Newsletter

The Society for Mucopolysaccharide Diseases

National Registered Charity No. 287034

Spring 2002

Samantha's Wish

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PARIS CONFERENCE BOOK NOW

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MPS Newsletter Spring 2002

'CARE TODAY, HOPE TOMORROW'

What is the Society for Mucopolysaccharide Diseases?

The MPS Society is a voluntary support group founded in 1982, which represents from throughout the UK over 1000 children and adults suffering from Mucopolysaccharide and Related Lysosomal Storage Diseases, their families, carers and professionals. It is a registered charity entirely supported by voluntary donations and fundraising. It is managed by the members themselves and its aims are as follows:-

- **To act as a Support Network for those affected by MPS diseases 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 MPS Society meet these Aims?

Advocacy Support
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 Young People and Adults with MPS
Empowering individuals to gain independent living skills, healthcare support, further education, mobility and accessing their local community

Regional Clinics, Information Days and Conferences
Information days and 11 regional MPS clinics throughout the UK

Regional Events
Social events held throughout the United Kingdom for mutual support

National Conference and Sibling Workshops
Held annually and offering families the opportunity to learn from professionals and each other

Information Resource
Publishes specialist disease booklets and other literature.

Quarterly Newsletter
Containing information on disease management, research and sent to members free of charge.

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

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

DIRECTOR'S REPORT

This is the last newsletter before the International Symposium on Mucopolysaccharide and Related Diseases to be held 20th - 23rd June 2002 in Paris. We are delighted that so many of you will be making the journey to Paris to join the foremost scientists and clinicians from around the world working on MPS and other Lysosomal storage diseases. I am sure we all stand to gain a lot by coming together. If you haven't yet booked there is a small number of subsidised places left for our members but do hurry.

Following the huge success of Jeans for Genes in 2001 the Society has been able to confirm the second year of a three year Project Grant to the Institute of Child Health, London and the Royal Manchester Children's Hospital totalling £190,787 to research:

Project 1
Expression of novel mutations found in patients with types I, IIA and IIIB

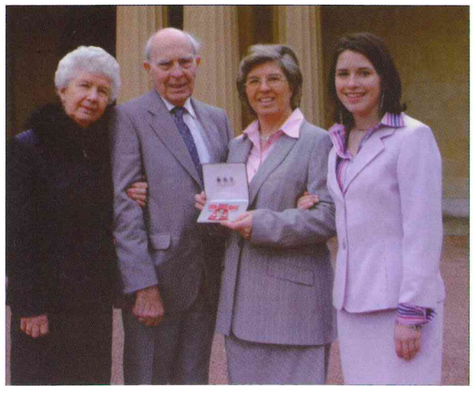
Project 2
Gene Therapy for Mucopolysaccharide Diseases using Herpes Virus Vectors

Project 3
Gene Therapy using marrow-derived mesenchymal stem cells and investigation of tolerance to foreign proteins after Gene Therapy

Project 4
Antibody production

A fifth grant has also been added to this project grant researching Autologous Mesenchymal Stem cells (MSC) as a Target of Genetic Manipulation in the Management of MPS II totalling £22,869. A sixth project to carry out this research for MPS IIIA and IIIB totalling £24,000 will also go ahead once we have sufficient funds to meet the cost of consumables.

The Trustees have been looking for some time at the need to enhance the clinical services to individuals suffering from MPS and related diseases based on current best practice. To meet this objective the Trustees have committed a Jeans for Genes grant of £40,000 per year to three years for this purpose and I hope to be able to give more detail in the next newsletter.



Recognising the importance members attach to our support and advocacy service I am delighted that two new Development Officers will join the Society during April to provide this service. We were sorry to say goodbye to Antonia at the end of February and thank her for all her hard work over the past year and wish her well in all she does in the future. Antonia is succeeded by Sophie who joined the team as Assistant Development Officer in March.

Finally may I express my utmost personal thanks to all of you who telephoned, wrote or sent cards and flowers on the occasion of my being awarded the MBE. The messages were most touching and very much appreciated. Robin stepped aside to let my parents and Lucy come with me to Buckingham Palace on February 13th, the same day that Rudolf Giuliani (ex mayor of New York) received an honorary knighthood from the Queen. It was a very special occasion and I am very grateful to my parents who gave a reception afterwards at St. Ermins Hotel.

Christine Lavery
Director

OFFICE NEWS

'21 years of Bone Marrow Transplants for MPS and Related Diseases - The Report'

This publication relays the extremely valuable information exchanged at the Conference held in June 2001.

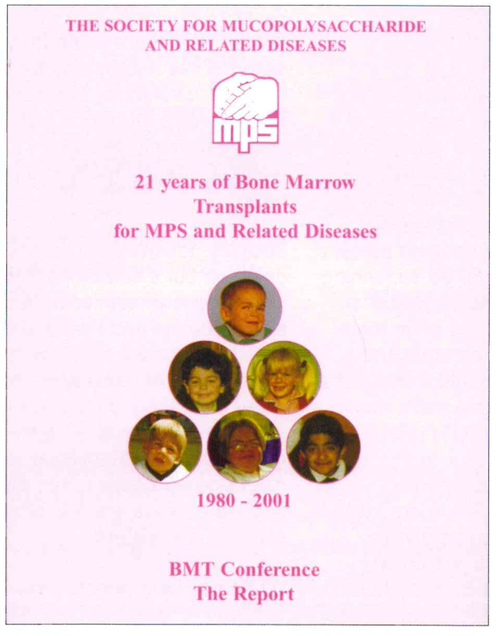
World recognised specialists review 21 years of Bone Marrow Transplantation. They give detailed information on the orthopaedic treatments many children and young adults have undergone. Angela Brown expresses a mother's perspective of BMT while Christine Lavery examines outcomes affecting the education of children suffering from MPS and related diseases post-BMT. In addition, the Report gives an insight on other forms of treatments currently being developed and on trial.

Whether BMT is an option you are considering as a parent or professional or you would like to find out about the advancement in therapies for Mucopolysaccharide and related diseases this publication will inform you.

To purchase a copy please fill in and return the enclosed order form.

Many thanks for sending me a copy of the proceedings from your conference of last year on transplantation for Hurler's Syndrome. I found the conference immensely informative, even as somebody who works in depth with this group of children. To see it committed to paper so that I can properly share it with all my colleagues is invaluable and I have already asked my nursing staff to purchase a copy of behalf of our transplant unit.

Dr Colin Stewart
Consultant senior Lecturer in BMT, Metabolic and Genetic Diseases




BONE MARROW TRANSPLANT CONFERENCE 2001

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'I've Got Morquio's' booklet feedback

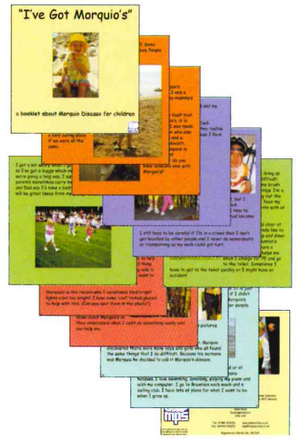
This is beautifully produced and easy to read. It comes over well as by children and for children. It will be excellent for explaining to a child's classmates and friends about the condition and open discussion and knowledge about other disorders as well. Useful in a classroom with a teacher to guide discussion.

The photo illustrations are particularly encouraging and positive.

Peter Robinson, Consultant Paediatrician, Hospital for sick Children Yorkhill, Glasgow

Thanks for the Morquio booklet. Just to say we are very pleased with the publication and felt that this needed to be done at some stage for younger children/teenagers to understand. It is also a good booklet for those who do not speak very good English.

Kind regards
Asma Seedat



MEMBERS' NEWS

'We don't know how long Bethany's here for'

This article appeared in *The Independent* shortly before the documentary 'Bitter Inheritance' featuring the Allen family was broadcast on BBC television. We reproduce the article here, by kind permission of the newspaper.

John Allen, a warehouse worker and his wife Joanne have three children, Jasmine (7), Bethany (4) and Thomas (10 weeks). Their daughter Bethany suffers from a rare disorder called Sanfilippo. which only occurs when both parents carry the gene. Bethany is expected to die before she reaches adulthood. They live in Tamworth, Staffordshire.

John

Bethany was diagnosed with Sanfilippo two years ago. We had taken her to the doctors with what we thought was croup, and so we were obviously devastated when we discovered she had a terminal illness. She stayed overnight with a specialist who became suspicious when he noticed something in her facial characteristics typical of Sanfilippo sufferers. They often have bushy eyebrows, a short neck and a high forehead. We thought it was just a family trait. I had no idea there was anything wrong with her.

They told us she had suspected mucopolysaccharide. There are various types and Bethany had type three which is Sanfilippo. It's present in everyone's bodies but Bethany hasn't got the enzymes to break it down, and eventually it starts attacking the organs, the central nervous system and the brain. I was devastated. We both were. We broke down completely. When the doctor told us there was no cure and that she would die from it, it was like she had died already. Everything was spinning and I kept thinking of all the things she would never be able to do.

It stretched the relationship between Joanne and I to the limit. We sat and talked and realised we had to stick together. We've ended up being closer. We want to get it right for Bethany and you can only do it as a team.

Although she's still in the early stages of the illness it can be very hard work; she's becoming more and more hyper-active. She doesn't seem to need much sleep, and gets up a lot in the night. Joanne and I take it in turns to stay up with her but we are both exhausted.

There are money worries too. We need about £20,000 for an extension to the house for Bethany, a bedroom with a special care bed and breathing equipment. I don't know how we'll ever raise the money.

People have been very supportive though. Jo's mum and dad and her sister all live nearby and we all pull together. Everybody is around to help, although Jo's mum is ill with Huntington's disease and it's getting worse. She's not really in a position to help.

Bethany doesn't seem to realise what's going on but it can be hard for Jasmine who is quite protective of her little sister. Joanne and I wanted another child because we didn't want Jasmine to be left on her own when her little sister dies. Even though we knew there was a one in four chance that a child of ours could have Sanfilippo we took the risk because I grew up an only child and was always envious of people with brothers and sisters.

When you hear people moaning about trivial things or when you see people shouting at their kids you want to make people realise what they've got, how lucky they are to have a healthy child. I don't know how long she's here for and it's an everyday battle but you can't give up. No "ifs" or "buts".

Joanne

I always felt there was something wrong with Bethany. She was very different to Jasmine. Bethany didn't seem to be bothered about anything. She wasn't even interested in playing. She wasn't interested in talking or walking and used to scream at people. She was frightened of strangers and hated any kind of eye contact with them. I felt like I was going mad sometimes. I was afraid that it might be my fault because I had had post-natal depression and didn't put as much time in with Bethany after she was born as I should have done. When she was diagnosed I was devastated, but in some ways it was a relief to find out why she was acting like that. When I found out about her illness I understood her and wasn't scared anymore.

John had always been the stronger one in the partnership whereas I'm more emotional. But when we found out he went to pieces for at least a couple of weeks. He was a walking zombie. I tried to keep up some sense of normality and routine but it was overwhelming. We do bicker a bit but we realise as soon as we start and pull ourselves together again. We've learned to compromise because we had different ideas about how to treat the kids. I don't want to spoil them. I try to treat Jasmine the same and take her out on her own so she gets a bit of extra attention.

Jasmine was six when we found out about Bethany. She understands that there's something wrong with her sister: The Society for Mucopolysaccharide Diseases has been brilliant. When we went to our first conference, Jasmine was looked after by people with experience and they helped her express her feelings about it.

I try not to think about the future too much. I worry about Mum as well but Bethany has to be my priority. I know what's going to happen and I've seen kids in the latter stages of the disease. It terrifies me, and I can't imagine Bethany being like that so I just try to take it day by day. When I hit rock bottom it's the people around who pull me through: my best friend, my sister and John. When either one of us is down we seem to pull each other up and help each other put things into perspective.

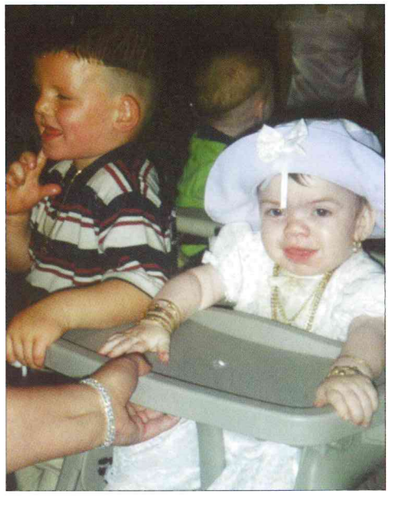
Interview by Xenia Gregoriadis

The Gee family trip to Disneyland Paris

The Gee family went to Disneyland Paris in November. "We took Ellie (Sanfilippo, aged 12) and Isabelle (aged 8) for a couple of days and a friend of ours came along and acted as carer. I think Ellie enjoyed it as she got to go on a few rides and Issy had a ball!"

