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support
research
awareness
Wicked GENES

The MPS Magazine

Summer 2013

Society for Mucopolysaccharide Diseases

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The articles in this magazine 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. Products advertised in this magazine are not necessarily endorsed by the Society.

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The MPS Society

Founded in 1982, the Society for Mucopolysaccharide Diseases (the MPS Society) is the only national charity specialising in MPS and Related Diseases in the UK, representing and supporting affected children and adults, their families, carers and professionals.

Our Aims:

Acts as a **support network** for those affected by MPS and Related Diseases

Promotes and supports **research** into MPS and Related Diseases

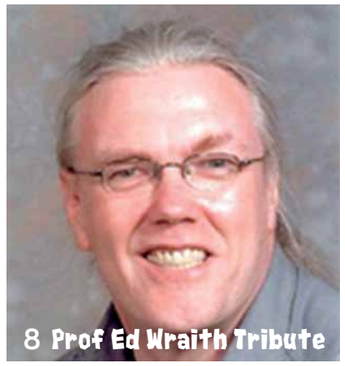
Brings about more **public awareness** of MPS and Related Diseases

MPS and Related Diseases

Mucopolysaccharide (MPS) and Related Diseases affect 1:25,000 live births in the United Kingdom. One baby born every eight days in the UK is diagnosed with an MPS or related disease.

These multi-organ storage diseases cause progressive physical disability and in many cases, neurological deterioration resulting in death in childhood.

At present there is no cure for these devastating diseases, only treatment for the symptoms as they arise.



Front Cover
Wearing It Blue for MPS Awareness Day!
Photo courtesy of Torquay Herald Express
www.torquayheraldexpress.co.uk

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Chief Executive's Report

Did you **Wear it Blue!** for MPS Awareness Day?

I did along with so many of you and I want to say a big thank you for helping to increase awareness of Mucopolysaccharide and related diseases and raising much needed funds and awareness for the MPS Society. So many played their part but a special thanks goes to Ethan's mum, Katrina Fanneran whose justgiving.com page supported by so many friends and family raised so much money for MPS.

Wear it Blue will be back next year but it is not too late to organise that 'Wicked Walkabout' for 2013. Trustee Wilma Robins and I did our 'Chess Valley Wicked Walkabout' joined by MPS staff, family and friends on Sunday 12th May 2013. Wicked Walkabout organiser packs are downloadable from the MPS website www.mpssociety.org.uk or on request from the office.

The Society has regular success in securing funding for special projects such as the Legoland trip coming up in August. However to secure these funds, run these events and deliver on all the other services offered by the MPS Society we do need your help raising unrestricted funds. We fully appreciate times are tough for so many of you so the staff team and Trustees too are really going the extra mile to help bring in those extra pounds.

When it comes to future clinical trials for Sanfilippo disease, they are like London buses. Nothing for decades then suddenly Enzyme Replacement Therapy, Genistein and even Gene Therapy are only just around the corner. An update of Clinical Trials can be found in this MPS Magazine or a more detailed overview is available on the MPS website www.mpssociety.org.uk.

If we are to benefit from clinical trials the MPS Society needs to continue its ongoing dialogue with NHS England to ensure access to high cost therapies for MPS and the Lysosomal Storage Diseases are embedded into the future plans and budgets for the new NHS. We are confident that the commitment to all of you by Earl Howe, Minister of Health, last Autumn is holding up at the moment but we ask that you let us know if you have any concerns or see changes to your treatment regime alter going forward.

Having said that, we are aware that to make efficiencies to the ERT budget, patients receiving their treatment in hospital are being required to have their treatment at home. All patients receiving Enzyme Replacement Therapy are also required to accommodate a small fridge provided by the Homecare company so that monthly deliveries of enzyme can be made. We do ask that you do your best to work with these arrangements as the savings will enable those newly diagnosed with Fabry, MPS I, MPS II and MPS VI to be put onto ERT as soon as possible.

For those with MPS IVA and their families I want to reassure you that we are asking all the right questions but NICE do not seem to be coming up with any answers. For this reason we will be contacting you to invite you to raise your situation concerning access to Enzyme Replacement Therapy with your Member of Parliament before the Summer recess.

For those of you that attended the MPS Conference Weekend in its new venue, the Coventry Hilton Hotel 28 – 30 June, we hope you enjoyed the programme. Please let us have your feedback. A feature on the conference will appear in the next edition of the MPS Magazine.

Christine Lavery MBE
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twitter
twitter.com/MPSSocietyUK

News from the Board of Trustees

The Society's Trustees meet regularly. Here is a summary of the main matters that were discussed and agreed at the Trustee Board Meeting held on 8-9 February 2013.

Treasurer's Report

Trustees considered the Society's year end accounts and agreed to report back to the Finance Officer.

Risk Management

Trustees agreed the Risk Register required a thorough review although it was acknowledged many areas have been addressed over the last 6 months including succession planning. It was agreed that a full review of the Risk Management Register take place at the next Trustee Board Meeting.

Personnel Report

It was acknowledged that the staff team are working very well together and the Trustees recognised the impact of the high level of work on the advocacy team due to a vacant advocacy post as a result of the lack of funding.

Clinical Management

The Chief Executive Officer updated Trustees on work related to the MPS specialist centres and challenges for members living in the South West of England. Trustees received a report on the LSD Patient Organisation Collaborative's engagement with Kate Caston, Director of Specialised Commissioning, NHS England and Barbara Howe, Director of Highly Specialised Commissioning and NHS England. We are still waiting for further clarity of the Clinical Reverence Group for Rare Diseases.

Advocacy Support

Sophie Thomas, Senior Advocacy Officer reported that times are challenging for our members with all the benefit changes coming into place in April 2013 and she expects large volumes of work in the coming months.

Fundraising

Whilst the Society is showing an 11% return on its applications to charity trusts and corporate donors the members unrestricted fundraising is well below that of this time last year.

To try to address this the Chief Executive Officer proposed that all Trustees organise a 'Wicked Walkabout' or an alternative fundraising event. Leading the way the CEO announced that she and Trustee, Wilma Robins were organising a 'Wicked Walkabout' in the Chess valley for 12th May 2013.

New website launch www.mpssociety.org.uk

As you will know over the last few months, there have been lots of developments at the MPS Society and a smart new look. We are delighted to announce that we have also launched our new website.

The most important change is that we have gone from being a co.uk address to a org.uk address to be in line with other charities and not-for-profit organisations. Don't worry if you forget and use the old website address as we have a redirection set up.

Following feedback that navigation on the old MPS Society site could be improved we have taken your comments on board and created our new site which includes the new branding. The updated website has easy to find tabs that include information about MPS and related diseases, treatment options, latest research, clear information about the support our Advocacy Team can offer, how you can get involved in fundraising and also our events.

We are also now able to offer an up to date clinical trials page which includes information about the latest trials for MPS and related diseases and information about who to contact for more details. There is also a brand new latest news section so as we receive news about latest research, changes to benefits that may affect you and details of our events we will put them on our latest news section.

When you visit our new website, don't forget to take a look at our updated shop, we have some new MPS Society T-shirts as well as our regular best sellers and you can even pay by Paypal now too.

Now we have our smart new site we will be updating it regularly to keep it relevant and keep you informed with all latest developments. We are hoping to increase the amount of traffic we get to the site and will continue to use our Facebook and Twitter sites to attract existing and new audiences. If you aren't following us yet on social media, then please do so at @MPSSocietyUK for Twitter and facebook.com/mpssociety

We have tried to include as much as we can on the site, but would welcome any feedback and suggestions, it's your charity, your site.

You can email your comments to **info@mpssociety.org.uk**

New members

Kathleen has recently been in contact with the Society. She has Fabry disease. The family live in Cumbria.

Jayden is 20 months old and was diagnosed just after is 1st birthday with Sanfilippo disease. Our family are from the Midlands.

Wayne has recently been in contact with the Society. He has diagnosis of Fabry disease. The family live in Tyne and Wear.

Congratulations

Congratulations to Tom and Liz Gill on the birth of baby daughter May Elizabeth, born on 27 March 2013. Little sister to Bobby (MPS III) and Tommy.

Congratulations to the Oulton family
David Oulton (MPS II) is now uncle to Tyler, born 15th October 2012 weighing 9lbs 6oz. Tyler adores Uncle David (Day Day).

Family home videos needed!

The MPS Society are looking at creating some short videos to raise awareness. These will be used online on our website and on social networking sites such as Facebook and Twitter. We hope that people will share these far and wide, connecting with new supporters.

To make these films engaging and appealing to viewers we would like a selection of snippets of home videos from families affected by MPS diseases. We are looking for a variety of settings – playing with siblings, family meal times, celebrations and holidays. We would be interested to have footage of treatments (in hospital and at home) and anything else you may have filmed!

Footage will be selected, edited and put to music. We don't need Hollywood quality. We need real life home videos.
If you can help us please contact us on 0845 389 9901 or wickedgenes@mpssociety.org.uk today.

Please update your contact details: We like to keep you informed of news, events, information and opportunities. To minimise our costs we aim to contact you by email wherever possible rather than by letter so it is vital that you keep us informed of any changes to your contact details and let us have your current email address. Please email **mps@mpssociety.org.uk** to advise us of your email address and we can amend our records.

Ollie sets up online business

Ollie was diagnosed in 1999 with MPS VI Maroteaux Lamy syndrome and was given a horrible prognosis of 5 years to live by a specialist in Leeds (even though he knew little about the disease). On making contact with the MPS Society, we realised that things were not quite as bad as this specialist suggested and that he may actually have a future. We were introduced to Prof Ed Wraith at Royal Manchester Children's Hospital and to cut a long story short, Oliver went onto Enzyme Replacement Therapy in 2006.

Not only has Ollie defied the specialist who gave him the original bad prognosis, (at the age of 4) but now he is 18 years old has just launched his very own business.

Ollie has taken inspiration by his own experience of having difficulties with certain household tasks and began to search the world for labour-saving gadgets. But he wants his business to reach out to all people whether they are able-bodied or with some form of disablement and believes his gadgets can help everyone. He has been working on preparing the business for the last six months and will not give in to the illness. He now has an e-commerce website, a Facebook page and is planning attending shows and fairs whenever he can!

Ollie still has his 4 hour infusion at home with Naglazyme and feels that this has slowed down the progression of the illness to such an extent that he is focusing on the future and refuses to let his health issues get him down. He still has trips to the hospital every few weeks where they are monitoring virtually every part of his body.

Over recent years he has had many operations including a spinal decompression operation. He is now awaiting a hip replacement because he is in constant pain and can only walk very short distances. He has also recently had a hernia operation, and is currently having vision and eye problems so is evaluating if a corneal graft can be done. But despite all this he is a very happy, cheerful enthusiastic person who is loved by all! Everyone who knows Ollie looks upon him as an inspiration and a really happy character and we feel this represents a true good news story!

Ollie is adamant that MPS patients can still have a life and even a successful career and is totally committed to demonstrating this. Take a look at **www.tasksmadeeasy.com** and check out his page on facebook! **Paul Moody**, father of Ollie.

What's on in 2013

CONFERENCES and REGIONAL EVENTS

MPS Family Weekend Legoland - Please contact the MPS office for availability
16-18 August

Childhood Wood Planting
13 October

MPS REGIONAL CLINICS 2013

MPS IV Clinic GOSH
9 July (tbc)

Manchester BMT Clinic (under 6 years)
12 July, 11 October

Manchester BMT Clinic (over 6 years)
5 July, 18 October

BMT Clinic GOSH
24 September (tbc)

Bristol Clinic
8 July

Fabry Clinic GOSH
27 August (tbc)

MPS III Clinic GOSH
13 August, 12 November

Birmingham MPS and Fabry Clinic
8 November

Adult Fabry Clinic Birmingham
13 August

The MPS Society pays tribute to Professor James Edmond (Ed) Wraith

Consultant Paediatrician at Central Manchester and Manchester Children's Hospitals NHS Trust

On the evening of Wednesday 10 April 2013 Professor Ed Wraith died suddenly and has left his family grieving for a much loved husband, father, brother and grandfather. The MPS Society's thoughts are with Ed's wife, Sue, and their three children at this very sad time.

Ed came into my and the MPS Society's life in 1987 when Ed's predecessor, Dr Immie Sardhawalla, approached the Society to ask if we could fundraise to support the appointment of Ed as a second paediatric consultant. From the moment the MPS Trustees met Ed they knew that they were about to support a human being whose priority as a doctor focussing on Mucopolysaccharide and related diseases was totally unique and selfless. Ed did not disappoint and will be hugely missed not just by families of children who have been under his care but clinical colleagues who viewed him as a friend or mentor across the world.

Twenty five years ago there was very little good news when presenting an MPS diagnosis to a family but Ed had a gift for supporting families whilst giving the worst of news. So many families recall the hours he would spend with them explaining their child's condition and he is infamous for his upside-down drawings to help understand the genetics.

Ed was a willing partner working with the MPS Society to take his expertise to all corners of the UK through the MPS Clinics. In the 1990s we made many visits to countries near and far, where at MPS Society family meetings, in his usual easy way he would share his knowledge and answer parents questions late into the night. Possibly a remedy for the many unusual places we expected him to rest his head including a monastery in Hungary, communal accommodation at St Alfred Haus in Germany to a caravan with a cockerel as an alarm clock in Northern Ireland.

He was always keen to progress knowledge of the science of these devastating MPS diseases and over the past 12 years embraced the opportunity to have pioneering clinical trials for lysosomal diseases in Manchester. He worked closely with colleagues, the pharmaceutical industry and the MPS Society to ensure MPS families had access to reimbursed Enzyme Replacement Therapy.

Ed was born in the North East of England, graduated from Sheffield University in 1977 and received his postgraduate qualification in paediatrics (MRCP) in 1980. In 1993 he became a Fellow of the Royal College of Physicians (FRCP) and in 1997 a Fellow of the Royal College of Paediatrics and Child Health. However I understand the award he treasured most was the Wellchild 'Best Doctor Award' where he was nominated through the MPS Society and presented his award by Prince Harry. It goes without saying Ed was a much valued medical advisor to the UK Society for Mucopolysaccharide Diseases.

Ed you will be sorely missed by the whole MPS Community at home and overseas.

Christine Lavery
Chief Executive

Here are some comments from the MPS Society facebook page:

The doctor you could sit across from and talk to like a mate, who made my boy feel so at ease every time he saw him! There are not enough words to describe what a brilliant, great bloke he was, thanks for all you did for my son

One of the most genuine, unassuming and warmest people I ever had the pleasure to meet and had a way of making you feel that everything was going to be ok

Shone a light in our darkest days

The man managed to give us more hope than anyone or anything we have read since

Thank you to those individuals and organisations who kindly made donations to the Prof Ed Wraith memorial fund on the MPS website.

In Memory

You're calling him what?!

Well, if ever a name fitted a person, Blaise's name fitted him! Once he was up and running that was it, he blazed a trail leaving chaos and havoc in his wake. Blaise means stammerer and true to form Blaise stuttered. I am proud to be Blaise's N n n n nanna...

Those of you that knew Blaise in his two to ten years will recall the whirlwind with an aim to rival the greatest of darts or discus throwers, and while the receiver was rubbing their head Blaise was off roaring with his inimitable belly laugh and a smile that could light the darkest of skies.

