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IN THIS ISSUE

MPS

support

research

awareness

Wicked GENES

The MPS Magazine

Winter 2013

Society for Mucopolysaccharide Diseases

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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:

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

To promote and support **research** into MPS and Related Diseases

To bring 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 can result in death in childhood.

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

On the cover...

Our lovely front cover image is of the MPS Society's Senior Advocacy Officer Sophie Thomas with Kamal Hoteit (MPS IV) on the ice skating rink at Lapland UK. Kamal was attending the MPS family day at Lapland UK with his mum, sister and grandfather. More photos and stories from the Lapland day out to follow in the spring edition of the MPS magazine.

Please don't forget to send us your stories and photos from any MPS family days you attend. Funding for these events is achieved as a result of written applications made by the Society throughout the year to Trusts and Grant organisations. Your personal experiences help us to demonstrate in applications how important these fun days out are to the children, young people and the families that the Society supports and helps us to secure future funding for similar activities.

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

Welcome!

It's 2014!

Welcome to your latest edition of your MPS magazine, and as usual it's full of latest news and updates as well as your inspirational stories.

Christine's Chief Executive Report on page 4 outlines recent developments in MPS, Fabry and related lysosmal storage diseases.

We've some lovely stories in this issue, not least Gracie meeting Katy Perry and Cinderella (p7)

Roma Drayne (p8) and Ian Hedgecock (p11) have both written about their experiences with home adaptations

You'll find a really helpful Advocacy education update on school exclusions (p14) and assessments for Special Education Needs (SEN) Statements (p15)

Read about the MPS Family days at Harry Potter World and Legoland (p18-19)

Sunday 13th October was our Childhood Wood Planting day, read Trustee Jessica Reid's moving account (p20)

Recent clinical trial updates can be found pages 22-23

Genistein Recruitment news is on page 24

Your incredible Fundraising & events stories from page 28

Best wishes, The MPS Team

Contents

This magazine was printed with the kind support of a grant from The Goldsmiths' Company.

Chief Executive's Report

As we move into 2014 we send best wishes to all our members and supporters.


Christine Lavery, MPS Chief Executive

We are conscious that for our newly diagnosed children and adults and their families and friends 2013 may have been a difficult year. Our thoughts are also with the families who lost their loved ones through MPS, Fabry and related diseases. In the past twelve months, the MPS Society has been only too aware of the impact of the financial crisis on our members and we are most grateful in this difficult climate to all those who fundraised for us. Every pound counts and the variety of ways you raised these tens, hundreds and thousands of pounds is truly inspiring.

These funds along with support from Charitable Trusts and Industry have enabled the MPS Society to maintain its core activities of support, awareness and research throughout the past year. Many of you have received support from the dedicated MPS Advocacy Team, participated in Regional Events and the Weekend Family Conference and received the quarterly MPS Magazine. In 2014 the MPS Society has plans for a Northern Ireland Conference, Regional Events and more but we can't do it without your fundraising and donations.

2014 is an important year for MPS IV Morquio and our affected members. In March we expect the Enzyme Replacement Therapy, Vimizin for MPS IVA to receive marketing approval in Europe. The MPS Society is actively working with clinicians, BioMarin and the regulators to achieve timely reimbursement in England for Vimizin. We also understand that regulators are fully briefed in Northern Ireland.

On 8 October 2013 the Scottish Government announced a new flexible approach to the evaluation of new medicines and the extension of the £20 million Rare Medicines Drug Fund in Scotland. We sincerely trust this is good news not only for those affected by MPS IVA but MPS I, MPS II, MPS VI and Fabry disease. We are only as good as the information we receive so if you or a family member are denied reimbursed Enzyme Replacement Therapy and live in Northern Ireland, Scotland or Wales please do contact the Advocacy Team.

After decades of working for treatments for Sanfilippo disease the level of pre-clinical and clinical trial activity is almost daunting. Further on in the magazine are clinical trial updates and an article on Genistein but as I close this piece I am delighted to announce that the Phase III Genistein study for MPS III, Sanfilippo A, B and C is now recruiting at the Manchester Children's Hospital.

Happy New Year

Christine Lavery MBE
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www.mpssociety.org.uk

The MPS website is constantly updated with latest news on research and treatments and events. There are lots of useful downloads and resources from our Advocacy Team from knowing your rights to understanding the benefits systems and guides on how to manage difficult behaviour. The site is full of advice on how to access help when and if you need it.

The MPS Society website is also packed with imaginative ideas for fundraising for the Society, whether you are running a marathon, holding a school cake sale, or planning a Wear it Blue event for MPS Awareness Day, there is something for everyone. We also have posters, sponsorship forms and guides to download.

If you are planning an event don't forget to visit our on-line shop for T-shirts, badges and bands . We are always looking for ways to improve our site, so if you have any suggestions or ideas please don't hesitate to contact us at **info@mpssociety.org.uk**

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 Meetings held in July and September 2013.

Appointment of New Trustees

Over the two meetings two new Trustees took their places on the Board. James Garthwaite and Katrina Fanneran were welcomed by the Chair who acknowledged neither are strangers to the work of MPS.

Election of Officers

Sue Peach was elected to continue in post as Chair of the Board for a further year. Wilma Robins was elected to continue as one of the Vice Chairs for a further year and Paul Moody was newly elected to the post of second Vice Chair for the coming year. Judith Evans was elected to continue in post as Treasurer for a further year.

Treasurer's Report

Judith Evans read to her report and at the September Board Meeting drew Trustees' attention to the pleasing fact that the MPS Society was only £229 over spent on its approved expenditure budget. Trustees' acknowledged that many grant applications are going out however the MPS Society still has some way to go to secure its approved income budget before the year end in December 2013.

Personnel

Trustees were advised that all staff annual appraisals have been completed and were updated on some staff changes. The CEO confirmed that her PA who had been on a year's maternity leave had decided to be a fulltime mum and had resigned. Toni Ellerton who was appointed to cover this maternity leave has had her post substantiated and will continue as PA to the CEO.

MPS House

Trustees were updated on the office adjoining MPS House and further considered the business case for purchasing the other half of MPS House and received a report on the cost of alterations required to ensure the space works for MPS. The Trustees scrutinised the costings which included the rental income of the upstairs office which will cover the extra mortgage and the grants being offered to cover the alterations. After a further visit to the building the decision was taken unanimously to purchase the adjoining building and instructed the CEO and Finance Officer to start the process.

MPS Commercial

Following professional advice the Trustees agreed unanimously to the proposal to establish MPS Commercial as a wholly owned subsidiary of the charity, the MPS Society and operate its patient access clinical trial programme and health technology studies programme through MPS Commercial. Any profits will be gift aided back to the MPS Society.

Risk Management

The Trustees received and considered the Health and Safety Report and interrogated the Risk Register. Trustees agreed to defer the discussion on succession planning until January 2014. Two Trustees undertook to develop the MPS Society's Business Continuity Plan.

Advocacy

The Senior Advocacy Officer provided a report and Trustees were advised of a number of advocacy matters by the CEO. The organisational arrangements between Manchester Children's Hospital and the MPS Society for the 'Ed Wraith Memorial Conference and Gala dinner' in his memory to be held Saturday 5 April 2014 at Old Trafford Cricket ground were agreed by Trustees.

Fundraising

Trustees considered the Fundraising and Communications report which outlined plans for current campaigns including 'Wear it Wicked' for Halloweeen. Trustees also discussed the merits of a MPS Charity shop in Amersham and the potential use of volunteer fundraisers.

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twitter.com/MPSSocietyUK

Trustees recognise Barry Wilson's outstanding service to the MPS Society

On 29 November 2013 Barry Wilson stepped down from the Board of Trustees after more than a decade of dedicated service to the MPS Society.

He took over as Chair of the Society at a time of change and has steered the Society to the heights it is today. In recognition of his service the Trustees presented Barry with a framed photo of himself at the Palace of Westminster celebrating the Society's 25th anniversary.

On behalf of the staff, Trustees and members we thank Barry for all his support and energy and wish him good health and happiness in his retirement.


Barry Wilson with his retirement gift from MPS Trustees and staff

New members

Trudy and Nick have recently been in contact with the Society. Their son Mason has a diagnosis of MPS I Hurler. Mason is 6 months old. The family live in the West.

Mr Hussain has recently been in contact with the Society. His daughter Maleeha has a diagnosis of MPS I Hurler Scheie. Maleeha is 7 years old. The family live in the Midlands.

Mr and Mrs Imran have recently been in contact with the Society. Their daughters Manahil and Wania have a diagnosis of MPS VI. Manahil is 10 years old and Wania was 9 in November 2013. The family live in the South West.

Mr and Mrs Rawles have recently been in contact with the Society. Mrs Rawles and their son Archey and daughter Maisey have a diagnosis of Fabry. Archey is 5 years old and Maisey is 3 years old. The family live in the South East.

Jen and Matt have recently been in contact with the Society. Their son Jacob has a diagnosis of Hurler. Jacob is 8 months old. The family live in the South East.

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.

Births

Many congratulations to Laura Troll and her partner Rob on the birth of their daughter Jessica Nancy Mae on 8th November 2013 weighing 6lb 8oz. Little sister to Sam.

Jessica Nancy Mae

What's on 2014

CONFERENCES and REGIONAL EVENTS

Ed Wraith Memorial Conference and Gala Dinner
5 April

All Ireland MPS Conference
9 – 11 May

MPS Awareness Day
15 May

13th International Symposium on MPS Diseases
13 -17 August

MPS REGIONAL CLINICS

MPS I - GOSH: 25 March, 22 July

MPS III - GOSH: 14 January, 8 April, 8 July

MPS IV - GOSH: 28 January, 24 June

N. Ireland MPS Clinic: 9 May

MPS I Post HSCT (over 6's) - RMCH
3 Jan, 4 April, 4 July, 3 October

MPS I Post HSCT (Under 6's) - RMCH
10 Jan, 25 April, 11 July, 10 October

Jensen's Surprise Trip to London!

During October's half term, Jensen Bechemin, 8, who lives in Jersey, Channel Islands was taken on a surprise trip to London for five days by his Nanny and Poppa. Jensen had started ERT for Fabry Disease in April and has been very brave during his treatment.

Whilst Jensen was in London he was due his fortnightly infusion. Jo, his nurse, visited him at his London Hotel and administered his infusion. It was all very exciting.

Jensen had a fun time going on the London Eye, visiting Hamley's toy shop, which he loved, even playing with some of the new toys. One day he went to the National History Museum to look at all the dinosaurs, his favourite being the Stegosaurus.

In the evening he was taken to see 'Charlie and the Chocolate Factory and he bought a T Shirt which looks as if it has melted chocolate all over it.

Jensen's favourite place to visit was the Regent Park Zoo. He loved seeing all the different animals, the giraffes, zebras, lions and colourful fish. His favourite place in the park was 'Penguin Beach', he loved looking at the penguins playing together, especially 'Ricky' the Rockhopper penguin.

A trip he will always remember.
Zoe Bechemin

Jensen Bechemin (Fabry)

Gracie meets Cinderella!

This year has been a very busy one for me! On May 4th my mummy organised a Gala Dinner, called All That Glitters..., to raise money for my super duper pink power chair! We even had some guests from Coronation Street, who also sang for us on the night! I really wanted to get on the stage to sing and dance, but I wasn't allowed to! I gave my mum a huge bunch of flowers to say thank you for helping me. My new power chair arrived a week later, and I love it!

In October I had some more excitement - I got to meet my favourite popstar, Katy Perry, thanks to the charity Rays of Sunshine!

I was driven around in a pink limousine all day, had tea at Planet Hollywood, stayed in the Hilton Hotel in London, and watched Katy perform before I met her! I also met Ellie Goulding, who was lovely! Katy gave me a bracelet from her last tour, and also gave me €45 of iTunes vouchers! She was beautiful and funky and let me ask her as many questions as I wanted to!

The excitement carried on at the end of October, as another great charity called Childflight took us on the holiday of a lifetime to Florida! We went to all the Disney parks, and SeaWorld too! My favourite park was Magic Kingdom where I got to meet my favourite princess - Cinderella. We had a giggle about glass slippers! It was just absolutely wonderful, but I missed all of my friends back home! Me and Daddy were ready to fly home, but mummy wanted to stay in Magic Kingdom, and become Tinkerbell! Silly mummy!

Thank you for reading my article, and I hope you had a great 2013 too! Lots of love from Gracie, aged 7.

All the Glitters Gala Dinner

Gracie and her power chair

Gracie meeting Cinderella!

