MPS magazine

Society for Mucopolysaccharide Diseases

Autumn 2016 • www.mpssociety.org.uk

What went on at Bonn?

NEWS FROM THE 14TH SYMPOSIUM

MEMBERS INTERVIEW

Stories on vlogging, knitting and driving

Back to school

your first day photos inside

National draw prizes on page 23

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, and can result in death in childhood.

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

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. We aim to:

- **act as a support network for those affected by MPS and related diseases**
- **promote and support research into MPS and Related Diseases**
- **bring about more public awareness of MPS and related diseases.**

Board of Trustees

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To submit content email:
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The articles in this magazine do not necessarily reflect the opinions of the MPS Society or its Management Committee.

The MPS Society reserves the right to edit content as necessary. Products advertised in this magazine are not necessarily endorsed by the Society.

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Front cover photo: Chloe Blanc on her first day of school
Icon on p. by Rockicon for the Noun Project

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A tough challenge but a great achievement

Try this!

Download the easyfundraising donation reminder for your internet browser and you'll never miss another donation opportunity.

It's free and simple to use. Just register at easyfundraising.org.uk and get shopping!

WELCOME

The summer is over and that autumnal feeling is upon us, with Halloween and bonfire night just round the corner – and that means the countdown to Christmas! Check out our insert for the new range of cards from the MPS Society this year and the merchandise leaflet for some stocking fillers.

In this issue you'll also find a book of raffle tickets for our National Draw. You can apply for more at any time, just let us know if you sell them all and please return any you don't sell. A list of prizes is on page 23.

This issue also sees a bumper number of fundraising pages which shows what an amazing job you've been doing to raise money and awareness for the MPS Society. Thank you for your hard work!

From the Group Chief Executive

Christine Lavery
Group Chief Executive

First of all I want to congratulate the German MPS Society on their huge success hosting the 2016 International Symposium on Mucopolysaccharide Diseases in Bonn this July. It is no mean feat to bring together over 1200 delegates that included MPS families from over 30 countries not to mention hundreds of MPS specialist nurses, doctors, academics and pharmaceutical companies from across the world.

The UK MPS Society was very well represented with families and young adults drawn from our membership participating in the Symposium. Apart from hearing cutting edge presentations on palliative care, clinical management and treatment and future research and clinical trials our members had the opportunity to meet and share their experiences with those similarly affected as well as the professional delegates who are always keen to have the patient and carer perspective. We are looking forward to our own Expert and Patient meeting on Maroteaux-Lamy disease (MPS VI) 15–16 October in Northampton and will feed back on this in the next issue. It's not too late to book! Find out about the latest speakers and download a booking form from the events page on the MPS website.

I now turn my thoughts to a subject that at best may feel mundane to our membership given the enormity of the MPS challenge you face day to day. When I have broached the governance needs of the Society with some of you in the past understandably your focus has been more around the importance of the advocacy service, the MPS Magazine and support to ground-breaking research that may lead to new treatments and therapies. In the words of one member 'What we like about the MPS Society is everything just happens and we don't have to worry'.

But how does everything just happen? Some of you may have a good understanding of how the MPS Society is governed as a registered charity and a limited company whilst others of you might wonder at how the MPS Society reaches the decisions it does? The MPS Society is very fortunate to have an experienced board of Trustees supported by a highly professional Senior Leadership Team. Whilst the Senior Leadership Team are employees of the MPS Society and provide the evidence, information and guidance to the Board of Trustees on everything from policies, health and safety, fundraising and budgeting it is the Trustees collectively that have to have a complete oversight of the MPS Society from a legal and fiduciary perspective and make best interest decisions.

> "We would be pleased to hear from anyone interested in knowing more about the Trustee role

The MPS Society is constituted to have up to 10 elected Trustees each serving up to two 3-year periods. There is also a facility to co-opt up to three Trustees who would then be required to stand for election at the next Annual General Meeting. The Board of Trustees meets five times a year in Amersham on a Friday evening and all day Saturday. Friday night accommodation is arranged and reasonable travel costs are reimbursed.

The role of Trustee for the MPS Society is very important and with three Trustee/co-opted vacancies on the Board I would ask you to consider if you have the time, commitment and skills to take an active role in the governance of the MPS Society? We are particularly looking for individuals with a background in financial management or business but would be pleased to hear from anyone interested in knowing more about the Trustee role. Please do email me at c.lavery@mpssociety.org.uk with an expression of interest and short CV. If you would prefer an informal telephone conversation please say this in your email.

Further information on the role and responsibilities of Charity Trustees can be found on the Charity Commission website: www.gov.uk/topic/running-charity/trustee-role-board

News from the Board of Trustees

The Society's Trustees meet regularly. Here is a summary of the main matters discussed and agreed at the Board of Trustees Meeting held
15–16 April 2016

Governance

Election of Officers

The following Trustees were elected to the following responsibilities:
Chair – Susan Peach
Vice Chairs – Wilma Robins and Paul Moody
Treasurer – Judith Evans

Financial Management

Judith Evans spoke to her Cash in Bank statement and confirmed that at the 11 April 2016 there was £979,770.67 in the MPS Society Accounts plus £116,343.87 in the MPS Society Research Accounts.

The Trustees agreed that they had received and read the Auditor's letter in respect of the Group accounts for year ending 31 December 2015. Trustees felt that the auditor's letter was positive but unqualified and agreed they would want the Finance Committee to meet with the Auditors through a TC when the Group Accounts for 2016 are being audited in early 2017.

Risk management

Christine Lavery advised the Trustees that all the changes agreed by Trustees at the Board of Trustees in February 2016 have been made. Mindful of the Board of Trustees legal and financial responsibilities the Board looked at a series of 18 questions that explore their understanding of the Trustees Annual Report (TAR).

Income Generation

Led by Gina Smith the fundraising team gave a presentation to Trustees on generating income for the MPS Society. The presentation was in two parts 1) Grants and Trusts and 2) Corporate and Community Fundraising. It was acknowledged that Fundraising for MPS is tough due to the rarity of the diseases. The presentation included schemes MPS uses to raise funds including the MPS shop with the new metal logo badge. The importance of the psychology of giving was emphasised. In the last two years MPS social media audience numbers have grown. Posts are made daily. The MPS website is much improved with good navigation system with 7,000 views in past months.

Charitable Trusts and Foundations are playing an increasingly important role in providing the funds to improve the MPS Society's services. Emphasis was put on the importance to research the Trust/ Foundation before applying and reporting back if successful. Over the last 12 months the MPS Society has had a 38% success rate in applying to charitable trusts and foundations with 30% being rejected with a response.

Policy strategy

The Donor and Fundraising promise which had been received in advance by Trustees for their consideration was approved.

The following policies were reviewed and agreed: Data Protection Policy; Confidentiality Policy; Employees Conduct Policy; Recruitment Policy; Financial Controls Policy; Adverse Weather and Travel Disruption Policy: Interaction and Relationships with the Pharmaceutical Industry; Vulnerable Supporters Policy.

Research grants

Professor Bryan Winchester advised the Board of Trustees that he has been giving considerable thought as to how the MPS Society might invest £50,000 to fund research that has a tangible outcome(s) to the MPS members. Bryan has also talked to some researchers. He sees the two options as to add on to an existing project or start a new research project. Christine Lavery asked Bryan if he was giving thought to the research avenues including use and benefits of bisphosonates that emerged at the ML Expert Conference. Bryan Winchester acknowledge the bone problems in ML and MPS is a challenging problem and suggested identifying biomarkers for ML II and ML III. Judy Holroyd said that the members are looking to the MPS Society investing in research that will lead to improvement in quality of life. Wilma Robins spoke of the information she gained at the LSD Collaborative meeting on the use of botox to reduce salivation in Tay Sachs patients. Christine Lavery said that excess salivation is a significant problem in MPS III stage three children and adults. It was agreed further discussions need to take place.

Advocacy Support

The Board of Trustees noted from the Report how hard the Advocacy Team are working and the diversity of the workload and expressed their appreciation of the Advocacy Team.

WHAT'S ON?

MPS Regional Clinics

MPS I Post HSCT (over 6 years) – RMCH
7th October

Adult Fabry – QE, Birmingham
11th October • 8th November • 13th December

MPS III – BCH
13th October (afternoon)

MPS II/MPS VI - BCH
14th October

MPS IV - GOSH
8th November

MPS I Post HSCT (under 6 years) - RMCH
14th October

Fabry – BCH
25th November

Conferences and Regional Events

Childhood Wood Planting – Sherwood Pines
16th October

MPS VI expert meeting
15th–16th October

Pantomime – Copthorne Hotel, Manchester
27th November

Lapland Family Trip – Finland
2nd–5th December

Sunday Lunch with Santa, Newcastle
4th December

SAVE THE DATE

MPS Weekend Conference 2017 – Hilton, Coventry
7th July–9th July 2017

Announcements

NEW MEMBERS

Caroline and Alan have recently been in contact with the Society. Their son has a diagnosis of MPS I Hurler Scheie Disease. **Mason** is four years old. The family live in Scotland.

Chloe has recently been in contact with the Society. Her son has a diagnosis of MPS II Disease. **Ronnie** is two and a half years old. The family live in the Manchester area.

Emma Siddall and Wayne Bond have recently been in contact with the Society. Their son **Harley** has a diagnosis of MPS III Sanfilippo Disease. Harley is three years old. The family live in Sheffield in the North of England.

Nicola has recently been in contact with the Society. She has Fabry Disease. The family live in Bedfordshire.

Naima Hamra has recently been in contact with the Society. Her son **Omar** has a diagnosis of MPS III Sanfilippo Disease. Omar is 5 years old. The family live in the South East of England.

Expert and patient meeting on Maroteaux Lamy disease (MPS VI)

15–16 October 2016
Hilton, Northampton

If you would like more information or are interested in attending the meeting visit our events page:
www.mpssociety.org.uk/support/events-clinics

Introducing Zoe, our newest employee working in the fundraising team

Hello, I am Zoë Van Niekerk and recently started at the MPS Society as a Fundraising & Information officer. I joined the Society because I am passionate about making a difference in people's lives. Even though I have only been here for a short while, it's wonderful to see that the team is so dedicated to achieving the charity's goals.