Maggie Stokes

This is a photo of Maggie Stokes at her Uncle's wedding



MEMBERS' NEWS

Keegan Lovick

Hi! My name is Melissa Al Qadi and I am Keegan Lovick's mum. Keegan is seven years old and has Hurler disease. I would like to let you know how Keegan is getting on.

We had a very busy time last year. Keegan had his tonsils and adenoids removed in June 2001 at the Royal Manchester Children's Hospital. He recovered well but, unfortunately, when he eats, food comes out of his nose. As you can imagine, this can be rather embarrassing especially eating out in public when Keegan has strands of spaghetti dangling from his nostrils!

On 14th September I re-married and Keegan gained a new step-daddy who he calls "Baba" which is Arabic for "Daddy". Keegan loves his step-daddy very much and is spoilt rotten. Baba is also by his side every time he goes into hospital (which has been often).

Keegan has oxygen and nebulised adrenalin at home which he now needs from time to time. Thankfully Keegan doesn't need this too often any more since he had the flu and pneumonia vaccinations.

In November we moved house and Keegan had his 7th birthday party in the new house two days after we moved in.

Keegan is doing much better than how we expected at seven years old. He is such a happy little boy. Everyone who knows Keegan remarks that they have never seen him angry or crying. He is always laughing and is a very loving little boy who loves cuddles and kisses. Everyone who knows Keegan loves him.

Melissa Al Qadi

Samantha makes a wish

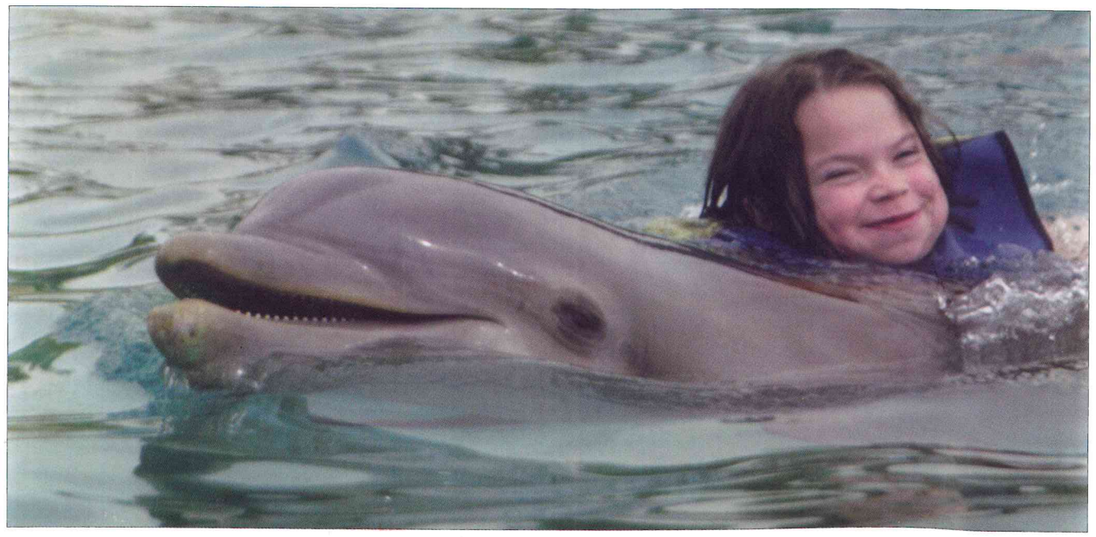
We all had a great time in Florida on our holiday, made possible by Make a Wish.

Samantha's wish was to swim with the dolphins which she did at Discovery Cove. While we were there she also snorkelled in the coral reefs among lots of tropical fish.

We also visited the Magic Kingdom, Animal Kingdom, Seaworld, Universal and MGM Studios. A very busy week!

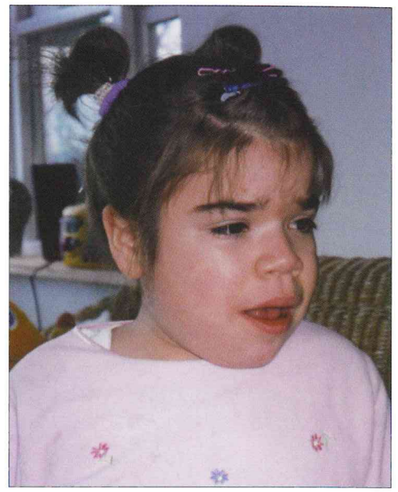
Make a Wish were excellent and we couldn't have asked for any more.

Vicki Brockie
01280 847315
www.make-a-wish.org.uk



MEMBERS' NEWS

Louise Lewis writes about her daughter Georgia



It is January 2002, the beginning of a new year, and I've finally got around to writing an article for the Newsletter, although by the time I've finished, it may not get published until next Christmas! I always read the Newsletters, and often think I should write in, but as always, I just never seem to get the time! Let me introduce myself and my family:

My name is Louise Lewis, I am 37 years old and live in Chepstow, Monmouthshire with my family - Jon is my husband, 39 years. We have been married for 8 years. Christopher is my son, he is 15, and last, but not least, there is Georgia, aged 6. Georgia has Sanfilippo A, and was diagnosed in January 2001. I can't believe it's been a year since the diagnosis, it's gone so quickly, and we've done so much. It still seems like yesterday that me and Jon were sat in the Consultant's office, hearing the devastating news. We were both numb with grief, and also, relieved that we finally knew what was wrong. In a strange way, a big weight was lifted from us, as we now no longer had to strive and push Georgia to do things that we knew she would never be able to do, but were able to just accept her for what she is.

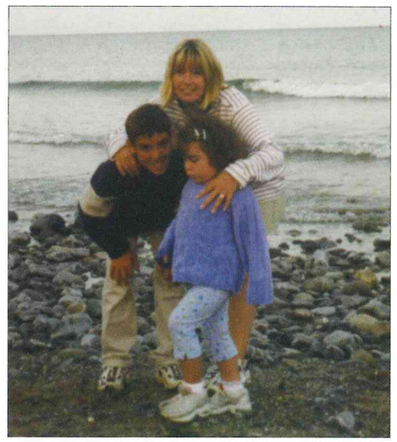
I'm extremely proud of how my family has coped and dealt with everything this year, including my parents and my sisters. A diagnosis like this affects everyone, not only family, and we're very lucky to have good friends who are very supportive, and who know Georgia well, which certainly helps!! Myself and Jon both work full-time. Jon is a Production Team Leader, and I have recently been promoted within my company, and now work as a Project Consultant. The Company I work for are very supportive, and together we have raised almost £900 for the "Jeans for Genes" campaign this year, and currently we are helping to raise money for Ty Hafan, the children's Hospice of Wales, where Georgia stays occasionally.

At home, we have had an extension built on the house, so Georgia now has a big playroom. We have made it "Georgia proof', which basically means no carpets (wooden floors), light switches/plug sockets out of reach, a TV and video, and no ornaments!! We're hoping to get some "soft play" equipment i.e. mats, climbing blocks etc. Georgia loves her new room, which has lots of different kinds of lights, which she loves to look at.

Well, what can I say about Georgia. She has all the expected characteristics of Sanfilippo, but I look beyond that, and I see "my angel". She is beautiful, adorable, funny, loveable, innocent, but more importantly, she is my inspiration. She makes me realise that life is for living, not to be taken for granted, and I do not worry about anything anymore! Everything is now put into perspective, and she has brought out the best in me, and all those around her. She is perfect in our eyes.

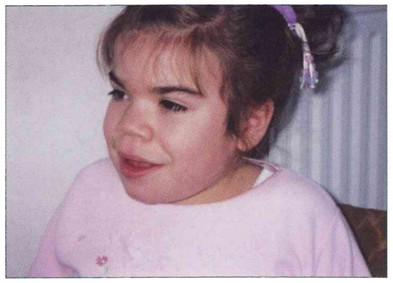
I would be happy if anyone wishes to contact me, please write via e-mail to:

louise.lewis@udex.com



MEMBERS' NEWS

A personal experience of the MPS Society's Befriending Scheme



My daughter, Georgia, aged 6, was diagnosed with Sanfilippo A, in January 2001. This last year has gone incredibly quickly, and initially I was anxious to get as much information as possible about Sanfilippo, and I relied a lot on the internet to provide all the answers. Through this, I now regularly have input, and correspond to people on the American MPS Website.

However, America is a long way away, and I really wanted to get in touch with people in the same situation as me, but a bit closer to home! Antonia Crofts, of the MPS Society asked if I would like to use the "Befriending Link", which is organised by the MPS Society, and I said I would. Very soon, I was sent a contact name & number (with consent of the contact person!) and I got in touch. It was great to speak to someone in exactly the same situation, and now we regularly e-mail each other, with advice and tips, and just a general chat.

I have now been given another contact number too, so there's somebody else I can chat to!! I think it's a great idea to use the Befriending link, as quite often, it's easy to feel isolated, but knowing there are other people who understand your situation makes it a whole lot easier!

If anyone wishes to contact me, please do so, either via e-mail or by phone:

louise.lewis@udex.com

What is the befriending scheme?

Over the last couple of years the MPS Society has established a befriending scheme which offers to link individuals and families throughout the UK and on occasions internationally. Such links are often requested by an individual or a family who want to be in contact with others who share a similar experience of MPS diseases, a particular issue such as BMT, or who come from the same ethnic or religious background or live in the local area.

How is the befriending scheme run?

- Family or individual requests a befriending link
- The Society identifies appropriate individuals or families to act as befrienders and writes to these individuals or families to obtain written permission to pass on names and contact details to the individual or family who requested the link
- Upon receipt of written permission from the befriender family we pass on the contact details to the original individual or family requesting the link so that they can make contact.

Confidentiality is a priority of the befriending scheme and no names, personal stories or contact details are passed to any third party without us obtaining written permission beforehand.

Who can request a befriending link?

Any member can request a befriending link, and many individuals and families have already used the scheme. If you want to take part, the Society will always try and find out what issues are important to you and why you are interested in making contact with another individual or family. In this way we can identify the most appropriate befrienders for your particular link and both parties can get something beneficial from being in contact.

Once the befriending link has been established...

Once the link has been made the level of contact you have is entirely up to you. Many individuals and families just have telephone contact whilst others meet up regularly. At all times the MPS Society is always available to offer support or answer any queries, but from the positive feedback and number of requests for links the Society receives it seems that the scheme is proving a very successful and worthwhile initiative.

If you want to take part in the befriending scheme please contact the MPS Society.

MPS CLINICS

Birmingham Clinic

The MPS regional clinic programme for 2002 is being supported by funds from the 2001 Jeans for Genes appeal.

In the first two months of 2002 two regional clinics have been held, Birmingham and Newcastle.

Hello, my name is Joanna Wilson, I am a Scheie patient. On Friday the 25th of January 2002, I attended the MPS clinic at the Birmingham children's hospital. This is the third clinic I have attended since I was diagnosed, I always look forward to these clinics, as it is an opportunity to meet all the experts on MPS; once again I was not disappointed.

At the clinic I find that I am able to talk freely about all my problems that I believe are related to MPS, everybody is so friendly and easy to talk to. I do find that the team, particularly Dr Ed Wraith, explain things to me in a way in which I thoroughly understand.

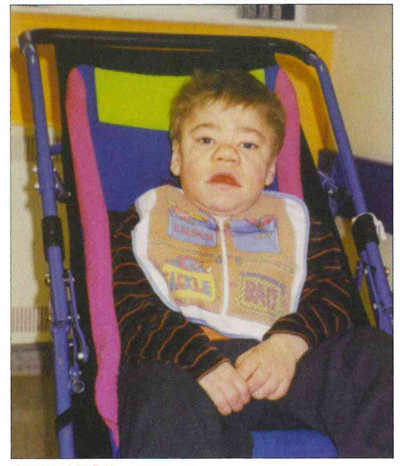
I was also pleased to see at this clinic Dr Chakrapani, who is the new consultant paediatrician specialising in metabolic disorders at the Birmingham children's hospital. This was my second meeting with Dr Chakrapani and I think that all MPS patients attending the Birmingham children's hospital will benefit from his appointment.

It is always lovely to see the friendly faces of the team from the MPS office, in this case Ellie and Antonia.