Blaise knew where everyone lived and the way to get there and woe betide if you went the wrong way, he was the original back seat driver. He also knew where every McDonalds was in a 5 mile radius or could spot one half a mile away, and if he thought he was en route to a McDonalds and a car got in the way... well let's just say road rage would raise its head. He would also practice his road rage in the garden in his cars, so much so that Terri-Ann had to bring him in in case the neighbours heard him.

Blaise loved his food, really loved his food and he had certain meals he liked to have at different places, our house was tinned spaghetti or SKETTI. He would march in straight to the kitchen, get everything out he needed, put the tin in the pan and onto the cooker and shout "sketti n n n nanna". At Francis House he liked to have spaghetti bolognese, at Manchester Children's Hospital's restaurant it was sausages. If the Children's Hospital was on TV he would shout "sausages mummy". He liked to share his food too, especially with our dog straight from his mouth... needless to say she misses him too.

Blaise stayed at our house most weekends, he was Alex's wrestling partner, Grandad's garden buddy and he liked to share Cheska's lipstick when

she was getting ready. He would sleep in the big bed with Nanna. Can any of you imagine what it must be like to sleep with a giant Jack in the Box? Believe me that boy could go from lying to sitting with the speed of a striking cobra, this would go on throughout most of the night as Sanfilippo children are not known for their sleeping skills. So we set up the spare room as Blaise's - friendly, all nice and redecorated. I woke the next morning to find Blaise playing and giggling, went in to get him.... and made a hasty retreat to put on a bath - his lovely new bedroom looked as though Willy Wonkas Chocolate Factory had exploded only it didn't smell of Cadbury's Roses! This happened every time so we reverted back to sleeping with Nanna, it was much easier to wake to a bra and knickers on my head instead!

Blaise loved music, his favourite song being 'Man, I feel like a woman', this changed to 'Blaise, you look like a woman'. Pink by Aerosmith was a favourite in the car with Nanna, Blaise did mainly the pink bits and Nanna had to sing the verses, he was very partial to a bit of Meatloaf too.

Blaise has loved his school life both at Lisburne and the Heatons, he would get grumpy and bored in the holidays, but the joy and excitement on his face on the first days back in was a lovely sight. Many thanks to his carers at both schools who have loved him almost as much as we do.

In recent years Terri-Ann met Andrew, who has been an amazing daddy to him, and Blaise became the proud big brother to two baby sisters and at Christmas his baby cousin Millie. He loved babies, apparently there is something very funny about a baby crying according to Blaise.

He was the ringbearer at Terri-Ann and Andrew's wedding last year, eventually, as we had to cancel the first time round as he was very poorly.

We have so many happy and funny memories of our very special boy, mixed in with proud moments, like when he made a plane full of people erupt into laughter on a very very rocky approach to land with his cheering at the bumpy ride, meanwhile his mummy was turning green, and what a mummy she has been to him. We are so proud of how she has fought tooth and nail for him and never ever given up on him. Thank you Terri-Ann for giving us the most remarkable grandson ever and we shall never ever forget him. He will always be Nanna's Blaise (Family joke) xxx

Our gorgeous son **Thomas L Fisher** aged eight years old passed away at Demelza Hospice Care For Children on 20th May 2013.

Thomas was a kind, gentle and loving boy with the most beautiful smile and infectious laugh. Despite the hard times, sleepless nights and unlimited banging we love you no matter what. You will always be in our hearts. You'll be with us forever

Love Mum, Dad, Mia and Mikkel xxx

Paul Franklin 1979 - 2013

Paul was born on 9th July 1979 and weighed in at 10lbs 6ozs. He was now the young brother of our daughter Emma who was born in 1975.

Five months later Paul was diagnosed as having Hurler's Syndrome.

We were extremely lucky to have a GP who was concerned that Paul was not thriving. He was not putting on weight and had what appeared to be a snuffle like a cold. After a couple of months of tests at our local Hospital the doctors detected this rare condition.

Our next piece of luck came when a close family friend who had actually fostered a child with Hurler's told us about doctors at the Westminster Children's hospital who were wanting to try a Bone Marrow Transplant (BMT) for an inborn error. Within a week we were at the Hospital speaking with Professor John Hobbs, Head of the Transplant Team, and Dr. Kenneth Hugh-Jones, Consultant Paediatrician.

It was decided to try a BMT, which at the time was experimental, and because he was too young to have complete body irradiation they used a toxic cocktail of drugs to remove his own bone marrow.

It was the first BMT in the world for an inborn error.

Paul was transplanted twice, one at 9 months (using my marrow) and the second at 11 months of age (using Anne's marrow) in April and June 1980.

We were both deemed 'compatible enough' to try the transplant. The second one took. However, despite the enzyme now being produced by his new marrow it did not reverse the damage that had already been done to his body, and during the next few months Paul became very ill a couple of times as a result of what is termed 'graft verses host disease' where the new marrow attacks the recipient, but his battling spirit brought him through.

All this time Paul was in a plastic bubble – two in fact. The first consisted of a cot in a big plastic tent. The second was a bed in an even bigger plastic tent in a different ward downstairs. He was to remain in the tents for 8 months.

How did we get him from the cot to the bed? A large plastic bag was passed into the cot tent and Paul was placed into the bag and handed to me so that I could pull him out through the air vent. The bag was sealed to prevent air getting in. I then carried him through the corridors, down the stairs (all lined by Doctors and Nurses in the event of a mishap) to the new ward. He was then passed into the bed tent via the air vent into the arms of Anne. This is when the only hitch occurred. Paul would not let go of the bag when we tried to get him out. This typified Paul's fighting spirit throughout his relatively short life.

While in the cot Paul would make the tent collapse when the odd nurse or doctor forgot to leave one or both of the plastic arms dangling outside the tent. He discovered that by pulling off the marigold glove (dark ages) that was attached with an elastic band (up to date technology) it would collapse the tent, thereby setting off the alarm which in turn brought nurses running from everywhere. He used to sit there with a grin on his face. This happened several times.

Paul was allowed home on Christmas Eve but went back on Boxing Day and spent several more weeks in Hospital until it was considered time to come home permanently.

During the next 31 years he lived life to the full. He was walking, running and kicking a football until his condition got the better of him and he was in his wheelchair by the age of 11.

However, he was always so positive, so outgoing, so friendly, so cheerful (except when Crystal Palace lost), considerate and helpful.

The love and support of friends and family helped us through the early years.

"Paul's Pennies" was set up by the Young Wives group at his local church. Money was raised by way of a number of events and happenings such as jumble sales, waitressing at wedding receptions (for a fee), church hall dances and pantomimes which were performed by all of us mums and dads in the church to sell-out crowds.

Paul's Pennies was started by friends who wanted to help in any way they could and raising money seemed to be the best way to do that. What if Paul needed treatment in America for example, or some sort of medical equipment was required. The funds would be there.

After his transplant most of the money raised was donated to the Bone Marrow Unit at the Westminster Children's Hospital.

Some of the money was used for us all to go to Lourdes and several years later the balance of the fund was used to buy Paul a video recorder for his new room as a special treat after operations on his back and neck which resulted in him wearing a Halo and back brace for several months.

Wearing the Halo and brace did not stop Paul from going back to school because he didn't want to miss the school concert.

He attended St. Giles School for the disabled which he loved and in spite of more than 20 operations (some small but Paul insisted on counting them) that he endured during the early part of his life he always wanted to get back as soon as possible. He certainly never grumbled about the problems that his disability presented – he just got on with life. He was a communicator and organiser with his group of friends from St. Giles and he also had a very wide circle of friends because everyone that he met became aware of his enormous personality and character.

One morning, about a week before he was due to go back to the Hospital to have his Halo removed, Anne walked into his bedroom to discover Paul lying on his back with his Halo collapsing around his head. The screws securing the frame to his forehead had come loose. Paul wasn't too bothered but panic set in and an Ambulance was called. First one arrived, that was no good – no spinal board. Second one arrived with the necessary board and off he went to Great Ormond Street Hospital. After a three or four hour wait on a trolley reading football magazines that Anne had bought and chatting to any passing nurse or cleaner he had his Halo and brace removed and was passed fit by the surgeons and came home.

Paul became an Honorary Beaver, a fully-fledged Cub and then a Scout. He was awarded the Scout's highest honour, The Cornwell Award. This is a special award which is reserved exclusively for Scouts under the age of 20 and is given in respect to their courage and endurance under extreme conditions.

Paul attended St. Giles from the age of 3 until he was 16 and he kept in constant touch with several of his school friends and saw all of them at least once or twice a year and others more frequently.

Even at an early age he was helping some of the other children with their various disabilities. Paul appeared in the school Nativity playing Joseph. Everything went well until the frankincense, myrrh and gold arrived. Paul put them in the crib on top of Baby Jesus. That was an 'oops' moment.

Paul left school with a GCSE in Art and attended Croydon College for a few years.

Paul appeared in Songs of Praise when it came to Croydon as well as a documentary about Bone Marrow Transplants entitled 'Chance of a Lifetime'.

Paul enjoyed a full and eventful life despite his disability. He made friends easily and kept in contact with many of them for years.

He attended the Eldon Fab club in Croydon, then the Waddon Youth club which he first went to as an after school pupil while at St. Giles and later as a volunteer with his best friend Daniel. Apart from generally helping out he would set football questions for quizzes from time to time.

He joined the Dance and Movement group in Croydon and ended up having his photograph on posters all over Croydon. Paul has a copy on the wall in his room. It shows him in his tipped back wheelchair, baseball cap back to front punching the air.

He appeared at the Concert Hall at the Fairfield Halls singing with St. Giles. and on another occasion at a Croydon Schools concert he was on stage the same time as his big sister Emma.

Paul enjoyed his 'Activenture' holidays in Ashdown Forest, Sussex. The British Red Cross and the Association of London Youth clubs joined forces to run the residential centre which was used for a couple of one week holidays a year.

During that time Paul would do abseiling, canoeing, swimming and archery.

Vitalise was another organisation that Paul enjoyed. They had centres in Southampton, Nottingham, Southport and Chigwell and over the years he visited them all. The week had a packed programme of events and always finished with some sort of a party which he proudly evidenced by showing us photographs on his mobile phone of him surrounded by several scantily dressed young female helpers.

Crystal Palace was his life. He didn't go to many matches because of his poor eyesight but when we did go it was in the Arthur Waite stand. Over the years he watched Palace at Coventry, Nottingham Forest and Birmingham City when he was away on a special trip with one of his clubs.

Paul has photographs of himself with Ian Wright and Steve Coppell and other members of the team over the years. He even has Nigel Martin's Goalkeeper's shirt signed by the whole of the Palace team.

We once visited the training ground when Steve Coppell was manager and after Steve Coppell chatted with Paul he took off his own baseball cap, signed it and gave it to Paul. Paul could always charm anyone.

Many years ago Paul was captain of a five-a-side football team at a local charity event. The team was called 'Paul's Posers'.

Paul played in goal and if he didn't stop the ball on purpose with his wheelchair he would often stop it painfully with his head.

For the last 13 years Paul worked at the local McDonald's for a few hours at lunchtime on Fridays either clearing tables or making up the children's meal boxes.

Tuesday afternoons were spent in Croydon with his friend Daniel, when they would have a meal, do some shopping and then go to the Waddon club. Wednesdays were usually reserved for the cinema in Croydon.

Paul loved socialising and at any get together or gathering he would chat to all and sundry about anything and everything. For the last 12 years we celebrated New Year's Eve in different hotels in various parts of the country with a group of friends where he had a chance to wear his dinner jacket and bow tie.

When Paul wasn't chatting to you face to face he would phone you up. He would talk about anything from telling his Granddad the lottery results to discussing the latest football news or the latest film releases. Or he could be simply asking how you are and telling you of the Franklin news.

His memory for birthdays, football facts and film trivia was phenomenal. Not only did he know the birthday of every family member and friend but also of film stars and other celebrities.

Paul was always confident, determined, brave and outgoing. A long time ago it earned him the nickname 'Paul I can do it myself Franklin' and it was always the case.

To prove the point we can recall when Paul was about 5 years old he would wake up very early in the morning, go downstairs to the kitchen, climb onto the fridge and turn the television on. When we discovered this he was punished by us taking his favourite toy, the A Team van, away from him for a day. This happened a few times until early one morning a week or so later Anne was lying in bed when she felt a tap on her shoulder. Paul produced the A Team van said 'e are' and went downstairs to the kitchen, climbed onto the fridge and turned the television on.

Our daughter Emma has two girls, Katie and Lucy and Paul was so proud of being an Uncle.

Paul is from the Latin 'Paulus' meaning small. "He is respected and strong, with great conversational skills. A communicator and teacher, although prone to chasing dreams. Gives his time for the good of others." How true.

I guess we thought someone so outgoing, so friendly, who enjoyed life so much would go on forever and yet there was always the history of vulnerability if we dared to think about it.

Paul had a persistent tickly cough for several months and for the last couple of weeks of his life he was off his food and generally not feeling 100%. But this was not uncommon and he usually bounced back.

However, this time it was not to be and Paul passed away without pain or suffering in his own room with Anne and I and Emma.

We will never regret the decision we made for Paul. We had 33 wonderful years which we wouldn't have missed for anything.

There are no words to express our thanks to all of our friends and family for their support and encouragement over the years and to all of his Teachers, Youth Workers and colleagues for the help that he was given.

Goodnight Paul. Sleep well.
Ray and Anne Franklin

Bereavements

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

Emily Otway who suffered from Sanfilippo disease and who passed away on 22 March 2013 aged 16 years.

Blaise Leslie who suffered from Sanfilippo disease and who passed away on 23 March 2013 aged 15 years.

Paul Franklin who suffered from Hurler disease and who passed away on 4 April 2013 aged 33 years.

Jake Lloyd who suffered from Sanfilippo disease and who passed away on 15 April 2013 aged 12 years.

Aaron Doherty who suffered from Sanfilippo disease and who passed away on 16 April 2013 aged 16 years.

Thomas Fisher who suffered from Hunter disease and who passed away on 20 May 2013 aged 8 years.

Correction: In the last edition of the MPS Magazine, it was wrongly recorded that Ian Shead passed away aged 40 years. Ian was 23 years. We wish to apologise for this error.

MPS Regional Specialist Clinics

The MPS regional clinic programme involves centres including Manchester, Belfast, Birmingham, Bristol, Cardiff, London and Newcastle.

The programme aims to enable individuals affected by MPS and related diseases to access a consultation with a tertiary centre consultant without having long distances to travel to their designated specialist centre. The clinics also aim to increase expertise in the regional centres on MPS and related diseases in the clinical management of those affected.

The MPS Society supports the regional clinic programme, which can include arranging and co-ordinating dates of the annual programme and funding the tertiary centre doctors' travel and subsistence costs to the regional centres. Specific arrangements for each clinic and for organising appointments differ for each of the centres. At least one member of the Society's advocacy support team meets individuals and families face to face to offer individual advocacy support.


Jordan


Melissa

Bone Marrow Transplant Clinic

It was a warm day on 3 May 2013 for the BMT clinic at Royal Manchester Children's Hospital, though we only saw the sun on the journey to and from Manchester! This was my first visit to Manchester's Children's Hospital and I was made to feel very welcome by the staff and families. It was a busy clinic and nice to see the families having a chat and catching up on each other's news whilst waiting to be seen.