I have a background in the HIV field. I started my career doing monitoring, evaluation and counselling in community outreach projects in South Africa. Prior to joining the Society, I have been involved in pharmaceutical healthcare funding for community outreach projects focusing on HIV and women's and girls' issues.

I have been married for two wonderful years to my husband Kurt. I love animals, going for day hikes, trying new foods and visiting new places.

I am looking forward to growing in my role as a Fundraising & Information officer and look forward to attending some of the fundraising events and meeting some of the families.

Back to school

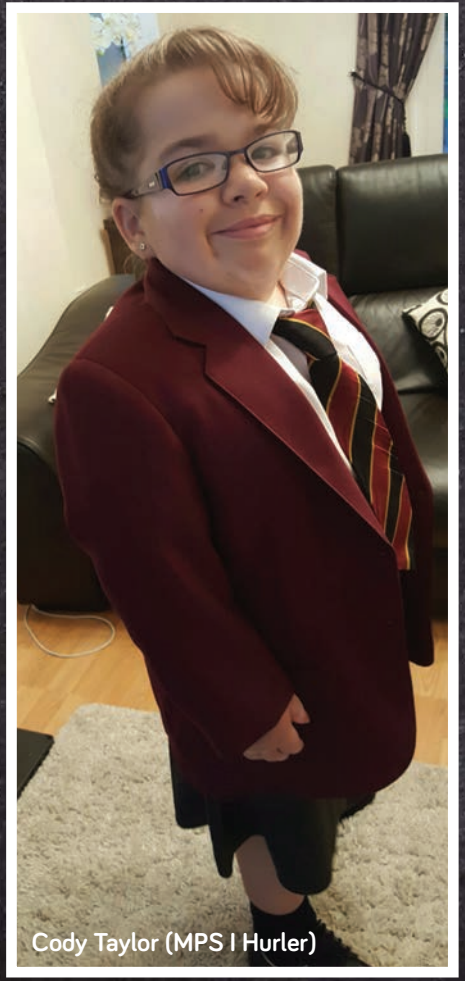
Celebrating all those starting and returning to school this term

Sam Brown (MPS IV Morquio) and his friend, Alex (left)

April Losztyn (MPS I Hurler)

Sophia Scott (MPS III Sanfilippo)

Emily Bradshaw (MPS I Hurler)



Cody Taylor (MPS I Hurler)

Oliver Gosling (MPS I Hurler)

Chloe Blanc (MPS I Hurler)

Daniel Molloy (MPS II Hunter)

Advocacy

Our advocacy support service is at the core of everything we do at the MPS Society. We know how isolating and challenging it can be living with MPS or a related disease so we want you to know that you are not alone and we are hear to help. We are always striving to improve the support we offer and to sure we respond to each individual need as best we can.

Our service is flexible and a wide range of support is offered on a needs led basis but here are some of the services we can offer.

Telephone helpline
We provide an active listening service, information and support by phone, including an out of hours service. You can reach us on 0345 389 9901.

Disability benefits
We provide help and support in completing in completing claim forms for Personal Independent Payment and, where needed, will take a representative role in appeals and tribunals.

Housing and equipment
We 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
We help 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.

MPS careplans
We undertake 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, by producing a careplan.

Respite care
We work closely with a number of respite providers and can make individual referrals if needed.

Independent living/transition
We provide 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.

Befriender service
We link individuals and families affected by MPS and related diseases for mutual benefit and support.

Bereavement support
We are here whenever you need us, especially at the most difficult times.

Advocacy Resources
The Advocacy Team have also developed a range of information resources focussing on particular issues which are free to download 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.

Sophie
Manages the MPS Advocacy Team

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

Rebecca
Fabry, MPS II Hunter, ML III/ML IV, Mannosidosis, Fucosidosis

Louise
MPS III Sanfilippo (shared with Steve), LAL D, Gangliosidosis

Alison
Currently on maternity leave. All Ireland patients (covered by Advocacy Team)

Steve
MPS III Sanfilippo (shared with Louise), MLD, AGU, Winchester, Geleo Physic, Dysplasia, SLY, Sialic Acid Disease

Each advocacy officer works to a high level of professionalism. To make sure of this the following skills, knowledge and person qualities are present, applied and reviewed regularly:

- Qualified Social work
- Public/statutory services
- Genetic services

Areas of responsibility

In order to provide continuity and a better quality of service to the Society's membership the MPS Advocacy Team have responsibility for a smaller number of MPS diseases. This is to ensure that all workers not only have a sound knowledge of all the MPS and related diseases but become more familiar and develop a greater knowledge base in a smaller group of diseases.

There will be ongoing training in all disease groups to enable workers to ensure they are fully informed and are kept up to date with any changes or developments.

There will also still be opportunities for joint working and there will be times when workers will have to undertake work for another disease group. For example in staff absences, taking forward emergency pieces of work, attending clinics, social events and conferences where necessary.

Our recent work

Since the summer issue of the magazine, the Advocacy Support Team have sent out new member packs, attended visits, meetings and clinics, visited new members at home and conducted general visits, attended a Children in Need meeting and provided clinical management, social care, education advice and help with benefits. The team also attended the Bonn International Symposium, a family weekend at Drayton Manor.

Our ongoing work includes the MPS pregnancy study, which includes writing clinical and experience papers and information leaflets where our data will feed into a larger study being conducted by Prof Hendriksz. As well as this we have the MPS III B Natural History study ready for publication, produced MPS II and MPS IV posters for the exhibition at Bonn, attended a Nice appraisal for LAL D.

We have written 47 key reports, letters or forms about the following:

- Housing –12
- Education – 4
- Benefits – 14 (request for support of DLA, PIP, ESA, reconsiderations and appeals)
- Social care referral – 8
- General support - 3
- Health – 3
- Charity application – 2
- Legal – 1

14
new member packs sent

23
visits, meetings and clinics attended

4
visits made to new member's homes

47
key reports, letters and forms completed

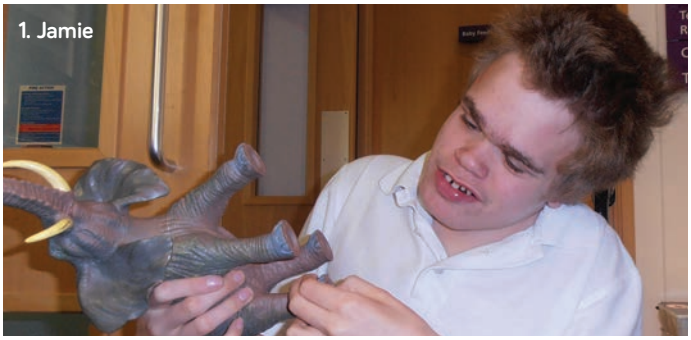
For more information on any of the above or if there is anything else that you would like to chat with the advocacy team about please contact us:

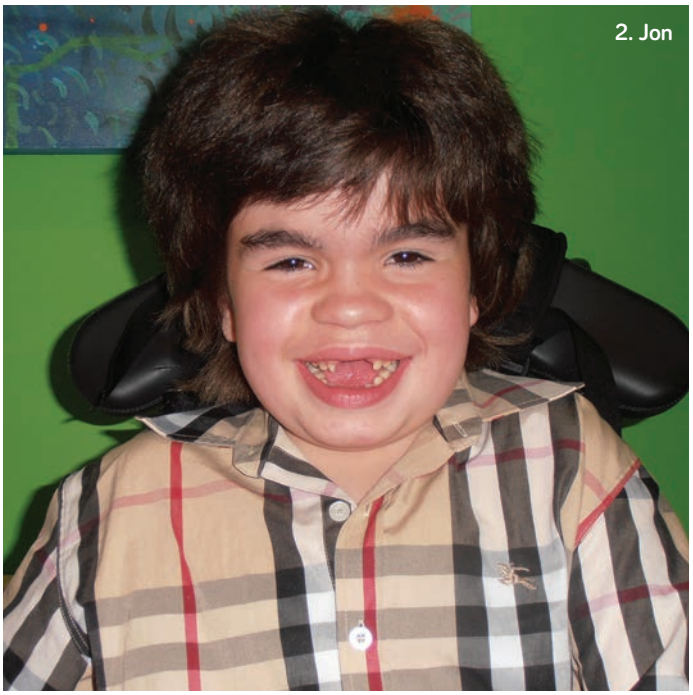
e: advocacy@mpssociety.org.uk
t: 0345 389 9901

Clinics

GOSH MPS III 28 JUNE

(1–2)


1. Jamie


2. Jon

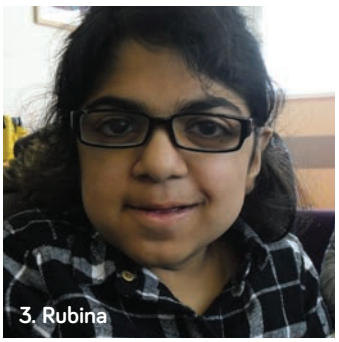
I attended a very busy clinic run by Great Ormond Street Hospital, on an afternoon where the weather could not make up its mind!

I was welcomed to the clinic by Nurse Specialist Sindi Mnkandla and enjoyed meeting four families that have young people with MPS III Sanfilippo Disease. It was especially good to meet one of the young people that the MPS Society has recently been supporting, and was exciting to hear how we had been able to support families in the past and to meet the other families that we may support in the future.

All of the young people where happy and a joy to be around, and I am finding that attending clinics is a good way to catch up with families to hear how they are. It was fantastic to hear that the young people were doing well educationally and that the families are happy with the support they are receiving from services so far. It was also good to hear that one family is in the middle of having their property adapted for their son, and that they are looking to continue to raise funds and awareness of the disease.

I look forward to meeting many more families at future clinics, to catch up and provide support as needed.