Not only do I feel that I benefit from attending these clinics, but I know that my Dad looks forward to them as well.

Joanna Wilson

Bethany - MPS III

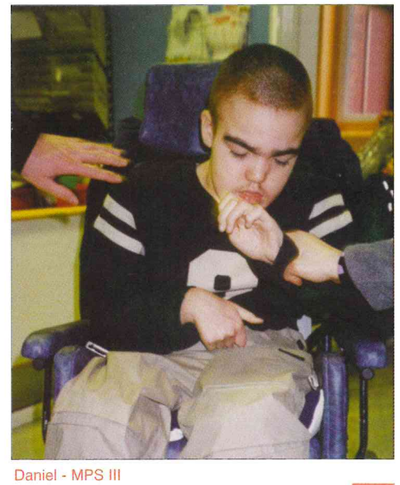


Jamie - MPS II

Retirement of Beryl Holmes, Clinical Nurse specialist - Birmingham

Beryl Holmes retired as Clinical Nurse Specialist in Birmingham Children's Hospital in the summer of 2001. It was to Beryl the MPS Society turned in 1998 when the need was identified for specialist MPS clinics to be held twice yearly in Birmingham. Beryl worked in partnership with the Society to achieve this MPS clinic. Since then a further 7 MPS clinics have been held and the legacy for these clinics has passed to Joy Wright and Dr Chakrapani working in collaboration with Dr Ed Wraith and the MPS Society.

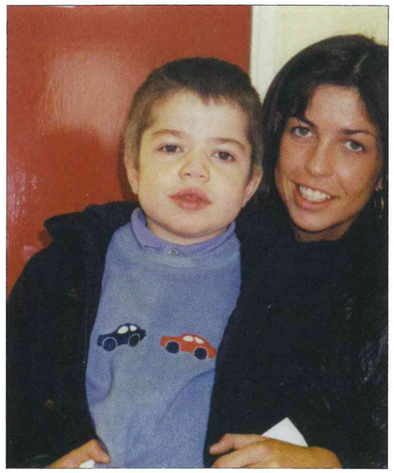
At Beryl's last MPS clinic in July 2001 she was presented with a bouquet of flowers in gratitude for all her work with those affected by MPS, their families and carers. We wish Beryl a very healthy and happy retirement.



Daniel - MPS III

MPS CLINICS

Newcastle Clinic - Tuesday 5th February 2002



Daniel - MPS II

It was a sunny winter's day when Antonia and I flew up to Newcastle for the MPS annual clinic held at the Royal Victoria Infirmary.

We received a very warm welcome from the team at the RVI, particularly Dr Martin Ward-Platt who was supporting the children attending the clinic before Dr Rylance who is to support this clinic in the future beginning in Newcastle in March 2002, and Dr Nicky Leech, an adult physician at the RVI.

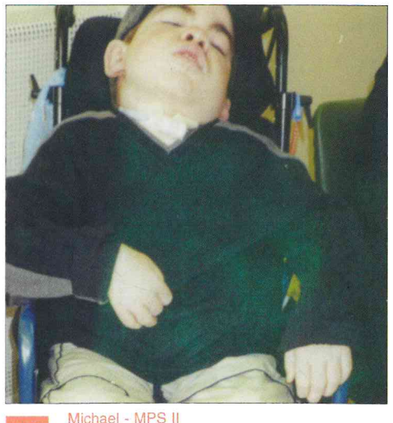
The MPS regional clinics are set up to provide a holistic service of advocacy support from the MPS Society's Development Team alongside medical support from Dr Ed Wraith and local medical staff. Even when an individual or family do not have specific advocacy needs the regional clinics enable meeting the Development team and learning of new developments and initiatives. The medical team at Newcastle ensured a high quality service was delivered and that every member of the MPS Society was met and offered the opportunity to be supported.

From the feedback received we know this regional clinic is very much appreciated by the Society's members in the North East. We extend our thanks to Dr Martin Ward-Platt and his team for hosting this year's clinic and look forward to holding the next clinic in February 2003 with Dr Rylance.

Our thanks also go to Dr Wraith for his continued commitment to the Regional clinic programme, and particularly for travelling to Birmingham and Newcastle in the winter months of this year.

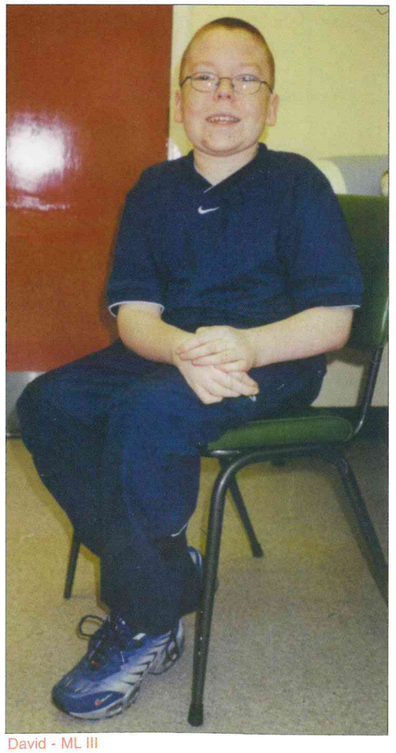
Ellie Gunary
Assistant Director

Andrew & Jeanette - ML III



Michael - MPS II

Daniel - MPS II



David - ML III

MPS Newsletter Spring 2002

MPS CLINICS

BMT Clinic - 1st March 2002

In addition to the regional clinics, the BMT clinic was held on 1st March 2002 at the Royal Manchester Children's Hospital and provided an opportunity to catch up with those children who have attended this clinic before and see the new faces of children who have had a Bone Marrow Transplant recently or are about to undergo a transplant.

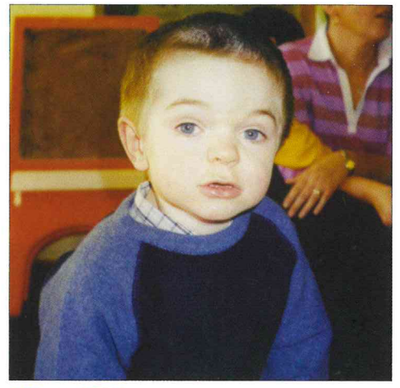
The clinic offers a very important multi-disciplinary approach to supporting children post transplant, an approach which includes an opportunity to meet the Development Team and be supported with any advocacy needs. For some families it is the first time they have met children who have had a transplant and much mutual support and information is shared.

This clinic was no exception and whilst the children played, parents met together in the waiting area.

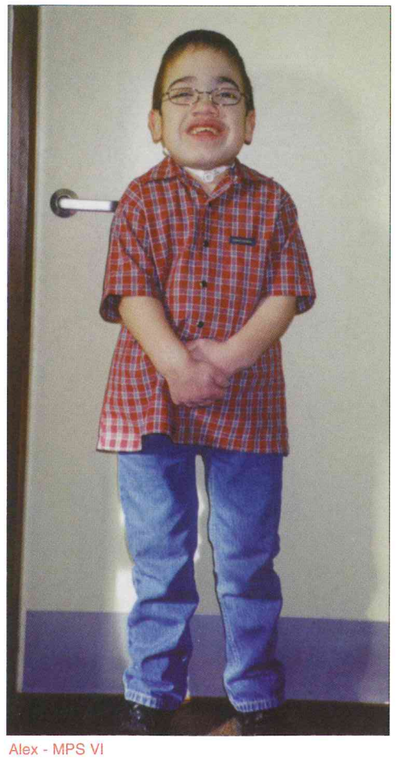
Bernie and Jill, at the Willink Unit ensured the clinic ran smoothly. Our thanks go to both of them and particularly to Bernie for arranging sandwiches, which were greatly appreciated. Our thanks go to all the medical team at the Royal Manchester Children's Hospital for their continued support and commitment to all children and adults with MPS, their families and carers including those who had the transplant.