Assistive technology for Roma Drayne

There are some things in life that we all take for granted. Really simple things. Take for example the ability to switch on your lights, open your curtains, open your windows or blinds, or your bedroom door, or to be able to see who is at your front door. And think too about how important it is to maintain your privacy, your heat/cold or the noise that stops you from resting. Suppose that you have to lie in bed during the winter, in pain and you cannot walk. You have to rely on someone to come and perform all these simple functions for you.

Aren't these things so absolutely basic, in fact so ordinary that it is actually difficult to comprehend just how important these things are – that is unless the ability to do these things was taken away from you? Maybe you should try it yourself, see how you would deal with all of this if you couldn't do these things.

These are all the reasons why I argued with Occupational Therapists for the provision of assistive technology (Environmental Controls) for my daughter Roma who has Morquio, and is now 21. Roma uses a powerchair, as she is no longer able to walk. She suffers from constant bone pain that affects her stamina, so she spends quite a lot of time in bed particularly in the winter months. She is unable to get into or out of bed by herself, and she is unable to reach for things. Roma's room is her castle and she spends most of her time there.

Roma is no stranger to IT, she uses it constantly, with laptop, kindle, apple, iphone, you name it she has got it. She is a student with the Open University and spends most of her life online. When in bed she needs access to her various devices and she will text me when she needs to get up, use the toilet or wants food or drink.

So I asked the OT's about the possibility of applying for a Disabled Facilities Grant to provide Environmental Controls that would enable Roma to control her own environment from one device. After all the position statement of COT (College of Occupational Therapists) states that OT's will enable service users to:

- Have choice and control;
- Live in an environment/society that is accessible and appropriate to their lifestyle;
- Manage their long-term condition; Have responsive and accessible information and services that are personalised and appropriate to their needs;
- Retain maximum dignity and respect...

Well, what ensued following my request was the mother of all battles, I first spoke to the OT in 2010 about this, and I am happy to say that in November 2013, Roma eventually has the system installed. It took over three years to fight for this provision and two days to install it.

Why did this take so long? Well, all I can say is there was a very considerable degree of resistance towards providing this equipment, which was caused by ignorance about EAT, professional hubris, and fear of actually applying for the equipment on the part of the OT from Children's services. There was also an assumption that it was my job to operate everything for Roma, despite all the other multitude of caring tasks that I perform.

The first assessment that was carried out on Roma entailed the OT watching Roma trying to open doors from her wheelchair, and the effort to do this wore Roma out who nearly fell out of her chair trying to get at the door handles. Part of the assessment involved asking me questions such as how often do you leave the house? My answers were honest; I said I only left the house to do school runs.

The OT concluded that yes Roma had difficulty with opening the front door only, and that I was at home with her all day. Little did I know that OT's were using that argument against me, - I was at home all day with Roma, thus I was on call all day to operate Roma's environment. However, I tried to tell them that the reason I was at home all day, or could not go out was because Roma needed me to do all these things for her.

I tried to argue my position with OT's; they came to a decision at a meeting that only a door opener would be provided. I was not at that meeting, and I do not know who made that decision. I wanted a copy of the assessment papers; they refused to give them to me. Eventually one of the OT managers agreed that I could get the papers but I had to request permission for them and there would be a charge. Roma has had a multitude of reports/assessments done by professionals and they were always given to me if I requested them.

Eventually after several months I received a copy of the assessment and I was then in a good position to argue for the provision of Environmental Controls. I could see clearly from the assessment that they were not taking the carer's position into consideration let alone Roma's. I had done some research and I knew that several OT managers had attended EAT courses, thus some at least were aware of its potential. I pursued the OT manager to provide with me a copy of the Criteria for Provision of EAT.

This was not forthcoming but I persisted for over 6 months to get a copy of this. I had no option other than to put in an official complaint straight to the Director of Children's Services. This at last meant things started to move, we had several meetings in our home with senior Allied Health Professionals and OT Managers. We had Alison our MPS advocate who was a tower of strength at these meetings and who really helped not only to referee, but also to fight both my corner and Roma's.

Eventually OT's agreed to Roma having her patio bedroom doors automated as well as the front door. Health and Safety arguments were used to provide this. Having now received a copy of the criteria for provision we persisted in arguing for Roma to be able to control her environment in her room. Eventually after more months of squabbling and about one hundred emails they agreed to the provision of an Evo Assist system, and that would be operated from Roma's ipad or iphone. The system would not only operate the environment in Roma's room, but it would also open the patio doors in Roma's bedroom. The subsequent application for DFG was posted to the Northern Ireland Housing Executive, and we thought we had made great progress.

Well, more disappointment followed, having spoken to the provider of the system several times, we eventually discovered that the doors could not be automated because the operating arms would protrude through the blinds and the curtains. I tried to argue for the provision of sliding doors, and despite being told by an OT manager that they would 'do what it takes' to help Roma, this promise never materialised. By this stage Roma's condition had further progressed. Her corneal clouding, a feature of Morquio had worsened. This accounted for Roma's need for electric light during the day, and it also made sense of her poor tolerance for light in the morning, preferring a gradual build up of light when she wakens up. No matter how hard I tried to explain this to the OT's they simply failed to appreciate the problem, advising that blackout blinds be used as a solution. But that would not solve the problem of the need for graduated light and the need to see outside, that function is only available via venetian blinds. A solution, which I proposed, was to provide sliding doors with integral venetian blinds that could be automated. The cost of this was extortionate, however it would still have meant the grant money was much less than most Disabled Facilities Grants.

After more arguing over this, I just got so fed up I told the OT's just to provide Environmental Controls in Roma's room and not to bother with automating the patio doors. I think I just wanted to give up at this stage. Surprisingly they agreed to this, but we are still left with a fire risk, Roma could not escape from her room onto the patio if there was a fire. Her bedroom door which leads into the utility room is automated but the door leading from there into the kitchen is not.

Despite the criteria for provision stating that media can be used on the Evo Assist System, the OT's nitpicked over this function so we have asked for it to be provided at our own expense. We argued for a light to be put in the adjoining wetroom, but they said no, but agreed to lights in the bedroom being automated. Extra functions will be added gradually which we will also pay for, we will in time replace the blinds and patio doors with ones that will be compatible with both the system and the need for curtains and blinds.

The Evo Assist will operate Roma's curtains in front of her bed, her bedroom door, her bedroom lights, TV and satellite stations, and she now has a camera on the front door which allows her to see who is there.

It has been such a long and protracted struggle to get this. One distressing aspect was when the OT Manager dealing with the case died. No-one bothered to tell me about this and I discovered this from reading the newspaper.

We are still getting used to the system, and it is really helpful for all of us. It is so nice for Roma to be able to open her curtains in the morning

allowing her time for her eyes to become accustomed to the light. This prevents disorientation that previously was a part of her mornings. One thing I would advise though is to think about installing this system at the same time as having main DFG work done, because the system cannot be put onto some features that have been installed retrospectively. For example we really would have liked Roma to be able to control the heating in her room considering the problems with body temperature that many young people with Morquio have.

I have learned from this experience that persistence pays off but I do not think I could have achieved all this without the MPS Advocacy Team. I have also been made aware just how hard it is for OT's working on the 'front-line'. Many do not seem to receive the training or CPD that their managers do. At times I simply lost patience with the OT's particularly when one described the ability to open curtains as an 'enhancement'. I am also sympathetic towards OT's who are constrained by a Trust culture, which just seems to dwell on the financial cost of things rather than quality of life. The nitpicking and haggling brought out both the worst and the best in all of us, but I am happy to say we are still friends.

Bernie Drayne

If you would like support from the MPS Advocacy Team:
advocacy@mpssociety.org.uk
0845 389 9901

“Research changed my life”

During November and December the MPS Society took part in a national campaign called “Research Changed My Life” to help patients to understand the positive effects that can result from getting involved in clinical research.

The campaign was organised by the National Institute for Health Research (NIHR) Clinical Research Network and used inspirational stories of people who have been involved in clinical research to help raise awareness.

One of these 18 individual stories was from 13 year old Sultan Ali who has Morquio MPS IV. Read his story...

Sultan Ali, Birmingham

Research changed my life because... now I’m really happy.

I am 13 and I was born with Morquio. It’s a condition which affects the way my bones grow; they are weaker and it means that I don’t grow as fast as other people. It affects my daily life as it affects my ability to walk and stand.

Four years ago I was offered the chance to take part in a research study by my doctor. He asked if I would like to be one of the first people in the world to try a new medicine made for people with my condition. The research study is designed to see whether the medicine can cure or just help people like me.

My mum and dad said that I could take part but it was ultimately down to me and I said yes because I wanted to take the chance. Before I got involved in research I didn’t know anything about it, but on the very first day I learnt so much about clinical research and how it helps people. I also learnt that research doesn’t just help people with my condition, it helps everyone.

I visit the hospital every week to have an injection which is put into my arm. At first I felt scared but now I understand what happens, I’m not scared anymore. Whilst the medicine is going in I just chat with my friends, do school-work or play on the PlayStation. I also get to speak to the nice research nurses every week about any problems I might have.

Since taking part in research I feel much stronger, I have more stamina and I don’t feel as tired anymore. I can play on my PlayStation for longer as my hands don’t get so tired which means that I can play with my friends more and work towards my dream career goal of being a game designer.

By taking part in research, I haven’t just got stronger, I’ve got to meet other people with my condition which is good because now I know I’m not the only one out there with Morquio.

If anyone asked me whether they should take part in research, my advice would be to take the chance - I’m a million percent happy that I took part.

After diagnosis: how can counselling help parents to cope?

By **Tim Copsey**

Counselling is a word with several definitions, including the giving of advice or legal direction. The British Association for Counselling and Psychotherapy defines it simply as “an umbrella term that covers a range of talking therapies”, and encompasses the traditional view of a gently empathic listener, an analytical cognitive therapist, or even the old school Freudian psychoanalyst delving into childhood memories.

After two years as a bereavement counsellor and a student on an MSc programme in integrative counselling, the best definition I can offer is that it is a shared journey where the client wishes to achieve some kind of internal or external change, whether by making a decision, learning the strategies to cope with a difficult reality, or simply by making sense of their own thoughts and feelings about a situation.

The role of the counsellor is to assist this exploration, by listening and by applying a range of specialised skills, by giving the client tools which will help them to adapt and take control again, and by pointing out details on that journey which may be significant and helpful.

Every therapist works in a different way, and not every client/counsellor relationship is a perfect match.

Research has shown repeatedly that the most important factor in successful counselling is the degree of trust and the working relationship between both individuals. It’s about finding someone who feels right for you, and a good counsellor will find the best way of working with their client.

My interest in counselling developed long before I had even heard of MPS, but my experience as a client began when my own daughter was diagnosed with Morquio syndrome six years ago. I experienced the diagnosis as a severe, traumatic event, and I found that counselling helped me to accept and adapt to my fundamentally changed reality, and to become positive about my child’s future. **CONT on page 11...**

The Nightmare of Alterations

To tell this story I really need to go back to the time. I was diagnosed with Fabry Disease in 2002 and after the diagnosis I was sent on a journey that involved the social services and so to the next 10 years of life with a new partnership.

Sarah was my first occupational therapist to help me with this disease and advise what I may need going forward. It was decided I needed a stair lift, a wheelchair and a stick and this was the first time that I had needed to give a full breakdown of the income that was coming in to the house and this was submitted to the local council as they were responsible for awarding the grant. It was approved and I had my stair lift. However, on one visit it was decided that there should also be an extension on the back of the house for the future so it was decided to go down this route.

As the best laid plans of mice and men went, the extension did not happen and we were advised to move. The bungalow came along and the best thing was that the bathroom had already been converted so the OT was very happy but things never quite run to plan, as we ended up with a new OT; Alan. In the time since Sarah had moved on and Alan came I had had 2 strokes and I could no longer walk without support, the current shower room was no longer big enough.

We knew a lot of work was going to need doing. It ended up with an external extension as well as work inside as we had to take part of the dining room. Not only did the shower room need work, but also things needed doing in every room that I use, such as all the plugs needed to be raised and light switches lowered. Also, as we had to widen the doors, it meant the floor covering needed replacing and walls decorating.

For all the work to be completed we needed all the different building trades to have a part in the project. This also meant that we needed to move out for a minimum of 6 weeks (as it was in the end 7.5 weeks) but I needed wheelchair access. If you have ever needed to move fast it is hard let alone to find a place that has a wet floor shower and is wheelchair friendly. It was hard but Ann did it and she found a brand new wheelchair friendly mobile home (on one of the holiday parks) and we could have it for 6 weeks. Wonderful.

So we moved into the mobile home with help from good friends who moved us out and into the temporary accommodation. The builders turned up at the bungalow and started to take the bungalow apart and after the first week they had cut out all the door frames for the widened doors and they had taken the side wall out to make the extension. As we very quickly found out our home was taken apart much faster than it was rebuilt.

