Newsletter

The Society for Mucopolysaccharide Diseases

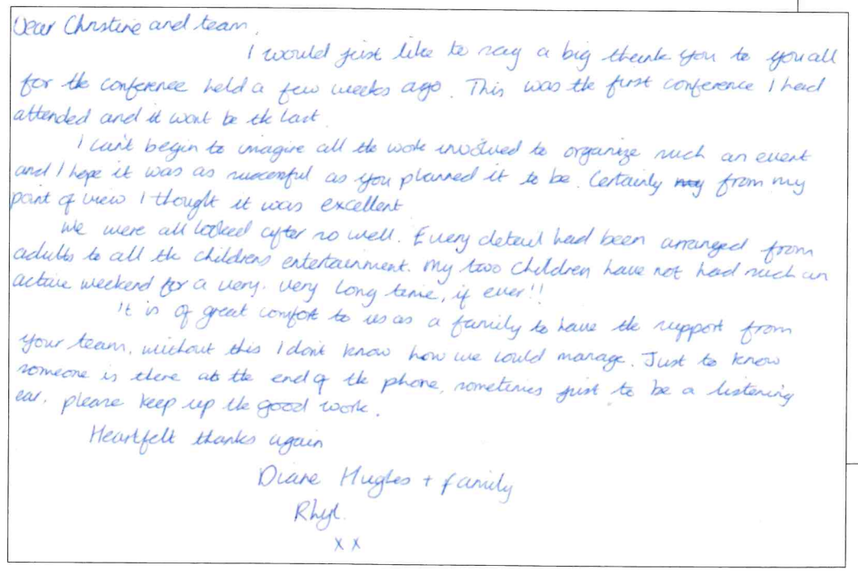
National Registered Charity No. 287034

Autumn 2003

MPS CHILDHOOD WOOD CELEBRATES ITS 10TH BIRTHDAY

CONTENTS

Letters



Dear Christine and team,

I would just like to say a big thank you to you all for the conference held a few weeks ago. This was the first conference I had attended and it won't be the last.

I can't begin to imagine all the work involved to organize such an event and I hope it was as successful as you planned it to be. Certainly way from my point of view I thought it was excellent.

We were all looked after so well. Every detail had been arranged from adults to all the children's entertainment. My two children have not had such an active weekend for a very, very long time, if ever!!

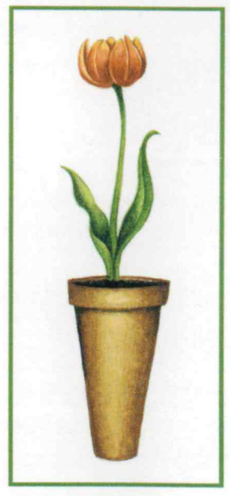
It is of great comfort to us as a family to have the support from your team, without this I don't know how we could manage. Just to know someone is there at the end of the phone, sometimes just to be a listening ear, please keep up the good work.

Heartfelt thanks again

Diane Hughes + family

Rhys

X X



Dear MPS Society,

I would like to thank you for a fantastic weekend you provided for the 21st MPS Conference. as you know it was our first conference since having Harrison diagnosed with Sanfilippo type A. I had mixed feelings about attending but had a brilliant time, made loads of friends, had fun and learnt lots of information about Harrison's condition. The children all had a fantastic time, Harvey didn't want to go home he kept saying he wanted to go back to his "room".

Harrison entertained many by jumping into the fish pond, and became a star with Ed Wraith and his gang with a photo session!

We had some very late nights, dancing, singing and chatting. I think everyone will agree that we all felt like one big special family that we didn't want to leave. For once it felt like everyone understood what each other were going through, good and bad.

It was lovely to meet the MPS staff who I'd spoken to many a time on the phone. You should be very proud of this invaluable team.

The doctors were great giving one to one time to those who needed support as well as the talks that they did.

Thank you once again to the Society and the hotel staff and the wonderful volunteers we all had, I'm sure I speak for many.

Hope to see you all in Germany for the next MPS Society conference next year.
Madeleine Luckham (Harrison King's mum)

'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 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
11 regional MPS clinics throughout the UK and information days and conferences in Scotland and Northern Ireland

National and International Conferences
Held annually and offering individuals and families the opportunity to learn from professionals and each other

Sibling Workshops
Specialist activities for siblings who live with or have lived with a brother or sister suffering from MPS or a related disease

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

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

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.

Assistant Director's Report

Ellie Gunary

Working for the Society never brings a dull moment and the past three months have been no different with the number of members ever increasing and the range of support given and requested, continually expanding. New publications continue to be developed with two new children's booklets recently published, 'I've got Fabry's' and 'Our brothers and sisters have Sanfilippo'. These bring to four the number of children's booklets in this range, a range which has been warmly welcomed by schools who are supporting a child with an MPS and related disease and their brothers and sisters. To enable you to acquire any of our booklets and the MPS video, an order form is enclosed with this newsletter.

Whilst the advocacy support team is actively supporting those members for whom Enzyme Replacement Therapy is now a licensed treatment, this team is also equally committed to the maintaining of the high standards of support given in areas of welfare benefits, home adaptations, equipment, education and palliative care which have been the hallmark of the Society's advocacy support over the past few years. All ends of the country continue to be travelled to, to meet members at home, attend multi-agency meetings and support members at formal appeal hearings.

Opportunities to promote the work of the Society and the needs of members have been actively pursued with an information day held at Rachel House Children's Hospice, Scotland, which generated much discussion amongst members and their local social services and education support staff, a training session run for nurses in Buckinghamshire and Christine and I were very honoured to be invited to speak to a team of grants officers of the Family Fund and raise the day to day issues of caring for a child with a progressive disability.

The advent of licensed products for Enzyme Replacement Therapy for MPS I and Fabry Disease has brought hope to many but it is extremely worrying that patients throughout the country are being denied funding for their treatment at a primary care trust level. Determined to ensure that treatment is available to all those for whom it has been medically prescribed, wherever they live in the country, the Society is actively supporting members when needed to pursue the appeals process whilst also planning a campaign of parliamentary lobbying should appeals be unsuccessful. After all the years of funding research we can see from a patient perspective how frustrating it must be to have waited all this time for Enzyme Replacement Therapy for it now to be unobtainable.

We eagerly await developments for treatments for the other MPS and related diseases, treatments which we are actively pursuing through the awarding of research grants from monies raised from Jeans for Genes. Our thanks go to our many members who joined thousands of people across the country on Jeans for Genes day, 3rd October 2003, to don their jeans and join this nationwide campaign to raise awareness of genetic diseases and raise much needed funds for research. Stories about how you supported Jeans for Genes and any press cuttings would readily be welcomed for inclusion in the next MPS newsletter.

On 4th July 2003, sixteen families gathered at the Childhood Wood to remember loved ones, some of whom died recently, some many years ago. The cake made for the occasion, celebrating ten years of the Childhood Wood and twenty-one years of the Society, was a work of art and is featured on the newsletter front cover.

It is always the articles written by members which are read most eagerly in the newsletter and about which we receive the most positive feedback. The stories about day to day life with an MPS or related disease are always of interest and I know from the many letters received by the Society's staff team that all our members have a story to tell. Please keep your newsletter articles coming in. They are much appreciated by all who read them, including professionals, volunteers and overseas readers, from whom we would also welcome relevant articles.

In this newsletter you will find member's stories as well as articles on research and the ever popular information exchange.

Whether this is the first MPS newsletter you have read or the eighty-second as it will be for the Society's oldest member, Mary Moulding, whose story is featured, I hope it interests and empowers you.

News From the Management Committee

The Trustees met in July and considered the following matters:

Election of Officers
The following officers were elected:
Chairman - Barry Wilson
Vice Chairman - Judith Holroyd
Vice Chairman - Bob Devine
Treasurer - Judith Evans

Support and Advocacy

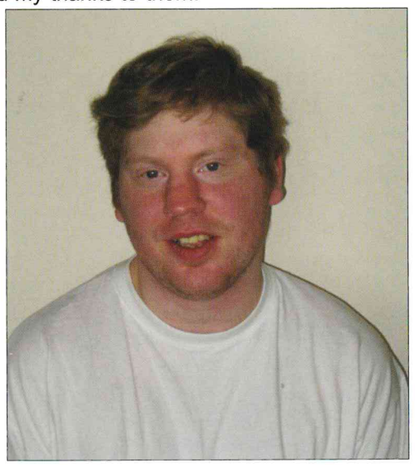
Trustees learnt that two members with Fabry disease have been represented by the Advocacy Support Team at Primary Care Trust (PCT) appeals for funding for Enzyme Replacement Therapy. A number of applications to PCT's for MPS I patients to receive Aldurazyme. Patients and their families are waiting anxiously.

Jeff Bawden

Hello I am Jeff. I am the new(ish) member of the Advocacy Support Team, having started work for the MPS Society in April.

I qualified as a nurse in 1996 and worked as a Community Learning Disabilities Nurse in Reading for 4 years. After this I worked in the private sector running supported living projects for people with mental health problems and learning disabilities.
So far my time with Society has been very rewarding and I particularly enjoy the wide variety in the work I undertake. The National Conference in June gave me the opportunity to meet many members and actually put some faces to names. Although the National Conference was very hectic it was also very enjoyable. The support and understanding shown by members towards a new member of staff has been most welcome and has greatly helped with my learning about MPS and related diseases. So thank you all.

I have joined a very supportive staff team, which has made me feel at home very quickly and I would like to extend my thanks to them.



Mary Moulding: Her Story

On a rainy day in July (typical English Summer weather!) I went on a trip to the Isle of Wight to visit one of the oldest supporters of the MPS Society.

In a prime location with a magnificent view over the sea, I found Mary Moulding (a spritely 82-year-old). Mary was one of the first supporters of the MPS Society as her daughter, Pam, had Maroteaux-Lamy disease.

Pam was born in 1946 and lived to be 40 years old. Mary planted a tree at the Childhood Wood for Pam at the first planting in 1993. Unfortunately she has not been able to make the long journey back up to Nottingham to see how the tree has grown since then.

Pam was at school when the family first moved to the island and went on to become manageress of a shop. Following that Pam worked at the Sandown Caravan Park where she was in charge of the bookings. She had her own car which enabled her to get around and she enjoyed her independence.

Pam used to love crocheting and made many things which Mary still treasures. Mary told me that Pam was never idle and always enjoyed being busy.

Pam had a big party for her 40th birthday and Christine Lavery accepted an invitation to attend. Mary has never forgotten this and speaks fondly of Christine and the support she received.

Mary looked after Pam herself and nursing her at the end. Mary has been an avid supporter of the Society from the start and continues to raise money and awareness of MPS.

Mary is clearly very proud of her daughter's achievements and has treasured photographs and fond memories of Pam although she doesn't like to dwell on the past. Nowadays Mary does very little. She spends her day looking out over the sea, reading, or writing poetry.

Mary is something of a local celebrity on the Isle of Wight. Her book of poetry 'Home Truths and Reflections from the Isle of Wight' was published in 1999 and she has pictures of Pam and posters supporting the MPS Society surrounding a well in her front garden.

Mary has been married three times (successfully she asked me to point out!) but now lives on her own. However, she has a large family and many visitors and continues to support the Society in any way she can.

She was an inspiration to meet and if I could demonstrate half her kindness, spirit and wicked sense of humour I would count myself a lucky person.

This photograph of Mary was taken at the home visit.

Getting to Know the Society's Trustees

The MPS Society is governed by a voluntary board of Trustees. Currently there are 9 Trustees. Most of the Trustees are people who have direct experience of living with MPS and related diseases, either because they have MPS themselves, or because someone close to them has one of these conditions.

The Management Committee is legally and financially responsible for the implementation of all the Society's policies as well as overseeing the Society's UK wide support, research and fundraising strategy. The Management Committee is responsible ultimately, for everything the Society does on behalf of people affected by MPS and related diseases.

Judith Evans

I am. ..Judith Evans, definitely 'over 21', married for many years to Graham and mother of Joanne who has MPS IV -Morquio's. Although Graham and I were both born and raised in England, we have lived happily in Scotland for 22 years and indeed Joanne was born here in 1986.

Joanne was diagnosed with Morquio when she was 3 years old and we went through the whole gamut of emotions that all of you will have experienced. It took me a long time to come to terms with Joanne's condition, and initially I couldn't bring myself to contact the Society, although I did receive copies of the newsletter, and how it has changed over the years emerging as the glossy, professional publication we all enjoy today.

So my association with the Society began very slowly and I think this is one of the unique aspects of the Society -each family or individual can take from the Society what they need and when they need it, secure in the knowledge that there is always a friendly, compassionate and caring staff team available to listen to their problems and offer help.

As the years went by we attended and enjoyed events organised by the Society and at the AGM in 2000 I was appointed a Trustee; I was more than a little apprehensive at the enormous responsibility of helping to run such a prestigious charity and, of course, the steep learning curve facing me. However, three years on, I've survived and recently stood for re-election to the Trustees Board which probably says everything about the job and I can honestly say I've thoroughly enjoyed my first three years, even latterly the position of Treasurer! The work involved is certainly challenging, but never onerous; the range of responsibility is huge but always interesting and the learning curve is endless but such a wonderful opportunity. The Board meetings throughout the year cover a wide range of topics but we are always wisely guided by our outstanding Chief Executive, Christine Lavery, whose vast experience ofall aspects of the MPS and related disorders is recognised worldwide, and we are all aware ofhow fortunate we are to have someone ofher calibre leading the way.

Judy Holroyd

My name is Judy Holroyd. I have been an MPS trustee now for 2 years.

As a family, we first made contact with the MPS Society in 1986, just after our eldest son, William, was diagnosed with sanfilippo disease at the age of 10 years.

In the years that followed we derived much positive support from our links with the Society. Sadly, William died in 1996. Our three other children are now in their twenties.

After 15 years at home as a housewife and mum, I took up a career in teaching and worked in a comprehensive school for 10 years. A few years ago I felt ready for a change and spent 2 years retraining as a genetic counsellor. Some of you may remember me from my research project on 'decisions about having a further child after the diagnosis of an MPS child in the family' that I undertook as part of my studies. I have now been working as a genetic counsellor in the NHS for 2 years.

Ann Green

Ann Green has been a member of the MPS Society for almost 20 years. She and her husband Roger had a son, Charles, who suffered from Sanfilippo disease. Charles was born in 1978 and was one of the first children to receive a bone marrow transplant at the Westminster Children's Hospital. The transplant was unsuccessful and Charles died in October 1982 - a few days before his fourth birthday. Ann has a daughter, Laura, who is now 26 and is unaffected by the disease.

After Charles's death Ann returned to work in the NHS. She was subsequently appointed to a number of Human Resources posts in various NHS organisations and over the past eight years was a Board level Director of Human Resources in two NHS Trusts - latterly coupled with responsibility to the Board for the local Child and Adolescent Psychiatry service.

In September 2002 Ann was seconded to work with the Human Resources Division of the NHS Leadership Trust in London, with whom she is currently working.

Ann is a member of the Chartered Institute of Personnel and Development (CIPD) and holds an MA in Human Resource Strategy from London University.