Jordan flew down from Scotland and was proudly wearing his Celtic sweatshirt and Mum was also wearing matching green. **Melissa** continued the colour theme by wearing lovely purple accessories to match her school uniform; she even had a purple drink! **Cody** wore beautiful silver shoes and agreed to have her photo taken with **Charlie** who had spent the night at a hotel after having travelled from Northern Ireland. **Jamie** also travelled from Northern Ireland and the Mums were glad of the IPads to keep the boys entertained. **Shantelle** also travelled from Northern Ireland on the overnight ferry which took 8½ hours. Shantelle enjoyed spelling her name with the letter tiles.

It was lovely to meet all the families and to put some faces to names and also to meet Jean and Pauline too.

Debbie Cavell Advocacy Officer


Jamie


Rachel


Cody and Charlie


Shantelle


Sonny

The MPS Advocacy Service

The MPS Advocacy Support Service has been established since the Society was founded in 1982. At this time there were only 40 known families throughout the UK. The support provided was on a voluntary basis and depended heavily on individuals and parents to provide support to individuals diagnosed within their immediate and surrounding areas.

However in 1991, the Society opened its first office and with this the advocacy service we know today was born.

The MPS Society provides, through a team of skilled staff, an individual advocacy support service to its members. The service is flexible and a wide range of support is offered on a needs led basis.

The rarity of these conditions means that in many cases, accurate assessments, support and advice are not given due to the vast majority of social care and health professionals knowing very little if anything about the diseases.

Support provided by the team

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

For more information on any of the above or if there is anything else that you would like to chat with the advocacy team about please contact us:

Email:
advocacy@mpssociety.org.uk

Telephone: 0845 389 9901

Advocacy Resources

The Advocacy Team have also developed a range of information resources focussing on particular issues which are available to download free of charge from the MPS website, www.mpssociety.org.uk

- **Disability Living Allowance**
- **Life Insurance**
- **Travel Insurance**
- **Hospital Travel Costs**
- **Disabled Access Holidays**
- **Carers Legal Rights**
- **Carers Allowance**
- **Wheelchairs and Flights**
- **Guide to Housing and Disabilities Facilities Grant**

SOPHIE
Manages the MPS Advocacy Team

Each of our MPS Advocacy Officers works with specific disease groups as listed. However, every member of the Advocacy Team has knowledge of all the diseases and may at times provide support in other areas dependant on need and individual assessment

Team members

REBECCA
Fabry
MPS II Hunter
ML III / ML IV
Mannosidosis
Fucosidosis

STEVE
MPS III Sanfilippo
MLD
AGU
Winchester
Geleo Physic Dysplasia
SLY
Gangliosidosis
Sialic Acid Disease

DEBBIE
MPS IV Morquio
MPS 1 Hurler BMT, Hurler Scheie, Scheie
MPS VI Maroteaux-Lamy
MSD,
ML II

ALISON
Supports all members living in Ireland

My first six months in the MPS Advocacy Team

Debbie Cavell joined the Advocacy Team in January 2013 and works with MPS I, MPS IV, MPS VI, ML II and MSD diseases.

As I approach my sixth months at the MPS Society it now seems a long time ago that I first started as an Advocacy Support Officer on a cold, dark January day and looking back it seems that I have learnt so much already and I have met and worked with so many inspiring people and families.

I joined the MPS Society fresh and eager as a newly qualified social worker. Advocacy work appealed to my sense of justice and determination to challenge discrimination of every kind. I had learnt the theories and laws at university – now I just needed to put my skills and knowledge into practice.

On my first day I was warmly welcomed by the team and everyone made me feel at home. Sophie, Steve and Rebecca from the advocacy team have always been there with good advice and a seemingly limitless extent of listening skills and knowledge which has proved invaluable to me. The other MPS Society staff are equally as warm, friendly and helpful with my early requests for assistance such as navigating the database and fixing the jammed photocopier!

When I was asked to write this article I started by looking through my diary to see what I have done and I am amazed at how much I have accomplished so far in such a short space of time. The role is very varied, which I enjoy, and here is a taste of my work so far.

On my second day I attended the MPS IV clinic at Great Ormond Street Hospital and since then I have attended clinics also at Bristol Children's Hospital and Manchester's Children's Hospital. This has been a great opportunity to meet families and get to know one another.

With the changes in benefits this year I have dealt with a lot of enquiries regarding DLA and ESA applications including appeals and I now have an ESA tribunal coming up too. Assisting with these has helped me learn about how the MPS diseases affect individuals on a day to day basis and the challenges that families face and I am constantly amazed at the coping skills and strategies that families have to manage living with MPS.

I have also attended several school talks. This involves presenting to staff information about the conditions and how the disease impacts on school life and changes that could be made to benefit the child. I have also presented school assemblies to children where the content needs to be adapted to the age of the children. Luckily, I previously worked in several schools and can draw from past experience. This means I don't find a group of primary school children with lots of questions quite so daunting!

I have also supported families and individuals in a variety of ways and I never know what the next call is going to bring. I have learnt a great deal about housing and adaptations, medical equipment, obtaining funding and respite care and bereavement. This has given me the opportunity to work with many families and already I have travelled far across the country to meet with members.

At the time of writing I am looking forward to attending my first MPS conference. From talk in the office this seems to be a lot of hard work, but immensely valuable for families. I am particularly looking forward to meeting families and professionals that I have not yet worked with.

Looking back on my first six months the best part so far must be meeting the families and working with them to empower them to find a solution to their issues – which is great as that is why I chose to become a social worker in the first place and I'm looking forward to meeting and working with many more MPS members in the future. **Debbie Cavell**

Employment and Support Allowance (ESA)

The MPS Society has recently had members contacting us to advise that they had their ESA benefit stopped without any warning.

Since January 2013 if you are on CONTRIBUTION BASED work related activity benefit this is for 365 days only (1 Year). If you have been receiving this benefit before January 2013 then it is advisable to contact your Job Centre Plus and find out when the benefit started and make sure you contact them well in advance of the end of the benefit.

Speaking to the Benefit Helpline, 'in theory' claimants applying after January 2013 should be advised in advance of the benefit stopping and enabling claimants to reapply.

There are likely to be teething problems with all the changes happening this year..! For advice please email **advocacy@mpssociety.org.uk**

All Ireland Advocacy Support

Northern Ireland MPS Clinic

The 23rd May was the biannual MPS Specialist clinic in Northern Ireland. As usual this clinic was held in Antrim Area Hospital but for a change the sun was shining!

The medical team of Dr Fiona Stewart, Dr Simon Jones, Dr Siobhan O'Sullivan, Ms Aoife Bradley (Genetic Counsellor) and Ms Valarie Peacock (Hospice Nurse) saw 14 MPS families while I supported families with any advocacy support needs.

We were delighted to welcome Ms. Valarie Peacock along to the clinic. Valarie is a Northern Ireland Children's Hospice Nurse who is well known to lots of our MPS families. Valarie was attending the clinic to expand her knowledge on MPS and related conditions but I think our families benefitted hugely from having Valarie there to talk about the services that the Children's Hospice can provide. It was lovely to have the NI Children's Hospice represented at the clinic and we hope that Valarie and her team will be a regular addition.

The Northern Ireland Regional Genetics Service were delighted to receive a supply of distraction aids from the Starlight Foundation for the clinic this year. Clinic can sometimes be a daunting place for children who don't understand the investigations they are having, so anything that can make clinic more fun is a truly welcome addition.

Our next clinic will be in November and the date will be announced on the MPS website.

It's been a very busy time for the All Ireland Advocacy Support Service and many of the things the Advocacy Team get up to cannot be reported in the magazine. However, over the coming months I will be holding a Northern Ireland 'Wear it Blue' Family Day and will be attending lots of conferences and courses that I look forward to updating you on in the next MPS Magazine.

I also hope to bring you a few stories for our members in Northern Ireland in the next magazine. **Alison Wilson**
a.wilson@mpssociety.org.uk

Sources of information and support

New information website for young siblings.

www.youngsibs.org.uk

Young sibs is part of the original Sibs organisation which is the only UK charity representing the needs of siblings of disabled children. The idea behind young sibs is to provide young siblings up to the age of 18 years with much needed information and support, whilst they are growing up with a disabled brother or sister. The website covers various topics based on previous knowledge of siblings needs and includes things such as information about disability, dealing with feelings and family life, and tips for coping with difficult situations. It has a letters section for siblings to receive personalised responses to their questions, and a safe chat area for young siblings to talk to other young siblings in the UK.

New 2013 Holiday Information Guide from HFT

HFT is a national charity, providing support to people with learning disabilities throughout England. HFT have produced a holiday guide for people with learning disabilities which includes helpful information and guidance to support you when choosing a holiday. The guide includes information on:

- Pre-booking information
- Information on guides provided by other organisations
- Tour Operators
- Places to stay in the UK and abroad
- Meeting specialist health needs
- Specific Interest Holidays
- Insurance
- Available benefits and help

This guide can be downloaded for free through the HFT website

www.hft.org.uk

Emergency Patient Information Cards

(only for members of the MPS Society)

What are Emergency Patient Information Cards?
The Society has developed with the agreement of all specialist centres Emergency Patient Information Cards. The card is the size of a credit card and includes relevant details about the patient, the condition that they have and contact details of their specialist centre. Included also is a link to the MPS Society.

The card has been designed so that it can be carried easily and if required could be presented to medical staff in an emergency, giving accurate information about your condition and who to contact for information.

Here is an example of how the card looks:

This patient suffers from a Mucopolysaccharide or Related Disease

Name: A Sample
Date of Birth: 00/00/0000
MPS Disease: MPSIVA Morquio
Hospital: Royal Manchester Children's Hospital
Oxford Road, Manchester, M13 9WL
Hospital Tel No: 0123 456 7890
NHS Number: 123456789
Consultant: Dr Sample

MPS

Society for Mucopolysaccharide Diseases

Front

Important Medical Information

MPSIVA (Morquio) is a rare genetic disease associated with significant short stature, physical disability and, in many cases, limited life expectancy. Significant skeletal problems include instability of the cervical spine which may have been treated by a cervical fusion. There is currently no therapeutic treatment; however some patients may be on a clinical trial for Enzyme Replacement Therapy.

Urgent Patient Specific Information:
Cervical instability
Intravenous Enzyme Replacement Therapy

MPS Society Number: 08453 899901
www.mpssociety.co.uk

MPS Society Out of Hours Office Number: 07712 653258

Back

How can I get a card?
If you are a member of the MPS Society and you would like a card for you and/or your child, please complete the form(s) included overleaf and return it to Jo Goodman, MPS Society, MPS House, Repton Place, White Lion Road, Amersham, Bucks, HP7 9LP.

Please complete one form per sufferer. If you need more forms they are available from our website or they can be requested by email at j.goodman@mpssociety.org.uk.

Additional cards are available but may incur a small charge; please contact us for more information.

When your child transitions to adult services please apply for a new card.

If you are not a member of the MPS Society and are interested in talking to someone about this then please get in touch.

Society for Mucopolysaccharide Diseases
MPS House, Repton Place, White Lion Road,
Amersham, Buckinghamshire, HP7 9LP
T: 0845 389 9901
W: http://www.mpssociety.org.uk

Society for Mucopolysaccharide Diseases Registered Charity No. 1143472. Registered as a Company limited by guarantee in England & Wales No. 7726882. Registered as a Charity in Scotland No. SCO41012

Emergency Patient Information Card Application Form
(only for members of the MPS Society)

Surname: ……..……First Name: ……...

Full Address: ……

……Postcode: ……..…

Date of Birth:…… MPS disease type: ……

Consultant: ……

LSD Specialist Hospital: ……...

Patient NHS Number: ……

Urgent Medical Information: (please tick relevant boxes)

[ ] Portacath [ ] Intravenous Enzyme Replacement Therapy

[ ] Intrathecal Enzyme replacement Therapy [ ] Haemopoietic Stem Cell Transplant – Date ……

[ ] Cervical instability [ ] Cervical Fusion [ ] Tracheostomy

[ ] Gastrostomy [ ]

Allergies……...

[ ] Anaesthetic risk

[ ] Other

……..…

E-Mail Addresses Required
In order to keep the Society's costs down and to be able to provide you with up to date information on events and other MPS information, can you please give us your email address details (please write clearly) and confirm that you are happy to be contacted by email by signing below.

Email Address: ……..…... Signature: ……
……

Live Laugh Lemonade

My name is Kendra Gottsleben. When I was four years old, I was diagnosed with Mucopolysaccharidosis Type VI. Today, I am 28 years old and I live in the state of South Dakota in the United States. I have not let MPS stop me from accomplishing all of my dreams. Thus far, my accomplishments include graduating from high school and college, having an incredible career, becoming a self-published author, and having started my own business.
My life has had its obstacles, but no one in this world can say they don't have obstacles they have to overcome each and everyday!

Overcoming obstacles to achieve dreams is a large reason why I decided to write my book. I wanted to share my life's challenges with others and also how I stay positive with each and every challenge I meet. Many people have told me that I have an amazing, upbeat attitude, even with all the barriers I have had to overcome throughout my life, and that I need to tell my story of never giving up.

In my book, Live Laugh Lemonade: A Journey of Choosing to Beat the Odds, I talk about how I know how difficult life can be as a child, teenager, or young adult who has a medical condition or disability, and that if I can make an impact on even just one person's life, I would be honoured. I have been tremendously fortunate even though I have MPS Type VI and am in a wheelchair. For the most part, I have been able to have a pretty "normal" life.

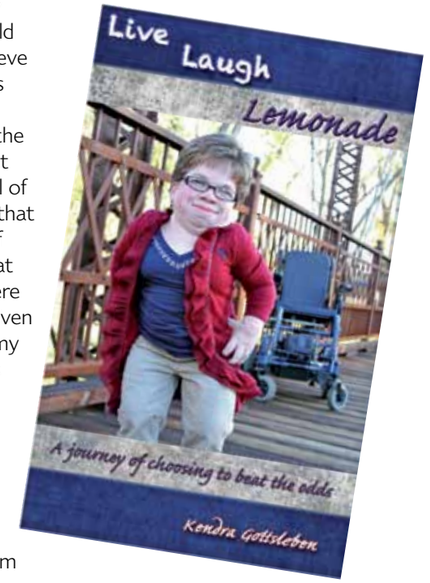
I have always tried to live my life with a positive outlook, believing that every hurdle I have to endure allows me the opportunity to learn something about myself and what direction I need to take in my future. I have never wanted others to pity me for my struggles because I do not pity myself. Having a "pity party" for myself will never help me obtain all my ambitions in life.

Everyone has barriers to conquer no matter who we are, whether we are able-bodied or not, small or tall, young or old, rich or poor, bold or shy. I acknowledge that we do need to allow ourselves time where we just need to cry, scream, and get upset with some of those obstacles in life. But we must not dwell on them for too long because thinking about them too much does not aid us in reaching our personal goals and dreams. Life is full of ups and downs for us all; that is just the way it is. As long as we work through the ups and downs, we will be able to accomplish something great!

I know that if I did not have MPS my life would have been completely different. Who knows, maybe I would not have been as motivated to achieve success or to help others if MPS was not in my life. I would not have had the awesome opportunity to be in the clinical enzyme trial. I also would not have had the pleasure of meeting all of the amazing and wonderful people that have come into my life as a result of MPS. I believe God only gives us what He thinks we can handle, even if there are times we wonder why He has given us such obstacles. That's why I live my life by the motto, "When Life Hands You a Lemon...Make Lemonade!"