The builders were left to do their work but we did need to keep a close eye on the work to be sure that we got (or I got) what I needed. I have to say they have done a very good job and it has made life a lot easier.

In all honesty the work was the end of the process, the application process takes a little time and you do need to disclose your financial situation. There is quite a bit of paper work to do before they can tell you if you need a deposit or in fact how much you need. We are going through the process again to get the outside altered so I can use my garden from the wheelchair so that is to come, so watch this space.

By **Ian Hedgecock**

Before and After!

CONT from page 10... I have kept an interest in the value of counselling to disabled individuals and their families, and now find myself in a position to carry out research into the possible benefits of counselling for adults who, like myself, have had to cope with the shock of adjusting to the diagnosis of a rare disease in their child.

The aim of my research is to qualitatively explore the experience of counselling for parents of children with MPS and related diseases, and whether it provided any benefits or fulfilled expectations. Adjusting to conditions such as the MPS diseases creates an exceptional set of challenges for a parent, and I am interested in finding ways that counsellors can be better at helping clients to overcome these challenges, by learning from the experiences of those who have had counselling in the past.

I will soon be inviting individuals to participate in this study. You will need to have undergone and completed or ended counselling or psychotherapy in some form, following a clinical diagnosis of a disabling condition in a child of your own. This does not have to have been an MPS disorder. I would ask you to be willing to engage in an interview about your experiences, lasting up to an hour.

Confidentiality will be rigorously maintained to protect the privacy of participants and other parties, and the research will be ethically overseen by Staffordshire University as well as complying with the ethical framework of the British Association for Counselling and Psychotherapy. I am interested in any experiences, positive or negative, as the eventual goal is to inform other counsellors of a best practice approach to counselling parents in this position.

If you have any questions about this article or would like more information, please contact me by email at **t.copsey@staffordcoll.ac.uk**. Recruitment of participants is expected to begin from 1st February 2014.

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** 0845 389 9901– 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 claim forms for Personal Independent Payment, 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

- **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**
- **Benefits including Personal Independent Payment, Benefit Cap, Council Tax Benefit and Universal Credit**

Each of our England based 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

SOPHIE
Manages the MPS Advocacy Team

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 I Hurler BMT,
Hurler Scheie, Scheie
MPS VI Maroteaux-Lamy
MSD, ML II

ALISON
Supports members living in Ireland

All Ireland Advocacy and Support Update

The winter season is always a busy one for the Advocacy Team and the same applies over in Ireland! There have been a few changes in the last few months and now seems like a good time to update the wider MPS community on what's been going on.

As some of you may remember from my introduction article back in 2010, I am a qualified Genetic Counsellor and as part of my role I provide one day per week of genetic counselling for the Northern Ireland Regional Genetics Service. I love this little bit of variety in my role but as time went on I was being called upon more often to support our MPS and Fabry patients in relation to their medical management.

So the decision was made in mid 2013 to focus my Genetic Counselling day on clinic coordination for MPS, Fabry and other metabolic conditions.

As genetic counsellor for metabolic patients the main duties include the following.

- Coordinating the MPS clinics, including ensuring all patient notes are up to date and complete
- Acting as a link person between patients and medical teams, including communication with local and community services
- Reviewing individuals medical notes ensuring patients are not lost to follow up
- Providing genetic counselling to families affected by MPS and related diseases
- Support to patients on Enzyme Replacement Therapy (ERT), liaising with home care agencies and dealing with any treatment related issues.
- Referrals to other services as appropriate and outside of the advocacy support role

We really hope that this will provide our members with a better, more comprehensive service.

Should anyone have any queries or concerns about this change please do not hesitate to get in touch with Sophie on 0845 389 9901 or Alison on 07786258336/02895047779.

Genetics on the Road!

Over the last few months the MPS Society All Ireland Advocacy Support Service has been represented at a series of information evenings hosted by the National Centre for Medical Genetics in Dublin.

These events were held in Cork, Sligo, Mullingar and Belfast – and for those of you not familiar with the geography these were selected to cover North, South, East and West Ireland. The information evenings aimed to update primary healthcare professionals about advances and changes in the world of genetics.

It's always great for the MPS Society to be represented at these types of events. Our talk focussed on the difficulties that parents, carers and individuals affected by MPS experience when filling in medical and social welfare forms. As you might expect, a few of the medical professionals took a sharp intake of breath when they were presented with the list of forms that families have to negotiate! **Alison Wilson**

Bereavements

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

Inayat Chowdhury who suffered from Hunter disease and who passed away on 23 September 2013 aged 6 months

Ben Richardson who suffered from Sanfilippo disease and who passed away on 25 September 2013 aged 14 years.

Andrew Millar who suffered from Sanfilippo disease and who passed away on 7 December 2013 aged 43.

In memory - Ben Richardson, 19/11/98-25/9/13

It is with heavy heart that I tell you my super special boy Ben Elliot Richardson gained his angel wings on 25th September 2013 age 14 years.

Ben has two younger siblings, Georgia 13 years old and Mackenzie who is 10, pictured right.

Ben enlightened and enriched our lives and it is a joy to be his mother.

I feel Ben was an angel who was lent to me to look after and it has been a privilege to have been part of his special journey.

We love you Benji boy more than words can say, you are and will always be the best thing since sliced bread.

Rest in peace our darling angel. All our love, always and forever. Mummy, Georgia and Mackie xxx

Advocacy - Focus on ...

IS YOUR CHILD BEING ILLEGALLY EXCLUDED FROM SCHOOL?

Illegal exclusions are more common than you think, especially for children with disabilities, special educational needs (SEN) and additional needs

Illegal exclusions can include the following;

• Requests for children to be picked up at lunchtime due to lack of support / supervision.

• Requests for children to be dropped off or picked up early.

• Children not being able to partake in class activities / school trips due to being assessed as not suitable or schools not being able to provide the additional support required.

• Requests for children to be picked up as they need time out or time to cool off.

• Being told that a child should be picked up or not to attend as it is for their own good as they are having a bad day.

• Children being put on a part time timetable.

A child with an identified disability, SEN or additional needs should never be excluded or prevented from attending or partaking in school activities whether on or off site due to lack of support.

Schools should be taking early action to prevent crises from occurring, working in partnership with other local authority bodies (including health and social care) to ensure that children have fair access to education with adequate support.

Where exclusion is necessary, appropriate statutory measures should be taken to ensure that these exclusions remain lawful.

A child can only be legally excluded from school for disciplinary reasons.

Any formal exclusion must include in writing the following;

• The reason for the exclusion

• The length of the exclusion

• Information for parents/ carers on how to appeal the decision and offer parents / carers the opportunity to meet with the school governors

• Parents / carers must be informed that during school hours a child is not allowed in a public place for the first five days of the exclusion

• That arrangements for provision of full time education will be arranged from the 6th day of the child's exclusion from school.

Informal or unofficial exclusions are illegal even if they occur with parents / carers consent. They are not subject to review and have no statutory timescales attached to them. It is therefore important that if you suspect that your child is being illegally excluded that this is addressed immediately and appropriate support given. Illegal exclusions can impact the whole family and have a negative impact on the child's wellbeing and success at school.

For further information and to read the survey (Falling through the net, illegal exclusions, the experiences of families with disabled children in England and Wales 2013) conducted by Contact a Family, please go to their website www.cafamily.org.uk

If you suspect that your child is being illegally excluded and would like further advice, please contact the MPS Society's Advocacy Support Team.

Further information

For more information, please contact the MPS Advocacy Team by email at **advocacy@mpssociety.org.uk** or by phone on **0845 389 9901**.

FOR SALE!

Turny orbit car seat with a Carony wheelchair base. The seat has various adjustable supports and a lap belt that is worn in conjunction with the vehicles adult seat belt.

This is a fully automatic seat with remote control that lifts the person in and out of the car. When lowered out of the car it can attach to the Carony wheelchair base with foot supports enabling easy transfer from the car to the destination.

The equipment was purchased and fitted through Motability for £4100.

The equipment is for sale for £800.

We also have a Crelling harness that can be attached to this car seat to enable postural support which cost over £100 new, now being sold for £40.

Please contact the MPS office for more information.

STATUTORY PROCESS FOR STATUTORY ASSESSMENT FOR SPECIAL EDUCATIONAL NEEDS

What is it: A statutory assessment is the legal process in assessing your child's educational needs.

Who can make an application: A parent or a school can make a referral to the Local Education Authority if a child is identified as having special educational needs and the school is unable to put in the extra support needed. Examples of this are; 1:1 support throughout the day, or input from specialist services i.e. speech therapy.

Initial request: Following the initial request the Local Authority has six weeks to consider the request and whether they will assess the child. The Local authority can refuse to assess your child if they do not believe that your child's difficulties are severe enough or that the school can provide the support.

Who is involved in the decision: On receipt of a request, the Local Authority will decide whether to carry out a statutory assessment. At this stage they will request supporting information from a number of people and this may include the following; parents, the school, educational psychologist, Social Services, doctor.

Time scale: The assessment process should be completed in 26 weeks, although if specialist or more in-depth advice is required then this timescale may be extended.

Draft Statement: Once all information has been gathered a draft statement may be drawn up for consultation and parents can say if they agree or disagree with the content.

Final Statement: After all the recommendations from the draft statement have been reviewed the Local Authority will issue what they believe to be the final statement. This may not have taken into consideration, recommendations for changes etc and therefore it is important that you read the final statement thoroughly and if you do not agree with the content you do not sign it.

If you disagree: You have to put in writing to the Local Authority that you do not agree with the statement and request that this proceed to the Special Educational Needs (SEN) Tribunal. The Local Authority should send you details or how to appeal to the SEN tribunal service.

Remember to keep copies of everything you submit or receive.

What is a Statement of Educational Need: A document covering; personal information about the child (full name, Date of Birth etc), information about your child (Eg about their diagnosis, their condition, how it affects them, their needs are etc), what support needs to be put in place, the name of the school, your child's non educational needs (Such as health and social care needs -physiotherapy, transport etc).

Statements are usually reviewed annually but can be brought forward if the child's needs have changed significantly.

What happens if the Local Authority does not issue a statement? If the Local Authority decides that your child does not require a statement they should write informing you of this decision with an explanation. The Local Authority should also inform you of your right to appeal to the Special Education Needs tribunal and the Disagreement Resolution Service. This must be done in writing within two months of the date shown on the letter. It is also advisable to inform your local authority and your education officer that you have appeal their decision.

Through my big brown eyes

Written by a family friend of the Brentnall family (Jacob MPS III) following their experiences at a soft play centre.

Wind swept hair and my special chair that doesn't bother me.
I like to explore like the other kids around, then rest and restore my energy, as I sit and look through my big brown eyes.

I may be strong, mighty and courageous but that won't stop me having to be last and wait for ages, I may be different in a certain way but I still love to laugh and play as I sit and look through my big brown eyes.

So please be kind and patient too. It may not come across but I really am not as lucky as some of you, so when I smile through my big brown eyes you have touched my heart and feelings inside.

I may not talk but signing I do, I can also let you know I can hear you, the world and the fear I have to face, sometimes all I need is some space looking through my big brown eyes.

If you find this hard to accept, take a look at your life and don't let words you have said make you regret, for what you do not see is what I see through my big brown eyes.

Written on behalf of a very special little boy, please think about being judgemental. Every child has a right to play regardless of any additional needs.

Gilastic Cataplexy

Gilastic Cataplexy is defined as an abrupt temporary loss of voluntary muscular function and tone, evoked by an emotional stimulus such as laughter, pleasure, anger or excitement (definition from the Academic Press Dictionary of Science and Technology).

Gilastic Cataplexy occurs when a person experiences an emotional stimulation – laughter, excitement, anger, stress etc. The stimulation leads to a very rapid loss of voluntary muscle control. This may take the form of the head flopping forward but often the person will collapse as a result and fall to the floor. The collapse occurs because the person can no longer control their leg muscles to remain standing. As soon as the stimulation is reduced, the person regains muscle control. This may last from a few seconds to a few minutes. The individual will remain conscious throughout and although they may appear a bit shaken (either from the fall or from the surprise of collapsing) they usually gain full control after a few minutes.

Gilastic Cataplexy is a feature seen in some of the Lysosomal Storage Diseases and is quite commonly seen In Niemann Pick Type C and occasionally in other LSD's (for example Sanfilippo disease).

Gilastic Cataplexy itself is not a serious medical emergency. However the abruptness of the collapse and the risk of falling against something, falling awkwardly or hitting their head when they fall can be dangerous.

Gilastic Cataplexy can sometimes be controlled through medication but where it can't the use of protective headgear may be required.