Sue Peach

Hello, I'm Sue Peach and I'm married to Dave, a laser engineer. We have been members of the MPS Society since it's beginning. Our daughter, Elisabeth, was diagnosed with Hunter's in 1981. In 1982 she entered Westminster Children's Hospital and had two bone marrow transplants. Sadly, she died in the hospital in July 1982. Our two sons, Matthew and Timothy, were the brave bone marrow donors. They are now grown-up, Matthew is a doctor and Tim has followed in his father's footsteps and become an engineer, working on hi-tech racing engines.

For my day job I teach Englsh and Drama in an 11 to 16 secondary school in Daventry. It can be challenging, exhausting but also very rewarding at times. My school has a large Special Needs Department and we have several wheelchair bound students and students with a range of problems -Spina Bifida, Cerebral Palsy and Downs- but as yet no MPS students. Every year I organise the Jeans 4 Genes Day and we usually raise over £800.

Outside of school, I'm a councillor on Rugby Borough Council which entails attending, on average, five meetings every six weeks. I'm also a Governor of a local school. Dave has got used to this and I think he quite likes the quiet times when I'm not there. I must be a glutton for punishment because I also represent my union at school and enjoy attending the annual conferences. In addition, I am a moderator for the GCSE Drama exam which means I get the opportunity to visit lots of different schools.

In my spare time -yes I do get lots- I enjoy reading, going to the theatre, having a pint or two with friends on a Friday night, cooking, doing crosswords and helping Dave in the renovation of our house - I choose the colours!

Bone Marrow Transplant - The Right Decision for Tara

Maria Murphy

Hello my name is Maria Murphy, mother of Kate and Tara. Our youngest daughter Tara was diagnosed with a metabolic disorder MPS I Hurler on 22 June 1998, aged 33 weeks.

Like all MPS parents receiving the diagnosis was the most devastating news you could hear but even more so as up until that time we were totally unaware that anything was remotely wrong with our daughter. To us Tara was just like any other healthy baby and to receive this diagnosis threw us into emotional turmoil. I can remember sat there and asking the doctor "is she going to die?" and he replied yes.

Due to being in a breach position and other complications, Tara was born by emergency caesarean section on 30 October 1997.

During post-natal checkups measurements of Tara's head circumference were taken as her head was quite large and a bit misshapen but this was put down to the breach position during pregnancy.

3 months after Tara's birth we moved house to a new area and therefore changed to the local Medical Centre. During a visit to the doctors in May to treat an ear infection that Tara had, the doctor commented on the fact that Tara's forehead was slightly more protruding than was nomlal and referred us to the Bristol Childrens Hospital. I wasn't concerned by this as members of family seemed to have quite high foreheads. An appointment came through for 1 June.

At this appointment we were introduced to Dr Chambers who examined Tara. He pointed out a slight curvature of her spine and that her tummy looked swollen but didn't proceed to tell us his fears at that stage. All sorts of tests were done on Tara, X-rays, blood and urine tests, amongst others regarding her height and weight. Thinking back I wonder why I didn't ask what all the tests were for but I couldn't see that there was any problem as surely something would have been said. We had a three" week wait until our appointment in which time we were still not unduly worried.

Dr Chambers sat both of us down and started to explain Tara's condition and gave us her prognosis. We had never heard of Mucopolysaccharide and couldn't even spell it. He had to write it down on a piece of paper for us. I don't know how long we were in that meeting but it was like time stood still. Our emotions were doing somersaults and I felt a tremendous feeling of nausea rising from the pit of my stomach - our beautiful baby daughter had just been given a death sentence.

We left the hospital feeling numb with pain and after visits to our parents we went home. Family and friends were constantly visiting and phoning offering their sympathies. It was awful. I just wanted to scream at them all to leave us alone and to stop being kind and sympathetic and longed for everything to be back like it was before.

Tara (right) with sister Kate exploring Pompei

The pain was excruciating when I thought of the changes that would eventually take place in Tara both physically and mentally. I didn't want her to look different, I didn't want people staring at what she would eventually look like, I wanted my beautiful baby to grow up normally not to have to face the progressive decline that this disease inevitably caused.

The next few days we spent in a haze of tears and unbearable hurting. It was the most desperate time of our lives and we were both emotionally exhausted. I remember one night wishing that I could sleep forever and not have to face the heartache of reality that waking brought. Ivan was so upset with me for having such terrible thoughts and made me realise that we had our 2 dear children to look after and had to think of them and the future no matter how bleak it looked.

That same week on 26 June, we had a meeting with a consultant, Colin Steward, who confirmed Tara' s condition and explained the effects it would have on her. We didn't think we could feel any worse than we did but Colin was painfully honest of what the future held for both Tara and us her family. He did mention the possibility of a bone marrow transplant as treatment for her condition but not a cure and explained possible side effects and even the chance that Tara could die during treatment. At this stage we didn't know if we had a bone marrow match for Tara.

The following Friday on 3 July we travelled to Manchester and met Dr Ed Wraith. Again he explained the condition and effects of the disease and talked about the possibility of a bone marrow transplant. He was optimistic about transplants and this made us feel hopeful that there was a chance for Tara.

We understood that the transplant was not the end of Tara' s condition but it was the start of everything that she would have to endure throughout her life.

It was the hardest decision that we had to make in our lives, one minute we both agreed, then next we changed our minds and then back again. Without the transplant Tara would become severely handicapped both physically and mentally and eventually die at a young age but there were no guarantees of what lay ahead after the transplant and this was a terrifying thought. Knowing what we know now and seeing how well Tara has progressed we shouldn't have even hesitated about her having a transplant but at the time we were afraid of possible side effects during treatment and if she would be both physically and mentally able after the transplant.

If we went ahead with the transplant no one could guarantee that there would not be a possibility of mental decline and there was also Tara's skeletal problems to consider. It was a huge worry that no one could say if she would still be able to walk when she reached her teens or say how much her bones would have deteriorated at that time. Also how would Tara cope and feel about the deterioration of her body and maybe her bodily movements as she grew older. It wasn't a cut and dried decision. It was the hardest decision trying to decide that if we went ahead what quality of life would she have later on both physically and mentally. Also I believe that people are very sympathetic and tolerate small children that are handicapped but as these children progress into adulthood I think peoples attitudes can change with lack of understanding and patience.

When we arrived back in Bristol we had come to the decision that we wanted Tara to have a bone marrow transplant. We didn't know what the future would hold for Tara and tried not to think too far ahead but we knew that without the transplant it was certain death.

The following week on 9 July Christine Lavery visited us and introduced herself. It was during her visit that we had a telephone call from Colin Steward to say that Kate was a tissue match for Tara and that Kate's enzyme level was above average. Kate was just 3 years old at the time. The decision was made.

Tara started her treatment on Friday 24 July. During the course of treatment Tara began experiencing Supra Ventricular Tachycardia or SVT , which is also referred to as palpitations and was prescribed heart drugs to control the condition. The flrst time she experienced SVTs the doctors used the method of holding Tara's head under water to slow down the heart. We weren't allowed in the ward while this was being done as they said it would be too distressing and I think they were afraid Ivan would punch them. The look on her face said she didn't like her swimming lesson much.

Tara's transplant took place on 4th August and took about 2fi hours. She was remarkable during her time in isolation and always had a smile for everyone. Every day we were are tenderhooks just praying that the transplant wouldn't reject and that her white count would increase. It felt very lonely during isolation and there was an awful lot of time to worry yourself senseless about the "what ifs". Tara lost her hair about 5 days after the transplant but when it grew back she had a head of Shirley Temple curls.

Tara was allowed home on 28 August and then spent the following six months in semi isolation at home to limit the chance of infection.

Two months later was Tara' s first birthday. We wanted to shout from the rooftops and celebrate how well she was doing but of course it had to be a fairly quiet affair and the family came in twos to visit, rather like Noah's Ark.

At this stage I was worried about Kate, that she was also being kept in semi isolation. With the help of our Social Worker we were able to get a place for Kate at a nearby Nursery which up until that time we were refused as we lived outside the catchment area. It was very important to us that things should be kept as normal as possible for Kate and attending Nursery helped a great deal where she made lots of friends and enjoyed herself.

Tara started attending a Social Services Day Nursery

when she was 2 years old. It was a very difficult and distressing time for Tara and terribly upsetting for me. She didn't want to go and cried to stay with me. At first we would stay for a short time and gradually increased her visits but still she would become very upset and cling to me. If it wasn't for my Mum telling me it was best for Tara, I would have stopped taking her but after about 4 emotionally charged months she actually started to enjoy going.

Eventually she settled into nursery routine and enjoyed participating in all the activities. She started interacting with the other children and Tara's speech slowly started improve enabling her confidence to grow. Tara really began to enjoy nursery and made lots of friends.

While Tara was at Nursery we were invited by our Parish Priest to visit Lourdes. We had a wonderful time and it was both a spiritually and rewarding experience. We spent our time there with the Glanfield Childrens Group which comprised of children of all ages and disabilities.

Tara left Nursery to start St Bernadette Primary School last September and a Statement of Educational Needs was awarded to her.

On her first day of school we felt tremendously proud of Tara and all that she has achieved so far. It seemed a long time ago since we were told of Tara's disease and couldn't believe that she was now all dressed up in her new school uniform ready to go to school just like her 5 year old friends. It was a dream that we never thought we would see and I cried with happiness as she walked into her new school.

It was important that great care and attention was taken during the transitional period from Nursery to School as Tara finds changes in routine upsetting. Fortunately Tara settled into school exceptionally well. She enjoys school and has made several new friends. Unfortunately, the Local Educational Authority reduced Tara' s Statement once. the transitional period was over and she has only been awarded enough points to cover her physical safety with no extra educational support in the classroom.

With advice and support from Tara's Consultant and the MPS Society we are aiming to get the Statement increased so that Tara is able to receive the help and support in the classroom that she is entitled to.

Last year after a lot of thought we decided not to have any more children. We decided upon this because if we did have another baby with the same disorder, we couldn't believe that could possibly do so well as Tara has done during and after treatment. We feel so lucky that Kate was born first and that she was a tissue match for her sister. We couldn't possibly be that lucky again.

It is likely that Tara will have to endure many hospital appointments and undergo orthopaedic surgery at some stage in her life and we don't know what the future holds. So we try to enjoy everyday as a family and not to think too far ahead and worry about the future. I would like to say that I feel one of the luckiest women, I have a wonderful husband and two beautiful daughters who love each other dearly. I wouldn't change my life for anything.

Douglas House

Jeff Bawden

In May of this year I was privileged to visit Douglas House in Oxford, a new respice unit for people between the ages of 16 and 40, which has only recently opened. Douglas House is situated alongside Helen House the local children's hospice which some of our members use. Douglas House is named in honour of a young man who suffered from Batten's disease who lived into adulthood but had to continue to use Helen House as there was no other appropriate service for him.

Douglas House has seven en-suite bedrooms and accommodation for families, a gymnasium, a fully equipped music room, study areas, a sensory room, a beautiful roof garden and most importantly a licensed bar.

When I visited I was impressed both by the physical design of the building and the commitment of the people who had designed it and those who are going to run it. Services for this age group are few and far between, yet are increasingly needed. Lets hope there are many more to come.

If you would like further details about Douglas House please do not hesitate to contact the MPS Society. The Society can also provide details of other hospice services throughout the country.

Disneyland Paris - The Holiday

Marianne & Adrian Stimpson

Some of you may remember an article that was in the last newsletter called "The Price of Being Disabled". Well this is the concluding part of the story!

The basic problem was that Eurostar/Thompson were forcing our son Dominic (Sanfilippo) to travel First Class and to pay the extra fare to travel in his buggy. They said that the Standard Class coaches weren't suitable for people who needed to travel in wheelchairs. Our argument was that Dominic was being discriminated against because he was disabled.

With the help of the MPS Society we contacted Disability Rights. Apparently, disabled people are entitled to the same areas and treatment as those who are able-bodied in everything except transport. They are currently working to rectify this.

We spoke to Eurostar/Thompson on many occasions but were getting nowhere until our local radio station - BBC Radio Norfolk became involved. At the mere mention of the BBC their attitude changed completely. They were so afraid of bad media coverage we were receiving phone calls and letters almost daily. Thompson upgraded our party to V.I.P. status. This meant we would get superior rooms, we wouldn't have to queue for any of the rides or to meet the characters and there would be gifts in the rooms for the children. Some of this turned out to be dissapointing. The "superior" rooms were no different to the others and the gifts were non-existent. However, they did refund some of the money we'd paid out and apologise for all the upset they'd caused. They were adamant that these problems didn't usually occur and even tried to tell us at one stage that they hadn't forced us to pay First Class fares for Dominic but we had it all written on an invoice so they had no room to argue!

The holiday itself was great and Eurostar is a good way to travel. They were very helpful with Dominic - helping him on and off the train. He had a large space for his buggy and a carer seat next to it. We stayed at the Hotel Santa Fe which was the only one that didn't have bunk beds. They wouldn't have been much use to us! There was a shuttle bus every ten minutes which was great but the people did not know how to queue. They didn't care whose feet they trod on or whose buggy they pushed out of the way, just as long as they got on the bus. To begin with we just thought the people were incredibly rude but by the end of the holiday we were just the same as them! We had to be otherwise we'd never have got anywhere. Our elbows are now very sharp and there are a lot of people with sore shins and ankles thanks to Dominics buggy! Not all the buses had disabled access but the drivers would radio for a minibus with a ramp if they couldn't get us on which was brilliant.

Dominic can now only manage pureed foor and it was a problem finding a restaurant that would do this to begin with. Eventually we found a steak house in the Disney Village who were really kind to us. They pureed his food beautifully and even garnished it for him. We took Weetabix for breakfast and heated the milk in the hotel microwave. For lunch we took tins of Big Soup, a flask and a hand blender. Again we used the hotel microwave. If you're thinking of taking a trip to Disneyland Paris, this information may be of use!

We have some terrific memories of our holiday. Dominic had a fabulous time, in fact a great time was had by all. The children's rides we wanted to take him on were all wheelchair friendly and he missed out on nothing. Dominic and our daughter, Jodie, laughed and played the whole time so in the end it was worth it.

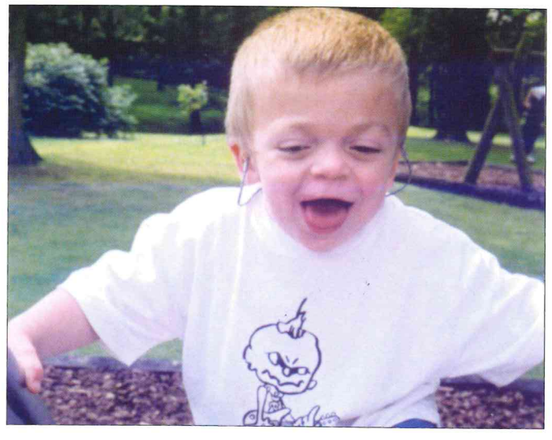
Bringing Issac Home - Part 2

Richard Dunn

For five weeks, Lou didn't leave the hospital. While Isaac was in the bone marrow unit she would go nowhere. She had to remain on watch. Tenderly guarding her son and the moments they had together.
Adam, Lou and Kay establish a routine. No one has time to relax and no one has privacy. Lou and Adam take turns at night. One lying awake in the parents room, waiting for another day to begin and the other sleeping in Isaac's room. Nurses come in every fifteen minutes to check on Isaac. The machines monitoring his progress send periodic signals to distant stars.