Louise Cleary
Advocacy Support Officer


3. Rubina

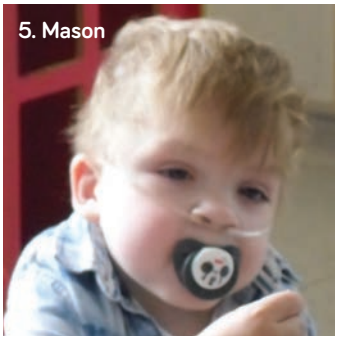
MCH MPS I 1 & 8 JULY

(3–7)


6. Mia


7. Demi


4. Morgan


5. Mason

There were 2 clinics in July to meet with the members in Manchester and it was lovely to see them all.

A special mention to Demi Leigh who became an Auntie that day!

Debbie Cavell
Senior Advocacy Support Officer

8. Sophie

GOSH MPS I 26 JULY

(8–11)

It was lovely to introduce myself to some families that I have not met before and to see how they are getting on. The girls were looking forward to the long summer break and having adventures and going holiday.

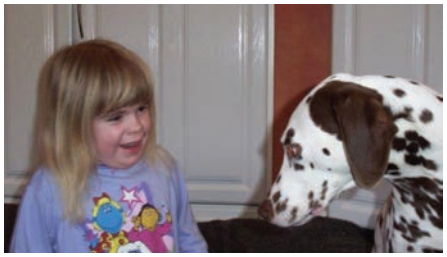
Debbie Cavell
Senior Advocacy Support Officer

9. Maurice

10. Hannah and sister, Emily

11. Abby

Remembrance



Sophie Summerton
20th May 1997
–9th July 2016

Our lovely Sophie died in July after a short illness peacefully at home with us beside her. As everyone says it was a big shock and it takes a lot of adjusting to life without her, a journey we are just beginning.

We wrote the words below and they were read at her funeral. They sum up Sophie's love for life and mischievous sense of humour.

About Sophie

In December 1995 we were blessed with the arrival of Will.
17 months later Sophie arrived on the scene with a very loud cry and masses of hair!

Sophie was a lively toddler and fairly wilful, not taking kindly to instruction. Many of our friends remember Sophie's screams as I tried to put her into her buggy. If I let her walk and then tried to put her back in, it could take three or four of us to succeed. She would scream Croxley Green down.

Sophie loved her holidays in Dorset where she and Will would spend time with all the animals, and they would take over egg collection and poo picking. She would take hold of a spare halter or lead rein and that would be hers for the week.

Sophie enjoyed her school time very much and pursued lots of activities there. Rebound therapy (trampolining) was one of her favourites, where she would gaze at Darren, her teacher, thoroughly absorbed.

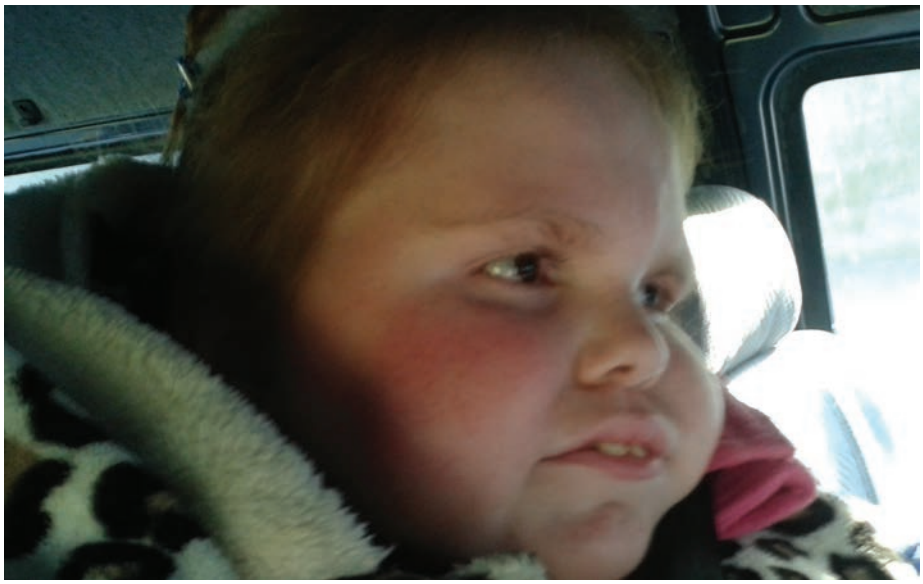
Hydrotherapy was another. She cuddled up to Denise, who was with her at school for years, and loved the whole experience.

As Sophie slowed down, she people watched and enjoyed any misfortunes people might encounter, that crafty smirk would appear – there was so much going on behind those big brown eyes!

Sophie left us very peacefully and we will all miss her forever. She brought so much into our lives and taught us a great deal too.

Love you sweetheart.

Tim and Sally Summerton



Helen Skidmore
14th March 1979–26th May 2016

Helen – "Our Precious Gift" by Pat

The house is so quiet,
No calls in the night,
And wish as we might
It goes on after morning light.
"Help me out of bed" you called,
"Put my phone on in case someone calls,
I'm missing gardening on TV,
So I can tell Mum what I see".

A room now empty where we sit and tears flow,
Remembering what has been,
What is to come we do not know.
Whatever life threw at us, you taught us how to cope,
With trust and endurance and a will of might and hope.

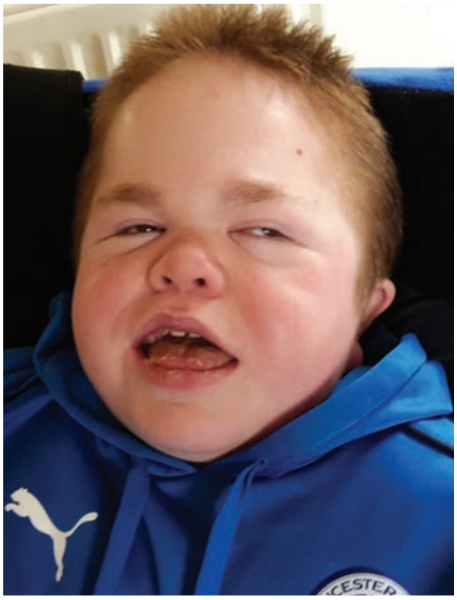
Forever we will remember the times we laughed and cried.
The joy you brought to simple things, until the day you died.
We always will be grateful for the happy times we shared,
But though we knew your time was short, we never were prepared.
You were a gift so precious, we could not let you go.
But your burdens had become so heavy, it was time for you to go.

Your battle here is over and ours has just begun,
The new pain is unbearable, can it ever mend?
It was such a privilege to care for you,
We would not change a thing
We hold you in our hearts for life,
Until we are one again.

Luke Edwards
8th September 2003–13th July 2016

"We were lucky to have Luke in our life for 12 wonderful years. He was loved by everyone who knew and met him and we miss him very much. We have many wonderful and happy memories and pictures which we will treasure forever."

Louise and Michael



Bereavements

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

Isabel Annakin who suffered from Metachromatic Leukodystrophy and passed away on 22nd June 2016 aged 10 years.

Connor McDougall who suffered from Hunter and passed away on 16th June 2016 aged 17 years.

Your stories

We talk to three of our younger members about their interests, achievements and living with MPS

Onaissa at her graduation

The knitter

Onaissa Jamil (left) was diagnosed with Alpha Mannosidosis in her 20s but managed to balance living by herself and studying to complete her degree in textile design

I was diagnosed with Alpha Mannosidosis at 23 years. Quite late! But I have always had balance and coordination problems since I was a child.

I started studying Textiles at school. I continued this at BTEC and HND level at College. My love for Textiles began getting more and more interesting. After a lot of hard work I gained an HND in Textile Design at college. I was then fortunate enough to go to university and study further.

What did you most enjoy about university?

I enjoyed university because I made great friends that hopefully I'll keep in touch with. I shared student accommodation with some really nice people. I experienced independent living and realised how tough it is doing everything by yourself! I liked that I lived on campus and I lived near my best friend so we saw each other every night without fail.

What inspires you?

My inspiration comes from travelling to different parts of the world. I love looking at different landscapes, architecture and Urban Cities. When I visited Morocco, I loved the sea views of the sand and the blue sea. I visited Istanbul and studied the architecture of the palaces for one of my University Projects.

Creativity is very broad. I put lots of creative ideas into the shapes, colours, textures and patterns in all the work I have done. Nothing is right or wrong! There is a huge range of designs out there you can experiment with.

How did your diagnosis affect you at university?

My sketching and drawings were never my strongest points, but with practice and different ways of expressing drawings I overcame this. Mentally it didn't affect me because I just got on with everything. I try and keep myself fit and healthy and do exercises to help with balance and coordination.

What challenges did you have to overcome to complete your studies?

While I was at university I have become more confident in speaking to new people. I find explaining things to people difficult, but this has got better at university. I have had to do lots of things by myself and this has sometimes been challenging.

At university I had to do presentations to the class. I was very nervous but again I overcame this challenge by practising and being prepared.

Not only were the projects hard at times but also living and supporting myself such as food shopping, eating and paying bills were sometimes difficult.

What support did you get?

My mum supported me with all my projects such as discussing ideas, looking at designs and ways of presenting my work. My tutors and the university were so supportive with all my work. I could go to the tutors if I felt overwhelmed with deadlines. I also had a note taker for lectures.

I even had support from the office for Student Accommodation. They were always helpful with any problems.

To be honest I have had such good support all round. I am grateful for everything and everybody who has supported me.

What advice would you give others with Mannosidosis based on your experiences?

Keep busy and be positive. It is difficult but I feel there is always a solution to a problem. Might be hard to find but it will come to you eventually.

What's next?

I have always had an interest in interior products such as cushion covers and throws and accessories such as hats and scarves. This industry is very competitive so I decided to start my own business and develop my ideas and designs. I have changed my bedroom into a workshop/bedroom. I have two knitting machines and have some orders already to go. I would love to share my passion with you too.

