bereavement care.

Ellenor Shining Lights Hospice at Home is a team of specialist children's nurses, doctors and other health and social care professionals, which works closely with families of seriously ill children. A 24-hour on-call facility, respite care, nursery nurse, play and music therapy are offered, within the comfort and security of the home. Nurses also carry out tests and treatments at home, preventing unnecessary trips and stays in hospital. Supporting in this way ensures the whole family is cared for.

ellenor
hospice at home

For more information visit **www.ellenorfoundation.org** or phone 01322 221315

Society for Mucopolysaccharide and Related Diseases

National Registered Charity No. 287034

Become a Friend of MPS

Would you like to show your support by becoming a Friend of MPS? We would welcome relatives, friends, overseas MPS families, professionals or indeed anyone interested in the work of the Society or the field of MPS & Related Diseases. This would encourage us, help us plan for the future and bring about more public awareness for this group of rare, genetic, life-limiting diseases.

What are the benefits of becoming a Friend of MPS?

Membership number and card
Quarterly colour MPS newsletter
Quarterly colour fundraising newsletter
Annual report and accounts
Regular publication updates
Information on and preferential rates at MPS events
Priority ordering of MPS & Corporate Christmas cards

Phone 01494 434156
or download a form from
www.mpssociety.co.uk