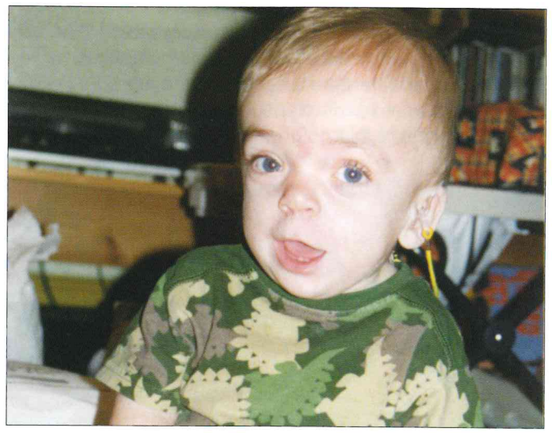
Each day they watch drugs being pumped into Isaac, ultimately to make him well but at the moment, to wipe out his resistance. To decimate him. They have to watch impassively as nurses, wearing rubber gloves and plastic visors, flush toxic drugs direct into his heart, through a tube in his chest. They watch, knowing it would happen but stunned by the ferocity of the happening. Torn into a thousand pieces as they see the lining of his throat disintegrate. As his hair falls out. As he vomits blood. So much pain we are forced to inflict on this little boy, in the desperate gamble to save his life.

Through the sealed window the drama is played for all to see. I look on in admiration as father, mother and grandmother, drawing on deeper reserves and with infinite care, fight for Isaac's life.



I watch Lou keeping emotions at bay until they bubble to the surface uninvited and trickle down her cheeks. I see her curled up in a chair too numb to move, for the moment overwhelmed by what she is facing. I watch Adam moving around. Edgy and restless but in control. Desperate to escape for a while. I see him take charge when Lou folds in on herself.

Perched on the stool by the window I observe a world I am forbidden to enter. I watch Kay, resolute, determined



and uncompromisingly optimistic. She is on a crusade to keep her children from sinking to their knees.

Everything goes into the room through a hatch and out through another, except for the sound. The sound escapes through the walls. I am struck by how quiet the bone marrow unit is, except for Isaac's room. In his room everyone has to smile. Often I see Kay, Adam and Lou singing, clapping and laughing, not in defiance, but to make Isaac smile. I hear a little boy with no lining in his throat and no energy to move, emit a quiet but joyful cackle. I sit by the window, paralysed by feelings I can't begin to understand.

The first transplant had rejected and they couldn't find another donor. The doctors decide to use a peripheral

stem cell transplant, using Adam's blood. This procedure had only been attempted once before on a "hurler" baby. We roll the dice again.

Lots of cards arrive on the day wishing good luck. Lots of people ring to ask how the operation has gone. Everyone has envisaged some sort of major operation. Doctors in masks wealding scalpels under blazing theatre lights.

The reality is something far more simple and poetic. Isaac sits on Lou's knee. Both his parents hold him gently, as the "CD34" cells from Adam's blood drip through the hickman line into his heart. Quiet, cool and unearthly slow. Each drop of liquid a gift from father to son. I watch the scene unfold on the other side of the glass. It is the 25th of July. Isaac is twenty months old. He sits lifelessly, cheekbones stretching the skin, tight across his face. My role as ever is to wait and watch and smile encouragement.

It's seven o'clock and I'm sitting in front of the sealed window again. I can't work out whether I'm inside or out. I'm looking into the room but somehow feel that I'm the one in isolation. I'm the one on view.
Sunday evening and it's time to leave. I wave to Isaac. He's sitting on Adam's knee. Both motionless; both lethargic. Kay and Louise come through the forbidden door for a final hug.

" I'll be back next Friday and I'll ring every day to see how he's doing."
"Thanks Dad. You look after yourself."
"I'll ring you tonight", Kay says cheerfully. We all cling to each other, leaving many things unsaid.

I pass through the first door and collect my coat. I wait for the first door to close before the second one can open. I think about an astronaut going back to Earth. Climbing through the air lock unable to open the second hatch until the first one is sealed. Alone now in his space suit, entombed by his private thoughts. Alone now. The sound of breathing his only companion. Alone now, I walk along the corridor with a sense of relief. A chance to return to a normal world. Submerge myself in work. I walk away feeling overwhelmed by guilt. I seem surplus to requirements but can't help feel that I am deserting them.

It's the same each week. I drive towards the motorway planning for the week to come. I use day to day practicalities to drown the sensations. Emotionless until I hit the motorway slip road and put my foot down.
My car accelerates onto the motorway like a spaceship in re-entry mode. I am like an astronaut returning to earth. The pain appears from no where. I cry out. Great sobbing tears. Intense but short lived. A cry of outrage, lost in the depths of space.

By the time I pass the Oldham junction I have folded and packed my emotions neatly away, like shirts in a suitcase. I have left the gravitational pull of the bone marrow unit. I have left my family for another week.

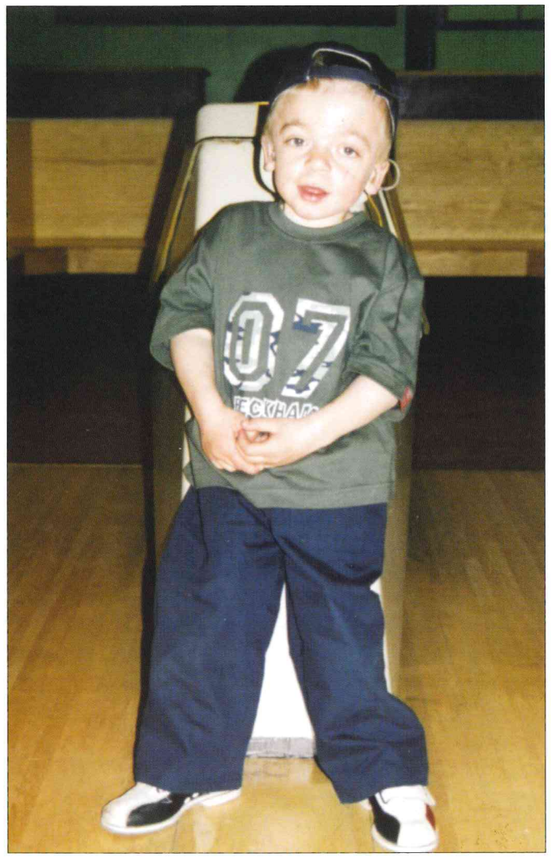
Strangers come and go like flotsam, washed up and down the beach by the tide. They say hello and goodbye with little consequence as they go about their daily routines

Sometimes though, some moments in our lives are significant, even momentous. Like milestones, they mark our memories. They are times when we stop and reflect. We feel regret or joy and our pulse quickens.

It is Friday 11th August. The time is 10 am. I'm at my desk and the phone rings. "It's your wife, Dick. I'll put her through." "Hi love, how's he doing?" "He's got enzyme. Isaac's got enzyme," Kay shouts gleefully down the phone. All my well prepared defences crumble. I'm smiling but I can't speak. " Are you there? Dick." I nod. There are tears running down my cheeks. I can breath for the first time in two years but I have no words left. Three weeks after Isaac's second transplant his tests show that he has developed the enzyme, which will save his life. This is our first fragile victory. Months of uncertainty lie ahead and no one dare believe yet that all will be well. We are still circling the planet but the glow of a new day seeps across the horizon. We begin to imagine bringing Isaac home.

Isaac Turner. Aged 4. "Isaac is a delightful boy with the support of terrific parents and extended family. He has a remarkable zest for life and a sunny disposition."

(Isaac's Grandfather)



What it felt like to be told your baby has MLII

Paul Sagoo

Nancy, our first child, was born on 18th June 2003 weighing 1.6kg. Naturally, my wife and I were elated and full of joy. A few weeks later, we received some devastating news about her condition. She was diagnosed with I-cell disease (Mucolipidosis Type II).

We were absolutely shell shocked and numb. It was like hearing a loud clap of thunder when least expected. We fell into an instant daze. Being in a dark room for a split second might be one description of our first and initial reaction. Our emotions were in chaos and playing havoc with our everyday expectations. Nobody could have written this script. My own first thoughts upon hearing the news were that this is unreal and not really happening - a terrible mistake.

After some time, we gradually began to try and comprehend piece by piece the meaning and scale of our devastation. Our way was to talk with friends and family with whom we could be entirely open and honest. We still have a long way to go but we have begun the uphill climb in a positive and assured way. There is no quick fix to deal with our plight and anxiety but there are methods that we have had to establish quickly to manage the daily needs of Nancy. In a strange way, it is like attempting to reroute the lines of the London Underground at incredibly short notice, without causing too much disruption!

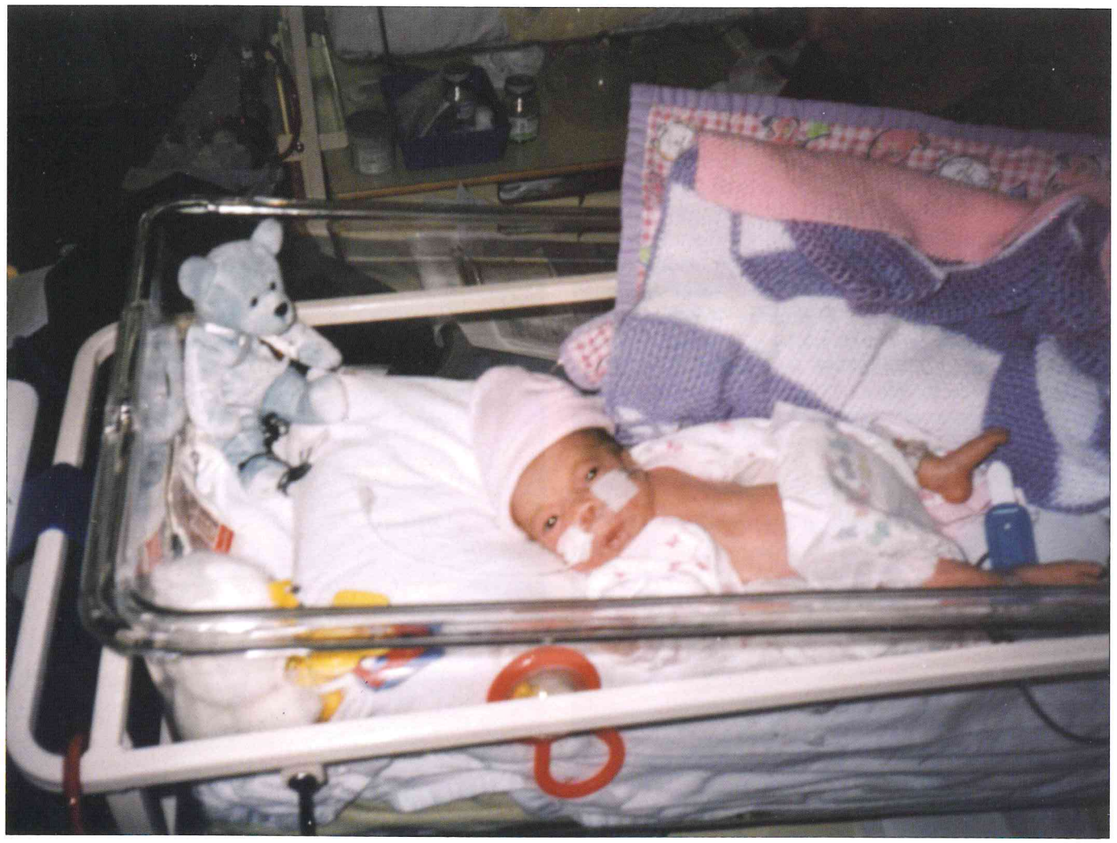
We have no choice but to survive and be strong for our loved one - our prized possession. We have to be sure that we are not alone and we must never lose sight of this. We realise that if we suffered in silence then the outcome would be extremely damaging and hence the enormity of the pain would rapidly become widespread.

Coping with this is hard and challenging. We remain committed and supportive of one another. This we feel is fundamentally important to ensuring that Nancy receives our undivided and united attention at all times. We have adjusted to becoming parents remarkably in such a unique situation.

Nancy is a beautiful girl - a real diamond. She is responsive and alert and she loves to be the focus of attention. She looks forward to her massage sessions with the physiotherapists! The nurses and doctors are tremendous and they continue to deliver a service to Nancy that all children in the world so rightly deserve - but only a few receive.

Our newly formed relationship with the MPS, will be critical to obtaining the support and information we need both for us and Nancy.

Nancy has a permanent place in our hearts and we love her dearly.



The days leading up to Nancy's farewell...

Paul Sagoo

Saturday 2nd August 2003

For the first time since Nancy was born, I decided to visit her in hospital on my own. Up until this point, my wife had always accompanied me. For the first time, there was a deep acknowledgement in my heart that Nancy would not survive. The time had come to ease the suffering of Nancy and us as parents. I wanted the support from the experts and the medical staff, which I readily received. They knew like I knew that Nancy's outlook was bleak - agonisingly grim.

Nancy's prognosis was poor and her dependency on respiratory aid was fast becoming a real necessity. This aid would only seek to perpetuate her existence and provide no quality of life - no light at the end of the tunnel. Complete isolation and despair. Her condition was deteriorating and any semblance of hope that we had diminished rapidly. Quite frankly, there was no hope. There was no miracle. Hopelessness and helplessness surrounded Nancy's whole world.

That afternoon, I returned home and consulted with my wife and told her that the doctors and nurses had informed me that it would be in Nancy's best interests to discontinue the respiratory aid. My wife accepted this reality calmly. From the bottom of my heart, I knew instantly that my wife was aware of the seriousness of Nancy's condition and that all belief had faded. My wife and I had to put our emotions to one side for a time somehow and quickly adopt a pragmatic approach in such difficult circumstances.

Thursday 7th August 2003

The time had arrived to initiate the decision - the decision to ease any more unnecessary pain and affliction to Nancy. There was absolutely no choice. Nancy was always being kept comfortable. All the medical staff and we owed Nancy a painless and peaceful path but above all dignity, respect and love.

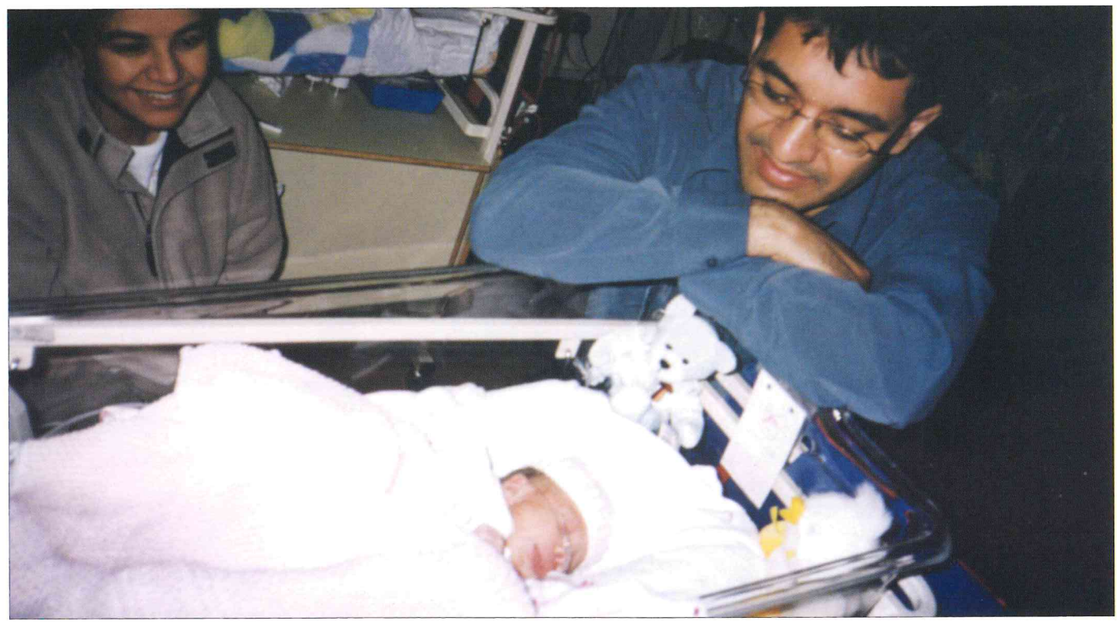
Saturday 9th August 2003

At this point, Nancy's breaths could be counted and between each one the interval got longer and longer. Nancy passed away in her mother's arms at 9.15am. She was at peace and her place in Heaven was rightfully secured.