Gilastic Cataplexy is often confused with seizures, especially if you are not familiar with the symptoms. Always get concerns checked but some differences to be aware of are: Gilastic Cataplexy occurs rapidly, it occurs during times of emotional stimulation, the person recovers almost immediately and the person is responsive during the event. With seizures, they will occur at both quiet times and periods of stimulation and tend to have more warning signs. After a seizure, the person will recover more slowly and may not remember what happened.

Some experiences from families;

"People out there must think that we are shocking parents as we tell our daughter to stop laughing! You'll fall over... which is normally what happens. She just sort of slumps down slowly, laughing all the way!"

"Cataplexy for us as a family was very difficult when it first started to present itself. Not only was it heart-breaking to see our daughter go through this but the constant worry of her hurting herself, which she often did, resulting in some really nasty head of facial injuries"

"Our daughter would suddenly stop dead, look very wide-eyed, and drop to the floor within seconds. Her body would become like a rag doll slipping through your hands as we tried desperately to support and protect her. Then within seconds, you would feel her body regenerate, back into life. It often left her wiped out for a few minutes. We do believe that she knew what was happening as she would suddenly look at you before dropping."

"Cataplexy is an extremely difficult condition to deal with. It is impossible to predict when she will suffer an attack. However, we soon realised that you cannot remove laughter from a child's life – now we just catch her or pick her up, dust her down and get on with life"

Editor's notes; this article has been updated from an entry in a previous magazine. The original piece was taken from article written by Jackie Imrie, who at the time was a clinical nurse specialist at the Royal Manchester Children's Hospital.

Help us paint the town... blue...!

We need you to get your blue thinking caps on! We want to make this year's MPS Awareness Day the biggest, bluest yet, but we can't do it without you.

Have a think about how and where you can paint the town blue for MPS Awareness Day on May 15th.

What can you do at work; will your company support a one day fundraiser for the MPS Society on 15th May? What about your local schools and nurseries? Last year we had some fabulous pictures of children in blue from across the UK, will your school do something? Do you need to ask the teaching staff if they can put May 15th into their school calendar now?

Can you think about where you live, shops, pubs, brownies, scouts, leisure centres? This year we really want to get people thinking nationally, about families affected by MPS, Fabry and related diseases.

Wear It Blue, hold a blue cake sale, a blue quiz, a blues night, hold a blue Wicked Walkabout, get sponsored to eat as many blueberries as you can in one minute. Get imaginative and please start planning now.

If you let us know what you are planning, we can support you with posters, balloons, sample press releases.

So whether you live in John 'O Groats or Lands' End, a busy town or a smaller village we want to try and have as many Wear It Blue events as we can across the country on May 15th.

Email **fundraising@mpssociety.org.uk** to let us know what you are planning and get your name down for our new Wear It Blue pack.

MPS Regional Specialist Clinics

Winter 2013

The advocacy team have been busy, attending clinics across the country.

Since the last magazine we have travelled to London for the MPS III clinic at Great Ormond Street Hospital, Birmingham Children's Hospital twice for an MPS clinic and a Fabry clinic, Manchester Children's Hospital, twice for the MPS I post-

transplant clinics and Alison Wilson held an MPS clinic in Northern Ireland.

The purpose of these clinics is to not only allow individuals to regularly see one of the specialist consultants but to also be able to see other medical professionals and hopefully limit the number of separate appointments individuals and families have to attend.

The clinics also allow individuals and families to meet together and to access support from the MPS advocacy support team, who attend these clinics. Thank you to all the medical teams who attended the clinics and helped in their organisation.

Below is a selection of photos from these clinics. **Sophie Thomas**

If you would like support from the MPS Advocacy Team:
advocacy@mpssociety.org.uk
0845 389 9901

Family Weekend at Legoland

In August 2013 the MPS Society organised a family weekend at Legoland Windsor Resort so that MPS families could join us for a weekend of fun-filled activities!

We were so pleased that so many MPS families could join us for the weekend. Arriving on the Friday at our hotel, the Copthorne Hotel in Slough, we started the weekend with a welcome buffet dinner and children's entertainment. This gave everyone a chance to get to know each other and everyone to receive their tickets for the park.

Saturday was a sunny day and everyone was up bright and early to take advantage of a full day in the park. Among the favourites were the Dinosaur Experience and the Big Lego car ride. The Duplo Splash Park was also open for this year so some of you had a great time.

The park was fully accessible to buggies and wheelchairs and Ride Access Passes were available to children and adults with disabilities to help cut down queueing time.

Saturday evening saw many of you enjoying some dinner in the hotel and chatting with other MPS families before heading to bed in preparation for another day in the park.

We would like to thank the following charitable foundations for their support in making this event possible: The Gosling Foundation, The Clover Trust and the William Brake Charitable Foundation. Here are a selection of photos from the day.

"We just wanted to say a huge thank you for organising the trip to LEGOLAND. We thoroughly enjoyed ourselves and the boys had lots of fun. James and I also wanted to thank you for organising the evening entertainment and meal on the Friday night - we thought it was a very valuable experience meeting other families for the first time. Joe (MPS II) particularly loved the big Lego car and Ben the dinosaurs." ***Emma, James, Joe and Ben (Hiller)***

Harry Potter Family Day

We were delighted to receive funding from Help A Capital Child to take families from the London area on an exciting group excursion to the Warner Bros. Studio Tour "The Making of Harry Potter", on Saturday 20th October.

All the families had a wonderful day, supported by MPS staff Gina, Sophie and Martine.

What an experience if you are a Harry Potter fan and even if you aren't, this journey into the making of the films will make you want to see them! We were amazed by the animatronics and astounded by the model of the castle that changed before our very eyes. If you ever wondered about a film set then this is an opportunity you won't want to miss! Here are a selection of photos from the day.

"We had only seen one film and bits of others ,when the MPS Society announced the day at Harry Potter Warner Brother Studio Tour, but thought what a chance to see something amazing.

After being given our tickets by Martine and Gina we went inside and after explaining to the staff that we needed to bypass the cinema we did unbelievably have the Great Hall of Hogwarts to ourselves for a few minutes.

We spent the rest of the day seeing some things we instantly recognised and some things we were not so sure about. Halfway round there was a place to get a coffee and see the night bus or Ron Weasley's car then stand outside the houses in Privet Drive , catching up with other families on the way round.

We ended up in the gift shop seeing which film to buy and we now have all eight films .

If you are thinking of a day out the staff at Warner Brothers were fantastic and nothing was too much trouble. They were radioing ahead telling staff of our progress making sure that Harry would be ok in the different areas.

It was a wonderfull day all made possible by the MPS Society. Thanks to the Society we as a family had a fantastic day out, one that due to Harry's condition would probably not have thought of going to see such a place.Thank You."
Ian Evans, father of Harry (MPS I Hurler)

MPS Childhood Wood Planting

On Sunday 13th October, 48 of us gathered at the Clumber Park Hotel in Worksop for the annual Childhood Wood remembrance planting.

We were welcomed with a tasty two course lunch and an introduction to the Childhood Wood. The Childhood Wood is a lasting memorial to children and adults who have lost their lives to an MPS or related lysosomal storage disease. The tree planting gives bereaved families the opportunity to plant a tree in memory of the child they have lost. The Wood provides a lasting and welcoming area where bereaved families can visit and remember loved ones.

We were privileged to have the continued support of Commander Judith Helen Swann, Councillor John Allin & Mrs Mavis Allin by their attendance. They helped to remember those who have recently lost their lives and whose families' couldn't be there, by planting the tree and letting the balloons go on their behalf.

After lunch, we departed for Sherwood Pines in convoy, with each family clutching one or two helium balloons with a child's name on a tag on the end. Most of us opted to follow the "white route" to the Childhood Wood - which was about a ten minute walk through the beautiful Sherwood forest.

We were very lucky and thankful that the wind and rain which had been present all weekend, managed to stay away literally just for the hour of the remembrance planting!

After a reading by Trustee, Wilma Robins entitled "Rembember" the families set off their helium balloons and then proceeded to plant their tree saplings.

This year we were remembering the lives of the following:-
Megan Elizabeth Fasey; Libby Fullalove; Landon Owen; Paul Franklin; Anabelle Rose Shepherd; Jamie Moxon; Alice Victoria Marston-Taylor; Jack Atkinson; Thomas L Fisher; Jake Lloyd; Emily Grace Otway.

I went to the Childhood Wood in 1993 for the first tree planting remembrance day. Back then each tree had individual inscribed plaques. However, as the Wood has grown over the years, the plaques have transferred to a memory board. The memory boards contain the names of each individual that have been commemorated with a tree along with a short message of remembrance from their family. Today there are over 300 trees and we even have wooden play equipment with games for children to enjoy and toadstool seats and picnic bench areas. If you get the chance to go to the Wood, I highly recommend it. The Childhood Wood is in a wonderfully tranquil setting – the perfect place to say goodbye to those we have recently lost, and to remember and celebrate their lives. **Jessica Reid** Trustee






Reimbursement of high Cost Therapies for LSD patients in Scotland

On 8th October 2013 the Scottish Health Secretary, Alex Neil, announced its drug approval process was to be revamped.

As a result Scottish patients will benefit from an increased range of new medicines with a package of measures that will ensure Scotland's drug approval system becomes more transparent and increases access to medicines for end of life care and treating rare conditions.

Alex Neil has directed the Scottish Medicines Consortium (SMC), the body which approves medicines for use in the NHS in Scotland, to apply different, more flexible, approaches to the evaluation of medicines for ultra-rare conditions like Fabry, MPS I, MPS II and MPS VI.

Additional Improvements Include:

• Extensions of the £20 million Rare Medicines Drug Fund to 2016.

• Imported patient support for engaging in the medicines approval process.

• Additional investment of £1 million to support SMC to make their work more transparent.

• Creation of a new peer approval system to allow clinicians to prescribe medicines that are not accepted for routine use by the SMC. (This will replace Individual Patient Treatment Requests).

• Opportunity for the SMC to appraise new medicines which have not yet been submitted by pharmaceutical company where these are considered clinically imported to NHS Scotland.

Following the announcement, the Inherited Metabolic Disease Scotland network hosted a regional meeting in Glasgow on Thursday 10th October 2013 to educate healthcare professionals from a non-specialist background, working in secondary or tertiary care, about the presentation, diagnosis and long term management of Lysosomal Storages Diseases chaired by Dr Bernd Schwahn.

The aim was to stimulate increased awareness of diseases and improve outcomes for affected individuals.

Dr Patrick Deegan, Clinical Director, Addenbrookes Hospital at university of Cambridge spoke about the 'closing of the gap between clinical presentation and diagnosis.' Dr Chris Hendriksz, Clinical Lead at Salford Royal Foundation NHS trust gave an overview of 'New Developments in ERT' and Catherine Stewart, LSD nurse at Birmingham Children's Hospital spoke on 'Home ERT – The practicalities'.

Scottish speakers included Dr Peter Gallaway Consultant Clinical Biochemist at Southern General Hospital Glasgow and Dr Peter Robinson, Consultant Metabolic Paediatrician at the Royal Hospital for Sick Children, Glasgow.

A small intimate group of twenty people were present of whom ten including four speakers are involved in the diagnosis and clinical management of children and adults in Scotland. **Christine Lavery**

Changes to Homecare update

Medco have now pulled out of the Homecare market in the UK for all of their contracts including the LSD ERT service for Fabry, MPS I, MPS II and MPS VI.

Medco have identified the opportunity to TUPE their nurses across to the other two companies Healthcare at Home and BUPA, therefore keeping the nurses the same for our members and also retaining their skill set.

It is anticipated that all nursing patients will go to BUPA whilst independent patients may go to Healthcare at Home of BUPA. **Christine Lavery**

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 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 listed on the website 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

MPS VII

Intravenous Enzyme Replacement Therapy for MPS VII, Sly Disease

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

Genzyme Fabry programme update

Genzyme, a sanofi company is currently enrolling young male Fabry patients aged between 5 and 25 years into “A natural history study of renal function in treatment-naïve, young male patients with Fabry Disease”. Different sites in Europe and the USA are expected to participate. Travel costs for the participant and one parent are provided for the planned visits. Detailed information may be found on www.clinicaltrials.gov. Please speak to your treating physician if you or your child would like to participate.

Amicus Postpones marketing approval filing with the FDA

Amicus Therapeutics Inc. announced on 17 June 2013 that it will delay seeking US approval of Amigal, its experimental treatment for Fabry disease, until the second half of 2014 at the earliest. Amicus and its partner on the drug, GlaxoSmithKline plc., may need to wait for an extra year of study beyond the 12 month plan to ensure approval by the Food and Drug Administration.