Facebook: Kendra Gottsleben
Twitter: @Kegottsleben
Pinterest: Kegottsleben

The book is available on Amazon.com



Find us on Facebook
www.facebook.com/mpssociety

twitter
twitter.com/MPSSocietyUK

Visit our online shop at www.mpssociety.org.uk.
Purchase our information resources, Christmas cards and MPS merchandise including our **NEW T-shirt**!

Children's Pages

Naomi is 13 years old and due to her Fabry she has missed a significant amount of school. This piece was written after her English teacher wanted to know how Naomi could attend some classes and not English. She now understands how Naomi feels when she has a 'Fabry Crisis'.

Despite all her problems Naomi took her GCSE Foundation Maths when she had just turned 13 years old and passed with a grade C. Naomi is taking her GCSE in Latin and Statistics in June 2013. Good Luck Naomi...

Pain Crisis

A storm was coming. Not in the weather, no, this was no matter that was discussed in small-talk. When it threatened to turn up in conversation, you discarded the thought, and searched your brain for a different subject, for if the subject was conversed, it meant that it was real. That it was really back, and I didn't want that. At the time, I didn't know how dark the sky was going to get, I didn't know how heavy the rain was going to be, I didn't know how viciously the lightning would flash across the sky, I didn't know how loudly the thunder would roar, I only knew a storm was coming. I didn't know how bad the pain was going to get.

I lay back in my warm bed, rearranging the duvet again, and again, to find that perfect position, in which I would be ready to sleep. The pain bothered me. It was just pain though, not too bad, manageable. It glared at me with contempt, wanting to strike, but was controlled, chained by this new painkiller, this new weapon of mine, cowering back into the darkness to avoid the bitter taste of the painkiller.

This new weapon was a brilliant ally, and I was glad to have it on my side, to not have to be locked up in my prison of pain, desiring the pain to go, but never expecting it to; your innermost desires, they never come true. There. There was that perfect spot, that warm, snuggly duvet under my head and cuddling my body. It comforted me; my aching body was consoled. I fell into that warm, safe home that is sleep.

The sky churned angrily above, it was controlled, used, but no more! No more. It would get its revenge, that painkiller, that weapon, would not control it, could not control it, because it was powerful, more powerful than the weapon. These chains would hold it no longer. The chains broke. It gleefully roared, a roar of freedom! a roar of contempt! a roar of war! In it's parade of hostility and danger, the lightning flashed, the rain poured, the thunder roared, the sky darkened, in crescending harmonies to the symphonic tune that was played.

I tossed and turned in my sleep, in my nightmare, my own nightmare of the storm, the storm that never ended, that never changed, that was free, evil, there, never-going, eternal, that encaged me in my own world of pain, despair and such solace! No one knew what it was to feel this pain, no one apart from me, I had no understanding person to hold my hand, to make it go away, there was no magic wand; there was just the pain and myself, predator and prey, evil and good, free and imprisoned, despair and despair, and nothing would make it go away, nothing would attend to my needs, I was nothing, left to endure.

It was happy; rejoicing in its sick display of power, hate, pain, and pain, and pain! It was winning, victorious, marvelling in its show of control, utterly insane, utterly explosive, widening, growing, increasing, all the while getting worse. And the rain poured down, but it was not refreshing, it was a rain of tears, languid and salty. The rain poured, the thunder roared, the lightning flashed and the sky darkened ever more.

I awoke and everything was fine. That would have been so much better, so much more desirable, but I must not tell lies; the dream had not gone away, merely a dream, beckoning to the demand of reality, no, reality had joined with it, a betrayal from the darkest pits of the endless realm of all evil. Reality was worse, because there was no hope; reality had been my last chance, the reason to continue, the dream was bad but reality would soon come and rescue me, my saviour come to pick me up and comfort me and save me from this, this, this. But I must not fool myself, the dream was real, myself was worthless, lost among this injustice of slavery to it. I had no choice; I had to obey what it told me to do, because everything else hurt too much. My only solace was the "rest" it offered to me, the throbs eased every so often, giving me "peace", but they always came back.

I hurt, I hurt everywhere, there was nowhere on me that did not ache, or sting, or throb, or tire; if you asked me "name somewhere that does not hurt" I would have turned you away for there was not such a place. Everywhere hurt and I mourned, I mourned for a time when the painkillers worked, for a time where I was under the illusion that I was free, to be unknowing of bad things and happy is better than to be knowing of them and despair.

I found the will to sit up out of bed, stand up and walk the five paces to mum's room. I was punished accordingly. Oh, how unfair it was! I was under the control of this master, my mind was not my own, my body was exposed to its doings, its evil, unmentionable doings. The pain exploded, but didn't die; it imploded but didn't decrease; it ignored the laws of nature that it should have abode by.

The day was long, the next day was long, and the day after was long. Three days and three nights of this insane explosion of sickness and hate and despair and the dark and no one else there but I. I was utterly alone, utterly abandoned by all logic and reason.

Distractions that previously amused me, meant nothing to me. I watched TV, but the characters on the screen moved and talked but that was all they did. What they said and did was funny, but I didn't laugh because that would encourage the pain and I would never help it, I allowed it because what could I do but allow it? I was powerless, under the control of this stranger who knew everything there was to know about me and exposed my secrets to myself for their amusement. Pain was not explained or even believed, but it was there, a meteor about to crash into the earth but ignored, a surfer on a surfboard ignorant of the shark behind them, a tragedy of which rest was unknown.

I lay there, powerless, enduring when I didn't want to endure, wishing the pain to end, but there was no genie to grant my wish, there was just I, mum and the pain, and we waited, through the endurance, waited for the first sign of change.

It rejoiced in its achievement, after so much time of having to wait, finally there was a reward. But it tired, tired as it was a cheetah that had failed in its hunt to catch a gazelle and could not keep up the race, while its prey endured the track and was a long distance runner, it was a sprinter who had lost their stamina. The race goes on and on but the hunt ceases before long. The salty rain poured, the thunder of laughter rolled in the sky, the lightning stabbed and the sky greyed.

Finally, after a millennia the crisis ended. The pain eased. The despair was replaced with hope. My Saviour had come. My prayers had been heard. I was not alone. My endurance was replaced with rest and my mind was my own. Change had come. **Naomi Carter**

For Sale!

Maclaren buggy with rain cover, basket (clips on underneath) and a padded seating cover (as shown in smaller picture). Seller will pay to courier so no cost at all. For more information please phone **01590 688014**

Large special needs trailer, made in Canada by Wike. They can take one or 2 children. £300 but open to offers. http://www.wicycle.com/special_needs_large_bicycle_trailer.php For more information please phone **01590 688014**

Clinical Trial Update

For full information about the trials listed here, please visit **www.mpssociety.org.uk** and click on the relevant diseases tab.

Clinical Trials that are currently recruiting

These clinical trials are active and recruiting patients. If you have any questions about clinical trials, please contact your clinician, the clinical principal investigator or the MPS Society.

MPS I

Intrathecal Enzyme Replacement for Hurler Disease
Laronidase (Aldurazyme TM) Enzyme Replacement Therapy With Haematopoietic Stem Cell Transplant for Hurler Disease

Pilot Study of Administration of Intravenous Laronidase Following Allogeneic Transplantation for Hurler Disease

A Study of Intrathecal Enzyme Therapy for Cognitive Decline in MPS I

A Study of the Effect of Aldurazyme® (Laronidase) Treatment on Lactation in Female Patients With Mucopolysaccharidosis I (MPS I) and Their Breastfed Infants

MPS II

Collection and Study of Cerebrospinal Fluid in Patients With Hunter Disease

Observational Study to Evaluate Neurodevelopmental Status in Paediatric Patients With Hunter Disease (MPSII) - HGT-HIT-090

Biomarker for Hunter Disease (BioHunt)

MPS IIIB

Natural History Study of Patients With Mucopolysaccharidosis Type IIIB (MPS IIIB, Sanfilippo Disease Type IIIB)

MPS IVA

Discovering New Biomarkers For Monitoring Disease Progression in Patients With Mucopolysaccharidosis IVA

Efficacy and Safety Study of BMN 110 for Morquio A Syndrome Patients Who Have Limited Ambulation

MPS VI

Mucopolysaccharidosis (MPS) VI Clinical Surveillance Program (CSP)

Fabry

Open-Label Phase 3 Long-Term Safety Study of Migalastat (MGM116041)

Canadian Fabry Disease Initiative (CFDI) Enzyme Replacement Therapy (ERT) Study

Natural History and Treatment Outcomes(Changes) in Fabry Renal Disease Study (LDN6702)

Stroke in Young Fabry Patients (sifap2): Characterization of the Stroke Rehabilitation

The Efficacy and Safety of Switch Between Agalsidase Beta to Agalsidase Alfa for Enzyme Replacement in Patients With Anderson-Fabry Disease (SWITCH)

Establishment of Biomarkers for Fabry Disease

Immune Response in Subjects With Fabry Disease Who Are Switching From Agalsidase Alfa to Agalsidase Beta

Sophisticated Assessment of Disease Burden in Patients With Fabry Disease (SOPHIA)

Fabry and Cardiomyopathy (FaCard) Epidemiological Study for the Analysis of Biomarkers and the Clinical Course of Patients With Fabry Disease and the N215S-mutation

A Study of Renal Function in Treatment-naïve, Young Male Patients With Fabry Disease

Clinical Trials that are active but not currently recruiting

MPS II

Extension of HGT-HIT-045 Evaluating Long-Term Safety and Clinical Outcomes of Idursulfase (IT)in Conjunction With Elaprase in Paediatric Patients With Hunter Disease and Cognitive Impairment

MPS III

Extension of Study HGT-SAN-055 Evaluating Administration of rhHNS in Patients With Sanfilippo DiseaseType A (MPS IIIA)

MPS IVA

Long-Term Efficacy and Safety Extension Study of BMN 110 in Patients With Mucopolysaccharidosis IVA (Morquio A Syndrome)

Study of BMN 110 in Pediatric Patients Under 5 Years of Age With Mucopolysaccharidosis IVA (Morquio A Syndrome)

Safety and Exercise Study of BMN 110 for Morquio A Syndrome

cont...

Fabry disease

Study of the Effects of Oral AT1001 (Migalastat Hydrochloride) in Patients With Fabry Disease

Study to Compare the Efficacy and Safety of Oral AT1001 and Enzyme Replacement Therapy in Patients With Fabry Disease

Open Label Long-term Safety Study of AT1001 in Patients With Fabry Disease Who Have Completed a Previous AT1001 Study

Enzyme Replacement Therapy Clinical Trial for MPS I Patients Post HSCT

Since the last MPS Magazine the parents of a handful of children with MPS I who have undergone a bone marrow transplant or Cord Blood Transplant have expressed an interest in their child participating in a two year clinical trial to look at the benefits of treating pre-pubertal children who have undergone HSCT for Hurler disease. The clinical trial principal investigator is Dr Paul Orchard and the trial will involve travelling to the University of Minnesota in the United States five times for assessment whilst receiving enzyme replacement therapy provided by the NHS.

Patients who meet the clinical trial criteria have been accepted on to the clinical trial on a first come basis as we only have three places. We hold a small waiting list of patients.

As this clinical trial does not have a commercial sponsor, the MPS Society working with its members is committed to raising the funds to support the three UK children recruited to this clinical trial.

Genistein Trial Update

The UK MPS Society awards first grant of £160,000 to the University of Manchester to fund a 'Phase III Double-Blinded, Placebo-Controlled Clinical Trial of High Dose Oral Genistein Aglycone in MPS III, Sanfilippo Disease'

In 2012 in collaboration with Dr Brian Bigger of the University of Manchester, the MPS Society began fundraising £650,000 to fund a clinical trial of high dose oral Genistein Aglycone in Sanfilippo disease. Although originally the cost of the trial was £800,000 the MPS Society working with Dr Bigger was able to reduce the cost of the trial to £650,000.

To date, the MPS Society with the amazing support of a small number of affected families in the UK, the National MPS Society in the USA, grants pledged from two trusts and contributions being raised by the MPS Societies around the world, we are guaranteed to raise £450,000.

With only £200,000 to go, the UK MPS Society is therefore delighted to announce we have awarded the first grant of £160,000 to the University of Manchester to begin the first stage of the Genistein clinical trial, recruitment for which begins this autumn. In starting the trial whilst still securing the remaining funds, it is recognised that children with Sanfilippo do not have time on their side.

This clinical trial has come about because of dedicated funding and support of pre-clinical research on Genistein and Sanfilippo disease over the past six years funded primarily by the MPS Society in the UK.

On behalf of the Board of Trustees, the MPS Society would like to thank its members, Share A Gift and the National MPS Society in the USA for their support in enabling us to start this important clinical trial.

There is still much to be achieved and the MPS Society and its members are committed to continue fundraising for the remaining £200,000. It is anticipated that further awards will be made as fundraising continues.

To make a donation directly to the Genistein Appeal via the MPS Society's website please visit http://www.mpssociety.org.uk/research/research-appeals/mps-iii-genistein-clinical-trial/

Icahn School of Medicine at Mount Sinai and Bene Pharmachem GmbH to collaborate on clinical studies for Mucopolysaccharidosis

On 15 May 2013, MPS Awareness Day, the Icahn School of Medicine at Mount Sinai, New York signed an agreement with Bene Pharmachem GmbH to collaborate in conducting clinical studies of Pentosan Polysulfate (PPS) in patients with Mucopolysaccharidosis (MPS). Bene Pharmachem is based in Germany and is the sole producer of Pharmaceutical grade Pentosan Polysulfate.

The original discoveries demonstrating the use of PPS for the treatment of MPS and the lysosomal storage diseases were made by Dr Simonaro in laboratories at Calogera at Mount Sinai. Dr Simonaro and Dr Edward Schuchman have demonstrated in MPS animal models that PPS has the effect of reducing the chronic inflammation associated with substrate accumulation resulting in improved mobility and slowing of bone / cartilage disease.

The effects were superior to other anti-inflammatory drugs. Their study, "Pemtosan Polysulfate: A Novel Therapy for the Mucopolysaccharidosis" was published by PLOSOne on January 24, 2013.

(http://www.plosone.org/article/info%3Adofi%2F10.1371%Fjournal.phone.0054459).

FSIG - Gene Therapy Fabry Stem-Cell Trial Launched

By Melissa James, FSIG Contributor

Canadian researchers have begun the world's first gene therapy clinical trial for Fabry. Promising gene therapy results in mice performed in the laboratory of Dr Jeffery Medin at the University Health Network in Toronto prompted this clinical trial.

The study, based in Toronto, will rely on expertise from Calgary doctors and scientists, plus the cells of Canadian citizen Chris Armstrong, 34, one of only 400 in the country known to suffer from Fabry.

Armstrong was diagnosed with Fabry in 2007 and recently donated his blood for important experiments for the first phase of the trial.

How the trial will work:
1. Researchers first remove a quantity of stem cells from Armstrong's blood
2. A working copy of a new gene will be inserted into the stem cells using a specially engineered virus
3. Researchers transplant these stem cells back into Armstrong and the new, working copy of the gene will make the missing enzyme.