Almost immediately after, we felt numb and empty but we could breathe a sigh of relief. Our arms and legs felt like lumps of lead. Our emotions were indescribable. Mere words cannot justify how we felt after the loss of our prized treasure. In our minds, we experienced an ordeal that was perhaps nothing short of a roller coaster ride in a desert storm.

However but thankfully, we knew that Nancy's suffering was over and her soul would encounter a new and fruitful beginning. Our connection with her spiritual existence was being realised and understood.

A few days later, I spoke with a work colleague and close friend. He and his family had been through a similar experience. Appropriately and as a testament to Nancy's strength, he said to me 'Your little Nancy has joined my little Sarah in God's world'. 'That's where all the beautiful angels go'.



Getting to Grips with Post Primary Education

Bernie Drayne

November can be a very stressful month for many parents as their children finally sit the 11 + exams. It marks the culmination of nearly two years hard work and preparation. Last November I watched the parents bring their children in to do the exam, the worry and anxiety was etched on the faces of both parents and children.

I have a special interest this year as my daughter has now reached primary seven, and like her peers she spends most ofher time doing 11+ practice tests. My daughter Roma should be sitting this exam this year, but as she is a pupil with a statement of educational special needs she will not be allowed to sit the exams, and will instead be assessed through course work and by an educational psychologist. I am sure there must be other parents among you who are in my position. As Roma has Morquio, she will need to use a wheelchair especially when she moves to post -primary school because of the larger campuses and distances between classes. She is academically bright but often lacks stamina which is caused by joint aches and pains.

The mainstream primary school which Roma attends have always been very caring and helpful, she has a classroom assistant and there are no stairs to climb. I have been advised to start looking around grammar or secondary schools which may be physically accessible for Roma. I have to make informed decisions and 'choices'. This is proving to be a very challenging and informative experience. Indeed, the whole process of the transition for physically disabled primary school pupils to mainstream post primary school seems to be an unknown entity to many professionals who should be involved in this process. My social worker did not know that statmented pupils are not allowed to sit the 11+ exam, and my occupational therapist sees it in terms only of physical accessibility, suggesting that my daughter should travel about 50 miles every day to school. Other professionals have suggested sending my daughter to a special school. All the infonnation sent out by the Education Boards, the Department of Education and the Code of Practice on the Identification and Assessment of Special Educational Needs completely ignore this important transition at age 11 to post primary school for statemented pupils within the mainstream system. Furthermore the Burns Report paid very little attention to these pupils.

I have many questions to ask, about this process and as a parent I am entitled to accurate accessible specific information. I have been told that this will all be sorted out during the last Annual Review at Primary School. But I have to be prepared for this review, I must know the right questions to ask, who to ask and where to go for this information. I have also been given contradictory infonnation about the provision which my daughter will receive in respect of classroom assistance. No-one seems able to advise me honestly on what to ask for when compiling the needs statement for the next school. Secondary or grammar school transition brings with it many problems. There are many more teachers and classrooms involved than primary school and all this has to be considered. Whilst the children who sit the 11 + repetitively sit mock tests I am not allowed to coach my child in any way through some of the tests which the educational psychologist will set for her. I have also been told that an early assessment is not possible. It would really help me if I knew at this stage or had some indication whether my child will be assessed as being of grammar or secondary school material, so at least I could cut down the number of schools which I have to approach.

I have sent out letters to several schools, explaining Roma's position and asking for a school visit. I received 5 very swift phone calls from principals all dissuading me from visiting. I detected a certain nervousness in their voices as they gave me various excuses for not accepting a physically disabled child. These excuses ran from, listed buildings, crowded corridors, building work in progress, to one saying 'Oh there's no-one like that in this school'. This is so disheartening and often depressing. It is so apparent, even just from the language used, that some professionals just do not know how to cope with the possibility of having a disabled child in their school and many are fearful of this happening. One school which I did visit however, was very welcoming; they had a 'can do' attitude despite the fact that not all parts of the school would be accessible. This was such a contrast to what had gone before. But there does not seem to be many such schools around.

Roma MPS IV

My daughter should be able to continue her education with her peers, she should be allowed to develop her full potential within a caring environment which believes in inclusivity.

This is going to be a challenge for us as parents. To get through this process one has to be articulate and assertive. Hopefully this will not involve going to a Tribunal to get what my daughter is entitled to. I do not want to send my daughter to a school with a begrudging attitude. I just wish there were more enlightened professionals out there.

I wish schools would realise that children like my daughter have so much potential and so much to give. Others have so much to gain to from these children, I can see how a caring and accepting attitude has been fostered in my daughter's current school through her presence there. People have a general notion that disability discrimination is being tackled but from my perspective it certainly is not. I have came across some really outdated attitudes. There is a gaping hole in both official literature and knowledge when it comes to finding out about mainstream provision for physically disabled pupils when they leave primary school and move on at age 11. 'Informed choice' here means that parents have to make an absolute nuisance of themselves. They must be assertive and not afraid to ask questions whilst remaining unemotional. Parents are left to battle their way through this system alone. They have recourse to a tribunal if they cannot get a school to accept their child, but this is 'after the horse has bolted'. Surely there must be better creative ways of doing things. I am sure there must be other parents among you who are in the same situation as myself or others who have come through it recently. I would like to hear from you, because I believe that something should be done for these forgotten children.

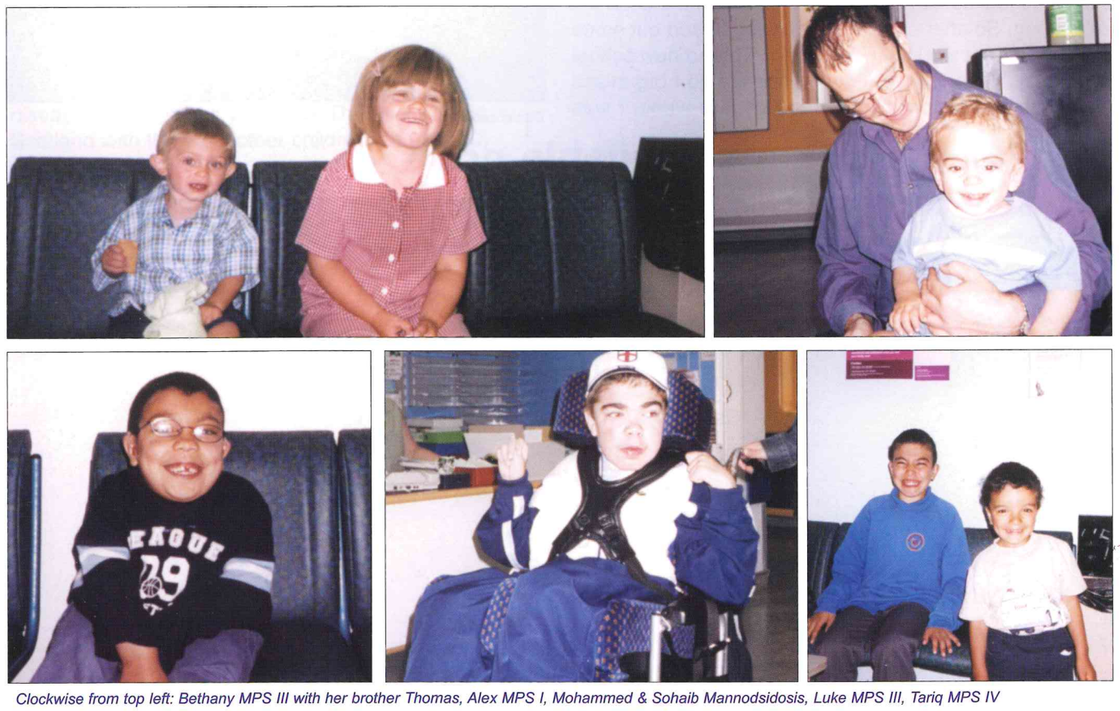
MPS Birmingham Clinic - 27th June 2003

Sophie Denham

Another successful clinic was held at Birmingham. 12 people attended the clinic, which was held on Friday 27th June 2003. The clinic ran relatively smoothly for Birmingham, which was helped by the prompt arrival of everyone for their appointments despite the major road works, which seemed to be happening around all of the entrances to the hospital! It was good to see so many of you at the clinic, both old and new. Some of you are now probably thinking that you did not see me and for some this is true as I got called away, but you were left in the capable hands of my colleague Jeff and if any of you wish to hire Jeff to read stories to your children, I have it on good authority that his story telling skills are superb.

Many of you in your comments about the clinic highlighted the fact that the waiting area is not user friendly and does not afford a social clinic. We are aware of the difficulties with the waiting area and we are working together with Dr Chakrapani in looking at how this can be improved.

Lastly our thanks go once again to Dr Anupam Chakrapani, Dr Ed Wraith and Joy Wright for the excellent work and for another successful clinic.



Clockwise from top left: Bethany MPS III with her brother Thomas, Alex MPS I, Mohammed & Sohaib Mannodsidosis, Luke MPS III, Tariq MPS IV

Life as Harrison's Brother

Daniel Luckham

Harrison has just had his 5th birthday and it's been just over a year since I found out that he'd been diagnosed with Sanfilippo. What I had always believed to be hearing and speech problems turned out to be such an awful illness, Sanfilippo disease.

Mum had known since February 2002 but didn't tell me until my exams were finished.

The day I was told I was so upset, I cried for hours until there were no tears left in me. It was the worst feeling, I knew that Harrison was different but this was so unexpected. He is such a happy little boy which made it hard to cope with "what would happen to him in the future". As time went by we all began to accept the situation and I realised that Harrison had not changed, he was still the perfect brother he had always been. Me and the rest of the family had decided that we would make the most of the time we had with Harrison and enjoy him to the full.

I started my first year of sixth form last September and became a volunteer for a local organisation called CHOS. I was nominated a family with a special needs child who I would go and spend time with once a week so that the parents could have some time to themselves.

Becoming a volunteer was the first way that Harrison's diagnosis had affected my actions. I wanted to help and make a difference to another family knowing how much we appreciate help that Harrison gets.

I had visited a couple of special needs schools that Harrison may go to. I felt that I could work in a special needs school as I loved the atmostphere and the job felt rewarding. So after christmas I decided to sort out some more volunteer work that I could do. A brand new school for autistic children had recently opened so I organised a day from sixth form to go there once a week. I was appointed as classroom assistant and really enjoyed working there.

In June it was the MPS Society Conference, we were all quite excited but nervous about going. The nerves soon disapeared as everyone was welcoming. The trips enabled us to mix with other families and volunteers. I made new friends, and although upsetting at times, made me want to be a volunteer at future conferences.

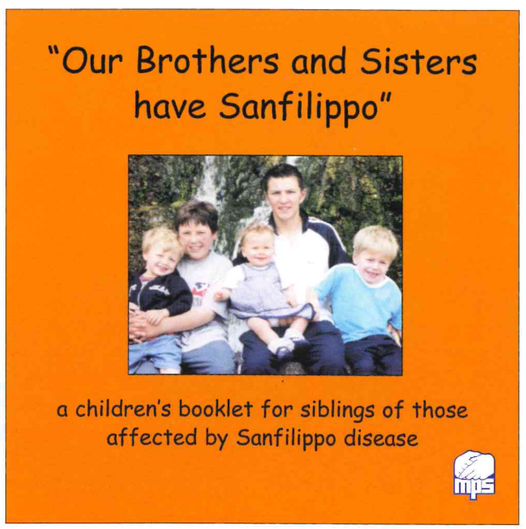
Recently I have started working at a Montessori school as a one to one for a special needs child. It has proved to be very rewarding and although it can be a challenge it is always a pleasure.

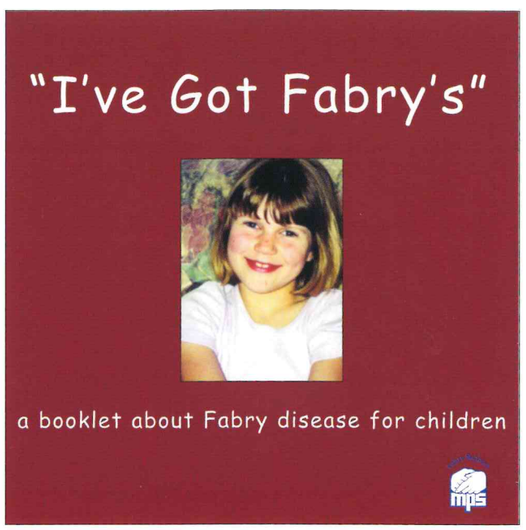
Because of the volunteer work I have been doing in the community and dealing with Harrison's diagnosis I have just been presented the Rotary Club youth endeavour award.

I know that Harrison's worst is still to come but I will endeavour to make Harrison's and other children's lives as happy as possible where I can.

Daniel seond from the right

New MPS Booklets available now





New Members

Kate and Andy Hall's son Isaac has been diagnosed with Hunter disease. Isaac is 3 years old. The family live in the Midlands

Paul and Gudia Sagoo's daughter, Nancy, was recently diagnosed with ML II. Sadly Nancy died aged 9 weeks old. The family live in the South of England

Adele has recently been diagnosed with Fabry disease. Adele is 33 years old. She lives in the North West

Ian and Jane Dearn's son, Alex, has recently been diagnosed with Hurler disease. Alex is 1 year old. The family live in the Midlands.

The Society has recently been contacted by Malcolm and Pailine Crocker. Malcolm has Fabry disease as does Malcolm and Pauline's daughter, Aimee. The family live in Wales.

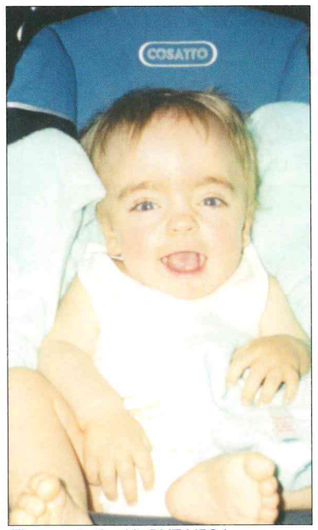
Carol and Graham Painter's son, Benjamin, has recently been diagnosed with Fabry disease. Benjamin is 13 years old. The family live in the North East

Anthony and Jayne's son Thomas has been diagnosed with MPS I Hurler disease. Thomas has recently undergone a BMT from an unrelated donor and is currently doing well. He is now 17 months old. The family live in the North East of England.