Onaissa has set up a business selling bespoke interior soft furnishings called Ojinteriors

You can see her latest designs on facebook:

www.ojinteriors.facebook.com/

Espen in his vlog about Vimizim

The vlogger

Espen Johnson has been a keen video blogger since 2008 injecting plenty of humour into his vlogs and sketches which often reference his experience of being in a wheelchair and living with MPS IVA.

How did you get into vlogging?

I first discovered vlogging in 2008, when a friend of mine showed me this guy called KevJumba on YouTube (which I had previously only known as the music/cat videos site). This random teenager, Kevin, was on the other side of the world but it felt like he was talking directly to me, connecting and sharing a story about his daily life in a way that I had never seen before, and he was really, really funny. It looked like he had a lot of fun doing it and, unlike film and TV, it didn't seem impossible to do, so I thought I could try it too. I then stole our family's camcorder (which never returned to my parents) and fell in love with filming and editing.

What's your favourite vlog post?

It's difficult to say what my favourite vlog is because by the end of the whole process I really dislike my videos; in editing you end up seeing the footage a thousand times, so the joke you thought was funny at first just becomes annoying, and so it's difficult to stay objective and say what you like. However a recent vlog I enjoyed making is called 'Entering the Paralympics?' and is about (some) people's expectations for me to be in the Paralympics because I'm in a wheelchair, as it's obviously that easy, right? The response to the video has been really great, and I had a lot of fun shooting it with a couple of friends of mine, so I think that's my current favourite.

How do you manage your time?

In reality I don't manage my time...at least not well. The type of vlogs I enjoy making take more time to do; I'm an aspiring filmmaker so I often use my vlogs to practise various aspects of filmmaking, but this means it is generally not something I can quickly do in a couple of hours. As a result you can see a gap in my uploads every year around spring as I shut down because of exams, and I simply can't make vlogs because of how time (and energy) consuming it is.

However vlogging is my main hobby, and I often use it to socialise with friends, so it is simply setting aside the time others might spend watching TV, or going to the pub (though I do those things too, just a bit less at times). Also, the great thing with vlogging is that it can be done in stages; I can script write one day, and film one segment another day, and edit one section another. So most of the time (exams are a special exception to everything) I can continue making vlogs in small sections regardless of how busy I am, though it means I don't upload as regularly as some.

How do you manage the practical elements (lights and cameras, etc.) whilst being in a wheelchair?

As with everything in my life, I've developed my own little techniques to managing the practical elements of filming. I've simply learned by doing it, and improving from my (many) mistakes. This is the great thing with vlogging on YouTube, it gives you the space to make mistakes and to learn; there's not any set way of doing things, you don't have a whole team of people who you have to work with (unless you choose to do so), who will be annoyed at you taking longer to set up a light. You can do it in your time, in your way (flipping my tripod upside down to let gravity extend the legs is a favourite of mine).

How does having MPS affect your vlogging? Or your subject matter? Or more generally?

I make vlogs about my life, and having MPS and being in a wheelchair is an integral part of my life, it shapes my whole experience, and thus it's often the topic for a video. However it's been important for me to create a space where I can talk about other things as well; MPS is one aspect of my life, not all of it, and I enjoy talking about my other interests. I treat it very casually, sometimes just making quick, off hand comments relating to it, sometimes not mentioning it at all (though you can clearly see I'm in a wheelchair). I also make a lot of jokes about it, as I find humour is a great way to bring people's guard down about disability; I enjoy making comedy, and I mostly draw from my own experiences, which includes MPS.

I hope that my videos will help normalise disability, that it will help people see that it's not something taboo or dangerous to talk about, it's just another aspect of my life, which doesn't always require pity, and certainly does not make me 'inspiring'.

Who are your favourite vloggers?

One of my favourite vloggers at the moment is Casey Neistat which, if you're familiar with the YouTube world at all, is the most cliche answer I could give, but it's true. Casey makes these incredible daily vlogs of his life which are filmed and edited so well, they are gorgeous to watch and I just feel inspired after every one to improve my own filmmaking.

See more of Espen's vlogs and sketches on his YouTube channel, Waist High View: www.youtube.com/user/WaistHighView

Or find him on Twitter @WaistHighView

Emma

The driver

Emma recalls her experience of living with Hurlers and her determination to get a driving licence

My name is Emma slater I am 23 years old and I have MPS I Hurlers disease. I had two bone marrow transplants in 1994 in Manchester Children's Hospital, I also have scoliosis for which I have had around 10 to 12 spinal operations, including Harrington rods which were lengthened every 6 months over a 10 year period. I also had a heart valve replacement when I was 18 years old in Birmingham Children's Hospital and most recently a total hip replacement on my left hand side at Salford Royal in Manchester.

I suffer some form of pain most days and prior to my hip replacement I was in pain every single day taking morphine and all the strong painkillers to help me get by. From an early age I decided I was not going to let MPS beat me and I would do what I wanted to do, as much as I could.

I studied hard at school and got four GCSEs. I then went to college on a child care nursing course for three years. Attending placements in various nurseries around the Worcestershire area. It was a long hard three years but I managed to complete the course and gain the qualifications required to be a nursery nurse, I was also awarded best student of the year for the whole three years I was at college.

During my time at college I asked my dad if it would be possible for me to drive, I started to learn to drive at 16 years old as you are allowed to if you have a disability. We had to get a new mobility car I needed an automatic and it had to be adapted with a special seat raiser so that I could see over the steering wheel and out of the windscreen. My dad took me out first so I could get the basic knowledge of driving and to see if I would like it, which I did. I then booked and took my Theory test which I passed with a score of 99%. I carried on my weekend lessons with my dad for a couple of months then when I felt confident I booked some lessons with a driving school. My driving instructor was called Rod, he was an old gentleman but very patient.

I took my first test and failed on a couple of minor points, the examiner said I was very unlucky to have failed, but I made one silly error on my return to the test centre which caused me to fail. I went out again with Rod, me and my dad practised and practised, and I was determined I was going to pass second time round. I took my test for the second time and passed with only two minors which was an excellent score the examiner told me. Now I had a driving licence and my own car, all I needed was some money to fill it with petrol and keep it going!

My final nursery placement from college was called Smileys Creche – it is attached to the Worcestershire Royal Hospital. I enjoyed it there and worked hard to see if I could get a permanent job. At the end of the placement and the end of my college course Smileys offered me a part-time job, to be honest I couldn't do much more than part time as my hip had worsened and I could hardly walk but I was grateful of the job and took the opportunity.

I started working 2 to 3 days a week and I also had a Sunday job at a local DIY store. Just as I got into the full swing of working up to 4 days a week my hip began to give way and started playing me up. The staff were really good and they noticed this and I was allowed to work in the baby room so I didn't have to do much walking around. It was at this point that I realised I needed something done with my hip. It took 2 years to finally get a hip replacement which was done on 8 March 2016. I am now out of the precautionary period of 12 weeks and can start to drive again and go back to work part time.

I am now looking forward to going back to work and I will also be able to start driving again. I can now walk without my crutches or a walking stick, I am not feeling as much pain in my hip as I did before which was the main reason I had my hip replaced in the first place. My walking is still not perfect, I suffer with stiffness in both my knees and ankles, this gets me down sometimes but I am not going to let that stop me doing what I want to do.

MPS 2016 BONN

International Symposium on MPS and related diseases

The 14th MPS Symposium, held from 14–17 July in Bonn, in Germany is over. The event welcomed 1056 participants from 52 countries of the world – families, physicians, scientists and other interested people.

It was an interesting conference that lit many new and important aspects of MPS diseases and also of basic research in the area of lysosomal storage diseases in general. Here's a round up of the presentations.

Thursday 14 July

There was a symposium on **Putting the Patient at the Center of Care**. This included presentations from:

- The Scientific Challenges to Delivering New Therapies for the MPS Diseases Brian Bigger, UK
- Clinical Trial and Access to New Therapies for MPS Diseases Emil D. Kakkis, USA
- Treatment and Monitoring of MPS Patients with Multisystemic Involvement Roberto Giugliani, Brazil
- Empowering Patients and the Future Role of Patient Organisations Georg Schetter, Germany

Alongside the scientific programme there was a satellite meeting on the **biology of the lysomal network** on the Wednesday and Thursday.

Friday 15 July

Our advocacy officers Louise and Debbie attended presentations on the Friday. The highlights of the day are summarized below.

The Natural History of Cognitive, Behavioural and Neuroimaging Characteristics of MPS III – Elsa Shapiro

This natural history study investigated the gross and fine motor skills, speech, behaviour and cognitive functioning against ages and stages of development for typical children. The grey matter was measured in the participants' brains through scans and observed a rapidly progressive group and a slower progressive group of children that visited their centres.

The study found that there were common behavioural symptoms for children with MPS IIIA and MPS IIIB, but that there were a few differences to be explored further. It was found that there was a faster loss of Amygdala compared to the loss of grey matter. Amygdala being the part of the brain that processes emotions linked to both fear and pleasure responses.

Intracerebral Gene Therapy for MPS III A – Samantha Parker

In 2011 Lysogene launched their phase I/II trial with four patients, which they have the results for. The initial study showed that much of the patients' hyperactivity was reduced.

In 2015 preclinical studies took place for the second stage of the trial. Lysogene are going to be using an Adeno-associated virus (AAV) Vector, where altered genes are put back into the virus. The aim being that the virus does not cause infection, and will hopefully support the reduction of glycosaminoglycans (GAGS) in the Central Nervous System. They will be delivering the drug straight in the subjects' brain by an intracranial approach, in the hope that the virus will spread into other parts of the brain cells. They hope to

be recruiting participants for the treatment study in 2017. In the meantime they are inviting patients to partake in a natural history study, as they still need to gather information, to compare their data against, to see how well the treatment will work.

Intravenous Gene Therapy for MPS VI – Generoso Andria

This treatment is being developed in Italy for those with no Central Nervous System involvement. It is hoped to gain approval to give a weekly infusion, and they currently are going through ethics of delivering the drug before Food and Drug Administration and European Medical Authority approval can be given.