Nearly a billion of Armstrong's CD34+ cells were isolated and will be sent to the lab in Toronto.

"We use a type of virus called a lentivirus, which has been modified in a couple of critical ways," Dr Medin told the media. "First, it's been stripped of any of its disease-causing capability and is safe. Second, it has the corrected functional GLA gene, which will cause the donor cells to make the correct enzyme. When the corrected cells circulate in the blood, they also secrete the enzyme, which is then taken up by unmodified cells. This effectively extends the therapy afforded by the modified stem cells."

Armstrong is currently receiving enzyme replacement therapy, which requires a home care nurse to visit his home every two weeks to administer the infusions. The disease has left Armstrong with a thickened wall in his heart.

"Even if the research meant that I had to come in once every six months for Enzyme Replacement therapy, instead of every two weeks, then it would still be another step toward living a normal life," Armstrong said.

"We hope this will one day be a form of treatment that effectively cures Fabry disease," Dr Aneal khan, a medical geneticist who is leading the Calgary segment of the national project, said in a press release.

Khan said if successful, the method could save Fabry sufferers from needing frequent enzyme replacement procedures and save the health system a lot of money.

"We hope it's a one-time transplant," he said.

The team hopes to treat the first human Fabry disease patient after several phases of the trial are complete and the pre-clinical experimental results have satisfied the regulatory requirements of Health Canada. They estimate that will happen within two years.

Learn more and watch Chris Armstrong in a video: http://calgary.ctvnews.ca/medical-trial-targets-cure-for-fabry-disease-1.1128320#ixzz2J1U1EiBZ

BioMarin Submits Vimizim MAA to EMA for the Treatment of MPS IVA

SAN RAFAEL, Calif., April 24, 2013 (GLOBE NEWSWIRE) -- BioMarin Pharmaceutical Inc. (Nasdaq:BMRN) announced today the submission of a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for Vimizim (BMN-110, elosulfase alfa), an enzyme replacement therapy under evaluation for the treatment of patients with the rare lysosomal storage disorder Mucopolysaccharidosis Type IVA (MPS IVA), also called Morquio A Syndrome. A Biologics License Application (BLA) for Vimizim was submitted to the U.S. Food and Drug Administration (FDA) in March 2013.

"The submission of this application to the EMA represents another notable milestone in our efforts to bring the first therapeutic option to patients with MPS IVA worldwide," said Jean-Jacques Bienaimé, Chief Executive Officer of BioMarin. "MPS IVA is a serious debilitating disease with no treatment options. We hope to leverage our expertise in developing enzyme replacement therapies to introduce a life-altering therapy and change the course of the disease."

Mr. Bienaimé continued, "Earlier this year, the EMA accepted our request for accelerated assessment for this MAA based on the premise that Vimizim could satisfy an unmet medical need and is of major interest from the point of view of therapeutic innovation and public health. We look forward to working with the European regulatory authorities in the coming months to bring this therapy to patients in need."

Ultragenyx Advances Clinical Development of UX003 for the Treatment of Mucopolysaccharidosis Type VII (MPS VII)

NOVATO, California – May 15, 2013 – Ultragenyx Pharmaceutical Inc., a biotechnology company focused on developing treatments for rare and ultra-rare genetic disorder, has announced a phase 1 / 2 study of UX003 for MPS VII or Sly disease.

UX003 is a recombinant human beta-glucuronidase intended as an enzyme replacement therapy (ERT) for the treatment of MPS VII, an extremely rare autosomal recessive Lysosomal storage disorder characterised by deficiency of the Lysosomal enzyme Beta-glueuronidase and severe multi-system disease. MPS VII has no approved therapies.

"Extensive research has been done on MPS VII for 40 years and unfortunately it is one MPS left behind," said Emil Kakkis, MD, PHD, Chief Executive Officer of Ultragenyx. "We are pleased to announce the first clinical study of MPS VII on International MPS Awareness Day. Our collaborations with Dr William Sly and his colleagues at St. Louis University continue to advance development of this urgently needed therapy, using innovative and creative approaches."

"I am pleased with the progress that Ultragenyx has made with UX003 programme." Commented Dr Sly. "After many years of research, we are getting one step closer to finding a potentially life changing treatment for patients with MPS VII."

Ultragenyx Pharmaceutical Inc.
www.ultragenyx.com

Morquio Registry - Scientific Summit

On the 17 – 18 May experts from around the world including Dr Simon Jones, Kathryn McBride and Dr Chris Hendriksz gathered in Miami for an interactive workshop to contribute to towards developing a refined informative MPS IVA registry. I was privileged to join this group to share my experiences from a patient perspective.

It is funny how things go. Two weeks previously ten parents of Morquio children and five adults with Morquio gathered in Coventry to participate in their own workshops giving their own perspective on the natural history of Morquio disease. I was immensely grateful to have sat in on the adult workshop and had no idea how useful the learnings of that weekend would be when participating in the Quality of Life workshop at the Miami Meeting. The one word I took away from Coventry and passed on in Miami was PAIN.

Christine Lavery
Chief Executive

Morquio Get Together 5 - 6 May 2013

On behalf of BioMarin, the Society was asked to arrange a focus group meeting to look at different perspectives of Morquio disease from both an individual's and family viewpoint. This was to include two separate meetings, one for individual adult sufferers and one for parents of children with Morquio disease. The meetings were to be closed meetings, held over two days, focusing specifically on the following:

- Understanding the patient/family perspective of living with Morquio disease
- Discussion on the differing symptoms and impact of Morquio disease on individuals/ family
- Review of sessions and discussions on management of the condition.

The meeting numbers were restricted and were independently facilitiated by an outside company.

As the meetings could not allow children to attend, the Society arranged activities for the children during the day.

On the Sunday; circus workshop skills – the children were allowed to sample many circus props with a view to presenting their new skills to all the parents at the end. This included plate spinning, stilt walking, juggling, stick balancing, dressing up, hoop spinning and ribbon work. The talent ran high, as did the laughs.

To allow parents to meet together socially we also took the children out in the evening to the local pizza hut followed by bowling.

On the Monday, the children all had the opportunity to learn how to decorate their own cupcakes using icing and fondant and were presented with a box to take their cakes home with them.

International News

FIN Fabry Expert Meeting, 26th – 28th April 2013

The primary aim of the Fabry International Network is to facilitate collaboration between Patient Organisations around the world to support those affected by Fabry Disease.

The Fabry International Network (FIN) which represents over fifty Patients Organisations around the world held its annual Fabry Expert Meeting at the Steigenberger Hotel, Frankfurt from 26th to 28th April 2013.

Christine Lavery, FIN President, welcomed representatives of fourteen Patient Organisations, representatives from the Pharmaceutical Industry, members of FIN Medical Advisory Board, guest speakers and Fabry Patients to the weekend conference that started with a welcome dinner on the Friday night, followed by a one day Fabry Expert Meeting and a Gala dinner on the Saturday. On Sunday FIN held its AGM meeting for 2012.

The objective of the FIN Fabry Expert Meeting in Frankfurt was to enable Physicians to share their experience and advice with representatives of Patients Organisations on the challenges to the diagnosis, management and support of patients with Fabry Disease.

Professor Atul Mehta and Dr Uma Ramaswami co-chaired the meeting. Dr Gabor Linthorst from the Academic Medical Centre in Amsterdam presented the practical considerations of screening and diagnosis of Fabry Disease. Dr Peter Woolfson, a prominent Cardiologist from the Salford Royal Hospital described the characteristics of a Fabry Heart. The current evidence of the long-term efficacy of the ERT in Fabry Disease has been highlighted by Dr Tarek Hiwot a Consultant in Inherited Metabolic Disorders at the University Hospital of Birmingham. Other presentations at the FIN Fabry Expert Meeting included: The Renal involvement in Fabry Disease by Dr Sandro Feriozzi (Italy), Early ERT in children by Dr Rossella Parini (Italy), Kidney and heart in paediatric Fabry Patients by Dr Uma Ramaswami, Consultant Metabolic Paediatrician at Manchester University Hospital and the reimbursement challenges for patients by Christine Lavery (MPS Society UK) and Lut De Baere (Boks, Belgium).

Other guest speakers shared their own personal experiences with Fabry Disease such as Jack Johnson from the Fabry Support and Information Group in the US or Anna Meriluoto from the Fabry Association in Finland and Erica Schenk from the Fabry Support and Information Group in the Netherlands. Both Anna and Erica described the challenges of Fabry Disease in women.

The Fabry International Network Board of Directors would like to thank all the participants and guest speakers for their valuable contributions and for making the conference weekend in Frankfurt a successful event.

For more information about FIN, please go to www.fabrynetwork.org

Megan Fookes resigns as President of Fabry International Network

Megan Fookes has been an active member of the Fabry Community for a number of years and has played a major role in developing and leading Fabry Support Group Australia.

In 2010, Megan was elected to the Fabry International Network Board and almost immediately took on the secretarial role where she had regular contacts with many Fabry Member Organisations. In 2011, Megan took over as President at FIN and brought considerable vision and forward thinking to the organisation. This has led to FIN appointing a Development Officer to provide a dedicated and continuous support to a growing membership.

In the past year Megan has put many things on hold including her family to meet the demands of FIN. It is with regret that Megan has decided to step down as President and resign from the FIN Board in order to take up a new role as the Director of Rare Voices Australia and to focus on her young family.

Following on Megan's resignation and with the unanimous support of the FIN Board of Directors, Christine Lavery is now the acting President of the Fabry International Network. **Nawel Van Lin** Global Development Officer Fabry International Network

Eurodis Members Meeting Dubrovnik, Croatia
31 May – 1 June 2013

The Eurodis Members Meeting is open to over 600 rare disease groups across Europe and provides a valuable platform to meet patient organisations that we may not usually gravitate towards.

During my time in Dubrovink I contributed to a workshop on 'Medicines, Side Effects and Patient Safety' where I spoke on the impact of the Fabrazyme / Cerezyme shortages and the lessons learned'. Although this difficult period for Fabry patients is over I was shocked to hear from François Houÿez, Eurodis, that shortages of drugs is a common problem. **Christine Lavery** Chief Executive

Fundraising

The posters are coming down, the balloons have deflated, the blue feather boa has gone back in the cupboard; MPS Awareness Day 2013 has past – but we don't want you to be blue!

Whether you didn't get chance to Wear it Blue or you gave the Smurfs a serious run for their money, we are brimming over with great fundraising ideas for you to keep the MPS Society flag waving through the Summer, let's keep the momentum going.

Get your families and friends out in the sunshine, organise a **Wicked Walkabout** and raise awareness and funds for people affected by MPS and related diseases. The McCauley family organised the first MPS Society Wicked Walkabout, Kevin Mc Cauley said; 'The walk was held in our hometown of Derry. On the day of the walk the sun was shining and we couldn't have wished for a better day.'

Contact us now for a Wicked Walkabout Guide, a step by step booklet to organising a safe, fun Wicked Walkabout

Perhaps you are looking for a way to motivate colleagues at work, or get your school involved in some Wicked fundraising. We have our MPS Wicked Fundraising guide will lots of hints, tips and ideas (ranging from fairly standard cake sales to the utterly bizarre - sponsored Wicked wax anyone?).

Quizes, sponsored runs, dress down days, coffee mornings, star studded ball - every penny counts and helps the MPS Society support individuals and families affected by MPS and related diseases, and to continue to fund vital research and raise to awareness.

Did you know that it is thanks in part to funding from the MPS Society that the labs have now developed 5 treatments for the 24 diseases supported by the MPS Society? We have come a long way and there is still so much we know we can do, but we need your help.

The MPS Society has been around for 30 years and is entirely dependent upon voluntary donations and fundraising to continue its vital work as the only UK charity supporting the MPS and related diseases community. We aren't well enough known yet, please help us change that.

Contact us now:
wickedgenes@mpssociety.org.uk

MPS Awareness Day
15th May 2013

How you helped us mark International MPS Awareness Day

MPS House goes Blue
I got into the office early to hang the balloons, streamers, posters and get out the blue food. (It did feel a little strange the evening before walking round the supermarket purchasing only food that came in a blue packet).

MPS staff didn't disappoint, we had everyone top to tail in blue - The Smurfs in Amersham. Some lovely blue nails, lipstick and eye shadow and some beautiful jewellery and accessories, although while the shower cap and goggles were blue, I'm not sure they actually qualified in the 'beautiful blue accessories category'.

Sophie Thomas (Senior Advocacy officer) arrived laden with home-made, beautiful blue iced cakes to go on the blue food table. Everyone dug deep and made donations for their blue attire and to help themselves to 'blue nibbles' and combined with the money raised from a recent office weight loss challenge we raised £63.32 - a great effort all round! **Laura Burrows**

Dave Peach and colleagues at GE Power Conversion in Rugby held a Wear It Blue day including a cup cake sale, raising £631 for the MPS Society! Thank you to everyone who took part.

Hannah Cooper (MPS IH post BMT) and her little sister Emily Cooper both attend Raynsford Church of England Academy, in the village of Henlow.

Hannah and Emily had been busy selling MPS. Awareness ribbons to friends and family. Their school, Raynsford Church of England Academy, fully embraced their fundraising efforts and held a "Wear it Blue Day" on Wednesday 15th May. The children sold MPS. ribbons a few days before the event and both children and staff wore blue accessories as well as their MPS Ribbons on "Wear it Blue Day". Mrs Wallis even made blue cupcakes for the staff to purchase in the staff room at break time! Thanks to everyone's massive support we managed to raise £263.81! Well done everyone!

Mucopolysaccharide Diseases were first brought to my attention when a baby girl named Anabelle (who featured on the front cover of the Spring 2013 magazine) that my mum child minded, was diagnosed with MPS I Hurler syndrome in 2012. It is heartbreaking to have to say that she passed away at the end of the year, but in the time that I knew her I learnt so much about the disease and the lack of public awareness regarding it. This year when I found out about MPS Awareness Day (Wednesday 15th May) and the Wear it Blue campaign I was eager to get involved. After e-mailing the Society and receiving a lovely and encouraging reply, I spoke to the Deputy Head Teacher of Priory School, where I am a Year 11 student, and as there were already plans in place for a Blue Day in support of the Tom Prince Cancer Trust we agreed to make the day a joint fundraiser, raising awareness for two charities in one day. Our Blue Day was very successful, with one student contributing £20 because he wanted to give more money to the MPS Society! I am so grateful for every penny that was donated to raise a wonderful £490 for a cause so close to my heart. I would encourage anyone who is able to organise an awareness event of their own, to help those with MPS and related diseases in the fight for their lives. **Charlotte Russell**

Employees at **Genzyme Oxford** raised £210 by holding a Wear It Blue day.

Hayley Thornley and the Greater Manchester Police donated £40 following a collection for MPS Awareness Day

Jane and her daughter Ellen organised a coffee morning held on 11th May at the High Street Methodist Church, Witney, raising £82.50 for MPS. They hope to do another one later in the year

Teresa Jeffery and her son Corey (MPS I) raised £344.67 on her justgiving page

The children of pre-school to year 6 and even their teachers at **Riverston School** in Lee Green, SE London had a wicked time raising money for MPS by dancing their socks off to tunes by Willow Smith, Shakira, One Direction and Michael Jackson! They all felt very proud to have raised so much money for this fantastic charity. Dylan Tonge is one of the pupils.