Ellie Gunary
Assistant Director

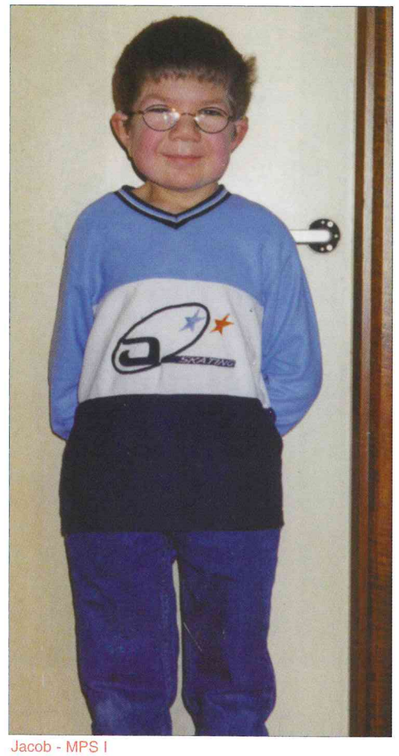
Isaac - MPS I



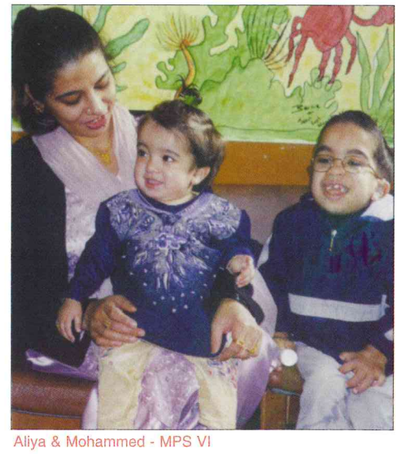
Matthew - MPS I



Alex - MPS VI

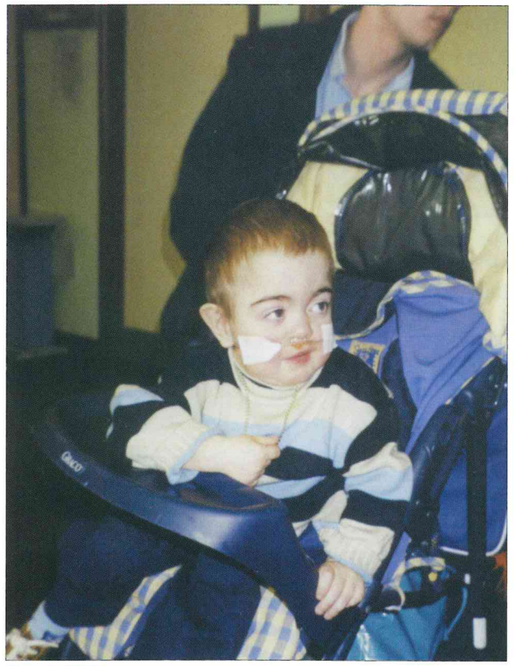


Jacob - MPS I

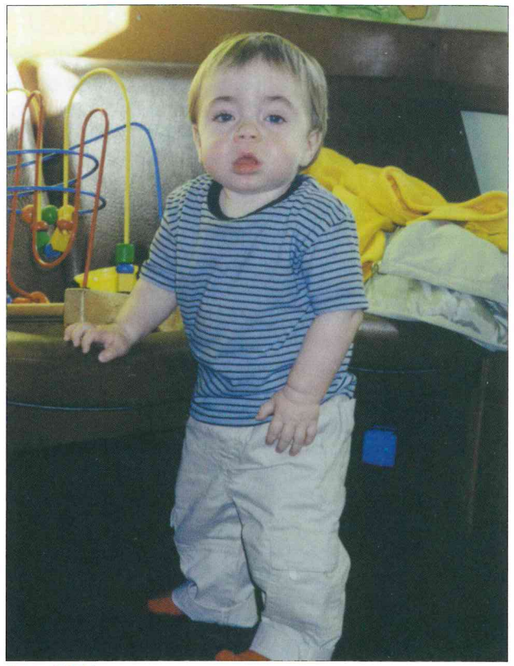


Aliya & Mohammed - MPS VI

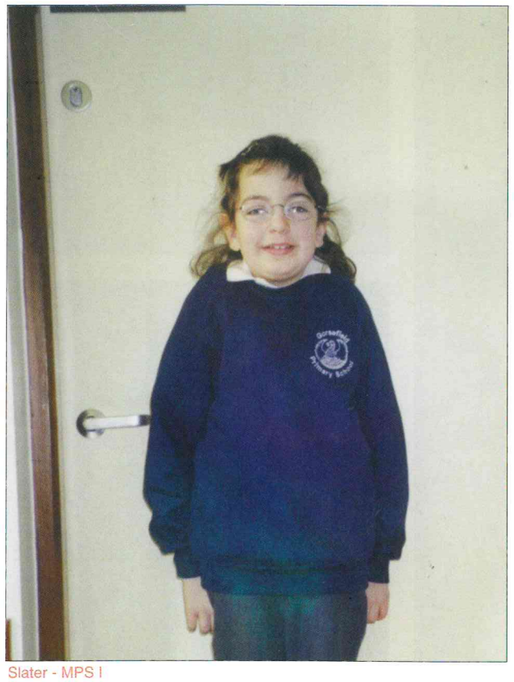
MPS CLINICS



Barker - MPS I



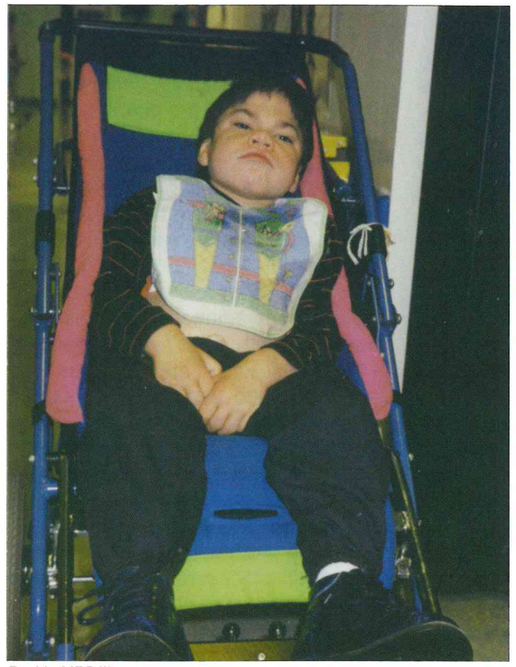
Jake - MPS I



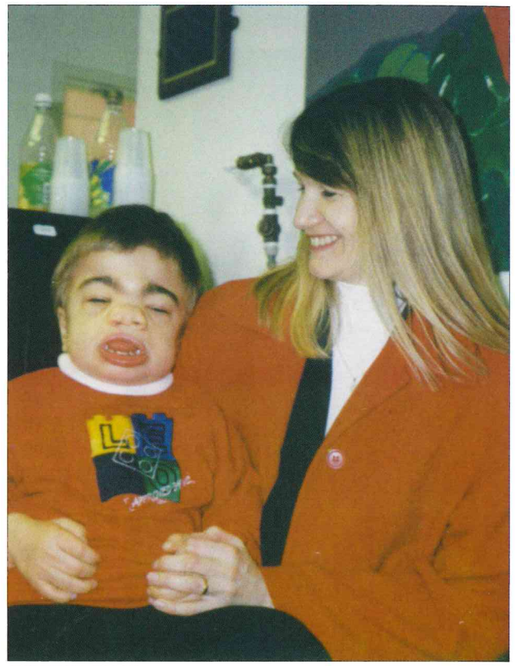
Slater - MPS I

Mohammed & Sohaib -

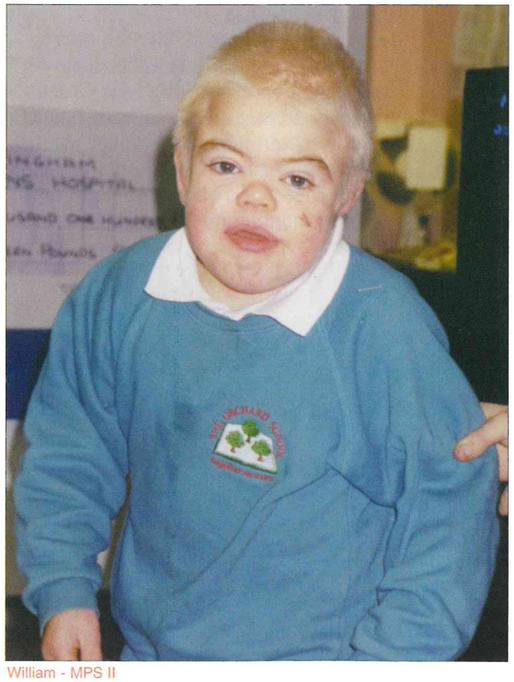
MPS CLINICS



David - MPS III



Alexander - MPS II

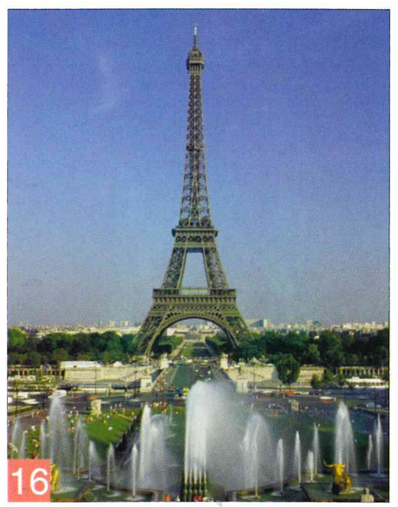


William - MPS II

Samantha - MPS III

PARIS CONFERENCE

There's still time to book your places!



7th International Symposium on MPS and Related Diseases
3rd Scientific Lysosomal Storage Disorders Congress
Paris, 21st to 23rd June 2002

We've had a good response, with firm **bookings** from quite a lot of families from all parts of the UK, as well as from professionals and families in other countries. If you're one of the many people who have expressed an interest in the event, but haven't got round to filling in the forms yet, please do so as soon as possible. There is a limit to the number of members we can subsidise, and it is vital that we know the number of participants for the purposes of accommodation, catering, childcare and so on. The better bedrooms will also be allocated on a first come, first served basis. Remember that, for MPS Society members, two adults can spend the weekend in Paris at a major conference at a luxury hotel for only £200, and children suffering from an MPS disease go free!

We do hope that as many of you as possible will be able to come to Paris. We appreciate that some of you may not feel able to cope with taking the whole family to France, and staying away from home. Remember though that you will probably be in the same boat as someone else, and there will be many people on hand to help once at the conference. You may also have financial concerns. We are able to apply to charitable trusts local to you, to assist with the costs of the conference registration if you need it, and there are travel bursaries available up to £200 per family.

The **conference** will be broadly along the lines of the UK National MPS Conferences that have taken place in Northampton. There will be presentations by professionals in the field of MPS and related diseases, and the children will be cared for by volunteer carers, many of whom will be familiar to you from the UK Conferences. The difference is that it will be on an international scale. You will therefore have the opportunity to hear about the experiences of a host of world-renowned doctors, find out about new therapies from the pharmaceutical companies that manufacture them, learn from the scientists about the research taking place now that could provide a cure of the future, and meet parents and sufferers from other countries.

The accommodation is provided in the conference venue itself, so everything is on hand. The **Hotel Sofitel** is very comfortable and attractively furnished, and offers high quality food. It is a 23-storey building, with wonderful views from the top, yet it is easy to get around thanks to the many lifts. There is even a swimming pool for the use of the guests. All the rooms sleep two people, but we can ask the hotel to add an extra bed, a cot, or a mattress on the floor. Some rooms have an interconnecting door to the neighbouring room, making them ideal for larger families, as an older child can sleep in the room next door with other siblings.

Friday evening is **free time** for everyone. You could take the metro into the centre of Paris – the Place de la Concorde, at the end of the Champs Elysées, is only ten stops from *Balard* station, about 5 minutes' walk from the hotel. Stroll around, soak up the atmosphere, and admire some of the famous monuments. There'll be musical events going on too, as Midsummer's Day is the Festival of Music in France. We'll be able to give you more information about this, and other attractions, nearer the time. If you would prefer not to have to travel far (public transport in Paris, like London, is sadly not very user-friendly for disabled people) don't worry because there is entertainment on your doorstep. Next door to the hotel is *Aquaboulevard*, a huge complex containing the largest water park in Europe (waves, fountains, jacuzzis, 7 slides, 'beach', air and water at 29°C...) plus food outlets including McDonalds, video arcade, gym, 14 screen cinema, and a few shops. Alongside the building are outdoor sports facilities and the heliport, set in a public park with wide tarmac paths and benches.

PARIS CONFERENCE

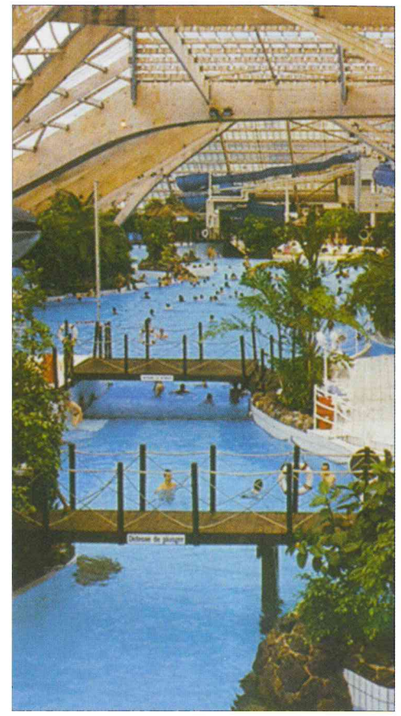
In an attempt to take some of the strain out of **travelling to Paris**, we have arranged coach travel for those of you who are interested. The coach, which will have wheelchair access, will leave from a mainline station in central London around lunchtime on Thursday 20th June and will pick up from a motorway services on the A2/M2 route in Kent shortly after. You will cross the Channel by ferry, and arrive at the stopover hotel by early evening. This hotel is likely to be the Novotel near Arras, located near the motorways and next to a development of shops and restaurants. Up to four people can share a room, and cots are available. The coach will take you on to Paris on Friday morning, and will bring you back to England on Sunday 23rd, arriving in London in the evening. The cost per adult is £75, and £50 for children of all ages. This includes return travel and bed and breakfast accommodation en route but does not include the evening meal on Thursday. We regret that we are unable to offer free places to MPS sufferers, although remember you may apply for a travel bursary towards these costs.

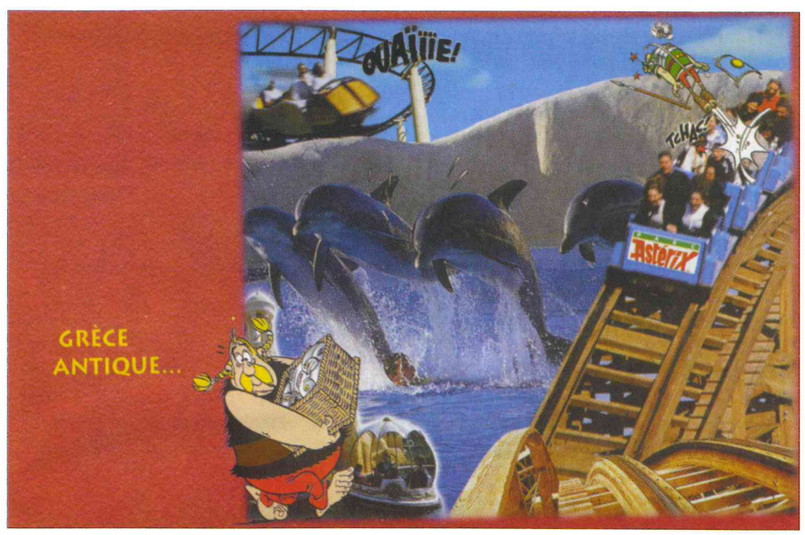
The region in which you live, or the needs of your children, may mean that it would be more convenient for you to fly, take the Eurostar train, or drive yourself. Groups of 10 or more often qualify for reductions, so you may want to try travelling with other families from your area. If you wish to extend your stay at the Sofitel, the conference rate of 148 € (approximately £92) per room per night will apply on Wednesday 19th, Thursday 20th and Sunday 23rd.

Sunday lunch at the hotel is an optional extra, at a cost of 28 € (about £17) per adult and half price for children. Those people travelling on the coach to England will not be able to stay for this, and others too may prefer to get something cheaper outside the hotel. If you want to join the lunch however, we must have the payment in advance.

If you have any problems obtaining **travel insurance**, try Travelcare Ltd. on 0800 181532. My thanks go to Gordon Rowe for helping me with this research! After being refused by a different company, his daughter Faye, who has Sanfilippo, was accepted by Travelcare with an additional premium of only £6.

The proposed trip to **Disneyland Paris** on Thursday 20th will not unfortunately go ahead, as it was proving to be an expensive and complicated option. The children aged 4 to 17 will however be taken to the highly regarded theme park *Parc Astérix* on Saturday.





(Opposite page from top)
One of the bedrooms and its ensuite bathroom,
The coach that has been booked for the trip,
The hotel where we hope families taking the coach will be spending the night
(Above) The Aquaboulevard water park
(Left) Parc Asterix

NEW MEMBERS AND IN REMEMBRANCE

New Members

Mr & Mrs Lloyd's sons Ben aged 4 and Jake aged 17 months have recently been diagnosed with Sanfilippo Disease. The family live in the Midlands.