The team are using a vector to deliver the treatment through an injection directly to the liver. The drug is currently being manufactured in the USA and they will be working towards using it in Europe.

Sleep Disorders in MPS: Changes of the Circadian Rhythm – Stewart Rust

Sleep depravity appears to be one of the hardest symptoms for families to cope with, as it means that enormous changes are made to daily living.

Parents have reported difficulties with:

- Settling individuals to bed
- Nocturnal waking
- Disruptive/Dangerous behaviour
- Early morning walking
- Reduced sleep at night and increased sleep in the day
- Aggressive behaviour

There has been research on eight children aged 2-15yrs with MPS III Type A and B Sanfilippo Disease, where the use of melatonin was stopped and saliva was collected. The data was collected through a sleep questionnaire and through data tags. It was reported that children's sleep worsened when taken off the melatonin, and also that as children became older their sleep difficulties increased.

It appears that sleep and behaviour are linked, so trying to break the cycle and put a routine in place is important even if the young person does not sleep during the night time.

Fusion Protein Therapy for MPS I and II – Roberto Giugliani

Roberto talked about how scientists have been looking to make a device much like a Trojan Horse in which to transport the drug to get across the blood brain barrier into the cells by building genetically engineered fusion protein. He explained that tests on animals have proven that the enzyme reaches the Central Nervous System (CNS) and reduces the glycosaminoglycans.

A two part study focussing on six adults who have Hurler Scheie or Scheie is underway. The conclusion so far is that enzyme replacement therapy with fusion proteins are a good way to target the CNS and the safety results look positive so far.

The Experiences of Women with MPS during Pregnancy and beyond – Deborah Cavell

The aim of this study was to, for the first time, interview women with MPS and related conditions who have had successful pregnancies in order to gain a meaningful insight into their needs and experiences during this time. It was also the aim of our group to take steps toward developing guidelines for the management of these complex pregnancies and to develop information resources for women with an MPS or related condition of childbearing age.

Saturday 16 July

On the Saturday the scientific programme included chaired sessions on the pitfalls and advances in therapy, new developments in related disorders, new approaches to therapies and breaking news. Some of the highlights are summarized here.

How to manage immunological reactions – Paul Harmatz

The mucopolysaccharidoses are caused by the absence or reduced function of lysosomal enzymes needed to break down glycosaminoglycans (GAGs). In the absence of lysosomal enzyme function, GAGs collect and result in progressive cellular damage and organ system dysfunction. Until recently, care has depended primarily on supportive care with hematopoietic stem cell transplantation (HSCT) providing the only specific therapy to address the deficient enzyme. Since 2003, enzyme replacement therapy (ERT) administered intravenously has been approved for MPS I, II, IVa, and VI to provide specific therapy. Despite this major advance in therapy, immune responses do occur to the infused protein in most patients and may lead to infusion associated reactions. These reactions are mostly mild to moderate in severity can be managed in most patient by slowing infusion rate, administering or pretreating with antihistamine, anti-pyretics or other immune system modulating medications. In rare patients, these reactions are severe and the care team should be prepared to manage anaphylaxis effectively. In this presentation, Paul described typical infusion associated reactions, possible mechanisms underlying these reactions, infusion protocols that may reduce the likelihood of reactions, protocols for managing typical or severe reactions.

Mannosidosis: genetic, clinical findings, options of treatment line – Gutte Borgwardt

Velmanase alfa (Lamazym or rhLAMAN) is a recombinant form of the human enzyme alpha-mannosidase, currently in development as enzyme replacement therapy for the treatment of alpha-mannosidosis. The biological activity of Velmanase alfa in correcting alpha- manno-sidase deficiency was demonstrated by a significant and sustained clearance of serum by oli-gosaccharides accumulation. A statistically significant benefit on patients' motor function was also documented as progressive improvement from baseline in 3-Minute Stair Climb Test (3MSCT). In addition, clear trends for improvement in 6-Minute Walking Test (6MWT) and in the Bruininks-Oseretsky test of motor proficiency, second edition (BOT-2) were observed in the pediatric population. Results support the relevant benefit of treatment of alpha-mannosidosis patients with Velmanase alfa, especially when treatment is started early in the pediatric age.

Respiratory therapy with music and iPad – Ruud van der Wel

Ruud is the founder of my breath, my music. He believes that music is a fun motivator to encourage breathing exercises.

So far he has developed the Magic Flute which is suitable for those of a smaller stature. He is also in the midst of a breath controlled app project called Groovtube. Ruud believed that the instrument could be adapted for even those with low lung capacity.

Sunday 17 July

The changing face of anaesthesia for Mucopolysaccharidosis – Robert Walker

Robert commented that it has been historically known that MPS I Hurler disease sufferers have "the worst airway problems in paediatric anaesthesia". He recommended that a thorough assessment takes place before any anaesthetic by a multi-disciplinary team. Also that there are various anaesthesia options and these may be considered dependent on the surgery to be performed. For example, laryngeal mask airway (LMA) is the preferred option for short procedures such as carpal tunnel surgery or fitting t tubes. LMA fibre optic intubation is recommended for IV access devices or cardiac or orthopaedic surgery. Spinal anaesthesia may be the preferred option for airway surgery or corneal grafting.

Pain management in Mucopolysaccharidosis – Chiara di Pede

Joint pain can occur due to the MPS deposits leading to inflammation, swelling and stiffness. Nerves can be entrapped due to MPS deposits, resulting in carpal tunnel syndrome, for example. Skeletal abnormalities can also cause pain and also headaches can be due to intracranial hypertension.

There are two types of pain – nociceptive (caused by damage to body tissue) and neuropathic (nerve damage) and individuals can suffer from a combination of both. Other causes of pain can be GE reflux, fractures, dental issues, renal calcinosis, pressure sores or procedural pain. Pain management often relies on pain evaluation and this can be done by a scoring system. When the patient has communication or learning difficulties there is the FLACC scoring system which uses observation of movement and tenseness to evaluate pain.

Acute pain can be relieved by pain relief and anti-inflammatory medication. The following can also be used to manage pain: physiotherapy, hydrotherapy, coping strategies, relaxation, distraction, controlled breathing, psychological support and hypnotherapy.

More information and more photos of MPS 2016 are available at www.mps2016.com

A video of the symposium is available online at https://vimeo.com/175370875

The Leonard family

With support from the UK MPS Society, we were able to participate at the 14th International MPS Conference with over 1,000 participants including medical professionals, carers, parents and those with a wide range of MPS conditions. There were talks and events for everyone. Tributes were paid to Christine Lavery for helping formulate the conference. MPS Trustee, Bryan Winchester contributed to several of the debates about clinical techniques.

One of the highlights was a walk accompanied by a German Band that took us through a wood to a lake where a Memory Tree had been placed. Many of us added a message of hope and afterwards we were treated to local German food and wine.

The conference provided a great opportunity to share experiences with a very good mix of learning and social activities. Our hosts were always very helpful, the hotel very suitable for the large number and variety of wheelchairs and thus, the conference worked successfully on many different levels.

Paul, Jean & Christopher Leonard

We are pleased to announce that one of our four posters presented at the 14th International Symposium on MPS and Related Diseases in Bonn was shortlisted for Best Poster!

Over 160 abstracts were submitted from 28 countries and our Poster for "Multistakeholder engagement leading to access to treatment for MPS IVA (Morquio A) a model for the ultra-rare disease community" was one of four that won. MPS Commercial worked to pull together the abstract and was supported by Helen (creator of our magazine) at the MPS Society who designed the fabulous poster. The win gave us the opportunity to present to the symposium about the campaign for access to Vimizim.

You can see the winning poster opposite and we will be featuring the other three posters across this magazine and the Winter 2016 issue.

Thank you to everyone who participated in the research surveys your involvement ensures that we continue to gain a greater understanding and demonstrate the impact of the disease.

MPS Commercial have a number of research projects on the horizon with the aim of continuing to collect data on MPS and related conditions which will help assist pharma companies for future trial designs and be referenced by NICE during their reimbursement decisions. We look forward to reporting our future survey results in the magazine.

Charlotte Roberts

David Besley and Charlotte Roberts

Multi-stakeholder engagement leading to access to treatment for MPS IVA (Morquio A) – a model for the ultra-rare disease community(BM)

Charlotte Roberts,[1] Christine Lavery,[1] Nigel Nicholls,[2] Mohit Jain,[2] Christian J Hendriksz,[3] Sheela Upadhyaya,[4] Edmund Jessop.[5]

[1]The Society for Mucopolysaccharide Diseases (MPS Society), Buckinghamshire, UK; [2]BioMarin Europe Ltd, London, UK; [3]Salford Royal NHS Foundation Trust, Salford, UK; [4]National Institute for Health Care Excellence (NICE), London, UK; [5]National Health Service (NHS) England, UK.

Objectives

To achieve reimbursement for elosulfase alfa for MPS IVA patients resident in England.

Background

- MPS IVA is an ultra-rare disease affecting less than 100 patients in England.
- In 2013, responsibility for the reimbursement decision making process for treatments for rare diseases, formerly governed by the Advisory Group for National Specialised Services, was replaced by a joint process involving the Highly Specialised Technologies Evaluation Committee of NICE and the Programme of Care Group of NHS England.
- The only treatment currently available, elosulfase alfa, was licensed by the European Medicines Agency on 28th April 2014.
- The UK had been a major contributor to the Phase III clinical trial with 35 patients being enrolled out of the 176 recruited worldwide.
- Interim funding was not available when elosulfase alfa was licensed and there was a high degree of interest and concern in continuing access to treatment in England.
- Although patients who had taken part in the clinical trial continued to receive free drug, other English MPS IVA sufferers had no access to treatment.

Methods

On the 21st November 2014, a 10 year old boy, supported by the MPS Society legally challenged NHS England's scorecard decision method. This marked the start of a year long process involving the engagement of all stakeholders to develop a workable solution for treatment access (Figure 1). Patients together with the patient organisation MPS Society UK, members of Parliament and clinicians canvassed NHS England and the Department of Health for a fair process with equal access to therapies as for common disorders (Figure 2).