Aisha Seedat (MPS IVA) held an awareness day at her college whereby all her support staff took part.

Aisha's uncle and aunt (Junaid and Raihana Seedat) who live in Malawi also wore blue and tried to create awareness. Aisha's cousins Sufyaan and Muhammed (from Malawi) did their special bit too.

Aisha continues to raise awareness and collected funds through her justgivingpage www.justgiving.com/aisha-seedat

Her total to date collection is now at £1747.00.

Here are a selection of some of the photos you sent us from MPS Awareness Day. Thank you!

Katrina Fanneran Burley raised over £6000 for the MPS Society in one week by asking everyone to wear blue for MPS Awareness Day 2012.

Katrina wrote on facebook: 'It was wonderful! I didn't have much time to organise it so took to social media and the response in support was outstanding. It felt like a celebration of my son which made me feel great too. Spreading awareness (and raising vital funds) like this is so easy. Wishing everyone the best of luck for 2013.'

Building on the success of last year's campaign, Katrina asked for donations to her existing Justgiving.com page, www.justgiving.com/ethansmps, for the 2013 MPS Awareness Day which has now reached £13,573.08.

Thank you to Katrina who also arranged for Sky Sports News presenters Millie Clode and Tom White (pictured above) to Wear It Blue for the MPS Society. Thank you to Millie and Tom!

Arriva Wear It Blue on the buses!

After the success of last year's non uniform day for MPS Awareness, since mid February I had quite a number of members of staff coming up to me and asking if we were going to be dressing up or down for the day in May. At the time, my reply was I hope so. After speaking with our new boss it was agreed that once again we could do our day's work without uniform or in fancy dress, with collection boxes on the buses.

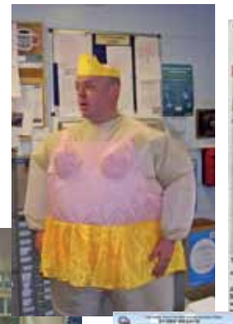
Pretty soon most of the drivers were talking about the day. Some of them asked if there was anything that they can do to promote the day even more. Our boss (Matthew Ashton) agreed to having posters on the buses. These were put on at the start of May and are as I write still on the buses. Then he told me that Dennis Mauger was planning something last year. Dennis spent the day dressed as Elvis. I went to see Dennis but all he told me was it's going to be better every year.

All I needed to do now was to get some prizes for the things that I was planning and sort out all the paper work. One of the prizes was once again very kindly donated by Louise and Constantine from the Hampden Arms in Great Hampden. Antonia and Laura from the MPS Society very kindly bought the collection boxes, posters and leaflets to the depot as they had come to Aylesbury to see the advertising boards that Arriva had given to the Society to be put on the back of buses. There have been many other offers of help which include a local post office also having a non uniform day.

The morning of the 15th came round and as my alarm clock woke me at 0345 I wondered if the day would be as much of a success as it was last year. I got to work at 0445 and waited to see what was going to happen. Lots of members of staff gave donations, having a go at the quiz, trying to guess how many rice puffs were in the bottle. Not too many fancied the cream cracker challenge but there were some. Dennis and his wife Janet came in dressed as airline pilot and cabin crew with the uniforms all made by Janet. During the morning there was a clown, avatar, cowboys. Some had blue shirts on, some just their normal clothes. One man had painted his head, hair and beard blue and had been collecting donations for most of the week. At around 1100 I went to the bus station and spent the day handing out leaflets and talking to the public with Terry (the man who had painted his head blue). He had also arranged for Bucks Council to display what was happening on the departure boards in the Bus Station,.With this and the MPS Society arranging for the local newspaper and radio station to be involved, many many members of the public gave donations to drivers on the buses and to us in the Bus Station.

I stayed at work untill around 2000 collecting the boxes in as the drivers finished then counting the money. From work I went to the Hampden Arms for the MPS Awareness Day quiz. Finally getting home at 2300. Over the next few days I collected all of the boxes and the donations from the wonderful work that had been done by Dennis and Janet. With all of the donations we have currently raised about £1800 and there are the promise of more donations and people at work wanting to do things to help the Society. I would like to thank Matthew Ashton ,Terry Dumpleton, Dennis and Janet Mauger and all members of staff at Arriva Aylesbury who helped me with the day or took part in the day.

Also thank you everyone at the Society without you, life would be so different for all of us. **Ian Evans**, father of Harry (MPS I Hurler)



Thank you to everyone who kindly supported our Wear It Blue campaign for MPS Awareness Day 2013...

Dr Victoria Powers raised £52.42 from a cake sale for MPS Awareness

Natasha Mitchell at Relish Events who did an office whip round raising £20

Sonia Lozano at the Speech and Language Therapy Department at Great Ormond Street donated £28 from their Wear It Blue day

Richard Cross donated £5 for MPS Awareness Day Wear It Blue

Elizabeth Heath's employer donated £5 for Wear It Blue, MPS Awareness Day 15th May

Ann Thompson donated £5 for the Wear It Blue day

Riversdale Surgery raised £60 on their Wear It Blue day event

Claire Garthwaite held an MPS Awareness Day walk and raised £210

Ashley Chattin, whose neice Sienna Taylor has MPS I Hurler, raised £83.35 from a Wear It Blue day on 15th May. Her employer, Rentokil Initial, kindly matched the money raised, making the total donation £167.70

The Hampden Arms pub held an Awareness Day quiz raising £116 for the MPS Society

Advanced Interior Solutions in High Wycombe raised £11 by wearing it blue

Argosy Components Ltd raised £43 by Wearing It Blue for MPS Awareness

Gail Tickle who kindly donated £2500 in support of MPS Awareness Day.

The **Northern Ireland Regional Genetics Service** held an Awareness Da coffee morning raising £171 for MPS.

Wrightbus Hybrid Dept. raised £30.50 for MPS Awareness Day.

Allens Croft Children's Centre held a fundraising day raising £102 for the MPS Society

Peter and Barbara Pollard donated £1000 in lieu of MPS Awareness Day

Robert Spencer raised £93.72 being the proceeds from a Wear It Blue collection at his office

Faizal Seedat donated £50 for MPS Awareness Day. His niece has MPS IV

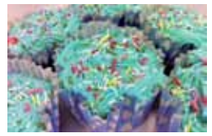
Barclays Corporate Team took part in our Wear It Blue day raising £86

Lloyds TSB, Spring Gardens in Manchester have raised £138 at their Wear It Blue event

WTCRF and ICH at **Birmingham Children's Hospital** donated £41.06 for MPS Awareness Day

Holly Nowell donated £125 as a contribution towards MPS Awareness Day

The **Robert Allen Group** in High Wycombe took part in our Wear It Blue day by wearing blue and baking cakes. They raised £37



Sandra Irvine (aunt of Nadine Murty MPS I) and colleagues at Cordia Encore Catering took part in Wear It Blue on 15th May raising £110

Find us on Facebook
www.facebook.com/mpssociety

twitter
twitter.com/MPSSocietyUK

MPS Trustee, **Jessica Reid**, held a Blue cake sale at her work Blandy and Blandy LLP raising £94.28. Thank you to Jessica and her colleauges for supporting this event

BioMarin held a Wear It Blue event raising £100 for the MPS Society

Deborah Thorburn, John and Sharon Hattle donated £500 on MPS Awareness Day

Year 4 pupils at **Fernwood Junior School**, Nottingham held a Wear It Blue day and donated £103.65

Donations from Tanya Jane Carter; Sarah Long; Jean Mercer; Lynn Brodie

Will you be part of our Wicked Walkabout?

You'll all be familiar with other big charity events such as coffee mornings, races and red noses. Well, we thought it was time we had our own and so we launched the MPS Wicked Walkabout this Spring and we are delighted to announce you have already held several which have raised funds and awareness and featured in local papers across the UK.

What is a Wicked Walkabout?

It's up to you! A Wicked Walkabout can be a gentle walk, a run or a cycle. We recommended a minimum of 5km, but that is just guide and it could be longer (or shorter if it's a Wicked Wellie Waddle!). The Wicked Walkabout is about increasing awareness and fundraising for those affected by MPS and related diseases. It may be you set a minimum sponsorship for people to enter your event or perhaps charge an entrance fee. It's your event, your choice!

What now?

If you think you would like to come together with family and friends and hold a Wicked Walkabout near you, you can use the poster we have enclosed with this MPS Magazine as a guide. It really is simple and good fun; just ask Kevin, Christine or Wilma who have all held Wicked Walkabouts

"On the day of the walk the sun was shining and we couldn't have wished for a better day." Kevin McCauley, organiser of the Derry Wicked Walkabout

"It was easy to arrange, great fun and there was a wonderful atmosphere. We'll definitely do it again next year" Christine Lavery, organiser of the Chess Valley Wicked Walkabout

You can also email **wickedgenes@mpssociety.org.uk** for a guide on how to hold a Wicked Walkabout if you need a bit of inspiration or look on our website www.mpssociety.org.uk

Eamonn, Michelle and Nathan Worsford organised a Wicked Walkabout raising £670 for the MPS Society.

Derry Wicked Walkabout

Hi my name is Kevin McCauley. On Saturday 6th April we held our first charity walk for the MPS Society. Our Wicked Walkabout was to help raise funds and awareness for MPS and to show support to our daughter Chloe now 16 who has MPS I.

The walk was held in our hometown of Derry. On the day of the walk the sun was shining and we couldn't have wished for a better day.

James Stewart who also has MPS and lives in Derry attended the walk with his family and we were delighted that James started the walk off for us.

Also attending the walk were our dear friends Michelle and Eamonn who travelled the whole way from Newry to take part in the walk. Michelle and Eamonn's beautiful boy Nathan also has MPS.

Overall about ninety family and friends came out to help raise funds and awareness for MPS. The walk ended once we had all crossed the Peace Bridge, where we then had a tea or coffee and were able to catch up with one another.

With the efforts of family and friends we raised £1065 but more importantly we helped raise awareness about MPS.

I would like to say a special thank you to all the people who took part, to James for starting the walk and to our friends Michelle, Eamonn and Nathan from Newry. Thank you for all your support.

Chess Valley Wicked Walkabout

A Sunday in May, you'd hope for warm weather, or at the very least dry. But it was not to be for our Chief Executive; Christine Lavery and MPS trustee Wilma Robins' first Bucks Wicked Walkabout. Did it make any difference – no it did not, it was great fun.

MPS House staff and families as well as local families met at the Wicked Walkabout starting point in Westwood Park, in Little Chalfont (Highlighted with a very smart Wicked Walkabout start sign!) and set off through the Chess Valley. Each walker was armed with a local quiz with questions to be researched on route. Ranging from 8 months to 91 years old and from two-legged to four legged participants, the event included a range of participants.

The first stop was St Michael's Church at the beautiful Chenies Manor, where Christine and Wilma had kindly arranged tea and cake. After a welcome pit stop the walkers set off, answering questions along the way about local sights heading towards the picturesque Chenies Village and over the Chess river. The walk included some beautiful scenery and even the slightly muddy fields didn't dampen the spirits. (There were a few piggy backs!)

Roughly two hours later, the team of walkers had completed the circuit to arrive back at the start, tired, but happy and ready to head home.

Christine and Wilma had made a terrific effort in organising the walk, ensuring everyone had a great time and got around the course safely. The local magazine did a short article about the MPS Society and the Wicked Walkabout and we are delighted to announce that Christine and Wilma's first Wicked Walkabout raised over £1,000, a huge thank you to everyone involved.

Chess Valley Wicked Walkabout 2014 here we come....

Events and Challenges

Thank you to everyone who took part in the Virgin London Marathon 2013 in support of the MPS Society. Here are your stories...

My name is Dan Colbourne and I ran in the Virgin London Marathon this year for MPS.

When I received my acceptance I was very excited, but I was also dreading it as it was my first marathon!
I had some strange dreams and even nightmares in the early days!

The training was gruelling and the long runs were very tough, especially with all the cold and rotten weather we had at the beginning of the year. As time went by I got stronger and more confident, then with 5 weeks to go the worst thing happened, Injury! I injured my Achilles and had to take 3 weeks completely off. I missed my longest three runs and I was worried I would not make the big day!

When I returned to training I was only allowed to run short distances and at 'marathon pace', hills were out too. Nevertheless I had the ambition to complete the marathon and I already had a fair few quid raised in sponsorship, so I went for it!

The day and the crowd were amazing and I set off exited about seeing London and being part of this massive event. I ran a good first half but I can definitely say I hit the wall in the second half, this was around mile 18. I had to walk / run from here as I just had nothing left, I felt sick and I was on an emotional rollarcoaster.

However with some help from friends, family and the awesome spectators I did manage to finish! It was an amazing moment and I enjoyed a nice cold pint with my nearest and dearest before I seized up. I must also be hooked as I have entered next year's ballot again!

I feel I need to do another as I was not happy with my performance.
I do feel that if I was not injured I would definitely of hit my target. I was aiming for 4 hours and I was on target up to mile 18, my finishing time was 4:42:55 in end.

In total I raised £1018 for this excellent charity. The Society for Mucopolysaccharide Diseases is a special charity to me, as my little cousin Cody has the disease. I have seen how much the Society has helped Cody and her parents. Keep up the good work.

Sophie's London Marathon

After having run a number of half marathons, I decided that 2013 was going to be the year of the marathon! My younger sister, Faye, suffers from Morquio's so naturally I decided to run for the MPS Society in the hope that other MPS sufferers and their families can continue to receive the support that we have received over the years.

I have to admit, the training was really tough! I started training properly in January and soon realised that I had not chosen a good year for it; the weather was horrendous, almost every training session was done in the bitter cold and sometimes even in snow. There were many occasions when I wondered what I had let myself in for and moments where I never wanted to see a pair of running trainers again but the donations and kind messages of support really spurred me on and reminded me why I had decided to run the marathon. In the end, I managed to raise about £1300 which I was really pleased with. It's such a great feeling to know that people care.

Miraculously, on the day of the marathon, the sun finally decided to come out. It was an absolutely fantastic day and I loved (almost) every minute of the race. The crowds were unbelievable and cheered me on the whole way. I managed to finish in 4 hours and 8 minutes – not too bad for a first attempt. For anybody thinking about taking part this year all I can say is 'go for it'... it really will be worth it!
Sophie Longley

Hannah's London Marathon

21st April 2013 – a date that has been etched on my mind for a year – and here it was, waking up at my old uni housemate's sitting room floor after a night at the pub catching up with old friends – (yes they did find it amusing that I wasn't drinking!).

Here I was, getting ready for the London Marathon – where, 11 years previously I was doing exactly the same thing but now married with two children! However this time I knew what lay in wait - and there was no going back! Somehow I managed to raise £1026 (thank you so much to the dinner ladies and teachers at the Weaver, bell ringers from St. Mary's Church, Nantwich and friends and relatives – sorry to keep asking you to sponsor me.) Therefore I had to run.....

Lots of texting in the morning making sure Edd and the children (Hannah – has MPS VI and Ben) were ok and getting final agreement where to meet en route, which side of the road to be on – and lots of texting from my training partner Jenny (thank you for joining me on those very long runs but being very sensible in only doing half the long distance routes and listening to all my moans!). She was reliving the marathon experience from the comfort of her living room as she took part two years ago and she was making sure I got to the queue for the toilets before they got too big! And here I was in the red waiting area...