In December 2012, clinical trial results showed Amigal performed no better statistically than a placebo, and the FDA refused to amend its plan for the failed study, prompting filing postponement. Forty one percent of patients given the drug were counted as responders, compared with 28% of the placebo group. A difference that failed to meet the statistical significance under FDA guidelines.

Good news for Fabry Patients in the Netherlands

On 3rd October 2013 Minister Schippers brought to a halt a year of uncertainty for Fabry patients in the Netherlands. Previously the Netherlands government had committed to cutting reimbursement for Fabrazyme and Replagal but that has all changed now with the announcement that Fabrazyme and Replagal are to be reimbursed from the basic healthcare insurance. According to the Minister a substantial price reduction has been agreed with Genzyme and Shire however the level of price reduction will not be made public as part of the agreement.

New Disease Standard Operating Procedures for Fabry

These new standard operating procedures for adults and children offer an overview of Fabry disease, clinical features and disease progression as well as principles of treatment. These SOPs also set out the standard of care a patient can expect to receive including recommended investigations and time scales.

For further information

www.specialisedservices.nhs.uk/library/23/FabryDiseaseStandardOperatingProceduresAdultsandChildren.pdf

Synageva conducting natural history and imaging studies in patients with MPS IIIB

Synageva is a clinical stage biopharmaceutical company focused on the discovery, development, and commercialisation of therapeutic products for patients with life-threatening rare diseases and unmet medical need. Synageva has multiple protein therapeutics in its drug development pipeline. The company has a team with a proven record of bringing therapies to patients with rare diseases.

MPS IIIB is caused by a decrease in alpha-N-acetyl-glucosaminidase (NAGLU) enzyme activity which leads to the buildup of abnormal amounts of heparan sulfate in the brain and other organs. The buildup of heparan sulfate leads to the serious complications of the disease. Synageva is investigating SBC-103, which is a recombinant form of the NAGLU enzyme, as an enzyme replacement therapy for patients with MPS IIIB. Studies in an animal model of MPS IIIB have shown that SBC-103 can reduce heparan sulfate in the brain and other organs.

Synageva is currently conducting natural history and imaging studies in patients with MPS IIIB to better understand the disease progression and characteristics. The first planned human clinical study with this enzyme will evaluate the safety and activity of SBC-103 in patients with MPS IIIB. This study is planned for initiation in mid-2014.

Further information regarding these clinical studies can be obtained from Synageva BioPharma:

Sponsor of clinical trials:
SYNAGEVA BIOPHARMA CORP
33 Hayden Ave, LEXINGTON MA 02421
UNITED STATES
Phone : 781 357 9900
Fax : 781 357 9901Contact:
clinicaltrials@synageva.com

Further information regarding MPS IIIB is available at the following patient organisations:

- The MPS Society) UK
www.mpssociety.org.uk/
- International MPS Network
www.impsn.org
- National MPS Society
www.mpssociety.org
- Canadian Society for Mucopolysaccharide & Related Disorders www.mpssociety.ca

Developing Therapies for Sanfilippo Disease: Genistein Clinical Trial

There are currently no approved therapies for Sanfilippo disease. The research initiatives that have resulted in clinical trials or are progressing towards clinical trial have one of two objectives; either to boost the enzyme levels or reduce storage (GAGs) through substrate reduction.

Genistein can be purified from soya beans and is recognised as a food supplement. In the pure Genistein the broad spectrum protein, tyrosine kinase inhibitor, blocks GAG production in patient cells from all MPS types tested.; MPS I, MPS II, MPS III, MPS VI and MPS VII. This was published by Piotrowska in 2006 in the European Journal of Genetics.

Genistein in MPS IIIB Mouse Model
Mice with MPS IIIB show similar behaviours as children with MPS IIIB, principally hyperactivity. Mice treated with 160mg/kg of pure Genistein were shown to behave very much like normal mice. There was a 30% improvement in Heparan Sulphate in the brain; 12% improvement in the astrocytes; 15% improvement in the microglia and as a consequence behaviour in the MPS IIIB treated mice were fully corrected and neuro inflammation was improved.

Safety Data for Genistein
There is a good safety data in dogs of up to 500mg/kg as well as in Dr Barbara Burton's MPS III patient cohort on 150mg/kg study.

Rationale for Higher Doses in Humans
The mice data clearly shows a minimum effective dose to treat the brain of 160mg/kg with toxicity at 500mg/kg. Human dosing is normally 1/5th or 1/10th of the mouse dose. However:

The Dutch clinical trial using 10mg/kg a day showed no brain effect despite plasma, GAG and Heparan Sulphate reductions and an aglycone level of 12.

Humans degrade Genistein quicker than mice thus having lower aglycone levels. To achieve an effective dose in the brain in humans the Genistein Clinical trial dose will be 160mg/kg.

Recruitment
The Genistein Clinical Trial will take place at the Manchester Children's Hospital and opens for recruitment at the beginning of January 2014. 24 children with MPS III Type A, B and C will be recruited.

Recruitment has been delayed because it took all this time to get the drug into the UK from Switzerland and it only arrived two weeks ago.

The Clinical Trial is for 1 year duration with 1 year extension and will not involve any crossover. The total duration of the clinical trial is 42 months.

The Drug is GMP (Good Manufacturing Practice) grade manufactured by DSM and provided at no cost. If the drug is shown to be effective it can be licenced from this clinical trial.

Patent Criteria
- Confirmed diagnosis of MPS III A, B or C
- 2-15 years
- Able to walk unaided
- Priority will be given to UK patients due to language and limited travel budget

Dosing
The clinical trial participants will receive 2 daily doses of 80mg/kg. Adverse events, toxicity and endocrine monitoring will take place. Clinical trial outcome measures will include Urine GAGs and a number of psychological and developmental tests.

Recruiting January 2014
Principal investigator
Dr Simon Jones
Consultant Metabolic Paediatrician
Manchester Children's Hospital
Oxford Road, Manchester M13 9WL,
UK, Phone: 0161 701 2137/8
simon.jones@cmft.nhs.uk

Teamwork within the International Rare Diseases Community

I had the privilege of meeting in person Maryze Schoneveld van der Linde on 18th November 2013.

Maryze was born in the Netherlands in 1970 and was diagnosed with Pompe disease at the age of eight years old. Maryze is a wheelchair user and is assisted occasionally by mouthpiece air ventilation to help her with her breathing. In her presence, you can feel this overwhelming energy and determination to live a normal life, with her own consultancy work, her involvement with the International Pompe Association and her numerous voluntary advocacy projects. I found her life story extremely inspirational.

Few weeks ago, Maryze and I worked together to respond to the urgent request from Miroslaw Zielinski of the Rare Disease Association in Poland to gather a list of countries around the world where both Fabrazyme and Replagal were being reimbursed. With the help of an international network of contacts, we were able to produce a comprehensive list of countries that offer reimbursements - information that Miroslaw Zielinski needed to advocate for Fabry Patients in Poland the need to get the ERT treatments reimbursed by the Polish Health System.

This shows the importance of working as a team within the international rare diseases community in assisting each other and the people who are really proactive in fighting for the rights of patients.

Thank you Maryze for your help and to all those contacts who provided us with information, including the Heads of Medical Agencies in different countries who responded promptly to our urgent requests. **Nawel van Lin**
FIN Development Officer

Nawel van Lin and Maryze Schoneveld van der Linde. You can read more about Maryze on: http://www.worldpompe.org/pompe-disease/patient-testimonials/314-maryze

A Phase III, Double Blinded, Randomised, Placebo Controlled Clinical Trial of High Dose Oral Genistein Aglycone in Patients with Sanfilippo Syndrome (MPS III A, B and C) - GENiSIS2013.

Stem Cell & Neurotherapies Group,
Centre for Genomic Medicine,
University of Manchester, Willink Biochemical Genetics Unit, Centre for Genomic Medicine, St Mary's Hospital, Central Manchester University Hospitals NHS Foundation Trust

We will soon be recruiting for a phase III, double blinded, randomised, placebo controlled clinical trial of high dose oral genistein aglycone in Sanfilippo diseases (MPSIIIA, B and C). This is funded by the UK society for Mucopolysaccharide Diseases, The National MPS society and the GEM appeal in a grant to Dr Brian Bigger and sponsored by the Central Manchester Universities Hospitals NHS Foundation Trust. We expect to begin recruiting in early 2014, primarily from within the UK.

The trial will be regulated by the UK MHRA, performed at the NIHR/ Wellcome Trust Clinical Research facility in Manchester using GMP grade genistein aglycone. The trial will be one year placebo controlled with one year open label extension with robust efficacy and safety endpoints.

The Manchester Group published preclinical data in the mouse model of Sanfilippo disease IIIB (MPSIIIB) showing significant delay in neurodegeneration and behavioural correction following high daily doses of the drug genistein aglycone delivered over a 9 month period (Malinowska et al. 2010).

Genistein is a substrate reduction therapy drug and works by reducing the levels of accumulated HS in the body as well as the brain. It is also an anti-inflammatory agent. Genistein in food supplement form has been tested in several clinical trials for patients with Sanfilippo disease. Most showed no measurable clinical effect, although one trial showed reduction of urine GAGs over a year (de Ruijter et al. 2012). These trials used low daily doses of genistein (up to 10 mg/kg/day) in a supplement form. This is unlikely to reach the brain in sufficient quantity to affect neurological disease.

Genistein in the preclinical study (Malinowska et al. 2010) was synthetically produced and is the pure (aglycone) form of the drug. Genistein can also be purified from soy extract but this is not necessarily the same product, as the naturally occurring form of genistein may not be absorbed as efficiently by the digestive system.

High daily doses of genistein aglycone are more appropriate for Sanfilippo as humans have a higher rate of breakdown of genistein (Glucuronidation) than that of mice. This means that higher oral doses are required to achieve equivalent plasma and thus high brain levels of active drug.

High daily doses of genistein aglycone have been delivered to patients over a one year period in an open label safety study in the USA providing significant safety data on the drug (Kim et al. 2013). In order to establish if gensitein aglycone is effective at treating the brain in Sanfilippo disease a placebo controlled clinical trial using high oral doses is required.

About the Manchester Group
The Manchester group handles over 15,000 diagnoses per annum for pediatric metabolic diseases from over 30 countries and see approximately 350 lysosomal disease patients every year. They are a nationally designated centre for lysosomal disease management in the UK, helped write national and international guidelines for management of many lysosomal diseases and contributed to several natural history studies.

They have been involved in over 30 clinical trials, some of which led to licensing of 6 drugs worldwide (of 9 available) for lysosomal storage diseases, including trials for mucopolysaccharide disease (MPS) I, II, IIIA, IVA, VI, Fabry, Pompe and Niemann Pick C. As a result, over 800 lysosomal disease patients now receive treatment in the UK and several thousand worldwide.

Research in the Manchester group has helped to move enzyme treatment into the home, thus improving quality of life for the majority of lysosomal disease patients on enzyme replacement in the UK. It has also broadened the scope of haematopoietic stem cell transplantation for lysosomal disease and reduced mortality, benefiting over 100 patients worldwide. Preclinical treatments in development for lysosomal diseases include high-dose genistein for MPSIII, haematopoietic stem cell gene therapy for MPSIIIA & MPSIIIB, and intracranial gene therapy for MPSIIIC.

The preclinical genistein publication is available for free from PLoSONE at the following link

http://www.plosone.org/article/info%3Adoi%2F10.1371%2Fjournal.pone.0014192

For information pertaining to the trial please contact Dr Simon Jones
Simon.Jones@cmft.nhs.uk

For scientific information please contact
Dr Brian Bigger
Brian.Bigger@manchester.ac.uk

References

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Malinowska, M., Wilkinson, F. L., Langford-Smith, K. J., Langford-Smith, A., Brown, J. R., Crawford, B. E., . . . Bigger, B. W. (2010). "Genistein improves neuropathology and corrects behaviour in a mouse model of neurodegenerative metabolic disease." PloS one 5(12): e14192.

LSD MasterClass for Middle East and North Africa

14 – 15 November 2013, Istanbul

A Multidisciplinary View of Fabry, Gaucher Disease and the MPS's

This meeting was attended by over 100 clinicians, Paediatricians, Cardiologists, Neurologists and Biochemists from across the Middle East & North Africa.

Over two very full days the participants learnt from Professor Atul Mehta (London), Professor Maurizio Scarpa (Wiesaden, Germany), Dr Christina Lampe (Wiesaden, Germany) and Christoph Kampmann (Mainz, Germany) the diagnosis and clinical management in particular of Gaucher disease, Fabry disease and the MPS diseases. With the exception of Turkey access to enzyme replacement therapy is not easily available in this region.