The Society has recently been contacted by Ian & Ann Hedgecock who live in Wales. Ian has Fabry Disease.

In remembrance

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

Russell Jenkins who suffered from Sanfilippo Disease
18 May 1988 - 2 January 2002

Richard Matthews who suffered from Sanfilippo Disease
12 November 1973 - 4 January 2002

Nicholas Hunt who suffered from Hunter Disease
3 April 1979 - 11 March 2002

Kim Eggleton who suffered from Sanfilippo Disease
30 October 1971 - 2 April 2002

Goodbye and thank you

Sid Shiff died on Sunday 10 February 2002 aged 77 years. Sid was a great supporter of the MPS Society over the years and raised considerable funds. We are deeply saddened to learn of his passing. Our thoughts are with his wife, daughter Selma and grandchildren David (MPS II) and Sarah.

Men Do Cry

I heard quite often 'men don't cry'
Though no one ever told me why
So when I fell and skinned my knee
No one came to comfort me.

As I grew to reasoned years
I learned to stifle any tears
Though 'be a big boy' it began
Quite soon I learned to be a man.

Then one long night I stood nearby
And helplessly watched my child die,
And quickly found to my surprise
All tearless talk was lies.

And still I cry and have no shame
I cannot play that 'big boy' game,
And openly without remorse
I let my sorrow take its course.

So those of you who cannot abide
A man you've seen who's often cried
Reach out to him with all your heart
As one whose life's been torn apart.

For men do cry when they can see
Their loss of immortality
And tears will come in endless streams
When mindless fate destroys their dreams.

From a SANDS Remembrance Service

MPS ADVOCACY SUPPORT PROGRAMME 2002/2003

2002

MAY

Friday 3rd May - **East Anglia clinic**
Friday 10th May - **Northern Ireland clinic**
*Wednesday 29th May - **Cardiff clinic (Amended date)**
Thursday 30th May - **Bristol clinic**

JUNE

Friday 7th June - **Scotland clinic**
Friday 21st June - **International Symposium in Paris**
Saturday 22nd June - **International Symposium in Pais**
Sunday 23rd June - **International Symposium in Paris**

JULY

Sunday 7th July - **Childhood Wood Remembrance Day**
Friday 12th July - **Birmingham clinic**

SEPTEMBER

Fabry disease and new therapies conference

OCTOBER

Friday 4th October - **Jeans for Genes day**
Friday 25th October - **Childhood Wood Planting**
Thursday 31st October - **Bristol clinic**

NOVEMBER

Friday 1st November - **Cardiff clinic**
Friday 29th November - **Northern Ireland clinic**

2003

FEBRUARY

Tuesday 4th February - **Newcastle Clinic**

DATES TO BE CONFIRMED

South East clinic

It is hoped that a clinic will be held in the south east of England. As soon as progress has been made to secure the support of a local consultant and a date arranged with Dr Ashok Vellodi, all the individuals and families who would benefit from this clinic will be contacted.

Regional Days

A programme of regional days is being planned to include events throughout the UK at different times of the year including Christmas Parties.

National MPS Conference

After considerable thought and due to the superb programme being offered at the International Symposium in Paris, it has been agreed to provide additional help to get as many individuals with MPS and Related Diseases and their families to the Paris conference as possible. As a consequence the Society will not be holding a National Conference in 2002. If you want to come to the Paris conference but are concerned about travel and costs please call Fiona now.

INTERNATIONAL CONFERENCES

New global collaborative forming to benefit Lysosomal Storage Disease Community

Interest Grows in GOLD

On 7 -9 February 2002 twenty-two experts from ten countries met in Cannes, France, under the sponsorship of Lysosomal Diseases Australia, to consider a new collaborative effort on lysosomal storage diseases (LSD). Although each LSD is uncommon, taken together they affect between 3-5% of the world's population, either directly or indirectly, so constitute a substantial health issue. Well-respected basic and clinical researchers, patient advocates and pharmaceutical company representatives, including Christine Lavery, Director of the MPS Society, debated how collaboration could best improve diagnosis, treatment and care for all those affected by LSDs. By the close of the two day session, GOLD, Global Organisation for Lysosomal Diseases, had been born. In the coming months, they will work to turn the dream into reality.

Led by a steering committee of Michael Beck, MD (Germany), John Hopwood, PhD (Australia), Edwin Kolodny, MD (US), Christine Lavery (UK), Abbey Meyers (US) and William Sly, PhD (US), Cannes meeting participants felt that collaboration was needed now because of:

- Increasing understanding of disease aetiology
- Growing number of patient registries and genetic information databases
- Promising/approved diagnostics and treatments
- Committed cadre of researchers, clinicians, and patient advocates/educators
- Model programmes for patient education and support
- Broadening recognition of the value of coordinated action

Participants also felt that the collaboration would best work to:

- Understand the global incidence, prevalence, and natural history of the diseases
- Promote basic research on each LSD
- Encourage the development of new therapies
- Provide accurate patient and physician education
- Increase funding for research, diagnosis, education and advocacy
- Speak with one voice to regulatory agencies and the public

GOLD Adopts Ambitious Goals

Participants summarised the mission of GOLD as:

"To develop an international collaborative effort to improve the lives of all patients with lysosomal storage disease. GOLD will promote research and education on lysosomal storage diseases and their early diagnosis, effective treatment and provision of support for patients or those at risk. Its priorities and programmes will be determined by an alliance of researchers, clinicians, patient advocacy groups and industrial partners."

Meeting participants agreed that success would come only by including any interested party in the new organisation. The conclusions of the Cannes meeting will be widely disseminated through conferences, newsletters and word of mouth. Reactions and active involvement of patients and family members, researchers, clinicians, public policy makers, industry, and all others is encouraged. Comments about GOLD and offers of volunteer assistance should be directed to Christine.

Progress will be reported at the International Symposium on MPS and Related Diseases/3rd Scientific Lysosomal Storage Disorders Congress, 20 - 23 June 2002, Paris. There the steering committee will review what has been done to set up GOLD as an organisation, develop its brand identity, and raise the necessary funds. Discussion will also focus on creation of a Web-based information centre and an inventory of patient registries.

Mark Krueger and Associates, Inc. helped develop the programme and facilitated the meeting.

JEANS FOR GENES APPEAL

Jeans for Genes is a consortium of four charities, one of which is the MPS Society, working together to raise awareness of genetic diseases and raise vital funds for research.

Jeans for Genes holds an annual 'Jeans for Genes Day' in October. The next one is Friday 4th October 2002 when schools, businesses and members of the public will be encouraged to raise funds by wearing jeans for the day.

As part of its strategy Jeans for Genes has agreed to diversify and support additional disease groups by giving up to 10% of the Jeans for Genes income for the benefit of research into other genetic diseases. This will bring new stories to the campaign, encourage wider participation and in turn help raise more funds.

Thirty seven charities applied for guest charity status from which eight were short listed for interview. Each charity was asked to present details of that organisation, how they would support the Jeans for Genes campaign and how the funds would be spent. Four charities were invited to be guest charities in 2002:

The Haemophilia Society
The Jennifer Trust for Spinal Muscular Atrophy
The Ataxia Telangiectasia Society
Alstrom Syndrome UK

A further charity was selected for guest charity status in 2003:

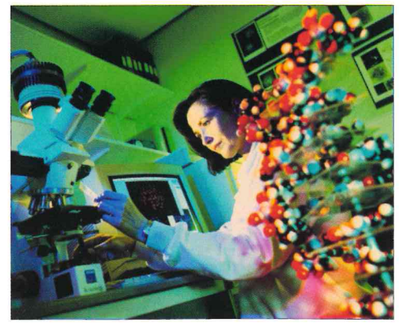
Rett Syndrome Association

The MPS Society along with the other Jeans for Genes partners extends a warm welcome to these five groups and looks forward to working together to raise an even higher profile for the Jeans for Genes campaign this year and in the years ahead.

So far last year's campaign has raised

£2,671,182.49

and counting.....



Visit www.jeansforgenes.co.uk

RESEARCH UPDATE

Epilepsy in patients with MPS

Parents of children with MPS know that in some of the disorders epilepsy can occur as one of the late complications. From the medical point of view there has always been some uncertainty of the exact risk in the individual MPS types. In addition there has been no clear guidelines on what treatment works best in individual disorders. In an attempt to answer some of these questions many of you helped by completing a questionnaire about your child and epilepsy and we would like to share the results with you.

237 questionnaires were sent out to parents who had attended the clinic in Manchester. Of these 148 (62%) were returned completed giving us information on 147 children and young adults. The questionnaire asked about the presence or absence and type of seizure and also the response to treatment.

The table summarises the results:

MPS Type	No seizures	Seizures
I	37	0
II	27	9
III	35	21
IV	16	0
VI	2	0

The results clearly showed that only patients with MPS II and III were at a significant risk of developing seizures and that even in these groups there are many patients who remain seizure-free.

In the majority of patients the seizures were simple tonic-clonic episodes of brief duration (<5 minutes):

Type of seizure	Number
Tonic-clonic generalised	15
Partial or focal seizure	4
Absence	4
Myoclonic	1
Nocturnal only	1
Gelastic (laughter)	1
Unclear/unknown	3

When we looked at treatment we found no consistent anti-convulsant regimen. Most patients were either on sodium valproate (epilim) or carbamazepine (tegretol), either alone or together. A small number of patients were on other anti-convulsants such as vigabatrin (sabril) or clonazepam (rivotril). Side-effects to the medications were reassuringly rare and the most common one was "tiredness", reported by 4 parents. In 14/30 patients the seizures stopped completely with treatment. In the remaining 16 seizure frequency was reduced but remained a significant problem in 5 patients.

From our small study we can conclude that epilepsy is only seen commonly in MPS III (at least 40%). Children with MPS II are at a much smaller risk (25%) but other types of MPS must only be very rarely associated with a seizure disorder. In affected patients seizures are not more severe than epilepsy in general and seem to respond well to the anti-convulsants commonly used in children.

We hope that this information will reassure parents in general about seizures in their children. If any parent has questions about this study or epilepsy in general we will be happy to discuss these with you further if you make contact.

Erica Houston & Ed Wraith

MPS Newsletter Spring 2002

RESEARCH UPDATE

Decisions about having further children - research results

Findings from a recent research study by Judy Holroyd into decisions taken by families diagnosed with a child with an MPS or related condition.

Background

In September 2001 I completed an MSc course in Genetic Counselling at Imperial College School of Medicine. The final part of the course involved a research project where I looked at the reproductive choices that couples made following the diagnosis of a child with an MPS or related condition.

My family has been a member of the MPS Society since 1986. I am now a Trustee of the Society. I presented the project at the family conference in September 2001 and below is a synopsis of my findings.

Description and focus of study

124 mothers, whose MPS child was diagnosed between 1994-98, were sent questionnaires using the MPS Society database. The response rate was very high. Confidentiality and anonymity were strictly maintained throughout.

The study focussed on three areas:
The reproductive decisions taken by couples following the diagnosis and the factors that influenced the choices made.
The sources and quality of information and support.
Links between decisions taken and the background / characteristics of the mother.

Major findings

1. For those couples whose families were not complete at the time of the diagnosis:

- 89% chose an option that avoided the risk of having another affected child.
- Two thirds of families did try for another child.
- Of these, 83% chose to use prenatal diagnosis.
- The remaining one third of families decided against having further children.
- The decision to have a further child was particularly significant in those families with only an MPS child.

2. The overwhelming degree of professional support came from the MPS Society, paediatricians and genetic specialists.

Mothers were asked about the information and support they received at different times following the diagnosis. The support received at the time of diagnosis in understanding the MPS condition was very high. However, for making future choices and supporting a subsequent pregnancy, the picture is much more varied and there is a significant proportion where no support was forthcoming.

The study found that there is a need for sustained quality support from all quarters. In the area of future family planning decisions, making such a choice is very personal, it can be very lonely and it is obviously a difficult and sensitive one. It is in this area where the amount of support at the moment is lacking.

The key provider of emotional and practical support was the father whose ongoing support was of critical importance.

Statistically, no significant associations were found between the reproductive choices made and the age, educational level or religious conviction of the mother.

Finally, a very grateful thanks to the mothers of the MPS children who participated in the study and to the MPS Society for their support. If anyone is interested in reading the full text of the project, please contact the MPS office.