I managed to overtake a Womble on the long walk of doom to the start line (an excellent boost of self-confidence in beating a womble!). And then we were off.... a few hundred yards I heard 'Where's Wally' (phew people knew what my costume was – this was my cunning plan for Hannah and Ben to play 'Where's Wally – Mum'!) The crowd was fantastic and around the Cutty Sark there were ten other Where's Wally's in the crowd who spotted me and just over Tower Bridge another Where's Wally marathon runner overtook me! BUT did Edd, Hannah, Ben and Uncle Neil spot me NO, did I spot them NO! (best well laid plans etc .. gone out of the window). I was a bit deflated but I did see my uni friends which was an excellent boast –

The atmosphere was amazing and very loud and even more spots of Where's Wally! However, blisters at ten miles (never happen in training!) and then realising after doing the proper training this time round I wasn't going to beat my time of 11 years ago and the disappointment of wondering where Hannah, Ben and Edd were – I had to keep going. My mind kept thinking of all those times in hospital. Hannah's ERT lasts for over 4 hours each week - well if Hannah can do this, spend four hours on ERT I should be able to keep going. She can't give up and say she's had enough of the treatment and stop half way - well I guess I can't stop either. Ben has to cope with all of Hannah's appointments and all that goes on with MPS VI so I could certainly try and keep putting one foot forward.

What did keep me going was I had to see Hannah and Ben at the end and get a big hug – a medal, and of course a t-shirt and knowing that a lot of money was being raised for a charity that is always there at the end of the phone!

So thank you for the marathon place, A HUGE THANK YOU TO ALL THOSE THAT SPONSORED ME – and hope the money raised helps all those people that help Hannah cope with MPS VI.

She has certainly coped with a lot (not sure if I could have got through all those operations) and for Ben who does think it's totally unfair that Hannah gets a day off from school to go to hospital (as he sees it, she goes and plays on the play stations and table football!). Well he does put up with a fair amount of travelling and had to understand from a very young age that a sprint down to the doctors is part of the routine!
Hannah Brock

Attracta Browning ran the Virgin London marathon In memory of her brave nephew Lewis Browning.

Lewis was born with MPS. Throughout his life he overcame so many challenges that this disease posed to him. Undertaking ongoing treatment to slow down the progression and effects of the disease, including several invasive surgical procedures. Tragically aged 13 he developed cancer and despite chemo, radiotherapy and surgery he lost his brave battle for life in Sept 2011 aged just 15.

Throughout his life Lewis showed such amazing bravery and courage. He always worried about everyone else's well being and managed to raise a smile in some of the darkest times. Lewis made such an impact on so many people's lives and his memory will live on forever in our hearts and minds. I chose to raise money for the MPS Society and Clic Sargent, two charities that provided vital support to Lewis and his family through some difficult times.

I trained hard and at times found this difficult as I experienced Achilles injuries requiring physio, but I was determined to keep going and do Lewis proud.

The day was a mixture of emotions at different stages. At around the 20 mile mark I found my legs tiring, however the songs I had downloaded on to my ipod kept me going. These were a mixture of artists that Lewis had liked, although emotional to listen to gave me the push and determination I needed to get to the finish line.

My husband and eldest son Ben, Lewis's sisters and mum were at the finish line and were all really proud and a great support.

I have raised over £1000 for the MPS Society, more including gift aid. I feel I have done Lewis proud and this money will go on to help other children with MPS, improving their treatment and research into this disease

Great Run events

We currently still have places available in the BUPA Great Birmingham Run on 20 October 2013. Please email us to register your interest.

Although we have now filled our other 2013 Great Run Events, please get in touch if you would like to register your interest for 2014 - the Great Manchester Run, Great South Run, Great North Run or the London 10k.

Cycle events

Doitforcharity London - Brighton Cycle 2013 - 8 September 2013. Please email us to register your interest.

Thank you to the following individuals who have taken part in events and challenges in support of the MPS Society...

Emily Garthwaite ran in the Virgin London Marathon 2014 and raised £4060 on her justgiving.com page.

Sarah Huntley took part in the Reading Half Marathon in March and raised £30 on her justgiving page.

Anthony Passey, Leon Francis and Ted Saward raised £241.42 from their participation in the Run the World Marathon at Bexleyheath Academy on 28 November 2012

Sarah Louise Goodwin took part in the Yorkshire Warrior 10 mile assault course raising £842 on her justgiving page.

Kate Humphreys ran in the Virgin London Marathon raising £1020 on her justgiving.com page

Ashton Cole did a sponsored tandem skydive raising £270 on her justgiving.com page

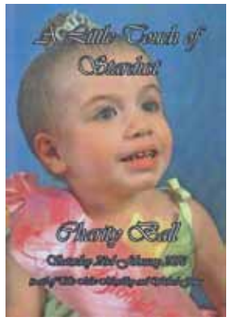
Ambrose Fullalove ran the Virgin London Marathon 2013 raising £1041 on his justgiving page.

David Tosh took part in the Wild Wadi Swim Burj Al Arab raising £500 on his justgiving page

Jason Vickers took part in the Great North Run raising £240 for the MPS Society.

Scott Bennett raised £619 in the Virgin London Marathon, to be matched to £500 by his employers Virgin Money.

Tim Richards ran in the Virgin London Marathon raising £1505 for MPS.



A Little Touch of Stardust

Michala Mawdsley organised a charity ball on Saturday 23rd February 2013 raising £5848.35 for the MPS III Genistein trial.

Jonah's Just Begun donated $5000 towards the Genistein MPS III clinical trial

Park Nursery in Bury raised £180 for the Genistein clinical trial. They held a fun day with bouncy castle and face paint. They also had a sponsored bike ride. Rifat Mohammed's son attends Park Nursery. Rifat says: 'I was very pleased with the outcome. It is so easy to raise awareness and ask for a helping hand. You don't need any specific talent or money to arrange the fundraising events. You just need the courage and the urge of getting the word out there that MPS exists. If I can do it, I know that everyone can.'

Thank you to **Jemma Rodzoch** who raised £170 from a Valentine's Day themed coffee morning. Thank you to the staff and residents of Campbell House in Bolton.

The Haddenham Mummers have been performing their short play over the Christmas period raising money for three charities. The Mummers presented Jenny and Andy Hardy with a cheque for £520 after they kindly received the cheque on behalf of the MPS Society.

Stamp House of **Queenswood School in Hatfield** raised £300.50 after one of their pupils, Rebecca Jones, friend of Molly (MPS III) nominated the MPS Society. Thank you to everyone involved.

6 degrees of separation – Who do we know?

Six degrees of separation is the theory that everyone and everything is six or fewer steps away, by way of introduction, from any other person in the world, so that a chain of "a friend of a friend" statements can be made to connect any two people in a maximum of six steps.

OK, we admit a bit of a random article in your Summer MPS Magazine but bear with us. In an effort to raise the profile of the Society, the families we support and the work we do, we wondered if any of our supporters and members have access to any celebrities, journalists or media contacts that might be interested in supporting the Society in its aim of raising awareness of MPS and related diseases.

While we may be hopeful that David Beckham is suddenly going to start fundraising for us (We are still clinging on to reality here at MPS House) we would be delighted to hear from anyone with any possible suggestions for contacts. You can email **wickedgenes@mpssociety.org.uk**

An African Adventure

What do three Dad's from Lymington in the New Forest have in common with film and TV star Jonny Lee Miller? Not a lot, except the fact that JLM has been touched by Sanfilippo.

It started like this. Dad (of Sophie and Tom with MPS IIIA) says to friend in pub on Christmas Day. "Do you fancy joining me on a 1000mile charity bike ride in aid of Sanfilippo". Friend rather hastily says "yes". Several weeks later I explain the route through Namibia down the north west of South Africa finishing in Cape Town. I explain the terrain, the very high temperatures, wild animals and the fact that we would need to train through one of the worst winters... friend still says yes! Another Dad also says yes (he too did not need any arms to be twisted!) and we become the Ride4Life trio. Our aim is to raise awareness of Sanfilippo and crucially to raise funds towards the Genistein trial.

Training starts in earnest in October 2012. The three of us are keenish cyclists who like to do an hour or two once a week. Doing ten 100mile days in succession is of course something entirely different. We are not young, have families, jobs and flabby bits. Slowly but surely we start longer rides – 30 miles, 40 miles and 50 miles. The weather gets colder and much wetter. Christmas and New Year come and go. By mid January we are showing signs of fitness. We can ride 60miles at a steady pace and don't succumb to heart failure. By mid February mild panic sets in as the prospect of our 17 March departure looms and the thought of riding ten 100mile days in Africa becomes all too clear. We ride through freezing weather (literally), snow, hail, sleet, wind and rain and now ride 2 or 3 times a week in between short sessions on our turbo trainers (bike set up on a static roller).

Whilst all the training miles are being put in, planning for the fund raising and awareness campaign are taking shape. Social media becomes part of our lives like never before and sponsorship opportunities are presented to a variety of companies near and far. The support for our venture grows rapidly. Sponsorship and donations start to arrive before we even depart for Namibia. During those cold and wet training rides the thought of people supporting us really helps to keep the motivation up. Our respective wives and children are husband and fatherless for several hours on Saturdays and Sundays as the ride durations are increased steadily. This really is a team effort.

7am, 19 March. Three Lymington Dad's set of through the morning "rush" hour of Namibia's capital, Windhoek. Our bright red and pink lycra outfits sets us off nicely against the brown African tones. And crucially car and truck drivers can see us! Sam is our support man and now has the pleasure of spending the next 10 days looking at our rears whilst driving the support vehicle at 30kph all the way to Cape Town. Luckily, Sam the Man, is the perfect companion and drives behind us in a protective manner whilst keeping his sense of humour and eyes on the road. The weather is hot. 40C plus in the first week – tea towels are configured into protective head gear to keep the scorching rays off our delicate white necks! Each day is conquered and we appear to get fitter and fitter as we push on. Namibia is simply stunning. Cars and lorries give us a friendly wave or peep as they go by. Petrol attendants ask us what on earth we are doing in the middle of nowhere. Good question!

We cross the border into South Africa. And then have a 52km climb in searing heat. This is the start of the hilly part of the ride and each rider knowing his strengths and weaknesses has to knuckle down to some testing terrain. People are still waving and peeping at us as we go through the barren Namaqualand. Our morale is tested when a bus scrapes past at speed and nearly send one of the team down. Luckily that evening we stay in a wonderful tented camp on a 20,000 acre farm with wonderful African bush views and our first big serving of carbohydrates. Thus far our diet had been meat, more meat and even more meat. Nightly varieties usually included beef steak, Impala or Kudu with a very modest selection of vegetables. The lager in Namibia and South Africa is excellent but sense prevails and we stick to soft drinks and the odd "light" lager.

Easter Friday is our arrival date in Cape Town. Our families have flown out to meet us (sadly Sophie and Tom cannot make the journey) and wait anxiously for us. The Cape "doctor" does his best with 40mph heads winds making our final day a punishing 10 hours and 40 minutes in the saddle. Ouch that hurt! All three riders had moments of doubt for the first time. The rolling countryside on a benign day would have made the final 115miles relatively straight forward. Instead we had to fight the headwind with all our energy reserves and resolve. The screeches of joy (and relief!) from our wives and children as we arrived are etched in our memories.

What made this trip so memorable? Lots of things, but the support of people near, far and wide who got involved with Ride4Life and our fund raising was incredible. The thought that we did this for children like Sophie and Tom is hugely uplifting and we urge everybody who wants to do something "worthwhile" to get out there and do it.

Massive thanks to everybody who donated, followed and supported us on Facebook / Twitter and our Blog. It has been a really humbling experience.

Jonny Lee Miller for his part recently run a 52mile ultra marathon in the USA in aid of Sanfilippo (he has now raised $130,000). So whilst we may not have his fame, fortune and good looks we have together all done a little something for Sanfilippo sufferers.

Paul Shields and the Ride4Life team have raised £50,607.73 on their justgiving.com page with a mixture of online and offline donations. Special thanks also to Posterscope UK and Paul Beaumont at Octopus HR.

Project Sanfilippo

The concept of Project Sanfilippo was developed by Karen Robinson, mother of Oliver who has Sanfilippo. Project Sanfilippo raises vital funds for the MPS Society to put towards research into Sanfilippo Disease. Here is a round up of what's been happening recently...

Mount Everest Challenge

Fundraising of any sort is very challenging and involves lots of hard work, commitment and compassion. I always loved kids and any chance to play with them and makes them happy in any way will be the highlight of my day...

I never heard about MPS or Sanfilippo disease until I heard about it from Rob who is my official transport most weekends. Rob told me how it affects kids and this dreadful condition take their lives before their teens. Rob then intoduced little Oliver, 14 from Fairford Gloucester who he takes to special school and that Ollie's mum is fundraising for a new drug to clinically trial on Ollie and other kids with similar conditions which cost £600,000 and is not funded by NHS or any big Pharmaceutical giants. I realised it's a mammoth task and I needed to do something special to see Ollie and all those kids who suffer from this condition to have many more happy years... So the same night after I found out about Genistein trial fundraising I took the decision to climb the highest Mountain ranges in the world, The Himalayas...climb up to Mount Everest Base Camp at 5360metres...

I had never done any mountaineering or trekking since I'm working full time as a Pharmacist Manager in Gloucester. The only hobby or sports is playing badminton after work. I gave up playing badminton to train climbing and started climbing hills and mountains in Wales, England and Scotland. I felt the pain the following day after 5-6 hours of climbing and it was satisfying as I know it's for a good cause and without pain no gain. I spent more evenings in the gym with a trek bag on my back weighing 20-30kg to prepare for the biggest trek, The Mount Everest. So I planned to be at the Everest Base Camp at 17,000ft for my birthday on March 10 and will be playing a game of badminton with my guide which I don't think anyone played in history as far as I know.

Thanks to all my friends, my work colleagues, Kirit Patel MBE (my boss) my family and above all Ollie who is

being an inspiration and drive to make it happen.and raised over £6000 for MPS trials.

I did it risking my life as I really suffered from altitude sickness and lost my breathing which I'm recovering now... So now no time to waste please dig deep and donate to the link below and every penny counts until it reaches the target. Thank you. **John John**

www.justgiving.com/john-john0

Farmor's School in Fairford held a Santa's Grotto raising £175.32 for Project Sanfilippo. Students made cakes to sell and small gifts and took donations for a visit to Father Christmas.

The school also held a Wear It Blue day raising £259.71.

www.projectsanfilippo.co.uk

Jump for Genistein

Karen Robinson, Lucy Cadbury, Sarah Cadbury and Andrew Butler did a tandem skydive raising over £2500 for the Genistein Trial. Thank you to them and to everyone who kindly sponsored and supported Project Sanfilippo and the MPS Society.

Other ways to support MPS Society:

Regular Giving

Over the past 3 decades your generous donations, have helped achieve some of the most significant MPS and related diseases breakthroughs in treatments, as well as supporting the individuals and families affected by these life-limiting diseases.

By making a regular gift of just £5 a month for example through direct debit you will make a big difference to the lives of children and young people with MPS and related diseases, helping to fund our unique advocacy support service and help to continue to fund vital research into treatments

You will be helping people like...