This resulted in elosulfase alfa for MPS IVA being referred to NICE for full evidence review and decision. During the NICE process, the MPS Society suggested a robust procedure whereby all patients that met a set of criteria would be able to access treatment (Figure 3). Stopping criteria were also included for the first time ever. This was incorporated by NICE and announced in their draft guidance in September 2015.

The development of the Managed Access Agreement (MAA) became a working partnership between NHS England, NICE, the MPS Society, BioMarin and a clinical expert.

The MAA was designed to be inclusive for patients, ensuring response to treatment in a minimum of 4 out of 5 criteria through consistent clinical and quality of life monitoring. An intensive follow up programme and multi domain assessments would be required and treatment would stop for those not meeting treatment targets (Table 1).

Results

On 16th December 2015 NICE guidance recommended elosulfase alfa for patients in England via the MAA.[1,2] As of 31st May 2016, a total of 46 patients have been recruited to the MAA through 7 hospitals in England. This represents 48% of the 95 patients known to have MPS IVA in England. Of these, 27 patients previously took part in the clinical trials for elosulfase alfa, and 19 patients are receiving this new treatment for the first time.

Table 1. Response criteria for continued treatment[3]

Response criteria	Naive patient (in 1st year of treatment)	Previously treated patients (2nd year or more on treatment)
Improvement of 6 MWT or 25ft Ambulation Test	10% Improvement over baseline	Remains 5% above baseline
Improvement in FVC or FEV-1	5% Improvement over baseline	Remains 2% above baseline
Stabilisation defined as no adverse change in the numerical value in two of the following three measures: • Quality of Life as measured by the EQ5D-5L or MPS HAQ Caregiver Domain • Beck depression inventory • Adolescent Paediatric Pain Tool or Brief Pain Inventory depending on age	Stabilisation	Stabilisation
Reduction in urinary keratan sulfate	20% Reduction from baseline	Remain reduced at least 20% from baseline value
Decline in ejection fraction as measured by echocardiogram	Decline of less than 10% from baseline	Decline of less than 10% from baseline

FEV: forced expiratory volume, FVC: forced vital capacity, MWT: minute walk test

Figure 3. The Managed Access Agreement criteria[3]

Start criteria
- Confirmed diagnosis of MPS IVA
- Confirmed enzymatic test, elevated urinary keratan sulfate and mutation analysis
- Sign up to the 'Managed Access Patient Agreement'
- Full set of baseline assessments obtained for patients over 5 years of age

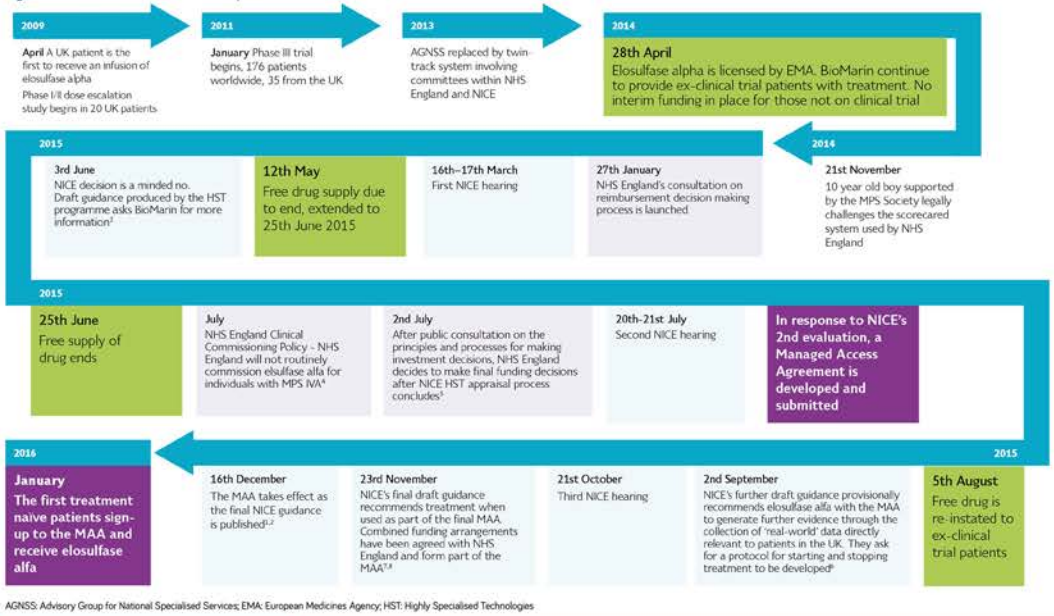
Exclusion criteria
- Patient is diagnosed with an additional progressive life limiting condition where treatment would not provide long term benefit e.g. cancer or multiple sclerosis
- Patient has a lung capacity (FVC) of less than 0.3 litres and requires ventilator assistance
- Patient is unwilling to comply with the associated monitoring criteria

Stop criteria
- Non-compliance with assessments for continued therapy (non-compliance is defined as fewer than three attendances for assessment in any 14 month period)
- Patient fails to meet 4 of the 5 treatment response criteria (Table 1)
- Patient is unable to tolerate infusions due to infusion related severe adverse events that cannot be resolved

Patients who are taken off treatment will continue to be monitored for disease deterioration and supported with other clinical measures.

FVC: forced vital capacity

Figure 1. The reimbursement decision process



AGNSS: Advisory Group for National Specialised Services; EMA: European Medicines Agency; HST: Highly Specialised Technologies

Figure 2. MPS IVA patients, families, the MPS Society and MPs, campaign for treatment access

- Engagement with 40 MPs, parliamentary questions led by Greg Mulholland, MP
- 3 meetings with the Minister for Life Sciences George Freeman
- 2 Adjournment Debates
- MPS Society hosted Westminster Hall event attended by MPs and peers, pharma representatives, patient organisations and the BBC
- 6 protests
- Parent met with the Prime Minister David Cameron
- Online petitions 'NHS England's scorecard system denies access to treatment for ultra-rare diseases' and 'Call for interim funding'
- Articles in the national and local press
- Social media campaign #fundourdrugsNOW #fight4treatment

Clockwise from left: Kamal (left) and Sam outside 10 Downing Street; Luke (left) and Olivia campaigning; Sunday Express cover story from 12 July 2015; and Kamal (left) and Sam campaigning with MPs, parents and members of MPS Society

> "There have been a couple of signs of Vimizim doing something...I have been in the garden for the first time in a long time last week and for the first time ever, I saw the legs of a caterpillar!! This may seem daft and simple, but due to the clouding of my corneas I have never seen much detail on anything.
>
> A patient's experience of treatment

Conclusions

In an environment where health systems are having to choose between high cost drugs and the funding of other health resources, the MAA, with a confidential financial arrangement, offers all patients meeting the treatment criteria access to reimbursed therapy in the first 12 months. The MAA will be subject to annual review under the chairmanship of NICE and the data collected will be used to assess whether NICE will continue to fund the treatment after the 5 year term of the MAA.

Whilst we are in the first year of this new initiative, MPS IVA patients have embraced the MAA and recognised that adherence to the MAA is the only way forward to ensure continued access to treatment. Only time will tell if the stopping criteria are fair and if patients affected by common disorders will become subject to similar requirements in the future to ensure equity across all aspects of health.

Society for Mucopolysaccharide Diseases
www.mpssociety.org.uk

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The educational journey of individuals with MPS II Hunter Disease in the United Kingdom(B)

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[1]The Society for Mucopolysaccharide Diseases (MPS Society), Buckinghamshire, UK

Introduction

- Hunter disease (MPS II) is the only known X-linked MPS disorder.[1]
- The deficiency of the lysosomal enzyme iduronate-2-sulphatase leads to a progressive accumulation of glycosaminoglycans in the body and an array of clinical manifestations, including skeletal and cardiac abnormalities.[1]
- Traditionally, individuals were classified as 'mild' or 'severe', based on the absence or presence of central nervous system (CNS) involvement; it is now recognised that the syndrome exists in a range somewhere between the two extremes, now classified as attenuated to severe.[1,2]
- Around two-thirds of individuals are estimated to have the severe phenotype (i.e. progressive CNS involvement); these individuals experience learning difficulties and neurological decline.[1,2]
- The aim of this project was to determine the cognitive variability in patients with MPS II in the United Kingdom (UK) and to understand their needs and support requirements in an educational setting.

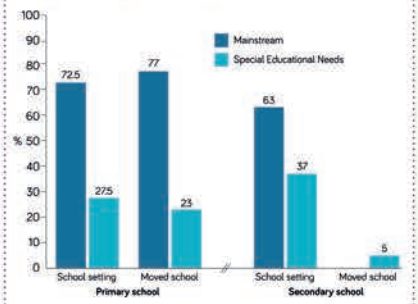
Methods

- Seventy-one individuals with MPS II resident in the UK were identified by the MPS Society and invited to take part in the survey via telephone interview.
- A specifically designed questionnaire was used to assess the individual's diagnoses, treatment, educational attainment and need for support from primary through to further education.
- Interviews took place in December 2015 and January 2016.
- Results for the education section of the survey only are presented here.
- Results for the diagnosis and treatment section of the survey are presented in Poster 165.