Judy Holroyd
February 2002

RESEARCH UPDATE

Prenatal Diagnosis in Pregnancies of Siblings of MPS Patients

Prenatal diagnosis for a MPS disorder has been possible for over 30 years. In the 1970's amniocentesis was performed in the second trimester of pregnancy, at around 15 - 16 weeks gestation. Cells present in the amniotic fluid were cultured and then used to test for the relevant enzyme. However, culturing took 3 – 4 weeks and generally a result wasn't available until around 20 weeks gestation.

A foetus starts to produce urine around the 11th week of gestation and it was found that if the foetus was affected with a MPS disorder the abnormal GAGs present in the urine of the affected brother or sister were also present in the amniotic fluid supernatant. We could, therefore, look at the GAGs in the amniotic fluid supernatant directly and obtain a result within days of the amniocentesis. An affected pregnancy could be terminated immediately and we also had the cultured cells available a few weeks later for enzyme assay to confirm the GAG finding. This technique was also very useful in families where an enzyme deficiency had not been confirmed in the index case.

In the early 1980's chorionic villus sampling became available and, provided the enzyme deficiency had been confirmed in the index case we could assay the enzyme in the chorionic villi directly and have an answer around 12 weeks gestation. This was less traumatic in many ways and socially more acceptable with termination being possible in the first trimester. However, it must be emphasized that neither amniocentesis nor CV sampling is without risk with the predicted miscarriage rate following amniocentesis about 1% and following CV sampling around 1.5%. To couples who have had an affected child this is a very low risk compared with the 1 in 4 chance of their having another affected baby.

However, the figures are quite different when the sister of an MPS patient becomes pregnant or a brother's partner becomes pregnant. We must be absolutely certain of the carrier status of this sibling to give accurate counselling. For many years the only way to detect carriers was to measure the activity of the relevant enzyme in blood. This was not entirely satisfactory as the results were often ambiguous, even within one family. There is a large overlap in activities of the so called obligate heterozygotes (i.e. the parents of the affected child) and normal controls which makes carrier testing even more difficult in unrelated individuals. Now, though, we have another tool to determine carrier status with very high accuracy – DNA analysis. This is available for MPS I, II, IIIA and IIIB at the Willink in Manchester and in London at The Institute of Child Health and Great Ormond Street Hospital. The Willink are also looking for the mutations in the MPS IVA patients.

Provided the mutation(s) have been found in the affected child or the parents of MPS I, IIIA, IIIB and IVA children or the mother of a MPS II child we can accurately define the carrier status of the unaffected sibling. We have found that some siblings that were predicted carriers by enzyme analysis are normal and indeed some siblings, thought to be non-carriers are in fact carriers.

If the unaffected sibling does not carry one of the abnormal mutations present in their family then there is no risk to the pregnancy. However, if the sibling is a carrier then what are the risks of the fetus being affected?

Obviously for MPS II there is a 50 % chance of any male fetus being affected and the chance of having an affected child far outweighs the risks of a miscarriage arising because of the sampling procedure.

In the other MPS disorders we have to ask what is the chance of the partner of the known carrier also being a carrier for the same disorder? Within the general population between 1 in 150 to 1 in 200 individuals are carriers for MPS I and MPS IIIA. For MPS IIIB and MPS IVA the carrier frequency is even lower. It is very difficult to accurately determine whether an unrelated partner is a carrier. Enzyme analysis, as I've already explained is often ambiguous and, at present, it is time consuming and very costly for the labs to screen for all the mutations that have been reported to give rise to the particular MPS disorder.

If we take the worst figure of 1 in 150 then, as we know there is a 1 in 4 chance of a carrier couple having an affected child the risk to each pregnancy is 1 in (150 x 4) = 1 in 600. This is obviously much lower than the risk of a miscarriage arising because of the sampling procedure and it is essential for the couple to understand these figures before embarking on prenatal diagnosis. For couples in these 'low risk' pregnancies who still want the reassurance of prenatal diagnosis we would recommend that following amniocentesis at 15 -16 weeks gestation amniotic fluid is sent to the Enzyme Laboratory at the Institute of Child Health or to the Willink, for GAG analysis. Obviously if prenatal sampling is being done for another reason e.g. maternal age, then there is no reason why some of the sample can't be sent for MPS testing.

What we really want to avoid is putting the laboratories under pressure for carrier testing. We would rather do carrier testing without having an absolute deadline hanging over us. Once we know the mutations in a family it is generally not difficult to do the test but nevertheless it still takes a few weeks to get the results so we don't want to be told that the pregnancy is already at 14 weeks. Are the mutations known in your family ? If the index case is no longer alive then we can test the parents. The siblings of many children diagnosed years ago are asking for our help now. Their parents aren't getting any younger so please make sure DNA is available. Even if unaffected siblings aren't worried their children may be and not knowing the mutation in the index case or the parents of the affected child will make accurate testing far more difficult for any family members.

MPS Newsletter Spring 2002

RESEARCH UPDATE

It is important that the mutation(s) in your family are known.

More recently DNA analysis has become part of the routine work up of a MPS patient but this wasn't available for many of the earlier patients. These families can ask their GP or the MPS society to contact the laboratories. If we have the results we will send them to your GP. Current families please talk to Dr Wraith or Dr Vellodi. We urge you to think of the future – NOW.

Elisabeth Young, Principal Biochemist, Enzyme Laboratory, Department of Chemical Pathology, Great Ormond Street Hospital for Children, London WC1N 3JH

Research from Australia

Dr John Hopwood - Women's & Children's Hospital Adelaide, Australia.

Many patients affected by Lysosomal Storage Disorders (LSD), including all Sanfilippo patients, have central nervous system disease, with clinical symptoms resulting from lysosomal storage of toxic substances in their brains. Treatment to prevent brain disease in patients remains a major objective of the Lysosomal Diseases Research Unit in Adelaide.

To achieve this, we have been evaluating and optimising enzyme replacement therapy (ERT) in which a corrective enzyme, specific for each disorder, is introduced into the brain to remove or prevent the storage of toxic substrates. We and others have shown that ERT effectively prevents disease-causing storage and halts disease progression in various organs in a number of different LSD such as Gaucher Disease, MPS I (Hurler/Scheie Syndrome), MPS VI (Maroteaux-Lamy Syndrome) and MPS VII (Sly Syndrome).

However, there is no conclusive evidence to suggest that ERT is beneficial in treating patients affected by brain disease. This is primarily because the brain is surrounded by a protective membrane called the blood-brain barrier. This barrier prevents the entrance of corrective enzyme from the body's general circulation into the brain. To systematically study and develop effective therapies for these LSD patients, we have been researching methods that will enable this transfer of enzyme to occur. Once this barrier is breached, it is expected that ERT will provide an effective therapy for all LSD patients suffering from brain dysfunction.

Our research plan uses four animal models to develop and demonstrate the efficacy of ERT for LSD that involve the brain. The four animal models we are using for our studies include a Sanfilippo "A" mouse (in collaboration with Professor Pamela Stanley, Albert Einstein College of Medicine, New York), a Sanfilippo "D" goat (in collaboration with Professor Margaret Jones, Michigan State University), an alpha-mannosidosis guinea pig and a fucosidosis dog.

Our logic for using four models is based on cost and an inability of any one animal model to match the clinical disease seen in humans. Differences in the blood-brain barrier between species are known, and importantly, the guinea pig's blood-brain barrier is thought to be more similar to that of humans than that of the mouse. Our hypothesis is that therapies based on delivery of corrective enzyme from circulation into the brain through the blood-brain barrier will be optimised in a small animal model such as the mouse and guinea pig before validation in a large animal model such as the goat and the dog.

Thus far we have characterised and compared the pathology seen in the brain of all four animal models with that seen in the brain of human Sanfilippo and other LSD patients. We have prepared and characterised significant amounts of corrective enzyme for ERT studies either planned or underway in each animal model. Recently, we commenced studies to develop, evaluate and optimise procedures to achieve the effective transfer of sufficient amounts of enzyme to reduce or prevent the storage of the toxic substrates for each animal model. Once we are able to demonstrate the principal and efficacy of a method to achieve the transfer of therapeutic amounts of enzyme in the larger animal models, clinical trials in the appropriate patients will be planned.

RESEARCH UPDATE

Mucopolysaccharidoses and spinal cord compression: case report and review of the literature with implications of bone marrow transplantation

Neurosurgery 2000 Jul;47(1):223-8; discussion 228-9.

Kachur E, Del Maestro R

Centre for Paediatric Neurosciences, Division of Neurosurgery, London Health Sciences Centre, University of Western Ontario, Canada.

OBJECTIVE AND IMPORTANCE: We present a patient with mucopolysaccharidosis with spinal cord compression, and we review previously published cases. This is the first published case of a patient with mucopolysaccharidosis with spinal cord compression who has undergone bone marrow transplantation.
CLINICAL PRESENTATION: A 2-year-old patient with Hurler syndrome underwent bone marrow transplantation. Although the bone marrow transplantation improved many of the systemic effects of Hurler syndrome, the patient presented at 8 years of age with a cervical myelopathy. Magnetic resonance imaging revealed soft tissue compression of the upper cervical cord. The literature review demonstrates that spastic tetraparesis, secondary to cervical cord compression, is the most common presentation of this subgroup of patients.
INTERVENTION: A suboccipital craniectomy and C l-C5 laminectomy and decompression with duraplasty were performed. Pathological examination of compressive soft tissue and lamina was consistent with mucopolysaccharidosis.
Postoperatively, the patient showed substantial improvement in neurological function.
CONCLUSION:
Mucopolysacharisoses can induce a compressive “metabolic myelopathy.” Decompressive procedures have shown significant improvement in neurological function in the majority of patients without spinal instability. Bone marrow transplantation may allow more patients with mucopolysaccharidoses to survive long enough to require neurosurgical treatment in the future. The effect of bone marrow transplantation on the prevention of spinal cord compression is unclear.
PMID: 10917366, VI: 20372034

Review of Article:
Spinal cord compression can occur in MPS disorders by 1) soft tissue infiltration of undergraded glycosaminoglycans and 2) congenital vertebral abnormalities. It has been suggested that the donor leukocyte lines (white blood cells) from bone marrow transplantation (BMT) would provide the missing enzyme, a-L-iduronidase, and prevent or reverse the abnormal accumulation of glycosaminoglycan by-products (storage materials). It is not know whether BMT can alleviate or prevent the accumulation of storage material around the spinal cord. This article prevents a child with MPS I who has BMT at age 2 years and diagnosis of spinal cord compression at 8 years. The article also includes a literature review of 19 cases of spinal cord compression in 19 individuals with an MPS disorder.

Following an improvement in clinical condition after BMT at age 2 years, the child at age 8 began to have difficulty with hand function with a decline in fine finger movements. She also developed daytime urinary incontinence. A cervical spine MRI revealed compression of the spinal cord at CI-C4. She underwent a suboccipital craniectomy and C l-CS laminectomy and decompression, and showed significant improvement in hand function and upper-extremity strength at her six week check-up. These improvements persisted at her two-year follow-up visit.

Of the 19 cases of spinal cord compression reviewed in the literature, six individuals had MPS I (two Hurler and four Hurler-Scheie), two had MPS II, two had MPS IV, and nine had MPS VI. The median age of presentation was 24 years (range 5-55 years); eight individuals were male and 11 were female. Fourteen individuals presented with a spastic tetraparesis (muscle contractions in all four limbs), four with spastic paraparesis (muscle contractions in the lower limbs), and one with spastic hemiparesis (contractions on one side of the body). Soft tissue infiltration of dura, bone and outside the external covering of the spinal cord were found in 11 whereas vertebral anomalies and subluxations (vertebra not in alignment creating pressure on the spinal cord) were found in six. Fifteen individuals had the cervical region affected and two had the thoracolumbar region affected.

Surgery was performed in 14 patients; 12 showed clinical improvement, with return to a normal or near-normal point of function in nine of the 14. One patient died during attempted intubation. A 36-year-old man with MPS VI required a second surgery following a brief period of improvement. A 44-year-old man with MPS II showed no improvement following surgery; this was attributed to the long delay before surgery was performed.

The authors note that recognition of this complication will promote early detection of spinal cord compression and intervention before irreversible injury has developed. Previous reports of individuals with MPS who have undergone a BMT indicate that BMT does not seem to reverse or prevent skeletal abnormalities, most likely due to poor penetration of the enzyme into skeletal tissue. It is not possible to determine whether the spinal cord compression in the individual discussed in this paper would have been more severe in the absence of her BMT. Should BMT be considered in patients with MPS in whom asymptomatic cervical cord compression is identified? Only a prospective controlled trial on the effects of bone marrow transplantation on spinal cord compression in patients with MPS would answer this question.