What was most heartening about this LSD MasterClass was the tangible enthusiasm and eagerness to gain all the knowledge possible and equally share their experiences of treating patients with LSD's in the clinic room. The last presentation on the first day was for me to share the 'impact of LSD's on Patients and their families.'

After all the science I was keen to bring all those present back to the reality that children, young people and their families endure in daily life with Fabry, MPS and the related lysosomal storages diseases. Thanks to individuals and parents who have consented to allow the MPS Society to use their images I was able to bring tears to the audience as well as show how so many children and adults in the MPS Society have triumphed over adversity.

My thanks go not only to the organisers of this LSD MasterClass but the members of the MPS Society who allowed their stories to be an inspiration to others in far off countries.

Christine Lavery

Invitation to the FIN Annual Fabry Experts Meeting

11 – 13th April 2014

The Fabry International Network would like to invite all its members Fabry Patient Organisations to attend its annual Fabry Experts Meeting with the participation of its key partners: Medical Advisory Board, Amicus Therapeutics, GlaxoSmithKline, Genzyme and Shire Pharmaceuticals.

The agenda is currently being finalised and we will share this with you in due course.

The objective of the FIN Fabry Experts Meeting is to enable Physicians to share their experience and advice with representatives of Patient Organisations on the challenges to the diagnosis, management and support of patients with Fabry.

The FIN meeting will coincide with the International Fabry Awareness Month of April and will be held at: The Steigenberger Airport Hotel in Amsterdam www.airporthotel-amsterdam.steigenberger.nl

Please feel free to contact Nawel van Lin to register your interest at nawel@fabrynetwork.org or on +31 486 83 00 23

7th Ceremony of the 'Cles Du Lysosome' 2013

College de France, Paris
18 November 2013

It was a great honour for me to attend Vaincre Les Maladies (VML) ceremony to recognise the lifetime work of four inspiring people in the field of Lysosomal Storage Disease and Rare Diseases.

Over 100 people gathered in the historic building of the College de France on the South Bank of the Seine to see awards presented to:

Professor Timothy Cox
Professor Marc Tardieu
Dr Jean-Michel Heard
Madam Christel Nourissier

They follow in the footsteps of others eminent in the field of LSDs including Professor Christian De Duve who discovered the Lysosome, Dr Roscoe Brady who discovered Enzyme Replacement Therapy and Professor Bryan Winchester for his lifetime's work in the science of lysosomal storage diseases.

The MPS Society has close links to Professor Cox who leads the clinical team in adult medicine at Addenbrookes Hospital, Cambridge and has a keen interest in the lysosomal storage diseases, particularly Fabry, Gaucher and Tay Sachs disease. In 2010 the UK MPS Society worked collaboratively on a natural history study on Sanfilippo disease (MPSIIIB) with Professor Marc Tardieu and Dr Jean-Michel Heard that has subsequently been published. Although Christel Nourissier may not be someone generally known to the MPS Society our members have a lot to be grateful to her for as Secretary General of Eurordis and her work to see the European Community legislate to require member states to introduce a Rare Disease National Plan.

The evening was made complete with a drinks reception followed by dinner. I would like to thank VML for inviting me and for all the work they do across France to support families of those affected by Lysosomal Storage Diseases.

Christine Lavery

A new opportunity for the diagnosis and therapy of MPSs in Europe: The HSK Rare Disease Institute and the Brains for Brain Clinical Research Institute

On January 1st 2014 a new specialised Center for Rare Diseases will be open in Wiesbaden, Hessen, Germany.

This new opportunity for patients affected by Lysosomal Storage Diseases (LSD) and other rare disorders will be offered after a collaboration of 2 key opinion leaders in the field of LSDs: Prof. Maurizio Scarpa MD PhD from the University of Padova, Italy and Dr. Christina Lampe MD from the University of Mainz, Germany, joining their expertise to the Department of Pediatrics at the Horst Schmidt Klinik (HSK), led by Prof. Markus Knuf.

The new Centre will be located in Wiesbaden, 30 km far from Frankfurt.

HSK Dr. Horst Schmidt Klinik

The HSK is a hospital of maximum care with 21 specialist clinics and 4 institutes (in total 1030 beds). The Children's Hospital of the HSK has 110 pediatric beds and 2 intensive care units. The Hospital offers all assessments needed to take adequate care for patients with multisystemic diseases. There is already a full neurological workup with all needed assessments available as well as a very experienced paediatric neurosurgical unit. During the last 2 years, more than 15 MPS patients (mostly MPS IV and VI) received decompression surgery of the cranio-cervical junction inside the pediatric clinics of the HSK. The Centre will be set up as a part of the Children's Hospital of the HSK in collaboration with all other Clinics of the HSK that are needed for the management of multisystemic and progressive diseases. The HSK is one of the certified centres for patients with tuberous sclerosis and other neurological disorders.

Beside organising a multidisciplinary rare disease team, the Centre is aimed to facilitate and speed up diagnosis, management and treatment for national and international rare disease patients. A major goal will be to improve the patients' and their families' quality of life and be the elective meeting point for stakeholders. Furthermore, the Institute will host a basic and clinical research unit for neurological diseases such as neurometabolic and neurodegenerative disorders, inborn malformations and epileptic encephalopathies.

BRAINS FOR BRAIN

The Centre will be integrated with the Brains For Brain Clinical Research Institute, as a joint venture with the Brains For Brain Foundation (www.brains4brain.eu) (B4B). The Brains for Brain Foundation was funded in 2009 after a donation from an Italian Family with a child affected by Metachromatic Leukodystrophy. It is now a well established international recognised international network involving 85 among scientists and clinicians of 60 major Universities in 14 EU Countries and USA, Brazil and Australia. The B4B Foundation is collaborating with 10 Biotech Companies, the major Neurological Organisations such as the European Brain Council and the major international patient associations. The International MPS Association is a funder Member of the B4B Foundation, Christine Lavery MBE is a member of the Advisory board. The B4B Foundation is very active inside the EU panorama and has organised 2 major events inside the EU Parliament in Brussels on 2010 and last November 2013 to address the attention of the Parliament to LSD problems and to start a new European PhD Programme on LSDs to attract and maintain in the field young scientists and clinicians.

All these programmes were in partnerships with the MPS Association UK. The B4B Clinical Research Institute at the HSK will be the site for basic and applied research projects, organisation of clinical trials and educational programmes concerning LSDs and rare diseases including presentations, seminars, meetings, journal clubs, scientific evenings etc. to increase awareness knowledge and interest in rare diseases.

The Centre for Rare Diseases at the HSK will be the first non-university Rare Disease Center in Germany.

In order to follow the recommendations of the National Action Plan for Rare Diseases in Germany (published August 2013) aimed at the establishment of a structure of certified centers, the Centre is in collaboration with the Network for rare diseases localised at the University of Magdeburg. The network with other hospitals and the sharing of different expertise in the field of Rare Diseases will lead to a better and more specialised care of rare disease patients. The HSK Rare Disease Institute and the Brains For Brain Clinical Research Institute represent a good example of the new concept of medicine based on the collaboration of excellences to create the critical mass needed to improve the management and the quality of life of MPS patients.

For more information please contact Prof. Maurizio Scarpa (maurizio.scarpa@hsk-wiesbaden.de) and/or Dr. Christina Lampe (christina. lampe@hsk-wiesbaden.de)

Fundraising

It's been a busy couple of months with lots of imaginative and exciting events. We have had family fun days, race nights, cycles and coffee mornings which you can read all about on the following pages, not to mention the Great Runs that have gone on north and South of the country in support of MPS.

We wanted just to take a moment, to thank you, our members and supporters for the hard work and effort that goes into creating your fundraising events. It doesn't matter how big or small your fundraising is, whether it's a gala dinner, a cake sale at work, an event at your local school or a church coffee morning. Every penny that you raise ensures the MPS Society can make a real and tangible difference to the lives of people affected by MPS and related diseases.

We may be a small, rare disease charity, but we have some wonderful supporters and members. If you are reading this and thinking, I would like to raise awareness where I live about MPS, Fabry and related diseases, but you are not sure where to start, drop us an email **fundraising@mpssociety.org.uk** and we can send you a fundraising pack which is full of helpful suggestions and handy hints to hold an event.

So whether you are planning on running, walking, wheeling, dancing, caking, jumping, racing or simply, straightforwardly donating, get in touch to see how we can support you

You're the reason we can keep providing our vital services, so no one has to face these diseases alone. Help us support people affected by MPS and related diseases and continue to research treatments.

Wear It Wicked!

Thank you so much to everyone that wore it Wicked for Halloween this year in support of the MPS Society and thank you for sending your pictures.

There was some ghastly goings on at MPS House this Halloween with Chief Executive Christine Lavery, getting Wickedly Witchy and the rest of the MPS team giving to get gruesome to support the Society. Advocacy Officer, Rebecca won best costume, good effort team!

Thank you also to the pubs and clubs that supported us this year by encouraging their patrons to donate a £1 to Wear it Wicked, schools and colleges that got spooky in school. We hope to make next year an even bigger and scarier event....

The Cock Inn in North Crawley held a Wear It Wicked event raising £40 for MPS.

BioMarin Wear It Wicked

Staff in the **Clinical Biochemistry Department** at Bristol Royal Infirmary raised £125.27 with a Wicked Cake Sale on Halloween.

Sandra Irvine and Encore Catering/ Cordia Outlets at the Glasgow Caledonian University held a Wear it Wicked day and raised £230.

The Meadows Primary School in Crewe held a Wear It Wicked day and raised £128.86.

Project Sanfilippo

Students from Davis House raised a total of £3,943.43 for The MPS Society Sanfilippo Project. The MPS Society has been the chosen charity for Davis House for two years and over this time a total of £8,824.32 has been raised by the students. Fundraising events that have taken place include cake sales, sponsored bike rides, walks, runs, coffee mornings, village market stall, coin collections, Carol singing, House evening, a disco and donations. All tutor groups have a target of £100 to raise over the academic year and many exceed this target.

Recently students have given presentations in assembly to nominate a new House charity for 2013-15. Following a student vote, the charity Davis House will support is Prostate Cancer UK.

Jane, Martin and Ellen Bate and Olivia Thornton and Edie held a coffee morning at High Street Methodist Church in Whitney raising £80 for MPS.

Sims Family Fundraising

Over the summer we held various fundraising events which were very successful. The events were to raise awareness for our daughter, Gracie Sims, who has ML II. We held a quiz night, garden party and a family fun day of music, games, raffle and even Mister Maker was there for a meet and greet with the kids (pictured left).

All events were very successful and in total we raised £1849.45 for the MPS Society. Thanks for all the support you have given us.
Lauren and Mat Sims

An Inspirational Evening

On Wednesday 14th November Vanessa and Brian Stottor held an Inspirational Evening at the DeVere Hotel in High Wycombe in support of the MPS Society. They have overcome personal hurdles and shared their story with an audience in the hope of inspiring others to live the best life you can. The event was a sell out and raised over £1800 for the MPS Society.

Congratulations to the Reid family

Neil Reid and Judy Hurry (now known as Reid!) married on 19th October 2013 at The Garden Barn, Little Bradley, Suffolk. It was a lovely day with close friends and family.

Rather than ask for wedding presents, Neil and Judy kindly asked guests to make donations to the MPS Society. A fantastic £650.86 plus gift aid was raised.

Neil's eldest son and first born child, Daniel George Reid who was born in 1977 had MPS II (Hunters). Daniel sadly died in 1986, aged 8. Neil has continued to support the MPS Society, including fundraising for them and his youngest daughter, Jessica is now a Trustee of the MPS Society.

Congratulations to Neil and Judy (the new Mr and Mrs Reid!) who we wish every success and happiness.

Race night in aid of the MPS Society

Jackie and Jim Mount with Jordan (MPS I)

On Friday 11th October we held a race night for the MPS Society. Our son Jordan was diagnosed with Hurler Syndrome on 29th March 2001 a day that would change our lives forever.

Over the past 12 years we have done various events to help fundraise for the Society, but this was mainly down to family organising as Jordan had so many hospital appointments and operations that a lot of time had to focus on getting Jordan through these procedures so we relied on my mother-in-law and my uncle to focus on some fundraising events. We had done cabaret shows and a sportsman dinner which were always a great success. I managed a few 10K over these past years which depending on how Jordan was, determined whether I would be able to take part. I have managed 4 but would like to try a half marathon before I get much older... but that's a different story.

Jordan is doing well at the moment so we felt it was time to try another fundraiser and we decided to have a race night. The idea came to me at Jordan's new high school as they hold a race night every year to raise funds for the school and this was the first one I actually attended. There was a twist, as my idea for a race night was watching a film on the screen, and the school used small wooden horses. We needed the audience to participate too and I knew we just had to try this as our family and friends enjoy a challenge.