Results

- Forty-one individuals agreed to take part in the study (58%), ranging in age from 1 to 36 years (mean 12.3 years).
- Of the 41 individuals surveyed, 54% (n=22) reported CNS involvement; 37% (n=15) reported no CNS involvement; and 4 individuals (10%) did not know whether there was CNS involvement or not.
- A review of all respondents data indicated all but 3 had some level of CNS involvement; 49% (n=20) had severe progressive CNS involvement.
- One patient, aged 1 at questionnaire completion, was too young to have attended nursey/primary school and is excluded from this analysis.
- Most individuals started their education in a mainstream school (Figure 1); one third of individuals (n=13) moved primary schools as their learning needs were not being met; individuals moved school at a mean age of 6.8 years.
- Three quarters of the individuals who moved primary schools were originally in a mainstream school (n=10) (Figure 1); of these, 8 moved to a special educational needs school (SEN); 1 individual moved to a mixed school, 1 did not answer.
- All individuals who moved from a primary SEN school (n=3) moved to an alternative SEN school.
- Individuals with central nervous system (CNS) involvement were more likely to move school than those without (55% vs 7%).
- Nineteen individuals had attended or were attending secondary school (Figure 1); 1 individual moved from a secondary SEN school to mainstream secondary school at age 12.
- Statements of educational need or educational healthcare plans (EHPs) were issued to 73% of individuals in primary school (mean age 4.9 years).
- The reasons for issuing statements or EHPs in primary school were learning needs (15%), physical needs (5%) or both (52%).
- Fewer individuals (68%) had statements or EHPs during their secondary education; 23% of these had been issued at secondary school (mean age 11.3 years).
- The reasons for issuing statements or EHPs in secondary school were learning needs (7%), physical needs (15%) or both (53%).
- More individual education plans (73% vs 42%) and flexible teaching (57% vs 40%) were available in secondary compared to primary schools.
- Flexibility included alternatives to physical education (PE) lessons (26%), support lessons, and options to drop a GCSE in mainstream schools; and totally individual lesson plans in SEN schools.
- A breakdown of the support provided in primary and secondary schools is shown in Figures 2 and 3, respectively.

Figure 1. Primary and secondary education.




Yusuf on his way to primary school


Clark ready for school

James in action at college

Figure 2. Support provided in primary school

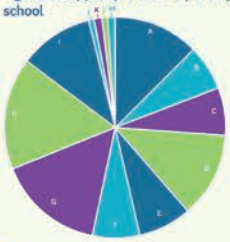
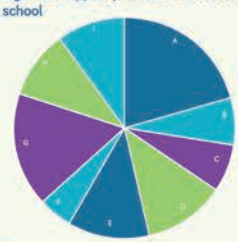


Figure 3. Support provided in secondary school



- The most commonly used specialist equipment in primary schools were chairs, pencil grips and laptops/iPads (all 15%).
- In secondary school, hearing and radio aids (26%), specialist chairs (21%) and laptops/iPads (15%) were commonly used.
- A breakdown of professional input for individuals in the primary and secondary setting is shown in Figures 4 and 5 respectively.

Figure 4. Professional input in primary school

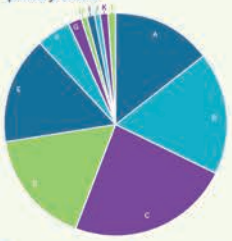
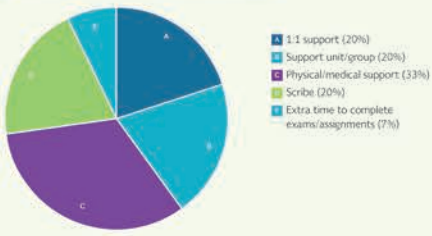


Figure 5. Professional input in secondary school

SENCO, special educational needs coordinator

- Sixty three percent of individuals felt that their support needs had changed from primary to secondary education; with reasons cited as a decline in mobility/more help to move around larger schools (31%), difficulty understanding work (57%), and deteriorating health/surgery (15%).
- Of the 13 individuals aged 16 or over, 69% had obtained GCSE or equivalent qualifications.
- Ten individuals were attending or had attended further education including 6th form, college and university; all received some degree of support throughout their further education, a breakdown of which is shown in Figure 6.

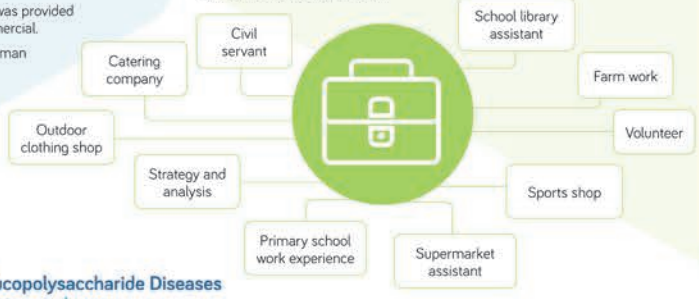
Figure 6. Support received in further education



- Individuals achieved a range of qualifications from 'A' levels through to Master's degree.
- Of the 8 individuals who had completed their education 50% were working in voluntary or paid employment in a variety of roles* (Figure 7).

Figure 7. Employment history of individuals with MPS II

*Some individuals reported more than one role



Conclusions

- Our findings report a lower prevalence of 'severe' phenotype than published estimates.[2]
- In the UK, there is a wide range of support available for individuals with MPS II in both SEN and mainstream education.
- Educational needs for those with CNS involvement cannot always be met in mainstream primary schools, but this is usually recognised, and more suitable SEN schooling found by age seven.
- Support requirements and the need for a flexible approach appear to change from primary to secondary school due to disease progression, the demands of moving around a larger school and the difficulty of the school work.
- Half of the MPS II sufferers surveyed who had completed their education had gained further education qualifications and found employment in the voluntary or paid sectors.

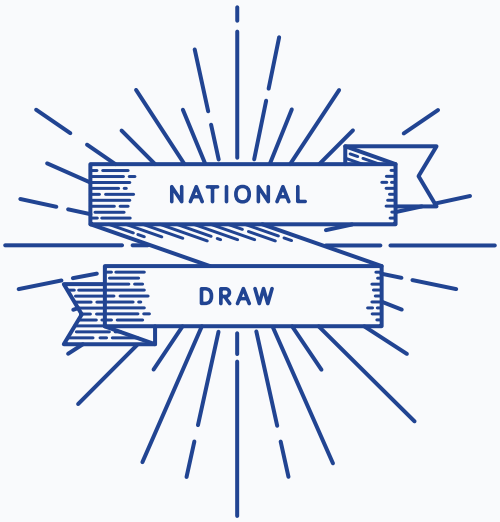
References

1. Wraith J, et al. 2008. Eur J Pediatr;167:267.
2. Muenzer J, et al. 2012. Eur J Pediatr;171:181.

Acknowledgements

- Rebeca Brandon, MPS II Advocacy Officer at the MPS Society, for all of her support and work with individuals and their families.
- Jaromir Miki (Shire) and Meng Wang (Evidera) who were active participants in the design of the questionnaire.
- Patient questionnaires were conducted by Jo Goodman and Benedicta Marshall-Andrew (MPS Commercial) and Alex Cavell.
- Medical writing and editorial support was provided by Jacqueline Adam, PhD, MPS Commercial.
- This research was funded by Shire Human Genetic Therapies, Inc.

Society for Mucopolysaccharide Diseases
www.mpssociety.org.uk



It's that time of the year again where the festive season of giving is near us and we are all making plans to spend time with our friends, family and loved ones. This time of the year also means that we are now running our annual national draw which has loads of wonderful prizes up for grabs this year.

We will be doing the draw on Saturday 26 November 2016 and would like to receive all tickets by Friday 25 November at the latest.

Best of luck to those taking part in the annual national draw and a big thank you for supporting the MPS Society!

The prizes include:

- **1st prize:** 3 night family stay (inc. breakfast) at the Adina Hotel, Hamburg plus a 3 course dinner for 4 in the ALTO restaurant (transport not included)
- **2nd prize:** £ 250 M&S voucher
- **3rd prize:** 3 night stay for 2 (inc. breakfast) at the Adina Hotel, Hamburg (transport not included)
- £110 Love to Shop voucher
- £100 Love to Shop voucher
- Family ticket for 2 adults and 2 children to Warner Bros "The Making of Harry Potter"
- 2 New Year's Day Club Enclosure tickets at Cheltenham Jockey Club racecourse
- ROCKI – the portable wifi music streaming device
- Rymans stationery set – iPad cover, letter book, winter wonderland colouring book, Alice In Wonderland diary, notebook, small change purse
- £50 M&S Vouchers
- £50 Ocado voucher
- 6 tickets to attend the Circus Starr winter tour
- 1 mobiliser session from Back in Action
- 2 entry tickets to Kew Gardens
- An online mindfulness course from Be Mindful Online
- 4 Empire cinema guest passes
- 1 Culti Decor Assolato Scented Candle
- £25 Clarks voucher
- £20 Boots Voucher
- Pebble Smartstick Phone Charger and Veho Portable capsule speakers
- iPad cover, Pebble Smartstick Phone Charger and hand warmer
- Delux Body Pilates Set which includes a Gym ball, 2 piece toning ball, Power band and pump
- £15 Entertainer Voucher
- 'Pronto!' Lets cook Italian' cookbook by Gino D'Acampo
- 2 cream tea vouchers at Van Hage Garden Centre
- 5 Top Wash vouchers from IMO car wash
- 1.35kg tin of Quality Streets and Cuddly teddy Bears
- 2x 2.26 kg Quality street boxes and a cuddly teddy bear
- X7 NERO VIP Drinks vouchers
- £10 Next Voucher
- Star Wars USB flash drive , I Cool USB fan and earphones

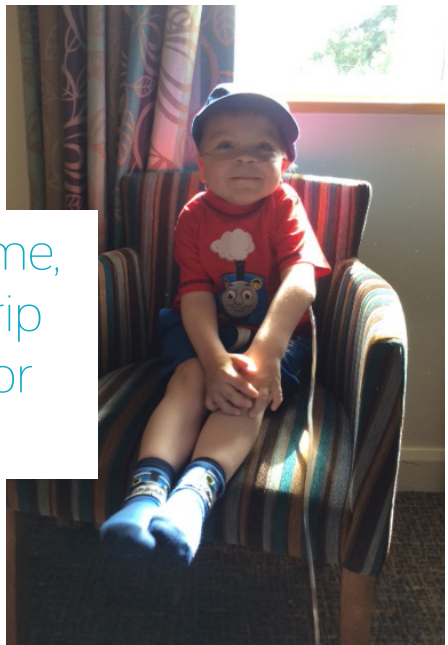
You may even choose to spread the festive cheer by asking colleagues, neighbours or school mates to purchase a ticket in support of the Society. We have included a raffle book within those magazines sent via post, but if you are interested in obtaining more, please do let us know at fundraising@mpssociety.org.uk and we will be happy to send it to you.