Ollie: *"I was diagnosed with MPS VI in 1999 when I was just 4 years old. At this time there was no cure or treatment to help slow down the progression of the disease but with the research largely funded by the MPS Society, eventually a treatment was developed called enzyme replacement therapy (ERT). At the age of 11 I had my first infusion in Manchester Children's Hospital. Within months I started noticing improvements in my health which included: increased energy levels, the straightening of my joints and most importantly after my height had halted to less than 4ft tall.. I began to grow once again!"*

Christian's family - Christian has Morquio disease: *"When we decided that Christian needed to have his bedroom on the ground floor, we immediately wondered how we would be able to afford to make the changes. Our house only had upstairs bedrooms and we needed to convert the ground-floor for Christian. Having got some quotes, we found out that the work would cost more than the local council grant. We knew that we needed help finding this money, so we contacted the MPS Society for advice and support. Their advocacy team made a home visit and were incredibly helpful, writing applications for grants to other organisations for support. After a lot of effort and hard work, the MPS Society managed to get grants from seven different organisations and from there; we were able to begin work on Christian's new room."*

Every day our Advocacy team are on the phone, visiting homes and clinics to support our members.

Why regular giving is so important

People who give regularly to the MPS Society provide on-going support for our advocacy service for our members and funding vital research. Regular monthly donations help us confidently plan ahead and contribute to treatments in the long term. It is also one of the most cost-effective ways to support the MPS Society. A regular gift will help us to continue our work in supporting those affected by MPS and related diseases.

How regular giving works

Regular giving is an easy and flexible way to support the MPS Society on an on-going basis. Each month your chosen amount is automatically debited from your credit card or bank account. You can increase, decrease or cancel your monthly donation at any time.

Find us on Facebook
www.facebook.com/mpssociety

twitter
twitter.com/MPSSocietyUK

What now?

If you have any questions about making a regular donation, please email us info@mpssociety.org.uk or phone the MPS Office on 0845 389 9901. Alternatively, if you would like to set up a direct debit, you can fill out the form enclosed in this magazine and return it to us.

Should you need to make any changes to your regular gift, please contact your bank and let us know.

Where does your money go?

A donation of £2 per month could help us to offer so much more support in so many ways:

- Access to clinical management and palliative care
- MPS Regional Specialist clinics
- Support with disability benefits
- Paving a child's way in accessing education
- Upholding rights in employment
- Advising on home adaptations
- Bereavement support

Please donate **online** at
www.mpssociety.org.uk
phone 0845 389 9901
text MPSS01 £2/£5/£10 to **70070**
or **post** your donation to our office, MPS House.

Thank you to Marina and Friends

We would like to extend a special thank you to Marina Foster and friends. Marina runs a charity shop in Bristol, Marina and Friends Fundraisers, donating the proceeds from the sale of second hand items to the MPS Society. So far, the cumulative total raised by Marina and Friends for research into Sanfilippo disease is **£103,066.15.** If you would like to support the MPS Society by providing items for Marina to sell, please find below the address for the shop: Marina & Friends Fundraisers, 44 Sandy Park Road, Brislington, Bristol, BS4 3PF. You can also follow Marina and Friends Fundraisers on facebook.

Special thanks to . . .

Grants, CharitableTrusts, Grant-giving Foundations and Community Groups
The Martin Connell Charitable Trust; The Gosling Foundation; The Rotary Club of Farnham; Rotary Club of Bude In Cornwall; The Felicity Wilde Charitable Trust; Baron Davenport's Charity; Joseph Strong Frazer Trust; Help a Capital Child; The Summerfield Charitable Trust;

Donations
Susan Lam; Derek Marshall; Monica Bowen; Holly Nowell; Jacqueline Best; Hannah Bujalska; M & I Vickery; Mr S M Brecker; Jackie Best; Mrs Baker; Claire Conway; Biomarin; Software Europe; Enkalon Foundation; Mrs Baker; Colette McDonach; Margaret Taylor; Pat Skidmore; Sue Kaye; Yvonne and Malcolm Leslie; Michael Hickey; Mrs Lois Brookes requested by D Russell; John Sanders; Liz Drew; John Roger Barker; Bob Lott; Rachel Overett; Janet Jones; Joyce Wilson; Tilmann Volk; Kerry Gowthorpe; Kyle Burrows; Mrs Slingo

In Memory
Wing Kin Yeung; Simon Lavery; Paul Franklin; Blaise Leslie; Matthew; Thomas Fisher

Collection Boxes
Sue Hollidge; Frutin Travel; Marcia Burnett; Deborah Thorburn, John and Sharon Hattle; Mrs Crespin

Stamps, foreign coins, mobile phones, ink cartridges; jewellery
Hannah and Ben Brock; Langlea House Care Home; James Garthwaite; Andy and Jenny Hardy; Derwent and Solway Housing Association; Mr and Mrs Croft; Ian Evans and Arriva The Shires; AG Office Supplies Ltd; Ellen Graham; Joan Crespin; Mrs A Baker; Karen and Andrew Weedall; Residents of Home Heights; Christine Hancox; Vivienne and Andrew Culley, Mrs Joyce Arnold, Miss Molly Rigby;

Dr Hugh Standing kindly donated £300 being the proceeds of a coffee morning in aid of the MPS Society. Dr Standing wrote that he has been well informed over the years by Andy and Jenny Hardy of the Society's work.

TWI donated £750 to the MPS Society in lieu of sending Christmas cards. MPS was nominated by Sarah Fowson.

Patrick and Karen Otway kindly donated £350 received in lieu of flowers at Emily's funeral.

Donations totalling £1660 have been kindly received by the MPS Society **in memory of Paul Franklin**. Thank you to the family for their support.

Ann Parsons donated £179 being the proceeds from the sale of trolley tokens at Asda Eastbourne.

Sheila Joiner donated £30 to MPS. The reason for their donation was a thank you to Michelle and Terry.

Sarah Huntley raised £24 being the proceeds of her participation in the Reading Half Marathon.

The Christadelphian Sunday School collected money for the MPS Society and have raised £34.80.

The class of 7jse at **Northampton Girls School** raised £230 for the MPS Society during their charity week.

John Casey, uncle to Jordan Mount (MPS I BMT), raised £300 from a sweepstake accumulator which he runs at the Castle Bar in Uddingston, run by John and Nicola Butler.

The MPS Society has received £250, a kind donation from Relief Chest Scheme of the The **Freemason's Grand Charity** at the specific request of Child's Hill Lodge No. L4275.

Kerry Hughes donated £310 to the MPS Society collected at her daughter Cameron's recent christening.

Claire and Suresh Weerasinghe donated £50 to the MPS Society for Cameron's christening.

Thank you to **Mix 96 Radio Station** for choosing the MPS Society to be one of the charity recipients of their Easter Egg Appeal.

The Ramada Hotel in Dover held two pamper nights on 30th November 2012 and 15th February 2013 raising £187.25 for the MPS Society.

Michelle Yard donated £40 being the proceeds of a collection on behalf of Jill and Philip Loveday whose nephew died from the disease.

Michele Brooks Daw recently celebrated her birthday and in lieu of presents, asked for donations to be made to the MPS Society. Family and friends kindly donated a total of £354.96.

Kim Mills donated £120 given by friends and family in memorium of her son Andrew's birthday. Andrew would have been 25 on 18th April 2013 but unfortunately passed away in December 2002. Kim asked that the money go towards research into Sanfilippo.

Mary Bird and Jill Stevens donated cheques totalling £205 given in memory of their mother, Hilda Marks, who passed away in April. One of Hilda's great grandchildren, Samuel Watts, has Sanfilippo.

Hepworth Garage in Shalford, Surrey donated £163. Hepworths has not been charging regular customers for headlamp changes, asking instead for a donation to their chosen charity, which this year is the MPS Society.

The **Inner Wheel Club of Yeovil**, Somerset donated £100 to MPS.

Northern Rock Foundation matched a donation made by Scott Bennett for £500.

Jill Loveday donated £125 from a recently held 60th birthday party. Donations were given to the MPS Society in lieu of presents in memory of Jill's nephew James Gooch

Staff at **Beauchamp College**, Leicestershire donated £155.46 raised during a Dress Down Friday

Caroline O'Driscoll raised £495 on her justgiving page for Erin's Baptism

Thank you to the **Shepherd family** and friends for raising £4504 on their justgiving page in memory of Anabelle.

The Society would like to thank the following donors for their regular contributions by either **Standing Order** or **Give As You Earn**.: D Forbes; G Simpson; W Cavanagh; L Brodie; E White; C Hume; A Weston; M Malcolm; E Mee; K Brown; J Dalligan; E Brock; M Fullalove; G Ferrier; R Parkinson; R Taylor; C Pierce; R Gregory; K Bown; S & J Home; E Moody; V Little; S & D Greening; Z Gul; M Reeves; P J Martin; J Casey; E M Lee; D Palmer; J & V Hastings; R Dunn; S Littledyke; N Saville; Marcia Tosland; S Bhachu; I Hedgecock; C Cullen; D Forbes; R Henshell; P Summerton; M Kalsi; D Holmes; A Sullivan; J Wilson; S Cadman; J & F York; K Robinson; M & J Wood; K Osborne; E Cox; Mr Thompson; Miss M Rigby; A Tresidder; A Byrne; C Lunnon; D Robinson; M Peach; C Garthwaite; R Arnold; J Ellis; I Pearson; Norman Saville; K Seeber; A Ephraim

Fundraising at School and University

MPS Society went back to University in Coleraine

Most UK universities have amazing RAG Committees (Raising and Giving) and through various activities it is estimated that these student-run university organisations raised between €6m and €9m for charity in 2011.

As we mentioned in the last magazine, Wicked Genes have been busy contacting universities to raise awareness of Mucopolysaccharide and related diseases and to put the MPS Society forward for RAG partnership consideration.

We would like to take this opportunity to thank the RAG team at the Coleraine campus of the University of Ulster, who recently selected the MPS Society to be one of their three charity partners. As a result, 3 of our member families went along to the Ulster University Student Recognition evening on Thursday 18th April and were presented with a cheque for £1004 for the MPS Society, a great effort.

If you are at university or are an alumni of one, perhaps you would consider putting the MPS Society forward for RAG partner consideration. RAG teams are normally based at the student Union and we would be delighted to support any student fundraising.

Please email us at **wickedgenes@mpssociety.org.uk** to find out more.

If you have any links to universities then please let their RAG teams, (normally based at the Student Union), know about the MPS Society. We would be delighted to support any student fundraising.

Fundraising at School

By the time you read this, it won't be long till schools brake up for summer holidays. We just wanted to take a moment to ask if you would consider contacting your local school about fundraising in aid of the MPS Society. Some schools decide this before the summer, others in the new term in September, either way we would really appreciate your support.

Why fundraise for MPS Society at school?

Fundraising in aid of charities and good causes is a regular activity in most schools; it is a vital awareness tool and income stream for charities, large and small.

The MPS Society has been fortunate enough to be the recipient of school charity events over the years; however, as a small rare disease charity we are not well known and are reliant on our members and supporters putting us forward as a school's charity partner. School communities are excellent places to raise awareness and encourage understanding of these rare genetic diseases.

Why is fundraising important to schools?

As vital as the schools support is to charities, the charity events, sponsored silences and fun days at schools are also invaluable to the school communities themselves.

The pupils involved often pick up valuable selling, marketing and organisational skills when planning an event, ensuring a fun activity for a good cause can also be a learning experience. The pupils also get vital team work experience as the motivation in pulling together for a common goal and learning about how and when to compromise are important life skills.

Instilling the importance of community and charity in schools by doing something for the benefit of others and not to be self-serving is a major part of any school fundraising activity, as is the development of creative skills that the thought process behind different schemes can bring.

School/Charity partnerships also provide a platform for pupils to explore curriculum based subjects. Whether it's applying shared common values in a PHSE lesson or understanding genetics in a Science lesson, there are valuable experiences to be made educationally as well as personally and socially for pupils engaged in school fundraising for the MPS Society.

What can I do now?

If you think your local school might be interested in hosting a fundraising event in aid of the MPS Society then please get in touch. Whether it's a one off event like a dress down day or school disco or you would like to talk about a calendar of events for the year we would be delighted to support you with posters, balloons, and where possible guest speakers.

Please email **wickedgenes@mpssociety.org.uk** for information on schools fundraising

We may be small but we mean business

- please help us get Wicked at Work

One way to support the Society and to raise awareness of MPS and related diseases is to get fundraising at work.

One of the biggest difficulties the MPS Society faces in trying to gain corporate support is that as a rare disease charity, not many people have heard of us, so when the staff vote comes round to elect a charity to support it is normally the big charity brands that are nominated.

We would really appreciate your help with this, as a personal contact and introduction is the most effective of introducing the MPS Society, the work we do and how we would benefit from your company's support. It is also an opportunity to raise awareness of these rare, life-limiting, genetic diseases and how they affect the individuals and families we support.

The great thing is that many companies have a really positive attitude to supporting charities that their employees are involved with. If you, a family member or a friend work for a company please see what you can do to help.

You might work in a small office or a large multi-national company; it might be a shop, a large retail outlet, factory or classroom. Wherever you work we are full of ideas of ways to engage, motivate and get staff having fun and raising money for a good cause and we promise to try and ensure it doesn't mean extra work loads for you.

We can provide press releases, case studies and photos which companies can use to put up on their websites, publish in internal magazines to give on-going exposure and advertising. We always write to say thank you and try to offer a local MPS representatives for cheque presenations

Fundraising at work is a great way to boost your employer's reputation and image with shareholders, suppliers and the community.

What do I do now?

If you think you might be interested in raising the profile of MPS and related diseases at you place of work and getting colleagues fundraising then drop us an email at wickedgenes@mpssociety.org.uk or call us on 0845 389 9901. We can send you our brand new super smart guide to fundraising, we can support you with ideas, posters, balloons and where possible guest speakers.

It might be that your company has a Corporate Social Responsibility Programme, and a Charity of the year partnership, we would really appreciate the opportunity to present the MPS Society for partnership consideration.

Once you have read this MPS magazine, please pass it on to your family, friends and colleagues.

Help us spread the word about MPS and related diseases and the work we do.

www.mpssociety.org.uk

Get Wicked at Halloween

Something spooky is a coming...

Yes, that really is a picture of a pumpkin a whole five months before Halloween. But we haven't gone 'batty' or made a 'howling mistake,' we are on a mission to recruit 'mummies' and (daddies for that matter) to start thinking about a Wicked Week of Halloween celebrations, 'Witch' we think is a great way to have fun, raise awareness and get into the 'spirit' of Wicked Genes; the MPS Society's fundraising initiative.

So what do you have to do?

At the moment... nothing, it's a whole five months until Halloween!

But... if you did want to get your thinking caps on about whether you would like to organise a Halloween party/disco/ball for adults or children at school, at work, at uni, with family or friends then you might need to think about venues etc. and how we can support you.

We are also going to be asking schools and businesses to get Wicked at Work and School. We will be looking for the best 'Wicked' outfits for Halloween. So, don't worry if you missed your chance to 'Wear it Blue' this year, you have a great opportunity to **Get Wicked** for Wicked Genes!

Drop us an email at

wickedgenes@mpssociety.org.uk

Wicked GENES

support

research

awareness

The Society for Mucopolysaccharide Diseases is the only UK charity providing professional support to those affected by 24 MPS and related diseases, funding research and raising awareness of these rare genetic diseases.

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