I booked the venue, some horses and some entertainment for after the racing and that was us ready to go. About 2 months before it, my friend Clare did a letter and we sent it out to some of the local businesses. Things were slow to start but we were soon inundated with prizes for the raffle. The response was amazing, but most people in our town have heard of or know Jordan and are always willing to help.

On the night a lot of guests did not know they were coming to a race night with a twist and it was my job to recruit jockeys for the horses. I soon stopped asking and was just telling people they were taking part... I just can't stop the bossy bit coming out in me.

Jim was in charge of the raffle and it wasn't long before they were sold out and we had 5 books! I take it Jim must be bossy as well or just a terrific salesman... he had a few recruits mind you. My sister-in-law, sister and niece were doing their bookmakers bit and working out the odds, I think they should consider opening a shop as they did an amazing job. Uncle John was in charge of taking in the money that was him happy as counting money is a favourite past time of his.

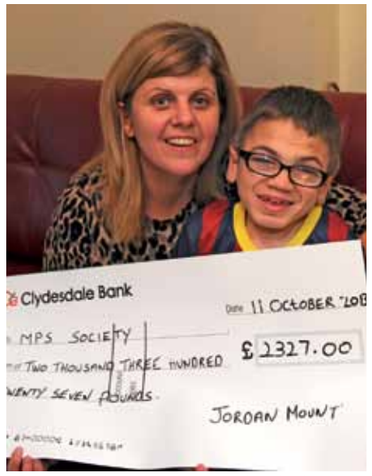
Well, with us all organised we were off. 8 people on 8 chairs having to wind in a wooden horse was a lot of fun. The competition was fierce and everyone really enjoyed taking part. As the alcohol flowed I think there was a fair amount of cheating going on... they know who they are. The last race was an auction race and that went well. So with the racing out the way, the raffle done, the fantastic David James took to the stage for some singing and the party was in full flow.

We managed to raise the grand total of £2,327 and I was absolutely delighted, I never expected to raise so much, but I should never underestimate my family and friends as they stop at nothing to help our special son who has captured the heart of our local community. The feedback from the night was amazing and we have been asked to do it again, watch this space as we are still trying to get over this year!

I would like to thank Kevin and Josie and also Karl and Edie who came from the other side of Glasgow to help us raise funds, as they also have children with Hurler Syndrome.

I have been threatening to do an article for the MPS for years, so that's it done now. I would like to take this opportunity to thank the MPS staff for the fabulous work that they do. The support they have given us throughout the past 12 years, the events we have attended... we have met the most amazing people and made some wonderful friends.

I would like to finish by saying although this was not the life that we ever imagined we would have, we feel truly blessed that we were given this special boy who has enhanced our life with so much and we are truly grateful he is our son. **Jackie and Jim Mount**



A fantastic total raised on the race night - thank you!

Run, cycle, jump for MPS - Please email us at **fundraising@mpssociety.org.uk** to register your interest in future running and cycling events or check out the fundraising section of our website **www.mpssociety.org.uk** for the latest news. We also advertise our places on **Facebook** and **Twitter** so keep checking these sites out too!

Find us on Facebook
www.facebook.com/mpssociety

twitter
twitter.com/MPSSocietyUK

Bedchester to Paris bike ride raises over £10,115 for MPS Society

Daniel (MPS III)

Our son, Daniel is eight years old and has Sanfilippo Disease, Type B. He was diagnosed with this devastating disease soon after we moved to Dorset. Our friends and the community have been amazingly supportive to us and Daniel.

Last year some of our friends undertook a three peak challenge to raise money for a charity to support awareness around brain cancer following the loss of a son by a local family. This year they were looking for a new challenge.

When my friend told me that not only were they planning to cycle to Paris from a small Dorset village, but they were planning to raise money for the MPS Society in honour of Daniel, I was overwhelmed! I was then a bit shell shocked at the suggestion that I was to form part of the team, however I was up for the challenge!

The following months were taken up by training for the challenge, usually consisting of extremely early morning starts for our Sunday morning cycle rides of 25 to 30 miles across the hilly Dorset and Wiltshire countryside.

The fundraising challenge began, with nineteen of us fathers raising money on a combined 'team' Just Giving page, www.justgiving.com/teams/Bedchester2Paris.

Daniel has a friend, Ella, who also has Sanfilippo type B. She lives locally and they both attend the same school. We are good friends with the family, and worked together in the fundraising appeal and publicity.

The cycling team were raising publicity through local press and social media to raise the profile of the campaign. Julia Bradbury, a friend of one of the fathers, also got involved by retweeting the appeal on Twitter where she has 4,700 followers.

The cycle challenge involved travelling from Bedchester, a small village near Shaftesbury in Dorset to Paris, a total of 300 miles. We were to spend three days travelling at 100 miles per day. We camped for two of those nights, finishing in Paris where we were to look forward to the luxury of a hotel and warm bed!

On the day we set off we all had to assemble in Bedchester before dawn. Getting out of bed at 4.00am to be faced with 3 days of intensive cycling was difficult enough, made harder by the relentless rain pouring outside! However, once we all grouped, we were off! Before we knew it, we were boarding the ferry at Poole en-route to Cherbourg.

Our ride took us past several D-Day locations in Normandy, including camping on top of the cliffs overlooking Omaha beach, the Normandy American Cemetery and Memorial at Omaha, cycling alongside the other D-Day beaches and onto Pegasus Bridge. The troops who captured Pegasus Bridge on D-Day set off from Tarrant Rushton Airfield in Dorset, where we had done some training rides. One the second evening, we were also welcomed with an official reception by Shaftesbury's twin town of Brionne in Normandy.

The ride was certainly eventful with many punctures and mechanical breakdowns. On the second day, we even managed to add 30 unnecessary miles to our journey by getting lost!! However, on the third day, Paris was in our sights. Just as we were approaching Paris, there were a couple more punctures, and a gear failure for one of the riders. However, as dusk was settling, we reached Paris, and it was a fantastic feeling to cycle around the Arc de Triomphe at the end of our epic ride.

We are very proud of our achievements, and are thrilled that we managed to raise over £10,000 for the MPS Society. We have been completely overwhelmed by the generosity from so many people.

Thank you so much to all the fathers - their commitment and sheer determination not only to complete the challenge to Paris, but all the planning involved to make the trip happen has been truly inspiring. **Edgard Zaldua**

Bedchester - Paris cycle team by the Eiffel Tower

Wicked Walkabout

My name is Sarah. I decided to organise a Wicked Walkabout after finding out my son had MPS III A and that there is no cure, then to find out there are trials available but they are very low on funding.

So I then decided I wasn't going to just sit back and wait for the money to be raised. A small group of us were sponsored to do a 5 mile walk and managed to raise £774 for The Genistein Trial to research Sanfilippo Syndrome. We also raised a further £210 on a charity fun day held at the local family pub.

I am extremely grateful for all the support I have recieved from the MPS Society and hopefully, with everyone working together, we can all help.
Sarah Miller

If you think you'd like to hold a Wicked Walkabout in your local community then please drop us an email at **fundraising@mpssociety.org.uk** and we can send you our Wicked Walkabout Guide, a simple, easy to follow collection of suggestions to get the most out of your event.

Throwing Punches With Good Intentions

Aaaaaand In the blue corner - raising money for the MPS Society is the Lloyd's Boxing Association.

Three years ago when I first started working in insurance and MPS were three letters that I only associated with stopping my unwanted mail, I attended a boxing event. Little did I know that in just a few years I would be organising the auction on the night for a charity that has become so close to my heart.

In 2012 my nephew Joe was diagnosed with Hunter Syndrome. The MPS Society have been a great support to my Brother and Sister-in-Law and so I wanted to do something to show my support.

In previous years the annual Lloyd's Boxing event has raised money for a charity called CRY which supports the families and individuals affected by cardiac risk in the young. This year the chairman of The Lloyd's Boxing Association suggested we also raised money for MPS and I jumped at the chance.

I considered boxing – that wasn't for me, I considered volunteering as a ring girl but I don't think my Dad would approve. In the end it was decided that I would be best suited to organising the auction.

Ten volunteers from the insurance market spent 6 gruelling months training for this event and sold 800 tickets between them. The hard work each fighter put into their training really paid off as they put on an amazing display of both physical strength and technical ability. One thing was for sure, these people had started off as volunteers and although they had transformed into boxers, they meant business and were there to raise as much money as possible.

After the fifth fight it was time for the auction.

The MPS Society helped to locate some amazing prizes and I would like to send a special thank you to our anonymous donor, without his incredible generosity we would not have raised anywhere near as much money as we did.

The auction prizes on offer on the night were;
- A signed Celtic Football Club shirt
- A signed Geoff Hurst Anniversary shirt
- Signed John Terry Football boots
- A signed Frank Lampard shirt
- A signed David Beckham shirt
- 2 tickets to a Manchester United football game
- A signed Marland Yarde England rugby shirt
- A signed London Irish rugby shirt

As if the prizes were not good enough to sell themselves, we had the help of the ring girls who worked their magic to show off each item and entice even more bidders.

The first item up for auction was a signed Celtic Football Club shirt, I had promised the donator of this item that I would not let it go for less than £50 and was secretly feeling smug as some friends of mine had agreed to bid three times as much to ensure the shirt was theirs. I could not believe it as the value of the bids climbed and eventually the shirt sold for £310.

This was just the start of things to come as the lowest winning bid for any of the items on the night was £150.

The star of the show however was a signed David Beckham shirt which went for an incredible £650!

The evening was a huge success and I am pleased to say we raised £2,150 for the MPS Society.

I think everyone involved would agree that the night was a success but the Lloyd's Boxing Association won't rest until the fight against MPS ends in a knockout. **Louise Hiller**

Events and Challenges

Lee Shepherd raised £1170 on Justgiving from the Great South Run. www.justgiving.com/LeeShepherd2013

Debbie and Mark Burniston sent in £125, part of monies collected in memory of Elizabeth Callum. £275 was also collected in cash making a total of £400. Debbie and Mark held a fun day on 7th September raising £621.87 for MPS. Their friend Paula Haliday has doubled the £621.87 and the £75 through a scheme at her work place.

Mark and Debbie also ran the Great North Run in September. Elizabeth Callum was Jake Corcoran's grandma. It is in memory of Jake that the Burnistons raise money for MPS.

Elliot Moody ran the Great North Run in September on behalf of the MPS Society. He finished in a time of 1 hour 50mins raising £540.

Tessa Nelson undertook an 11 mile wheelchair trek raising £478.18 for the MPS Society.

Lynne's Great North Run

Lynne Nappin is secretary at the Arriva Bus Company in Aylesbury. One of her colleagues, Ian Evans, a driver, has a son called Harry who suffers from MPS (Hurlers). Ian is a stalwart at fundraising from the MPS Society. Lynne ran in the Oxford Half Marathon in October 2013. She was generously sponsored by her work colleagues and one very kind person from her gym raising a total of £730.

Ride24 Event - Craig Hudson took part in the RIDE24 cycling relay around the Thruxton circuit on 7&8 September 2013 in support of the MPS Society and Sam Brown.

'My cousin's son, Sam is 5 and has a variant of MPS called Morquio Syndrome. It affects his growth (he will reach between 3-5 feet), with knock-on effects for his physical abilities and related long-term health problems. He has had a number of major operations and spends a day a week at Manchester Children's Hospital on a clinical trial of treatment. Despite all of this Sam is in every other way a typical 5 year old boy, and charges at life at 100mph. He sets a great example to us all.'

A huge thank you to Craig Hudson who raised £1,885.00 on his JustGiving Page http://www.justgiving.com/allabouthealthride24-mps

London to Edinburgh and Back by Bike - Ian Kellar cycled 1,400 kilometres in five days to support the MPS Society and the Neuroblastoma Society for 2 Otley boys who are friends with his son Seth. The two boys, Harry Buckley who has neuroblastoma, a rare and aggressive form of childhood cancer, and Sam Brown who has Morquio MPS IV both attend Wharton School in Otley.

Sam's mum Katy along with Sarah Buckley and cyclist Ian Kellar managed to raise £2,302.00 on their Virgin Money page. This will be split equally between the MPS Society and The Neuroblastoma Society.

A big thank you to all our fundraisers who raised money online through Justgiving, Virgin Money Giving and Do It For Charity.

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. 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.

Thank you to all our donors including . . .

A colleague of Kath Hiller donated £100 on the occasion of her 50th birthday by asking friends to make donations to MPS and Cancer Research UK in lieu of presents.

Tim Summerton and colleagues at GE held a Halloween collection raising £38.60.