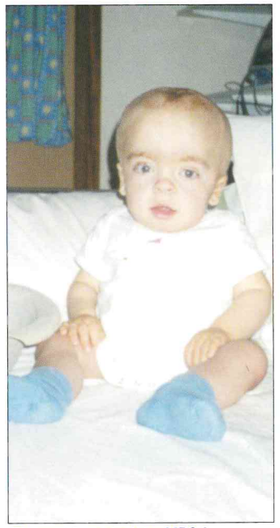
Mandy Wavell and her two sons, James and Kane have been diagnosed with Fabry disease. The family live in the South East.

Heather and Robert Reynolds' daughter Hayleigh has been diagnosed with MPS I Hurler Scheie disease. Hayleigh is 5 years old. The family live in Scotland with their two other children, James (10) and Emma (3).

Issac Hall MPS III



Thomas starting his BMT MPS I



Thomas 6 weeks later MPS I

Births

Megan Broadley at two weeks old. Megan was born on the 27th May 2003 and is a sister to Lewis, MPS III

Childhood Wood Remembrance 2003

Sophie Denham

The Planning of the Childhood Wood

Planning for the Childhood Wood officially started in January 2003. As it was the Society's 21st Birthday and the Wood's 10th Birthday this year, we wanted to try and do something extra special on the day. All staff were asked to put their thinking caps on for ideas of events that could happen on the day. After weeks of deliberations we finally came up with the idea of having a teddy bears picnic, a teddy bears competition, a balloon release and a children's activity.

The next step was the designing of the invitation where I found myself sitting on some extremely small chairs, in the children's section of the library, looking at pictures of teddy bears to use on the invite. This was due to none of the MPS staff being budding Picassos! Once found we used the image to create the front cover based on the theme "teddies in the wood". Families were asked to complete and return the booking forms if they wanted to attend.

Before any of the above could be implemented I needed to confirm the date of the Remembrance day with the wood, secure a plot for the marquee, order a marquee, tables and chairs, arrange the catering and pray for nice weather.

All members who had lost a child or an adult to an MPS or related disease were invited to the Remembrance Day. We also invited dignitaries from Nottinghamshire to join us at the wood.

A friend of Christine's mum and dad made the joint 21st & 10th Birthday cake and I am sure you will all agree from the picture on the front of this newsletter it was superb!!!

Everything came together relatively smoothly apart from the choosing of the 10 categories for the teddy bear competition. It took quite sometime to decide which ones to have, which to leave out, which ones would have the most appeal, which ones would be most popular etc etc… but we did finally come up with a definitive list.
Once all the numbers were finalised I was able to

Category
Most Loved Bear
Oldest Bear
Best Dressed Bear
Biggest Bear
Scruffiest Bear
Most Travelled Bear
Smallest Bear
Bear Most Like Owner
Bravest Bear

confirm catering, how many children would possibly be partaking in the children's activity and order the balloons for the balloon release.

With all the planning done Alison and I set off to clear the wood

Clearing of the Wood

Phew!! What a Scorcher! With our shears, bottles of cold water (which towards the end of the day turned to boiling water) at the ready, we set off to clear the wood in preparation for the next day. Prior to visiting the wood, we were warned that, although it looked very pretty, it was very overgrown and that the grass around the trees could not be trimmed due to birds nesting in the undergrowth. On approaching the wood it did look extremely beautiful but I did have those initial pangs of panic when I realised that some of the over growth was nearly up to my shoulders (Alison's knees of course)! However all wasn't as bad as it seemed and we set to the task of clearing around the trees.

Due to the intense heat we had to continue the clearing on the Friday morning as by now our water was warm and the suntan was changing to a rather rosy shade of red.

The Adventures of the Cake

The cake had a very strange and eventful journey from the cake makers to its arrival at the wood.

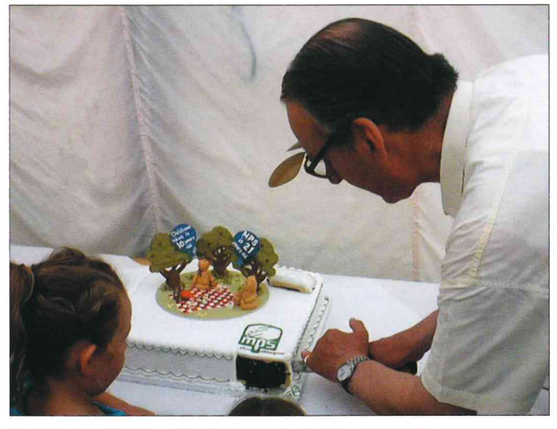
Its journey started from the cake makers to Christine's mum. It was then transported to Christine's kitchen and after an overnight stay made its way up the M1 motorway safely on the back seat of the car. There was one more overnight stay before it made its way to the wood on Ellie's lap. The cake finally arrived at the wood, had a short stay in the boot of the car (all staff were praying that it did not melt before we got back to it) before taking pride of place on its very own table in the Marquee.

The Childhood Wood Remembrance Day

16 families came together to remember the children and adults they had lost to MPS and related diseases, on Sunday 13th July 2003. The day began at 12 noon with everyone gathering at the wood, where Councillor Reg Strauther who was joined by his wife Lady Strauther gave a welcome speech, reflecting on the wood and what it means to Nottinghamshire County Council. Bob Devine read out the names of the Children and Adults being remembered before Wilma read out the poem "Remember" which as always was recited beautifully. After a few minutes silence Councillor Reg Strauther was asked to unveil the new information board, which explains what the wood is about. Families were then invited in their own time to make their way up to the marquee where the refreshments and the rest of the day's events would take place. Once everyone had arrived Sir Andrew Buchanan gave a welcome speech and reflected on the past 10yrs of the Childhood Wood and about his involvement since the first ever planting in 1993. After his speech all children present were invited to join him in the cutting of the cake.

While everyone was enjoying their lunch Alison and I set about setting up the teddy bears ready for Sir Andrew Buchanan to do the judging. It was a great relief to see that so many families brought their teddy bears with them, as previous to the day, I had only received 3 entries and panic had begun set in.

After lunch most of the children went out with Andrew Norman, the forest ranger, to do a nature trail, exploring the different aspects of life in the woods. Whilst the children were on their expedition it was time to blow up the balloons ready for the balloon release. This turned out to be a mammoth task and we were extremely glad of the offers of help to blow up the balloons and tie on the string. Once all the balloons were done families were invited to come and choose a balloon and write a message if they wished. Everything was going to plan until we realised that the weight of the string and the message prevented the balloon from flying. After much deliberation and experimentation we discovered that one solution was to tie more than one balloon together and the other was to cut the string and tie the message to the top of the balloon. At the time of the release the majority of them flew extremely well and those that did



not manage to make it the first time were successful on the second attempt.

Due to the intense heat of the day most people had decided to leave the wood early to try and miss the inevitable queues on the roads. Before everyone left there was just enough time to announce the winners of the teddy bear competition and give them their prizes.

The day went extremely well and we could not have asked for better weather. I would be very glad to hear from anyone who wishes to pass on their thoughts of the day, what went well, what didn't go so well and any recommendations you may have for future events or ideas at the wood.

Winners of the Teddy Bear competition held at the Childhood Wood - Sunday 13th July 2003

Category	1st Prize winner	2nd Prize Winner
Most Loved Bear	Hannah Russell	Alfie Tucker
Oldest Bear	Lisa Martin	_____
Best Dressed	Amie Martin	Eleanor Tucker
Biggest Bear	Hollie & Annie Stuart	Kirsty Hearn
Scruffiest Bear	Monty Russell	_____
Most Travelled Bear	Gemma Russell	_____
Smallest Bear	Sue & Peter Stuart	_____
Bear most like owner	Amie Martin	_____
Bravest Bear	Joe Tucker	_____

Overall Winners

1st Prize	Hollie & Annie Stuart
2nd Prize	Monty Russell

Deaths

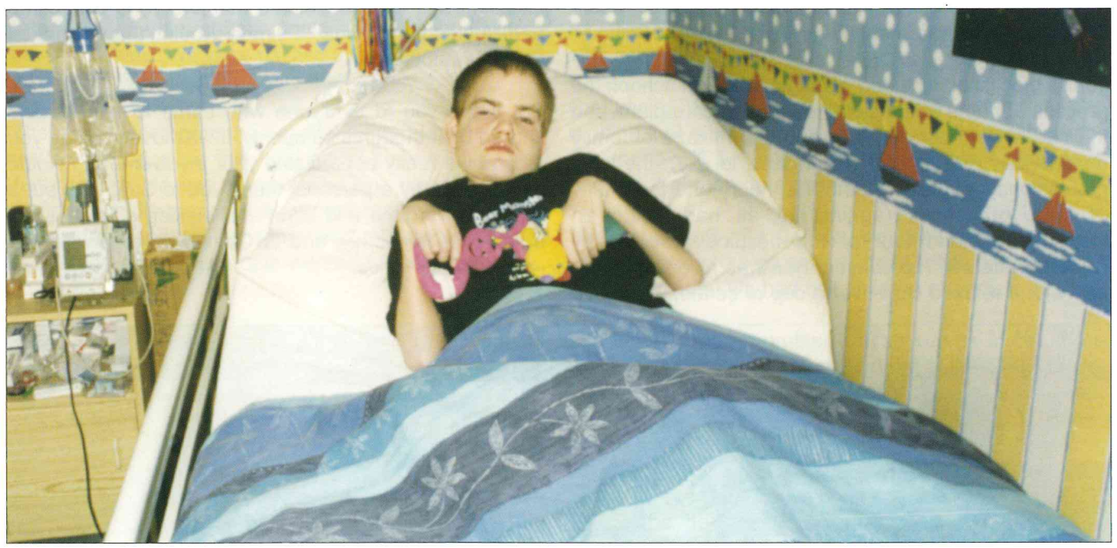
We wish to extend our deepest sympathies to the family and friends of

Nancy Sagoo who suffered from Mucolipidosis type II 18 June 2003 - 9 August 2003

Lauren Cawthorne who suffered from Hurler disease 31 August 1993 - 28 August 2003

Faye Rowe who suffered from Sanfilippo disease 29 August 1981 - 19 September 2003

David Seymour (below) who suffered from Sanfilippo disease 27 August 1985 - 16 September 2003



A Loving Tribute to Nancy

Paul Sagoo

Our little girl, Nancy, was an absolute treasure. Though she was in this world for only a short time, she gave everybody immense and unmeasurable pleasure. Nancy enjoyed our attention and she had her day-to-day needs, all of which were very small. For instance, she hated a dirty nappy and always informed us of when she required feeding!

There was a powerful and unmistakable twinkle in her eyes which brought us closer than we could have ever imagined. She also enjoyed being pampered by all the doctors and nurses, not forgetting our family members and friends. They, like us adored her especially when she smiled. Those smiles were widespread in terms of feeling and emotion. Smiles that will be etched in our memories forever.

Nancy is our inspiration and she will no doubt give hope to many children like her in Heaven. Heaven was always her ultimate destination.

We never got the full chance to become the parents and carers that we so wanted. Though in a strange way, our parenting role has now begun. We will campaign for her and help those children that suffer from the same illness that Nancy did. Everybody can be assured that the seeds have already been sewn. Our efforts will yield fulfilling results and dreams for all.

We will help Nancy every step of the way. We owe it to our beautiful daughter - a natural and carefully crafted gift. We know that our aspirations will be met. The much needed support from all corners will be important.

Nancy has taught us that waking up in the morning is a bonus and seeing the day through is fortune.

We love you Nancy and we miss you terribly. May you rest in peace in a world of angels all of whom will bring harmony and echo to your spirit - an ever-lasting spirit.

Scottish Information Day at Rachel House 3rd September

Alison West

The MPS Society, in association with the Community Fund, held an Information Day at Rachel House on 3rd September for families whose children are eligible to receive respite care at the hospice. We had a good response from our initial enquiries and a small group of families and professionals met at Rachel House to share experiences and ask any questions they felt they would like to ask in an informal environment.

Topics discussed included managing MPS at school, the process of home adaptations, setting up care plans and the role of the hospice. What with the mixture of family experiences (not all bad I'm pleased to say), the local professionals explaining both the theory and the reality of local processes (again, not all bad), plus an impromptu guided tour of the hospice provided by Gilbert Watterson (no comment), it was nice to end the day with a relaxed chat over a cup of coffee. This gave the families the chance to speak privately to individual members of staff at Rachel House, or members of the Advocacy Support Team, about any issues which may have been raised through the course of the day.

The feedback we received from the families was excellent and we are pleased that everyone who came participated so strongly. It simply wouldn't have worked as well without you.

So, thank you to everyone who came to see us and shared their thoughts so openly. It was lovely to see you all and we really appreciated your involvement. I would also like to say a personal thank you to Sue, who put a great deal of time and effort into making sure that this day ran so smoothly, and to Carol who organised the refreshments for us.

A day in the life - Callum's story gives hope to others

Action Medical Research

At just 10 months old, little Callum Pollock had a bone marrow transplant. The donor was his seven-year-old sister Chantelle. Such desperate measures were needed to give Callum, who has Hurler Syndrome, the chance to live a life that otherwise would have been denied him.

Callum is now seven. He attends school, goes to Tae Kwondo, plays football - and is something of a celebrity in his home town of Blackpool.

With his mum Carla, dad Donnie, brother Donnie junior and sister Chantelle, he has recently been on holiday to Majorca, where a stranger approached his mum.

"I know you," she said. "You're from Blackpool - I recognise Callum."

Carla told Touching Lives, "It happens all the time, everyone seems to know Callum, no matter where we are. He's a bright, cheerful little boy who will talk to anyone. Sometimes, it's hard to believe we've been through so much as a family, and the hardest part is that we still don't know what the future will bring. Callum's condition is so rare that no-one can predict what will happen."

Although he was a bouncing 10 pounds at birth, Callum became a sickly baby, vomiting and apparently having difficulty breathing. After several trips to the doctor and the hospital, at five months his family still didn't know what the problem was.

Desperate

"It was my mother-in-law who pushed me to make a stand and insist that we get some sort of diagnosis," said Carla. "I didn't know what to do but we knew that there was something very wrong and he was becoming so ill that we were desperate.

"When I was told he had Hurler Syndrome and without radical treatment probably wouldn't live beyond nine I was devastated. We were packed into a tiny room at the hospital and I just couldn't take it all in.

"Then other doctors in the hospital started to come and look at him because they had never seen a child with Hurler before. He was the youngest ever diagnosed and the first case diagnosed in Blackpool, but I hated the fact that he seemed to be some sort of novelty. I can understand now why people wanted to look, but at the time it was terrible."

Carla was contacted by the MPS Society, a support group for families of Hurler children, but most of the literature she read about the condition painted a very gloomy picture.

A chance

She said, "When he was diagnosed, the consultant said he needed a bone marrow transplant quickly, even though he was only a baby. I wanted to know more about the condition, but didn't read anything about Hurler Syndrome being treatable. We were terrified, but decided that we had to give Callum a chance.

"Without the transplant he would deteriorate mentally and physically and die. The transplant itself carried a big risk - at first he was too weak to go through it and we had to wait - but we saw it as a chance, and we took it."

Big sister Chantelle was found to be the perfect match, and though Callum was in hospital for a year after the transplant, his life was transformed as a result.