INFORMATION EXCHANGE

The 150th anniversary of Great Ormond Street Hospital for Children

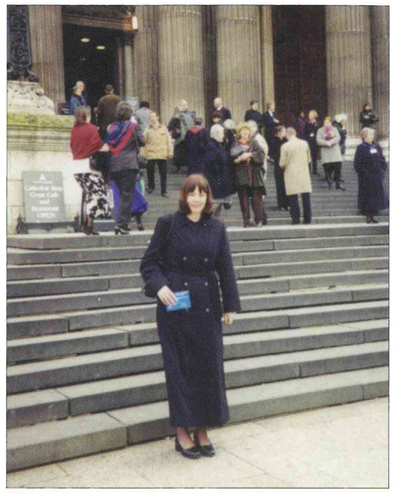
Thursday 14th February 2002 heralded the 150th anniversary of Great Ormond Street Hospital for Children. Christine and I were invited to a service of thanksgiving at st. Pauls Cathedral, followed by lunch at the Guildhall. The service included contributions from people representing all walks of life in relation to the hospital, from the Clinical Director of Surgery and the Chief Executive, to a Charge Nurse, a Health Care Assistant, a parent and a patient.

The congregation likewise consisted of people from a wide variety of backgrounds, from patients to nurses from many years ago, celebrities to those at the forefront of the services offered by the hospital today.

Afterwards at lunch in the Guildhall Christine and I met colleagues from the Institute of Child Health and the Jeans for Genes appeal, and the Prime Minister, the Right Honorable Tony Blair MP, spoke to mark this occasion.

Great Ormond Street Children’s Hospital has not always been the centre of medical excellence renowned and repeated world wide as it is today. Throughout the day there were reminders of how the hospital has grown from very humble beginnings. A history of the hospital was included at the back of the service sheet.

Ellie Gunary
Assistant Director



Ellie on the steps of St. Paul’s Cathedral

GREAT ORMOND STREET CHILDREN’S HOSPITAL

HOW IT ALL BEGAN

On a summer’s evening in 1851, Bloomsbury GP Dr Charles West was walking home through Coram Fields after dinner with friends. He turned down into Lambs Conduit Street and again into Great Ormond Street. It was here he noticed that No. 49 was for sale. This was a house where he had spent much time as it had been the home of his friend, Dr Meads, physician to Queen Anne.

Charles West had found the perfect place to realise his long-held vision – to create the first hospital especially for children in the UK.

Life for the Victorian child was extremely hard. Over 50% would die before their 15th birthdays from childhood ailments often caused simply by poverty. Dr West was appalled that there was nowhere to treat acutely ill children. There was very little medical knowledge to treat them and hospitals at that time only cared for adults. Opponents to his plans for a hospital for children included Florence Nightingale who believed, as many other medical and nursing professionals did, that hospitals were not an appropriate place for children.

Enlisting the support of wealthy friends, including Charles Dickens, Dr West bought No. 49 Great Ormond Street. A few months later, on 14th February 1852, the Hospital for Sick Children opened its doors for the first time with just 10 small beds in the converted drawing room of this old house. Unlike other hospitals, children were admitted purely on clinical need.

And on the 17th February George Parr aged 2 years was admitted as Great Ormond Street’s very first patient suffering from catarrh and diarrhoea.

Fundraising has always played an important part in the Hospital’s success. One of the first supporters was its neighbour, Charles Dickens who organised an annual dinner which raised enough in the first year to buy the house next door and double the size of the hospital.

Another famous author who has contributed so much to the hospital over the years is JM Barrie who gave the copyright of his play Peter Pan "providing the sum raised is never disclosed".

And in 1987 it launched its famous Wishing Well Appeal to redevelop the Victorian Hospital and provide new parent accommodation. The Appeal raised £42 million, and set new targets in the field of fundraising appeals.

Today the Hospital has 335 beds and treats over 100,000 children every year with the rarest and most complex childhood diseases. It has more specialties under one roof than any other hospital and employs 2,500 staff including 350 doctors and over 900 registered nurses. With its research partner, the Institute of Child Health, it is recognised as a centre of excellence in child health renowned and respected throughout the world.

As we celebrate our 150th Birthday, Great Ormond Street Children’s Hospital owes a huge debt of gratitude to Dr Charles West and his vision, as well as the many thousands of wellwishers whose support and encouragement is remembered today.

The Prime Minister and Cherie Blair cutting the birthday cake

INFORMATION EXCHANGE

Naidex & Kid e Quip 2002

14 -16 May 2002 NEC Birmingham Hall 1

Naidex, the UK's largest disability and rehabilitation event, has joined forces with Hid e Quip, the UK's leading event dedicated to children with disabilities.

- See, touch and try hundreds of new products from over 250 exhibitors
- Free seminars and advice
- New products showcase
- Mobility Test Track

For your free tickets call 0870 429 4428 or pre-register for fast track entry on www.naidex.co.uk

For further information call 020 8332 0044 or email naidex@touch-stone.co.uk

New Benefit Update

Main Benefits	Now £/week	April '02 £/week
DISABILITY LIVING ALLOWANCE		
Care Component		
-highest	55.30	56.25
-middle	37.00	37.65
-lowest	14.65	14.90
Mobility Component		
-higher	38.65	39.30
-lower	14.65	14.90
INVALID CARE ALLOWANCE	41.75	42.45

Carers and Disabled Children Act 2000

LEGISLATION

Vouchers for respite care were due to be introduced in April 2002, having already been delayed from last year. They have now been further delayed and will not be introduced before June 2002 at the earliest. This is due to a court case concerning provision of accommodation under Section 17 of the Children Act. To get the voucher scheme implemented, primary legislation is needed so the Government are adding a section to the Adoption Bill currently before Parliament.

As part of the consultation process, Contact a Family sent a written submission objecting to a sentence in the proposed guidance, saying "For example, if one particular voucher holder is redeeming vouchers at a rapid rate, the care manager may wish to check that the individual understands the operation of the scheme." They asked for this to be changed to read: "For example, if one particular voucher holder is redeeming vouchers at a rapid rate, the care manager may wish to check that this is not an indication of a rapid deterioration in the home situation which may require reassessment and provision of additional services." This change has been made, which is good news.

INFORMATION EXCHANGE

Support to individuals with Fabry Disease

In line with extending the membership and support services of the MPS Society to individuals with Fabry Disease, their families and carers I have been invited as a representative of the "Patient Support Group" (not my terminology) to sit on a working group, set up by the Department of Health to look at introducing enzyme replacement therapy for Fabry Disease to the National Health Service. Another member of the group is Alan Dickerson, a sufferer of Fabry Disease. It has been encouraging that the Department of Health are involving patient and patient support groups at this early stage and for me to be able to report back to the Society's members first hand news. Unfortunately, the first news to report is disappointing. Applications from each of the four centres below to the National Specialist Commissioning Advisory Group (NSCAG) for the development of a service for the diagnosis and management of Anderson's Fabry Disease have been unsuccessful:

The Royal Manchester Children's Hospital and Hope Hospital, University College Hospital, London and Great Ormond Street Children's Hospital for Sick Children, Royal Free Hospital, London, Addenbrooke's Hospital, Cambridge.

This in effect means the Government will neither fund nor designate a national service to be provided by local health authorities to sufferers of Fabry Disease.

On a positive note, in the absence of support for NSCAG, a firm commitment was made by the Chairman (the Chief Executive of the Eastern Region Specialised Commissioning Group) of the working group to ensure that the regions work together with health authorities and primary care trusts to fund the cost of providing Enzyme Replacement Therapy throughout England. Once the treatment costs are funded the committee will need to examine ways of funding the cost of the service.

Representing the MPS Society my key messages to the group were firstly the need to ensure that all eligible individuals throughout the UK (not just England) have equal access to these services, and secondly that clear information and support be provided to patients and carers.

Each of the centres must also provide both available treatments and give patients unbiased information on why a particular treatment is offered.

Ellie Gunary
Assistant Director

Campaign for travel costs for families visiting children in hospital

Contact a Family has campaigned for a number of years for the Government to review the issue of travel costs for parents visiting their children in hospital. This was recently recommended by the Kennedy Inquiry into children's heart surgery at Bristol Royal Infirmary. The campaign is backed by the Royal College of nursing and Andrew Lansley MP has tabled an Early Day Motion in Parliament.

Contact a Family has asked that any individuals and families in contact with their MPs urge them to sign the Early Day Motion.

At present families can apply for a means - tested refund for the costs of travelling to hospital for treatment. However, the costs of travelling to hospital for visiting can only be met by a discretionary fund which is frequently over-stretched.

Contact a Family is looking for families who may be interested in speaking to the media about journeys they have had to make or are making to visit their child in hospital. If you are interested in discussing this issue please do contact Anna King at Contact a Family on:

020 7608 8715 or
anna@cafamily.org.uk

INFORMATION EXCHANGE

Holidays available from React

React - Rapid Effective Assistance for Children with potentially Terminal illness have purchased mobile homes throughout the UK to enable families to enjoy a well earned break from the stresses of everyday life.

Please note that React mobile homes are not suitable for wheel chair use.

There are four locations of the mobile homes:

Thorness Bay - Isle of Wight

Travel across the water to visit the scenic Isle of Wight. The React mobile home is situated on the very popular and friendly camp site. Guests can enjoy all the facilities this has to offer.

A full entertainment programme catering for young and old alike can be enjoyed all day and every evening. (Please note there are no bus services from the Port to Thorness Bay, which is not easily accessible without a car). Full details of Thorness Bay Holiday Park may be obtained from the brochure.
Call: 01983 523109.

Coastfield - Skegness

Close to the famous resort of Skegness, with its funfairs, long sandy beaches and donkey rides. Coastfield is the ideal way to enjoy a traditional family holiday. This site has an outdoor heated pool and sun terrace and is a few minutes walk from the sea. It offers a wide range of activities and entertainment and is within easy reach of shops, pubs and restaurants.
For a brochure please call: 01754 872592

Combe Haven - Hastings

Combe Haven is situated just outside the historic town of Hastings. The complex is a bustling hive of activity with a full entertainment programme for young and old alike. Placed conveniently on the south coast enables trips to other seaside venues. This popular holiday resort provides two swimming pools, ten- pin bowling and its very own disco and cabaret club. For those who enjoy a sporty element there is an all weather sports court and bowling green. Full details may be obtained from the site, telephone: 01424 427891.

Wemyss Bav - Renfrewshire

The truly beautiful location on a wooded slope overlooking the Clyde, Wemyss Bay, is perfect for enjoying the scenic beauty of the West Coast of Scotland. Run by British Holiday, this small friendly park has a welcoming atmosphere with a great range of activities for all the family, especially in the evening when the family clubroom really comes alive. For a brochure please call 01475 520812.

In addition there are two flats:

Blackpool

React can also offer a holiday at Donna's Dream House in Blackpool, which is reserved for wheelchair use. Two privately owned, self-contained flats are available for our families to use throughout the year offering accommodation for the disabled. Alternatively, React is also able to offer bed and breakfast accommodation to families looking for the excitement of a Blackpool holiday based near the centre of the town.

If you would like to apply for a holiday in a React mobile home you can telephone React for an application form.