Damien and Dinah Adair and family donated £1000 to the MPS Society in honour of Roma Drayne's 21st birthday. They think the world of Roma who suffers from MPS IV and wanted to wish her all their love, continued good health and much happiness.

Dorothy Robinson & two friends held their fifth craft workshop and raised £200 for the MPS Society.

Hassell C.P School in Staffordshire held a 'Wacky Hair & Clothes Day' and raised £263.10 in support of one of their pupils Tisiphone Copsey who has Morquio.

Lynn Longhorn raised £150 for Sanfilippo Research at her Christmas Fair.

Sue Beaumont held a jewellery sale and asked for donations in lieu of gifts at a 50th birthday raising £250 for the Genistein Clinical Trial.

Peter Vickery recently retired from the Cheam Probus Group and selected the MPS Society as his chosen charity to receive a £200 donation.

Pippa Gearing & the Amersham Rock Choir held a concert and raised £225 for the MPS Society.

Gary Lashmar donated £125 from the Lloyds Boxing Event 2013.

Woodland View Primary School raised £113 with an idea from Mackenzie Richardson.

Dennis and Janet Mauger collected £182.22 at the Horton Village Fete on a shuttle bus.

Janet Pears donated £40 on the occasion of James Garthwaite's birthday.

Peter Andrea donated £200 to the Genistein clinical trial raised from the Tough Mudder event in aid of Bobby Gill.

Mr and Mrs Loader, family friends of the Eaton Family, donated £500 to the Society.

Teresa Padden raised £100 in cash donations for her London to Brighton cycle ride as well as her £300 raised on the Do It for Charity website.

Day Lewis Pharmacy donated £200 from their Community at Heart Scheme.

Kirsten Westly donated £20 in support of the Square Mile Fight Club evening.

Ken Pierce raised £3,000 at a Ball for the Society.

Christine Borland donated £200 to the Genistein clinical trial.

Amanda Sherman raised £40 by doing online psychic readings

James Garthwaite donated £120 given in lieu of presents at his recent birthday and £50 CAF voucher.

Sally Summerton held a coffee morning and raffle at the Coco Café in Croxley Green and raised £314.20

Friends of Heather Gordon donated £25 to the Society.

Friends and family of Jason and Elizabeth Heath donated £1328.70 in memory of their son Jack.

Dennis Mauger, friend of Ian Evans, raised £167.60 at the Chearsley Fete with his shuttle bus including a £100 donation from a Peter Dickson.

Stuart Robinson, friend of Ian Evans, raised £70 with a collection on his 65th birthday.

Vickie Brindle raised £308 with her English Half Marathon

Fiona Byrne donated £225 which was given in lieu of presents from staff on the occasion of her retirement from Rutherglen High School.

Elaine and Joe Quinn donated £1,250 in memory of their son Dan in lieu of flowers.

Amersham Trefoil Guild raised £105 with their raffle.

Louise McCreadie donated £200 after she ran the Glasgow Half Marathon in memory of her sister.

Caroline and David Lentaigne made a charitable donation of £100.

Kath Hiller donated £40 with her sale

The Quadrant Business Centre in Sheffield selected the MPS Society as their charity of the month and raised £72 selling second hand books and cds.

The GE Money Home Lending Health Ahead team in Watford raised £365 with their sponsored 2 hour spinning class.

Delia Jackson and colleagues at the School of Language, Literature and International Studies in Preston held a garden party in memory of Delia's son-in-law and raised £100.

James Hiller ran in the Great Birmingham Run raising £740 for the MPS Society.

J. Langford Stacey donated £110 from the sale of greetings cards and items of stationery.

Pupils at Meadows Primary School held a sponsored danceathon raising £532.39 for the MPS Society. One of their pupils is Emily Bradshaw.

Karen Hurst donated £30 to the Ed Wraith memorial fund

Kevin Thompson and family donated £278 in support of Tommy Thompson in lieu of flowers at Kevin's brother's funeral

Mrs M Brock sent a cheque for £50 on behalf of a family friend

Yvonne Pearson donated an additional £160 in donations from her and husband's wedding blessing.

Anne and Ken Hooper held a sponsored walk and ploughman's lunch on behalf of their grandson Jamie and raised £2,281 for the Society.

Phil Powell and his wife run a haberdashery store, Bids and Pieces. So far, they have raised a total of £168.30 from the sale of second hand knitting patterns.

Andy & Jenny Hardy held a Christmas card sale and raised £425.05.

The Rotary Club of Yeovil ran a Charity Golf day and raised £1,200 towards the Genistein Clinical Trial

The Emma Hiller's local village donated £100.

Chris at ASM Metal Recycling Ltd donated £275.

Woodside Junior School in Amersham raised £104.20 with a Wear It Blue Day.

The Towersey Morris Men raised £600.

Dawn Moody organised a charity fun day with colleagues at Barclays Bank Wyke branch in Bradford on 22 September raising £816.27 for the MPS Society. This is being matched by Barclays bank under their matched fundraising scheme.

Hepworth Garage Ltd in Shalford donated £350 money raised for the MPS Society as their chosen charity. Instead of charging customers for changing headlamps they ask them to make a donation.

The Shiney Company Store in Bristol raised £100, proceeds from a raffle at their August Bank holiday craft event. The money has been donated to support the Marina & Friends shop in their fundraising for research into Sanfilippo disease.

MPS National Draw 2013

Thank you to everyone who purchased MPS National Draw 2013 raffle tickets. Winning tickets were drawn by MPS Trustees on 30th November 2013. A full list of winning tickets can be found in the latest news section of the MPS website www.mpssociety.org.uk

A Special thank you

We always try in our magazines to say thank you to you for your super fundraising events and share your inspirational stories and pictures. However, we perhaps haven't really acknowledged our regular contributors enough, so this is for you...

We want to take this opportunity to say a HUGE thank you to those supporters and members who are our regular contributors. Every month, (or in some cases every year) you quietly support the Society with direct debits you have set up. Your contributions help us plan ahead for funding our Advocacy Service and research into treatments and cures for MPS, Fabry and related diseases.

Our Super Advocacy Team

From a single advocacy officer, back in 1986 funded by BBC Children in Need, covering the whole of the UK, the Society's highly skilled Advocacy Team is now five strong and supports over 1,200 families and individuals in all aspects of social care and housing issues as well as access to special educational needs and clinical management across the UK. The chances are you have probably met Sophie, Steve, Rebecca, Alison or Deborah or spoken to them on the phone.

Treatments

Thanks in part to funding from the MPS Society there are now 5 treatments available for the 24 diseases we support. We have come a long way and we know we can still do so much.

If you are reading this and think that you might be able to manage a small amount every month then firstly, thank you for even considering making a regular donation to MPS.

A regular gift allows us to plan for the future as we know that we will have a steady income stream that we can rely upon.

Thanks to better diagnosis and treatment, the MPS Society's membership continues to grow whilst at the same time, the squeeze on public spending and the Government's recent changes to the benefits system have affected many of you. As a result, the number of calls to our advocacy service has risen and we want to ensure we can continue to provide the same level of care and support to all our members, wherever you are in the UK.

By making a regular gift of just £5 a month you will make a really big difference every day. Your donation can ensure we can always send our Advocacy team to any family in the UK, where ever they are needed, always be on the other end of the phone to help you however we can and ensure that we can keep funding world class research.

Also, don't forget that thanks to the government's Gift Aid scheme, if you are a UK taxpayer, the MPS Society can reclaim the tax on all of the donations that you make.

Setting up a regular donation is easy you can either:

1. Go to the Society's Direct Debit page at http://www.justgiving.com/sfmd/Donate
2. Call us on 0845 389 9901

Do you know other friends and family that you think might want to support the Society in this way?

So thank you for you for all your support, past, present and future. You're the reason we can keep providing our vital services, so no one has to face these diseases alone. Help us support people affected by MPS, Fabry and related diseases and continue to fund research into treatments.

Grants, CharitableTrusts, Grant-giving Foundations and Community Groups
The Eveson Charitable Trust

Donations
Christopher Stanley, Adele Jones, Trevor Brown, Mette Fisher, Mrs G.S Vivier, Norman Saville, Janet Croft, Patricia & Carole Mould, F.G Robinson, Elizabeth Powell, Samantha Matthews, Monica Hartwell, Vivienne Culley, Kate Tate, J.M Brown; Mrs M E Horsley; Marlene Sanderson; Mrs Y Puddy; Mrs E M Mee; Maureen Loveday; Judith Swan; Ann&Rick Coleman (Friends of POW), Dr & Mrs Rudham, Mrs Joan Marsh, Mr W Plow; Pam Hope; D.Minett; RJL Byrom; RC Devine; R Shrimpton; Mrs J Haskell; Ms R Wheeler; Ms M Tosland; Mrs E March; Associated British Ports; Graham Bell; Peter Dine; Jean Armsby; Barbara Harris; Mrs D Duckett; Catherine Love; Alexander Kafizas; Pat Skidmore; Margaret Leask; Mrs E Mee; S.Bhachu; Lesley Cranfield; Chris Hume; Kathleen Chivers; Mrs A Baker; Mr & Mrs J Keenleyside; Spreadex Ltd; Iris Lay; Deborah Irwin; Joan Ingram, Colette Lee; Pauline Selwood; Jasmine Mclaren-Hall; Sandra Irvine; Mrs C Westland; Coney family; Terri McGinn; W.J McGinn; Mrs A Johnston; Ms J Johnston; Mr Ian Mahoney; Mr M Briggs; Iris Hitter; Roy Parkinson; RS Watson Jones; John Sanderson; Linda and howard Matthews; Luke Bown's Great Nan; Heather Fisk; Pat Pillinger; Mrs A Baker; Linda Wright

In Memory
Mr John Hemmings; Ben Richardson;

Collection Boxes
Ian Evans's local Post Office, Lady Gosling's office, Mr R Burgess, Bids & Pieces; Warren Tea Rooms; Susie Hammon

Stamps, foreign coins, mobile phones, ink cartridges; jewellery
Judith Evans, Bob Stevens; Ian Evans; Rachel Todd; Jenny and Andy Hardy; Ellen Graham /Your Derwent & Solway Housing

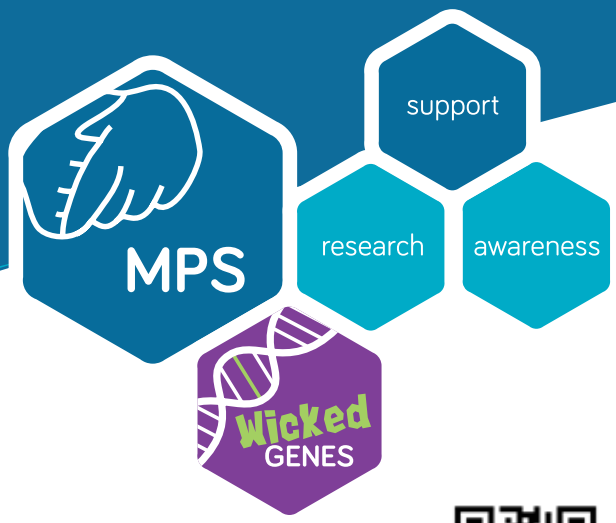
The Society would like to thank the following donors for their regular contributions by either Standing Order or Give As You Earn.
S Littledyke; R & K Dunn; Marcia Tosland; Norman Saville; S Bhachu; I & A Hedgecock; C Cullen; D Forbes; G Simpson; M Kalsi; R & J Richards; P Summerton; A Weston; E White; C L Hume; William Cavanagh; A Sabin; L Brodie; A Ephraim; J Dalligan; M Malcolm; E Mee; K Brown; E Brock; M Fullalove; G Ferrier; R Parkinson; R Taylor; R Gregory; R Henshell; K Bown; E Moody; S & J Home; V Little; M Reeves; Z Gul; S Greening; J Casey; M J & V Hastings; E M Lee; Anthony Lockyer; A Sullivan; A Byrne; D Robinson; S Cadman; J Wilson; J York; M Rigby; A Tresidder; E Cox; M Wood; Mr Thompson; K Robinson; J Heritage; C Gibbs; D Peach;

We support families
We advise on benefits, education and housing
We visit homes, schools and clinics
We travel all over the UK
We help fight discrimination
We organise family fun days
We help with transition and independent living
We fund vital research
We co-ordinate clinical trials
We support 24 different diseases
We raise awareness
We campaign for change
We are always there, when you need us.

Need support?
0845 389 9901

The MPS Society has come a long way in the last 30 years supporting those affected by MPS, Fabry and related diseases and funding research into treatments for these rare genetic diseases.

We know we can continue to make a difference, but we need your help. Can you spare just £1 a week to help support our vital work? If you think you can make a regular contribution please visit **www.mpssociety.org.uk** or email **info@fundraising.org.uk**



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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