"I started to see the Hurler symptoms being reversed," said Carla. "He was still very prone to infection and was in and out of hospital for several years, but he's at the stage now where he seems better able to fight off the chest complaints he used to get and I don't worry about him quite as much as I did. We take the view now that we all have to get on with life - whatever it brings."

Pressure

But having such a sick child inevitably put a huge amount of pressure on Carla, Donnie, who works as a painter and decorator, and their older children.

Carla said, "I was living at the hospital, but the other kids and all the family were just brilliant. I had worked right up until having Callum, but I've never been able to go back. I decided to enrol on a computer course when he started school, but the day I started I got a phone call to say he was ill. I think I was called home five times during the course."

Callum and his family have lost count of the operations he has had. Hurler children can suffer from curvature of the spine and a procedure to try and straighten his back has not been successful.

Callum's spine is the worst doctors have ever seen, curving in two different directions, and his X-rays have travelled the world as his consultants try to find an expert who can help.

Carla said, "We're just getting on with things, and I'd like to think that other parents who have a child with Hurler Syndrome will be able to read about our experiences and see that there is a chance for them.

"We're not soft with Callum, we don't treat him much differently from the other two, and I think in many ways it was good that we had two children already.

"He still has some problems. The curves in his spine mean that he walks on his toes most of the time, and he suffers bad migraines, which seem to come on when he's excited about something.

"But we decided that we have to get on and really live our lives, even though we don't know what the future will bring. The bone marrow transplant has undoubtedly prolonged Callum's life, but his doctors can't say what will happen, or when.

"Callum's story is in the MPS literature now and that makes me feel better, because other mums and dads in our situation will be able to read that there really is hope."

New treatments under investigation

Action Medical Research has given a grant of £105,000 to a project at the Department of Haematology, Royal Manchester Children's Hospital, to investigate the modelling of new therapies to treat Hurler Syndrome.

Hurler Syndrome is a single gene disorder causing the deficiency of an important protein. It results in the build up of mucopolysaccharides - long chains of sugar molecules - that in most people are continuously broken down and recycled. In Hurler sufferers, these accumulate instead and can cause severe damage. Sufferers have progressive mental and physical problems and rarely live beyond the age of nine.

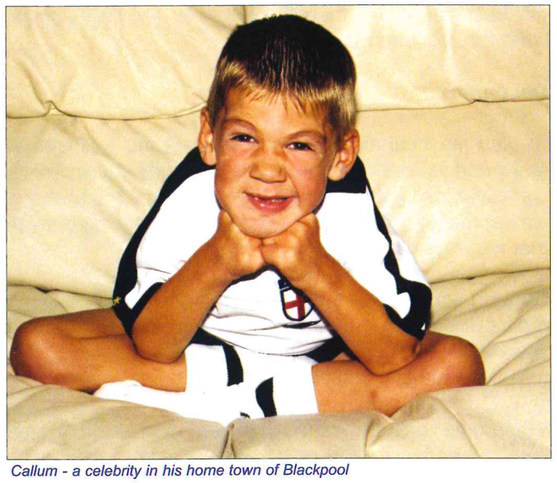
Dr John Grainger is leading the research into new therapies. At the moment, Hurler Syndrome can be treated with bone marrow transplants - but this is limited by the availability of donors, toxicity and the problems of rejection.

He said, "The research is investigating the use of mesenchymal stem cells (msc) administered alongside

genetically modified bone marrow cells. Mesenchymal stem cells can be derived from the affected individual and are able to develop into other tissues of the body. In addition msc can dampen down the immune reaction to bone marrow cells reducing the toxicity and the possibility of rejection.

"To date we have established a protocol for isolating msc and demonstrated how they can develop into different tissues and for genetic modification. We have now moved into a transplantation model to investigate the potential to aid engraftment and establish the fate of transplanted cells."

Action Medical Research is one of the country's leading medical research charities. For over fifty years they have funded pioneering research into a wide range of illnesses and conditions. www.action.org.uk 01403 210406



Callum - a celebrity in his home town of Blackpool

MPS I Under 5 Trial - Willink Unit RMCH

Jean Mercer - Senior Trial Coordinator

Four patients on the under 5year old trial with Aldurazyme for MPS I started having infusion in December 2002 and the fifth patient started in January 2003. The patients (from all over England) still attend weekly for their IV infusions via totally implanted venous access devices (T.I.V.A.D.S), which were inserted under a General anesthetic just before their first infusion.

The children and their families are over half way through the planned trial. They do all seem to enjoy coming to the unit and are very happy to see us and come to see their friends- in fact they are remarkable considering their young age.

The Willink is not a quiet place to be on a Monday with a combination of Teletubbies, Harry Potter, Music and Nursery rhymes! We also have extra colouring, sticker charts, trucks, baby buggies and lego bricks to add to the assault course for the parents and nurses alike!

The parents feel that they are beginning to see some improvement in the children.

As it is very difficult for me as the nurse to comment on the changes in the children I have approached the children's parents so here are some comments about changes they have noticed:

" We feel that her hearing is better. She is now able to get up and down stairs without support and moves easier. "

"Since my son has been on the trial I think there has been a few improvements in him. His tummy has gone down and he has a lot more energy now!"

"The changes have been visible signs such as Jessie's energy levels increasing dramatically, her stomach and umbilical hernia decreasing and her speech becoming faster and more articulate. Also her fingers seem to be slowing down in the amount that they bend and her overall condition of her joints is improving the longer she is on the trial."

"Since he has been on the trial he has improved a lot. He can now weight bear and he can stand for a minute or two on his own. He is very active and has had 3 bikes since December as he keeps wearing them out! This would have been impossible for him to do before the trial. His breathing has improved and he doesn't seem to be in as much pain at all anymore."

" The most noticeable improvement in Sophie has been her increased endurance and confidence in walking. Last summer she would barely walk outside in the garden without help and needed a pushchair for any distance. She now loves playing outside, can walk on all sorts of uneven surfaces and can walk for long distances (eg. one end of the park to the other or the length of the seafront) without tiring."

All the parents expressed a big thanks to everyone involved in the care of MPS patients and one claimed that the nurses in the Willink Unit are completely nuts!!! They obviously find support through each other and are stunned by the 100% dedication of the children and parents.

Overall the compliance has been good. We recognize Clinical Trials are very difficult for all the family and shouldn't be entered into lightly as the timing of investigations and visits can land at difficult times for the families. The trials are also very strictly controlled.
As you all know we couldn't get data needed for the under 5 year age group without our patients, their families, our excellent team of Research Nurse, and the other departments (which still have their normal Clinical workload to do. Although our lives at the Willink have been transformed since the beginning of the year we couldn't do it without our SUPERSTARS!!

MPS I Under 5 Trial - Parents Perspective

Suzanne Miles

My name is Suzanne Miles. I have a son Adam who has Hurler disease and is currently receiving enzyme replacement at the Willink.

Adam is 5 years old and started the trial in December 2002. Prior to Adam starting the trial Adam could not walk or weight bear. He was also in quite a lot of pain. He slept a lot and even doing the simplest of things left him exhausted and he would cry a lot. His breathing was quite poor and he had a lot of sleep apnoea.

Since he has been on the trial he has improved a lot. I didn't expect a miracle with the drug, as Adam was quite poorly, but he can now weight bear on his legs and he can stand for a minute or two on his own. He is very active and has had three bikes since December, as he keeps wearing them out. This would have been impossible for him to do before the trial. His breathing has improved and he doesn't seem to be in much pain at all any more.

Adam really enjoys coming for his infusions and the nurses are really nice to both of us. He has got to know them and is happy to stay with them. We do not know what the future is for Adam, but what I do know is that he is a lot happier since we started the trial.

MPS I Under 5 Trial - Parents Perspective

Paul Hambley

Apart from the obvious visible signs such as Jessie's energy level increasing dramatically, her stomach and umbilical hernia decreasing and her speech becoming faster and more articulate, there are other benefits for Jessie since she started on the trial. The doctors have found that her liver and spleen have shrunk down to near normal size, which is fantastic after only 6 months. Also her fingers seem to be slowing down in the amount they bend and the overall condition of all her joints is definitely improving the longer she is on the trial.

Hopefully with Jessie receiving the treatment from a very early age, she may not experience the problems associated with MPSI HS with such severity as her elder sister, who is waiting to hear about her application to receive Aldurazyme on the NHS.

After coming every week for over 7 months now, I have to say a big thanks to all the nurses and staff at the Willink who (although completely nuts) always give 100%, and also a big thanks to the other parents on the trial who also give 100% dedication, which makes it easier for me to keep coming 2 days a week, every week. Also the children, who outshine all of us and truly deserve medals.

Clinical Trials Update - MPS I

Aldurazyme, administered once-weekly, has been approved in the 15 countries of the European Union for long-term enzyme replacement therapy (ERT) in patients with a confirmed diagnosis of MPS I, to treat the non-neurological manifestations of the disease. As the first orphan drug approved for MPS I in the European Union, Aldurazyme has been granted 10 years of market exclusivity. Applications to market Aldurazyme also are pending in Israel, Canada, New Zealand and Australia; the companies expect a regulatory response in these countries in late 2003 or early 2004. Additional information can be obtained at www.aldurazyme.com.

Clinical Trials Update - MPS II
TKT Press Release, Long-Term Hunter Syndrome Phases I/II Data Show Continued Improvement in Patients

BRISBANE, Australia, Sept. 4 /PRNewswire-FirstCall/-- Transkaryotic Therapies, Inc. (Nasdaq: TKTX) today announced that its lead clinical investigator, Joseph Muenzer, M.D., Ph.D., of the University of North Carolina at Chapel Hill, presented long-term data from TKT's Phase I/II clinical trial evaluating iduronate-2-sulfatase (I2S) enzyme replacement therapy for the treatment of Hunter syndrome, also referred to as MPS II, a debilitating disease for which there is currently no effective therapy. The one-year data showed improvements in a variety of clinical measures and continued to demonstrate a favorable safety profile. These findings were presented at the IXth International Congress of Inborn Errors of Metabolism meeting being held this week in Australia. Additional data from this study will be presented in November 2003 at the American Society of Human Genetics 53rd Annual Meeting.

"The significant clinical improvements observed in these patients treated with I2S at both six and twelve months indicates that this product provides a clinical benefit and appears to represent a promising treatment for Hunter syndrome," said Dr. Joseph Muenzer, Associate Professor of Pediatrics, Division of Genetics and Metabolism, Department of Pediatrics at the University

of North Carolina at Chapel Hill.

After successfully completing the six-month randomized, double-blind, placebo-controlled study evaluating three doses of I2S (0.15 mg/kg, 0.5 mg/kg, and 1.5 mg/kg) bi-weekly for 24 weeks, all 12 patients elected to enroll in the open-label extension study. The six-month data showed a significant reduction in urinary glycosaminglycan (GAG) excretion, significant reductions in liver and spleen volumes, as well as reductions in left ventricular mass and improvements in forced vital capacity. Patients receiving placebo that crossed over to I2S also showed a similar treatment response. Patients showed a greater improvement in their joint range of motion and with the six-minute walk test at one year. Any significant infusion-related reactions that occurred were successfully managed by slowing the infusion rate and using pre- medications. Data from the six-month Phase I/II study were previously presented at the American Society of Human Genetics Annual Meeting in October 2002.

TKT is preparing to commence a pivotal study of I2S in September 2003. The primary objective of the study is to determine safety and efficacy of I2S as a treatment for Hunter syndrome. Ninety patients will be randomized to three treatment groups to receive either weekly or every other week infusions of I2S at a dose of 0.5 mg/kg or weekly infusions of placebo. The primary endpoint will be a single composite variable designed to evaluate the efficacy of I2S therapy across multiple outcomes. The composite variable will combine two clinical measurements: forced vital capacity as a measure of respiratory function and the six-minute walk test as a measure of functional capacity.

"The final preparations for the pivotal trial in patients with Hunter syndrome are being made and we are on track to begin enrolling patients in the trial this month," said Michael J. Astrue, President and Chief Executive Officer of TKT.

About I2S
I2S is a human iduronate-2-sulfatase produced by genetic engineering technology intended for long-term treatment of Hunter syndrome. The rationale for the therapy is that I2S would replace enzyme that is deficient in patients with Hunter syndrome and either stop or reverse disease progression. I2S has been designated an orphan drug in both the United States and Europe and is the only known enzyme replacement therapy in development for the treatment of Hunter syndrome.

Clinical Trials Update - MPS IV-A

MPS IV-A BioMarin Pharmaceutical Inc. has an active program aimed at developing ERT for MPS IV-A. There currently is no timeline for a human clinical trial. Studies in MPS VI and VII animal models suggest that, if given early, the enzyme can potentially change the outcome of bone and cartilage disease. Work to produce adequate quantities of enzyme for clinical use is still in progress; at this time, there is no clinical trial planned. The BioMarin Web site is www.biomarinpharm.com.

Clinical Trials Update - MPS VI

MPS VI BioMarin Pharmaceutical Inc. announced July 7, 2003, that it is set to enroll patients in its phase III trial of AryplaseTM (arylsulfatase B) in MPS VI. The phase III trial follows both a phase I dose ranging study, and a phase n study that evaluated a variety of clinical and biochemical safety and efficacy parameters. Additionally, BioMarin conducted an international survey study of 1 21 MPS VI patients to assess baseline disease characteristics to determine the most appropriate clinical design for the phase III study. The major components of the phase III trial are: .Six months in duration, randomized, double-blind; placebo-controlled, conducted internationally at six sites and will enroll approximately 36 patients- .The primary endpoint will be the change in endurance compared to placebo as measured by the distance walked in a 12-minute walk test. .Secondary endpoints will include the change in urinary glycosaminoglycan (GAG) excretion, a measure of biochemical activity of the enzyme, and the change in the number of stairs climbed in three minutes- .Safety measurements will include monitoring for adverse reactions and immune response to treatment. Aryplase was well-tolerated in the phase II and II trials. Adverse events attributed to the enzyme have generally been mild and did not require treatment. Antibody responses to Aryplase have not correlated with the infusion-associated reactions observed.

Infusing patients (at 1.0 fig/kg) in the phase III trial will begin shortly. Following the six-month, placebo-controlled portion of the trial, patients receiving placebo will receive weekly Aryplase infusions. If the results of the double-blind portion of the trial are positive, BioMarin plans to file applications to market Aryplase in the United States and European Union (EU) in the second half of 2004.

Clinical Trials Update - MPS VII

Drs. Emil Kakkis and William Sly have received a grant to develop enzyme replacement for MPS VII. Good progress is being made in production development. At this point there is no timeline for a human clinical trial.

J4G Research Grants Awarded From the Proceeds of the 2002 Campaign

J4G/17/00 Two Centre Project Grant

Project 1
To establish the molecular basis of novel mutations found in patients with MPS 1 – Hurler disease and MPS 111 – Sanfilippo disease.

Project 2
To develop gene therapy for MPS diseases using herpes virus vectors.

Project 3
To develop gene therapy using bone marrow cells and to investigate how the patient tolerates these new cells.