DONATIONS & FUNDRAISING

The Society is grateful to the following:

Donations

Mr & Mrs Davies – Oxfordshire
Jessica – Ely
Mrs J Simmonds – Andover
Stone CEC School, Aylesbury
Mr & Mrs Ingram – Sheffield
Alan Byrne– Glasgow
Clinical Biochemistry Dept – Bristol Royal Infirmary
Mersey Internal Audit Agency
Japanese Women's Club
Peter Adshead – Aylesbury
Mr M Ismail – Leeds
Lloyds TSB
Marton Institute Ladies Bowling Club – Blackpool
Towersey Morris Men – Aylesbury
M J Mc Tiffin – Hants
Genzyme
Jennifer Norsworthy
V Robins – Gloucestershire
Graham Anthony – Ipswich
Grace Smale
Genzyme – Oxford
O G S – Abingdon
Clydebridge Steel Works
Cap Gemini Ernst & Young
Spiffing Stationery
Provincial Grand Lodge of Buckinghamshire

STAMPS/FOREIGN COINS

Towersey Morris Men
Mrs T Lewis

COLLECTION BOXES

Steam Packet Inn – Isle of Whithorn
Kirkinner Inn – Kirkinner
Costcutter – Whithorn

Fundraising

Lynn Longhorn – Christmas fair
Dawn Jones – Christmas fair
C & G – Gloucester
Haddenham Mummers – 'The Doctor'
Whitchurch Village Social Club in support for Tara Murphy
Nigel Ratcliffe – 10K Run
Andrew Ratcliffe – 10K Run
Graham Ratcliffe – 10K Run
McCormick's – Haddenham
Highfield School – Ely
Mrs C Dickey – Belfast
Mrs K Boyde – Belfast
NIKU UK
Mrs N Longdon – Sale of Recipe Bk
Claire Garthwaite – Burns Supper
Jo Richardson – local Playgroup
Damon Singers – Concert
Haddenham St Marys Church of England School
Sue Lowry
The Sandringham Pub – Raffle
Marina/Dave – Car Boot
Gaynor Catteroil – Knitting/Selling Xmas Bells
The Swan Public House – Oxon – Raffle
Charity Golf Day – Slinford Park West Sussex
Mrs M Thomas – Easter Bingo
Beaver Scouts of Stoke Row – Talent/Supper Evening

In Memory

G Robins
James Edwards
Steven Grandidge
Sarah Kilvert
Richard Matthews
Keith Blanchard
Edward Nowell

FUNDRAISING

Sponsored cycle ride

This is brief description of our sponsored cycle ride and reasons for undertaking it. This is the second year running that we have chosen one charity only, not as in the two years previous where we had all chosen our own charities. Last year we raised over £2,700 and donated it to a local Meningitis trust. This year we managed to raise £2,636 which we have donated to the MPS Society (The Society for Mucopolysaccharide Diseases). We were prompted to choose this charity by a local couple whose young son has this terrible disease, which to date there is no known cure. This sum of money was not raised by the cycle ride alone, but also by events organised and sponsored by our local pubs, The Fox & Hounds Ennerdale, The Shepherds Anns Hotel Ennerdale and The Stork Hotel Rowrah.

Day one

With all the bikes etc, loaded into the mini bus on Thursday evening, everything was in place for a 06:00hr start on Friday morning. We arrived at Bell's Bridge in Glasgow just gone 09:00hrs (the place we'd finished our journey from Inverness 12 months earlier). It took us nearly half an hour to carry out our final preparations and checks, before setting off on our 253-mile ride back to Ennerdale. The first day turned out to be one of those I think most of us would like to forget as it was mostly through urban areas with all the problems of the last twenty miles of last year's ride i.e. no signs, signs turned around etc. After nearly 8hrs in the saddle plus lots of debates and back tracking we finally reached our accommodation in Ayr at around 17:30hrs, 56 very frustrating miles behind us, thank goodness.

Day two

We knew this was going to be the longest and toughest day of the ride, but we hadn't anticipated just how long and tough. After a good quantity of the local brew the previous night, off we set just after 09:00hrs. The first couple of miles were along the sea front and was that sea breeze needed (just how much we were about to find out). We didn't have too long to wait, as it was up and over a massive hill to a place called Maybole. From Maybole we headed on to a small village called Crosshill, where we started the longest climb of the ride, up and up for close on 5 miles, by which time a head wind had got up, followed soon after by rain. Again once reaching the hilltop it was a steep descent down to not much above sea level. This was again followed by another 3 mile climb and the wind seemed to be getting stronger or was it my legs getting weaker, I'm not sure. On reaching the highest point the road started to run gently down for the best part of ten miles towards a place called Glentrool. We never quite made it into the village as we beared left at this point onto a 26 mile off road stretch of the route, which eventually led us to our second over night stay in Castle Douglas. This part of the route was very picturesque and probably the best cycling of the whole ride; but with 79 miles behind us and a few people's sense of humour rapidly disappearing I was quite pleased to see Castle Douglas, as by this time it had just gone 20l5hrs. This was a good hard day's cycling behind us and in my opinion an excellent one, which was everything the first day failed to provide. We had beautiful scenery with nice quiet country roads and tracks to cycle along. Weather could have been better but you can't have everything, "after all we were in Scotland".

Day three

Another 69 miles ahead of us, but today the terrain and weather were to be well in our favour. We set out again around the 09:00hr mark and headed towards Dumfries, a journey of just over 20 miles. This part was quite hilly, but nothing compared to the day earlier. Once through Dumfries we headed south towards the Solway coast, where we turned and headed in a north easterly direction. Close by the Solway we came to Annan, where we stopped for a bite to eat (and a pint). We'd made excellent time, as we had a good breeze behind us and we hadn't seen a hill of any note since we left Dumfries nor were we to see one again that day. After Annan we pressed on through Gretna and back into England, then on to Longtown before turning south and into Carlisle via Rockcliffe. We completed the day's cycling around 16:00hrs, we'd only cycled 10 miles less than the previous day and in over 4 hours less.

Day four

This took us out of Carlisle via the excellent new River Caldews cycle way to Dalston, from there we went on through Raughen Head, Hesket Newmarket and into Caldbeck where we stopped for a bar meal (and a pint). There was an almost ghostly feeling about the whole area, not an animal in sight, in fact we could not remember seeing an animal in either the whole of Cumbria or most of Dumfries for that matter. It really hit home how this terrible foot & mouth disease had totally devastated these areas. We then headed along Caldbeck Common, before turning left and through by Over Water Tarn, down passed the Castle Inn, on through Embleton, Lorton, Loweswater before hitting Fang's Brow. Fang's Brow is a daunting enough climb with fresh legs let alone legs with four days' hard cycling behind them. Eventually reaching the top of Fang's we only had one good climb to go, up out of Lamplugh and over the Leap's, descending down into Croasdale, then down into Ennerdale village and The Shepherds Arms for some well earned sandwiches & chips and a pint or two, before heading home to rest some very tired and weary limbs.

On the whole it had been a long trail and the lads' patience had worn very thin on occasion's, but in my opinion due to the foot & mouth crisis most of the lads had not had the opportunity to get out and about, to get enough training in, as in previous years. Apart from the first day the cycle ride had been a very good one, a good challenge, but maybe with a few less miles per day, we would have been able to take in a bit more of the surrounding areas. I would like to say a very big THANK YOU to the following people, because without their help and support we would not be able to do the cycle ride and raise the money we have: Donna; Michael Rowe who drove the support vehicle, who did an excellent job; Sharron & John Walker who were an excellent help with fund raising; Ronnie & John, landlady & landlord of The Fox & Hounds Ennerdale, Norman, landlord of The Shepherds Arms Hotel Ennerdale, and Ronnie, landlord of The Stork Hotel, Rowarh, for all their sponsorship & help with fund raising; Irene Robinson of Satin Stitches Cleator Moor for the polo shirts; and Chris of Ainfield Cycles Cleator Moor for keeping us on the road throughout the trip. And last but not least the lads who carried out all the cycling, fund raising etc with myself, Bob Watson, Stephen Wilkinson, Howard Wilson, and Gordon Fletcher.

Many thanks.

John I Ireland

FUNDRAISING

MPS 21st Anniversary

Everyone loves to celebrate. So why not have a good time but also raise money to further the work of the Society for Mucopolysaccharide and Related Diseases.

Fundraising need not involve organising a huge event. It can be a simple coffee morning for a couple of dozen friends. If every family or supporter on our mailing list organised a celebration raising £100 it would bring in £50,000 which would fund two support and advocacy Development Officers or eighteen months of a vital research project.

The Society is planning a number of high profile events as well as a programme of regional events throughout the United Kingdom.

Whatever you plan to do to celebrate our 21st anniversary let us know. We will have special fundraising packs available in September..... Have fun!

Barry Wilson
Chairman

FOR SALE Scanbed 710

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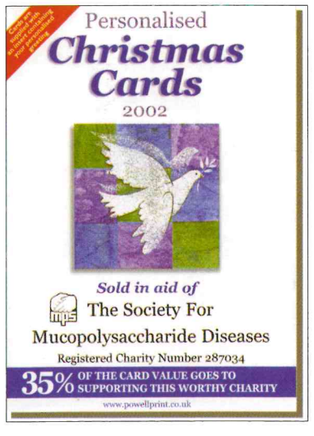
Christmas Cards



The response to our Christmas cards last year was our best yet. Thank you to everyone who purchased our cards. Many of you also made a donation to MPS at the same time as purchasing Christmas cards and this is most appreciated. A new range of five Christmas cards has been selected for 2002 and we hope even more people will support the Society by buying MPS Christmas cards this year. For companies, businesses and those who prefer to send personalised Christmas cards, a brochure will be included with the Summer and Autumn newsletters. For every order placed the MPS Society will receive 35% of the total cost.

CONTACTING THE SOCIETY

MANAGEMENT COMMITTEE

Chairman	Barry Wilson	
Vice-Chair	Steve Butler Judy Holroyd	
Treasurer	Judith Evans	
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	Alex Roberts	Project & Information Officer
	Fiona Woodcraft	Project & Information Officer
	Mounira Hadj-Rehouma	Development Officer
	Alison Britton	Assistant Development Officer

NEWSLETTER DEADLINES

SUMMER
30 June 2002

AUTUMN
30 September 2002

WINTER
17 December 2002

SPRING
31 March 2003

Do let us have your family stories and any helpful hints you would like to share with our newsletter readers. If you have a question that you would like to see answered in a future edition of the newsletter, please do write to us.

To submit information to the newsletter please send materials (preferably via e-mail for text) and mail photos to the address on the left.

The articles in this newsletter do not necessarily reflect the opinions of the MPS Society or its Management Committee.

The MPS Society reserves the right to edit content as necessary.

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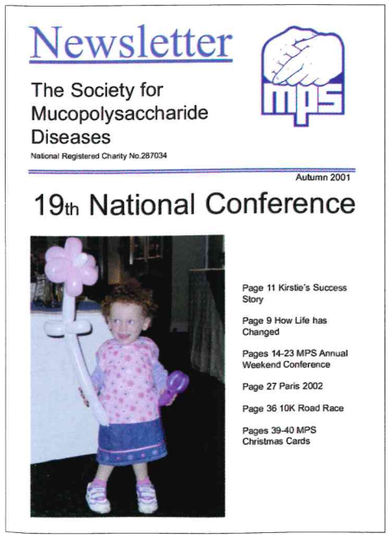
Fabry: www.fabry.org.uk


Newsletter

The Society for Mucopolysaccharide Diseases

National Registered Charity No.287034

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Newsletter

The Society for Mucopolysaccharide Diseases

National Registered Charity No. 287034

Winter 2001

MBE for Christine!

The Director of the MPS Society, Christine Lavery, has been awarded an MBE in recognition of services to the MPS Society in the Queen's New Year Honours.

Christine founded the charity nearly twenty years ago and has since worked tirelessly raising awareness of MPS diseases, supporting hundreds of affected families, as well as encouraging fundraising to promote vital scientific research. Dr Ed Wraith, who has worked closely with the Society for many years, said, "I doubt there has ever been a more deserved award", and her colleagues at the MPS office were obviously delighted with the news.

The award ceremony will take place at Buckingham Palace later in the year.

Back Issues

If you have missed an edition of the MPS quarterly Newsletter or know someone who would like one, you can order a back issue. We currently have Autumn 2001 and Winter 2001 in limited stock.

Each copy is £2.50 including postage & packaging. You can pay by sending a cheque payable to 'MPS Society' to the address above, or by credit card over the phone.

SPECIAL DAY OUT AT UNBEATABLE PRICES ALTON TOWERS

FOR MPS MEMBER FAMILIES

ATTENDING THE MPS SOCIETY'S AGM

ALTON TOWERS HOTEL

9.30am SATURDAY 11th MAY 2002

All MPS parent and adult members can purchase a maximum of 2 adult tickets and a ticket for each of their children for a total of £10.

This exceptional rate is dependent on the parent members attending the Society's AGM and tickets will be handed to families at the end of the meeting.

The AGM will start promptly at 9.30am and it is anticipated that it will be over by 10.00am.

MPS families may reserve accommodation at the Alton Towers Hotel on Friday 10th May and/or Saturday 11th May 2002 for £145 per room per night for up to 4 people including continental breakfasts. Please book directly with the hotel stating that you will be attending the MPS AGM.

Notice of Annual General Meeting

Notice is hereby given that the Annual General Meeting of the Society for Mucopolysaccharide Diseases will take place at the Alton Towers Hotel, Alton, Staffordshire on 11th May 2002 at 9.30am

HOW TO BOOK FOR THE ALTON TOWERS HOTEL

You need to have this information ready as you phone:

- The dates you wish to stay
- The number of children and adults in your party
- The number of rooms required
- Any special requirements you may need
- Your method of payment
- State that you are attending the MPS AGM

Now phone 08705 00 11 00