Project 4
To produce antibodies for use in gene therapy

Dr Ed Wraith Willink Laboratory, Royal Manchester Children's Hospital
Dr Rob Wynn Willink Laboratory, Royal Manchester Children's Hospital
Prof Bryan Winchester Institute of Child Health, London

The Mucopolysaccharidoses (MPS) result from genetic defects in enzymes involved in the turnover of large molecules called glycosaminoglycans (GAGs), which are important in maintaining the body's skeleton and in brain function. Recent research has indicated that it might be possible to replace the defective enzyme in MPS by putting a good copy of the gene that is responsible for the synthesis of the defective enzyme into a patient's cells – gene therapy. The major problems with gene therapy are rejection of the replacement protein by the immune system of the body, getting the replacement enzyme or gene into all tissues, particularly the brain and the short life of the replacement gene or enzyme. These projects seek to solve these problems.
Year 3 of a 3 year project grant £203,079

Project 5
Autologous Mesenchymal Stem Cells as a Target of Genetic Manipulation in the Management of MPS II and MPS IIIA and IIIB

Dr Rob Wynn Royal Manchester Children's Hospital

The use of Mesenchymal Stem Cells (MSC) to target cells to correct the enzyme defect in Hunter and Sanfilippo patients. To test the hypothesis that MSC from Hunter syndrome can be transduced with the retro viral vector containing the IDS gene.
Year 2 of a 3 year grant £35,786

J4G/21/02 Global report on the outcome of Bone Marrow Transplant for MPSI

Dr Colin Steward Bristol Children's Hospital

To fund the publication of this report as a supplement to a professional scientific journal to enable clinicians and scientists worldwide to access state of the art information on the clinical management and outcomes of BMT for MPSI.
£8,000

J4G/18/03 Research project to investigate the Psychosocial Outcomes of Bone Marrow Transplantation and Enzyme Replacement Therapies for Mucopolysaccharide Diseases

Cheryl Pitt The Society for Mucopolysaccharide Diseases

Bone Marrow Transplant (BMT) is considered to be the gold standard of treatments for the more severe form of MPS I (Hurler Disease). However, the treatment is not a cure, is high risk and has its limitations. The main limitations of BMT are that it cannot reverse neurological damage already caused by the disease prior to treatment, and it cannot prevent joint and bone problems that are characteristic of MPS I, from developing. Children with MPS I who have had BMT therefore go on to experience varying degrees of learning difficulties and physical disability. Over time their mobility is likely to decrease and they are likely to experience a forced dependence on their families. However, the psychological and social development of these children has, to date, been neglected as a topic of research. The MPS Society is undertaking a research project that aims to explore the psychological and social outcomes of BMT for this group of patients. This project will also cover the psychological and social outcomes of Enzyme Replacement Therapy as increasing number of patients begin this treatment.
Year 1 £20,000 (6 months).

News from the Czech Republic

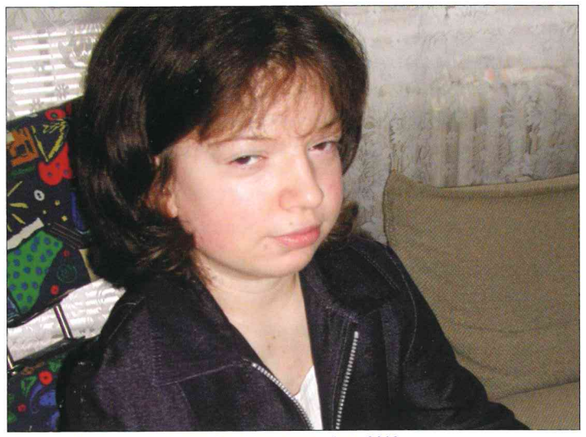
Lenka ˇvábová

I received the letter from you. I was very pleased to have such nice booklets, though the ones on Lauren and Jessica´s are so sad and emotional. The booklets say everything about this disease and in spite of the fact we think we know everything, we are always affected. I must say that our Hana is very similar to Jessica - they are the same age, and they look nearly like sisters.

Thank you very much for your kind help, keeping in touch and best regards to you and the people in the U.K. MPS society!

Yours Lenka and Hana (and the family)

Jessica sporting her red nose



Jessica after her first infusion for aldurazyme - June 2002

Plea for Photos for a New Booklet

The MPS Society is currently working on a new booklet about the Pattern of Inheritance of MPS and Related Diseases. To accompany the text we are looking for photos of families or perhaps groups of brothers and sisters which we can use to illustrate the booklet.
If you can help and are in interested in sending in some photos please contact Antonia Crofts at the MPS Society.

Grandparents - the Society needs your help!

The Society produces a wide range of information materials aimed at individuals with MPS and Related Diseases, their parents, carers and professionals to raise awareness and provide information on the presentation and clinical management of MPS and Related Diseases.

We would now like to put together a publication for Grandparents of those with MPS or a Related Disease. This publication will feature stories and photos of grandparents who have grandchildren with MPS, information on MPS diseases, how they are inherited and how grandparents can help the Society by fundraising and making a donation. There will also be a section on how the Society can provide support and information to Grandparents.

We need your help in the following ways:

Do you have a grandparent who would be willing to provide a story or send us a photo to publish -perhaps a photo of themselves with their grandchildren?

Are you a grandparent who would like to take part in the publication? Do you have any good stories to tell, for example, about fundraising, days out with your grandchildren or the difficult time when your grandchild was first diagnosed?

Do you have any ideas on what sort of information should be included? Is there any particular issue you would like explored in depth?

If you can help us in any of the ways listed above or want to find out a bit more then please contact Antonia at the MPS Society as soon as possible who would be very happy to provide you with more information.

Book Review
An Intimate Loneliness - Supporting Bereaved Parents and Siblings by Gordon Riches, Pam Dawson

Mary Jones - Bereavement Counsellor and Trainer

Many of the current books on bereavement focus on a clinical or developmental interventionist approach to working with children, which has limitations. It was therefore a joy to read a book that takes a holistic approach to working with families. *An Intimate Laneliness* looks beneath the surface and explores the concepts and theories of the bereavement process within the social context of the family, and modern society.

Anyone working in this area will know how complex the issues can be as each family struggles to interpret and make sense of its grief in its own way. It is this uniqueness of response, and its effect on how each member reacts, that challenges all bereavement workers. The title illustrates the paradox that grief after the death of a child can drive apart those who normally would be expected to give support to one other, isolating individuals from their most intimate relationships. Understanding these relationships and their effect on the family and the diverse roles members perform, we become better equipped to recognise how families pick up the pieces and carryon with the rest of their life.

The background for the book is Riches and Dawson's own ongoing qualitative research into how members of a family grieve. This means that theoretical models can be explored alongside the voices of the bereaved people themselves and those who work to support them. There is a richness of material, as current research and accepted concepts are illustrated, or sometimes challenged, by the experiences of the families. The authors build on many of the themes set out in Tony Waiters' book*.

The early chapters look at the problems of adjustment for parents and siblings. the importance of social relationships, and gender and diversity issues. Although death ends a life it does not end a relationship. The authors consider how the personal. social and cultural resources of parents and children can affect their ability to make sense of loss. Issues such as identity struggles. difficult deaths and complicated grief are also discussed in detail.

The final chapters cover bereavement support and help available. In our post-modern world. bereavement supporters are seen as explorers, guides or companions. The quality and intimacy of the relationship between a supporter and a bereaved family is linked to the willingness of each to learn from the other, and the relationship can be threatened by an over dependency on simplified models of grief. The authors petition for a flexible and open-minded approach, eclectic use of grief models and sensitivity to the diversity of beliefs. I particularly liked their concept of "it tie ladders and big levers' -things that can help shift perspectives for parents and siblings.

An appendix provides a substantial, well- documented reference list and each chapter has a concise summary, helpfully consolidating the main points. Reading the book I found myself, again and again, relating the material to many of the children and families with whom I have worked. I have recommended it to colleagues, students and those wishing to understand and appreciate grief in families. It should be compulsory reading for bereavement courses.

*On Bereavement:The Culture of Grief. Buckingham, UK: OUP. 1999.

Congestion Charge Exemption

Most people are probably are aware by now of the introduction of a five pound congestion charge in central London. What you may not know if you don't live in London is that holders of a Blue Badge (formerly known as Orange Badge) are eligible for a 100% discount. In order to obtain the discount, Blue Badge holders need to register in advance with Transport for London, and pay a one off fee of £10 to cover administration. For more information, ring Congestion Charging London on 0845 900 1234 -or look on the website: www.cclondon.com, which has a registration form that can be printed off.

Tripscope

Tripscope offers expert advice and information to people with impaired mobility on overcoming travel difficulties. The staff who operate the information and advice service all have a personal understanding of disability and can advise on travel both in the UK and abroad. Tripscope produce the AA's Guide for Disabled Travellers, which is free to AA Members and £4.99 to non members, and can be obtained by phoning the AA Disability Helpline.

AA Disability Helpline: 0800 262 050
Tripscope: 08457 585641 www.tripscope.org.uk

Travelling

Two useful websites if you are planning a holiday abroad or in the UK:

If you need help finding accessible accommodation, in the UK or anywhere in the world, somewhere to hire a vehicle that will take a wheelchair, or a contact for wheelchair repair or hire, you could try www.access-able.com

“We have information and everything else you need to know about: travel with disabilities, mature travel, disability magazines, access guides, wheelchair travel, scooter rental, accessible transportation and more!”

Subscribe (at no cost) to their monthly Email Newsletter which carries tips about places to go and stories of people’s experiences.

Respite Care

A voucher scheme to assist service users and their carers to arrange respite care was launched by the government last week, aimed at giving carers and users greater choice over when they take breaks and provide an alternative to the direct provision of services or direct payments.

Clothing

We have had a number of inquires about where people can go to find clothes that fit over large heads and around enlarged stomachs. A number of you have got back to us with some places to shop. They are: La Redoute catalogue, Vort Baudet catalogue, Primark, Quality Seconds, B-Wise and New Look.

If anybody knows of anywhere else that clothes that fit can be brought please do contact The MPS Society so we can share the information with others.

Anyway Up Cups

Kate Hall informed us that replacement lids for the anyway up cups by Mandy Haberman can be brought for £1.20 for two instead of £2.99, if you go through them direct and explain that your child has a disability. It could save you having to buy new cups every time the spout is chewed off! When phoning you need to ask for Wendy.

Obi Buggy

The Society has recently heard from a member that the Obi buggy has been crash tested. This is significant if you have a child who needs to be transported in their buggy in transport to school or whilst at respite as it is common practice that transportation will only be offered if the buggy is crash tested .

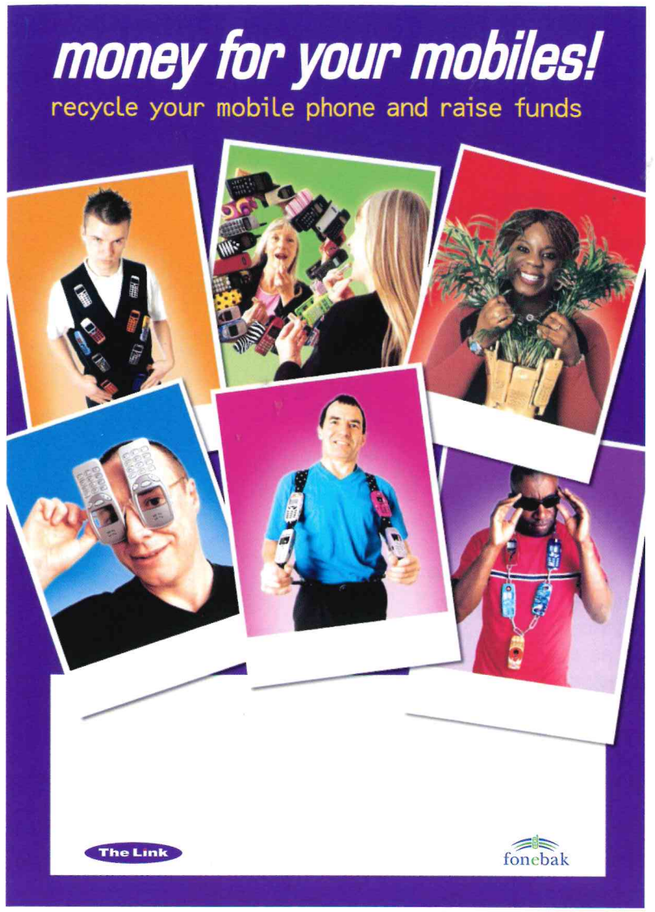
AGU befriending link

The Advocacy Support Team have had an enquiry from a family with a 13 year old son with Aspartylglycosaminuria (AGU). They would like to speak to others in a similar situation and asked if the MPS Society could help. Although this family is based in the UK we would welcome responses from across the world. So if you have a child who also has AGU and would like to share your experience with another family, please contact Alison at the MPS Office.

Communication Aid

Kate Hall has asked us to share with you a communication aid she found on www.liberatorcommunicationsdevices.co.uk It is an electronic 24 sleeve photo album, where messages can be stored on each individual page. It could be used as a way of communicating, for example; with photos or drawings and a word or message recorded. It is priced at £35.

Money for your Mobiles



The MPS launches mobile phone recycling scheme to help conquer the UK's growing mountain of 15 million discarded mobile phones each year

In cooperation with Community Fonebak, the scheme aims to bring in old and unwanted mobile phones in exchange for cash or vouchers to support MPS.

Under the Community Fonebak scheme, phones are collected and sent to a specialist facility where each phone is rigorously tested to determine whether it will be resold or processed for materials recycling. The aim is to reuse as many phones as possible as this has the least impact on the environment.

In the first 12 months to end August 2002, over 1 million phones have been marketed for reuse and over 105 tonnes of handsets, batteries and accessories have been processed for materials recycling. This equates to a saving in landfill of over 250 tonnes.

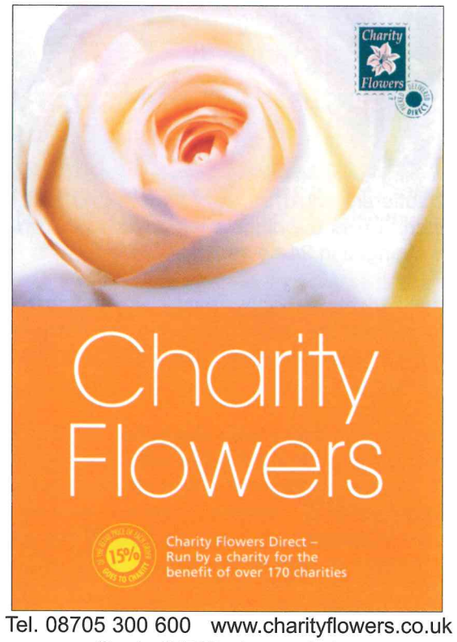
For more information, please contact: Gina Page at the MPS office.

Giving through the Self Assessment Tax Return

This new scheme from the Inland Revenue allows any individual to nominate a favourite charity to receive their tax repayment as a donation.

From April 2004 your charity’s name and code number will be listed on the Inland Revenue website (www.inlandrevenue.gov.uk). For the purposes of this scheme the MPS Society’s code is: RAB71QG.

This method of giving will only be available on Self-Assessment returns from next year. It cannot be used before the 2003/2004 Self-Assessment returns issued in April 2004.
For more information please contact Gina Page at the MPS Office.



Tel. 08705 300 600 www.charityflowers.co.uk
Quote ‘MPS’ when ordering

Please help my sons pleads mum

Caroline Bullock - Farnham Herald Newspaper (printed Friday, 23rd May)

US treatment 'only real hope' for boys suffering from incurable disease.

A Badshot Lea couple's happy family life was cruelly shattered when both their sons were diagnosed with a rare and life-limiting disease.

Now Bob and Claire Stevens are appealing to the local community to support the MPS Society, a charity which looks into pioneering new treat- ments for the disease which currently has no cure.

The couple's sons, Oliver, 3, and Samuel, 1, were diagnosed with Hunter disease, a form of mucopolysaccharide disease(MPS), which prevents the body breaking down waste products that are then stored in major organs and results in progressive physical and mental disability and often early death.

Bob and Claire now face the agonising prospect of watching the gradual decline in their sons' conditions with their only hope being enzyme replacement therapy (ERT), currently being trialed in America, which has been shown to ease physical symptoms, working as a treatment rathenhan cure, in a similar way insulin helps diabetes.

Bob & Clare Stevens with sons Oliver and Samuel

"Our lives were changed for ever overnight and our only real hope is getting ERT on the NHS," said Claire. "This is why we need to tell everyone about this because it's such a rare illness and it needs all the publicity it can get."

ERT has not yet been passed by the European government as a treatment for Hunter disease and, costing £100,000 per child per year, is not available on the NHS.

"I'd always planned for my kids' future, so I could retire early and Claire and me could enjoy life," said Bob.

"But now pensions don't mean anything. When we found out that sufferers could be severely affected and face early death, it was the biggest jolt anyone could have, our lives changed in 24 hours.

"Everyone expects a happy, family life and then this happens, but you just have to carry on. You think about it every single day, it never goes away, the thought of the boys not making 10."

The goodwill and generosity expressed by the local community in fund-raising for the MPS Society, which supports couples like Bob and Claire, and the organising of a fundraising ball at Farnham Castle this summer, has become an important and positive focus for the couple as they struggle to cope and come to terms with their situation.

Looking at Samuel and Oliver laughing and engrossed in a cartoon, they appear just like any healthy young children which makes it easy to forget how ill they are.

But even at this stage, the disease is already taking hold. Oliver's heart is 20 per cent larger than normal and if it gets much worse he will need open heart surgery. His mobility and hearing are also impaired and he is likely to require several major operations. Samuel now has a heart murmur.

Not surprisingly, the routine of normal family life, has been shattered with weekly hospital appointments. The couple have been unable to get away for a holiday or have a full night's sleep for the last few years.

Bob said: "It disrupts family life completely. When the boys get any kind of infection, it can turn very nasty, so there is always a sinking feeling when we have to take them to hospital. It's an exhausting battle. If we get any time to ourselves we just sleep."

It has put an enormous strain on the couple's health. Bob suffers from high blood pressure and takes anti-depressants and the sudden death of his father eight weeks ago proved another cruel blow.

Claire has rerumed to work part-time at Lloyds Bank in Bordon to get a break from the stress.

"I probably coped better in the beginning than I do now, and I definitely have some bad days. But I find when one of us has a bad day the other has a good one. Work has been very supportive and if I get upset and need to cry, I can go round the back."

The couple cite ERT as their only real hope.

Bob added: "If we didn't have the trials to look ahead to, I don't know what we'd do. I don't even want to think about it. But the treatment has to be passed by the government, so it's down to the politicians basically, 'which is quite disconcerting."

Results have been promising, showing improvements in the heart and lung function of sufferers and reducing liver and spleen size, which are both enlarged in those suffering from MPS disorders.

"We try and make the best of family life, we are fortunate that we have each other, and although we have our moments we always sit down and talk things through," said Bob.

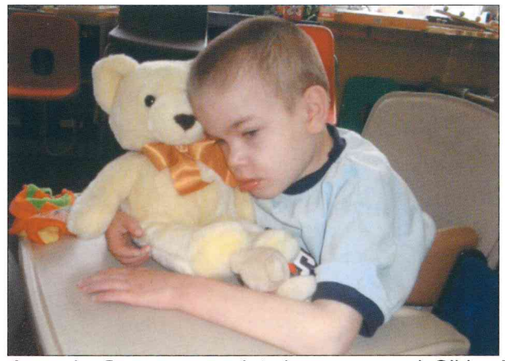
"But it never goes away and we think about it all the time. The boys are such great characters always smiling and laugh ing. They adore each other and cope with everything very well because tlley don't know any different."

The Midsummer ball was held in a marquee in the grounds of the castle on June 21 and was sold out. For details of the businesses and celebrities who supported the event visit the web site -www.farnhamball.com

Our thanks go to Claire, Bob and all their family and friends who raised in excess of £24,000 for the MPS Society.

From left: Darren Stone, Bob Stevens (holding Oliver), Kirsty Stone, Claire Stevens (holding Samuel), Heidi Teague, Siobhan and Paul Smith.

Glider Plane Ride for MPS



Amanda Gregory completed a sponsored Glider Plane Ride for MPS with a work collegue, they raised £200. Amanda works with Craig McLean and says Craig is a special little boy and this enspired me to raise money for the MPS Society.

Signed England Shirt

Signed 2001 England Football shirt. To buy send bid all bids on an envelope with bidders name and address to be reveived in the mps office by 30th November

Fundraising

Mr K Allinson – 3 Peaks Walk
Northgate Information Solutions
Lollipop Tree Nursery Group – Farnham
Lyn Longhorn – Fundraising Lunch
Amanda Gregory – Sponsored Glider Plane Ride
Employees of UVEX (UK) Ltd – Farnham
Edwards Hurdle – Windmill Marathon
Elizabeth McDowall – West Highland Way Walk – 55 miles
John Sanderson – 10K Road Race - Manchester
Graham and Margaret Moore – Coffee Morning
Williamston Primary School – Primary Seven pupils
Rupert Avenue Sewing Group
Mrs Margaret Jones – Talks
Chiddingfold Golf Club – Bridge Afternoon

Stamps & Foreign Coins
Norman J Wigley & Partners
Mrs F McConnell

In Memory
Arun Rohit Bansal
Jacqueline Maria Turner
Florence Aspin
Mr Anthony Frank Moulding

Donations

The Mercers Company
Dr Goel – Roselawn Surgery
Bansal Family
TKT Europe – 5S
Britannic Management Services Limited
Sue Lowry
Peter Rennoldson
The Oddballs Golfing Society - Newtownbreda
Chrisopher and Elspeth Thomas Trust
Mary Moulding – Isle of White
St Mary's Church – Haddenham
Annette Jones
Michael H Briggs
Bank of England
Dr John and Mrs Jane Heritage
Shirley Wavell
Flag Telecom
John Lewis Partnership
The Fitton Trust
Helen and Alan – Avon
Data Connection
Vauxhall Motors – Employees Fund
Bridget Butler
The Union of Catholic Mothers Malpas
Thales Charitable Trust

Collection Box
Rawtenstall County Court
N. C and B Lunt Pharmacy
C M L Jones & Partners Dispensing Chemist

James Wilson & Peter Glover ran the Great North Run 2002 which raised £650.


Lyn Longhorn donated £400 to Sanfilippo research raised at a fundraising Lunch for 48 people.

Take up the Challenge

Come and join John Sanderson on his sponsored trek in Peru in 3rd -12th June 2004. Trekers raise a minimum od £2,500 and the Society arranges everything else.

This is a challenging trek on high, remote mountain trials through breathtaking Andean landscaps to Machu, Picchu, the legendary Lost City of the Incas. Incan paths will take you through lush cloud forrest, green valleys and over high passes, passing fascinating Inca temples and settlements along the way.

If you are interested in taking the challenge please contact Gina Page at the MPS Society.

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YOUR HELP NEEDED

Do let us have your stories, experiences 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 MPS Society at the address below.

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.

PHOTOGRAPHS

If you would like copies of any of the photographs inside this edition please contact the office.

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email: mps@mpssociety.co.uk
Web site: www.mpssociety.co.uk
Fabry: www.fabry.org.uk

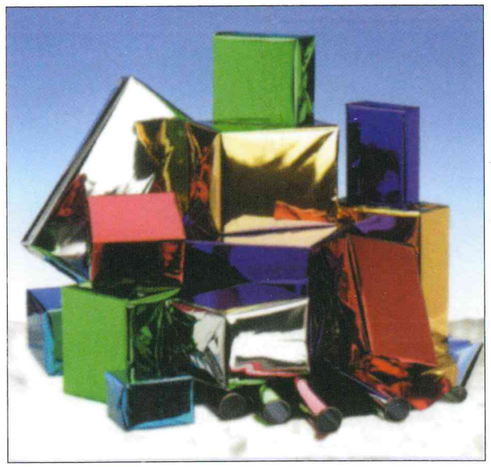
NEWSLETTER DEADLINES

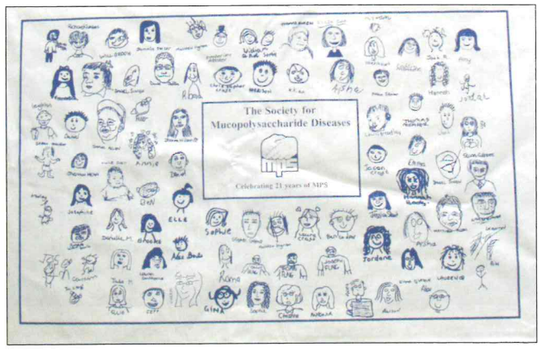
Winter
17 December 2003

Spring
17 March 2004

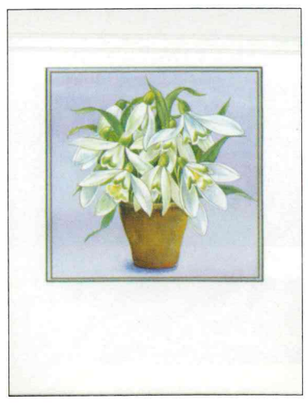
SUMMER
17 June 2004

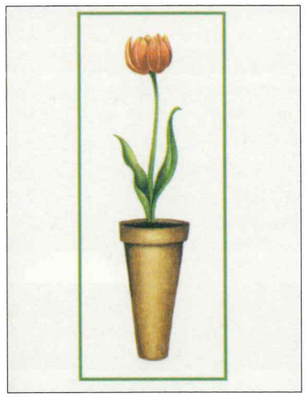
Autumn
17 September 2003

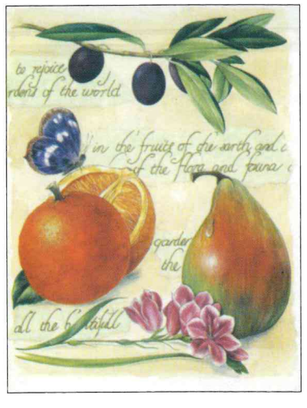


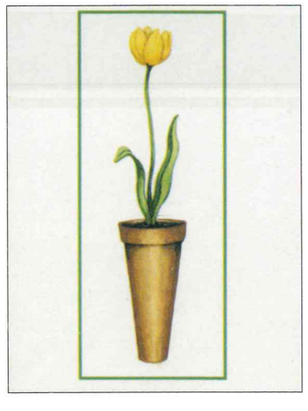


Notecards









Promotional Goods & Christmas Card Order Form

Promotional Items	Price	Quantity
MPS Pens (pack of 2, 1 blue + 1 black)	£1.20	.....
MPS Pencil	£0.25	.....
Whistle Pens (red, white or blue - please indicate preferred colour choice)	£1.00	.....
Yo Yo Key Rings (red or blue - please indicate preferred colour choice)	£0.75	.....
MPS Tie Pin	£1.20	.....
MPS Video	£5.00	.....
MPS 21st Birthday Tea Towel (individual)	£3.50	.....
MPS 21st Birthday Tea Towel (pack of 2)	£6.00	.....
MPS 21st Birthday Mug	£2.99	.....
MPS Promotional Pack (Pencil Case/Ruler/Bookmark/Key Fob)	£1.00	.....
MPS Key Fob	£0.50	.....
Bookmark	£0.40	.....
Ruler	£0.20	.....
Pencil Case	£0.50	.....
Christmas Stock		
Foil Gift Wrap (5 sheets/5 tags)	£3.50	.....
Star Design Gift Wrap (5 sheets/5 tags)	£2.99	.....
Moon & Mistletoe Cards (10 pack)	£2.25	.....
Santa's Animals Cards (5 pack)	£1.60	.....
Please refer to Main Order form for all new Christmas Cards for 2003		
Notecards		
Fruit and Flora (10 pack of 2 designs)	£2.50	.....
Snowdrop (10 pack of 2 designs)	£2.50	.....
Tulip (10 pack of 2 designs)	£2.50	.....
Clothing		
MPS Adult Design - Boy/Girl T-Shirts	£7.00	.....
MPS Child Design T-Shirt (only age 5-6 Years available)	£5.00	.....
Polo Shirts & Sweatshirts (various sizes and colours, please call for availability)	£4.00 - £7.00	
Christmas Cards		
Reindeer & Robin	£2.80	.....
Christmas Morning	£3.00	.....
First Christmas	£2.75	.....
Teddy & Puppet	£2.75	.....
Santa's Washing Line	£3.10	.....
Snowdrop Robin	£2.80	.....
We Three Kings	£2.95	.....
The Magic Begins/A Christmas Wish	£3.00	.....

(postage & packing for xmas cards only: 1-4 packs = £1.50, 5-10 packs = £2.00, 11+packs = £3.00)

Sub total £.....
Postage & Packing (xmas cards only) £.....

☐ I enclose a cheque/postal order payable to "The MPS Society" for £.....
☐ I wish to pay by Master Card/Visa Card No ..... Exp: ...../.....

To reduce fraudulent claims on your card please supply us with the last three digits of the security code on the signature strip on the back of your card.....

Name: .....
Address: .....
..... Post code .....

Please return this form when completed with your chosen method of payment to:

Gina Page
MPS Society
46 Woodside Road
Amersham
Buckinghamshire HP6 6AJ
England

Christmas Cards 2003

Mucopolysaccharide and Related Diseases cause progressive physical and mental disability usually resulting in death in childhood.The Society for Mucopolysaccharide Diseases is the only National Registered Charity, providing information, advocacy, and practical help to families of affected children and young adults. At present there is no cure and the monies raised from the sale of these cards will help towards supporting over a 1000 affected families in the UK.

Sold in aid of:
The Society for
Mucopolysaccharide
Diseases
Registered Charity: 287034

427 Reindeer & Robin (100x100mm)
"Happy Christmas"
£2.80 per pack of 10 cards

412 Christmas Morning (171x121mm)
"With Best Wishes for Christmas and the New Year"
£3.00 per pack of 10 cards

431 First Christmas (98x130mm)
"Happy Christmas"
£2.75 per pack of 10 cards

430 Teddy & Puppet (98x130mm)
"With Best Wishes for
Christmas and the New Year"
£2.75 per pack of 10 cards

DO66/02 Santa's Washing Line (110x210mm)
"With Best Wishes for Christmas and the New Year"
£3.10 per pack of 10 cards

BO38/02 Snowdrop Robin (125x125mm)
"With every good wish
for Christmas
and the New Year"
£2.80 per pack of 10 cards

CO25/02 We Three Kings (121x171mm)
"With every good wish and the New Year"
£2.95 per pack of 10 cards

CO20/02 The Magic Begins/ A Christmas Wish (121x171mm)
"With every good wish and the New Year"
£3.00 per pack of 10 cards
(5 each of two designs)