Drayton Manor

"We had a train-tastic time, thank you so much. A trip not only fun, but good for the soul!" *Teresa*

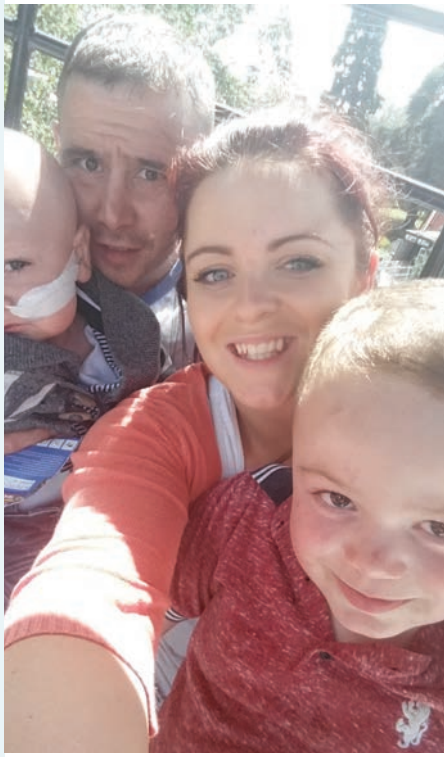
We can't thank you enough for giving us the opportunity to visit Drayton Manor. We had a amazing time meeting other families, hearing their stories, making new friends that we know are always at the end of the phone for the good days and bad. I've never seen such happy children, parents, siblings and members of staff at the party on Friday night. Thank you so much again.

Trudy, Nick, Mason and Mollie x



We had a fantastic time. We loved meeting the other families and Emily especially loved meeting Corey, Joshua, Ethan, Chloe and Carlos. What a fantastic party on the Friday night. The highlight for Emily was meeting Marshall and Chase from Paw Patrol. Nev and I enjoyed getting to know the other parents and sharing experiences and support. One of the best weekends yet.

Gayle, Nev and Emily



"Thank you for an amazing weekend it was full of laughs and happy memories to cherish forever." *Jasmin*

So so tired today, but it was in a VERY good cause. Namely enjoying ourselves all weekend at Drayton Manor theme park.

The weekend was organised by the MPS Society so we were surrounded by other member families.

It was of course great fun to try out the rides with the boys and eat too much rubbish for a few days, but when I asked Twiglet to guess what my favourite part was, he astutely said 'spending lots of time with your friends'.

The deal that had been organised was superb value (two nights accommodation and two days pass to the theme park) but the chance to relax and chat with other parents was priceless. No need to let them know how treatment is going, or explain any medical terms. Age, background, education – all irrelevant when faced with the fellow feeling that comes when talking to another MPS parent.

On the Friday night, there was entertainment laid on for the children – bubble machine, character meet-and-greets, party games, boxes full of Haribo. Needless to say, they were happy as larry, and as a parent it was great to watch knowing that any typical MPS II behaviour would be understood.

Extract from Sally' blog
www.hunterslife.co.uk

International

www.fabrynetwork.org

Treatment news from Fabry International Network

Amicus Therapeutics oral therapy Galafold Previously known as Migalastat, is being appraised through the NICE highly specialised technologies process in England. The UK MPS Society is leading the patient submission for the use of Migalastat in Fabry disease and this may well influence other players in Europe. Unfortunately the FDA requires more data on Galafold and therefore is not available for reimbursement in the USA

FDA fast-tracks development of Genzyme oral drug, GZ/SAR402671 a new investigational oral substrate reduction therapy for the treatment of Fabry disease.

Japanese ERT to use cloud technology in Fabry research – Medidata, the leading global provider of cloud-based solutions for clinical research in life sciences has announced that its cloud-based technology platform has been adopted by JCR Pharmaceuticals. The pioneer in biotherapeutics is leveraging the Medidata Clinical Cloud to support research on a therapy for the treatment of Fabry disease, bringing greater speed and operational efficiencies to the organisation's development programme in Japan.

Protalix announces positive clinical trial results – Protalix BioTherapeutics has announced interim data from the company's phase I/II clinical trial of 1mg/kg of PRX-102 for a treatment of Fabry disease. PRX-102 is a recombinant plant cell expressing, chemically modified version of the human alpha-Galactosidase-A enzyme. The phase I/II clinical trial of PRX-102 for the treatment of Fabry disease is an open-label, dose-ranging study treating up to 18 naïve male and female adult patients.

Trial to start for moss-made ERT for Fabry disease – Greenovation, a German Biotech company, has announced a phase I clinical trial application for its drug candidate moss-alpha-galactosidase (moss-agal).

Natural history survey – MPS IVB Morquio

A research team at BC Children's Hospital in Vancouver, Canada, is currently investigating the natural history of MPS diseases - in particular Morquio B or late-onset gangliosidosis (LO-GM1). Their objective is to better understand the clinical progression and patients' quality of life by connecting with international communities representing people who have these disorders.

For the investigation they have created a patient survey which has been translated into several languages. The survey consists of 72 questions, and takes about 20–25 minutes to fill out online. All information collected is anonymous and the data will be safely stored on a secure clinical database system.

To take the survey follow this link:
https://neurodevnet.med.ualberta.ca/surveys/?s=K99PHPMN78

For any questions, please contact maria.bleier@cw.bc.ca

My Journey from ERT to Chaperone Therapy by Anne Grimsbo

I learned about Fabry when I was five years old. My father's torso was covered by angiokeratomas. I asked what it was and was told that my father suffered from the same disease that my uncle had recently died from. I remember seeing my uncle in a wheelchair and my grandmother handling a spelling board for him.

When I was eight I had a couple of severe flu attacks with high fever. I had violent pains in my fingers and toes and was told that I suffered from the same disease as my father.

At the age of eighteen a Danish professor wrote to my father asking if he cared to be examined because he knew the disease was in our family. I wanted to be examined as having children was one of my future plans. As expected my father, my sister and I were diagnosed with Fabry.

At the age of 53 my father broke down with kidney failure. He died at 55.

I got pregnant in 1990, had prenatal diagnosis, placenta biopsies, and was told that it was a boy with Fabry. I had an abortion. My second pregnancy had no fetus. My third pregnancy was another boy with Fabry followed by another abortion. My fourth pregnancy I lost. My fifth and sixth pregnancy in 1993 and 1995 were my two boys with no Fabry.

I never thought of myself as a Fabry patient and I never had any troubles other than the pains when in fever as a young child.

I received annual examinations from 2002. In April 2007 my doctor recommended that I start treatment. In September 2007 I started ERT for the first six months I received treatment at the hospital and after that I had home treatment and my friend or my husband inserted the needle.

My mutation is A156T which means I can receive Migalastat. From 2009 I had a pause in ERT in order to take part in AT 1001. The 16th of December 2010 I had my first Migalastat capsule. The following year I had three kidney biopsies as a part of the trial.

Migalastat continues to work fine for me. This fall my doctor told me that I no longer have microalbuminuria. I have a heart condition and for the time being I have a loop recorder inserted in my chest. I receive medicine for that. For me it is a great advantage to receive Migalastat instead of ERT. I feel less like a patient. I am taking the capsules as if it was vitamins (only every second day, of course).

When you receive ERT you are very much a patient every second weekend and you have to take all kinds of precautions practically, hygienic and planning wise.

It is a great freedom for a Fabry patient to be treated with Migalastat.

The Danish Fabry Patient Organisation was founded in 2002 by my cousin and another patient. Today we have 66 members a mixture of patients and relatives. We have 4 board meetings a year and an AGM. We do a family weekend every second year and a day meeting the opposite year. I became chair in May 2013. In 2014 I joined the board of FIN.

My focus is sharing experiences between Fabry patients, listening to them and show them that a happy life is possible even when diagnosed with Fabry.

Claire tells us about her research into the link between MPS III and Autistic Spectrum Disorder

Hi! My name is Claire Wolfenden and I am a trainee clinical psychologist at the University of Manchester. I am currently in my final year of training and am working on my doctoral thesis. I became very interested in MPS III during my second year of training after learning how rare the condition is and the difficulties that families and children face. As I have to carry out a piece of research to complete my training I really wanted to look at something that would be of value to someone and so I decided to look at MPS III. In particular I am looking at whether children with MPS III also have symptoms of Autistic Spectrum Disorder (ASD). I am working with Dr Simon Jones and Dr Stewart Rust (who some of you might know from Central Manchester University Hospital) and I am being supervised by Dr Dougal Hare (Cardiff University) and Dr Anja Wittkowski (University of Manchester).

This is an interesting area because observations from clinicians and families in addition to previous research have indicated that many children with MPS III have behaviours that are often associated with Autism Spectrum Disorder (ASD). ASD is a pervasive developmental disorder that is characterized by difficulties with social communication, restricted interests and repetitive behaviours. If we see that children with MPS III do have symptoms that are typical of ASD, it is hoped that services will be better able to identify and provide appropriate behavioural support that will ultimately improve the quality of life of the child with MPS III and also the family around them.

The research will aim to find out the probability of a child with MPS III also having symptoms associated to ASD. We are asking parents/carers of a child with MPS III (sub-types A, B & C) aged between 2 and 16 years to complete three questionnaires that look at the behaviours of their child. My plan is to then share the results of the research with the families who have participated, and more widely, to help make a difference to anyone living with MPS III.

Thank you for taking the time to read this and I look forward to hearing from you!

If you would like to find out more about the study or should you wish to take part, please contact me via the details below:

Telephone -0161 306 0400 or 07710 639856
Email – claire.wolfenden@postgrad.manchester.ac.uk

There are three different ways of taking part, depending on what suits families best:

1. Questionnaires can be sent in the post to families with a stamped returns envelope, or

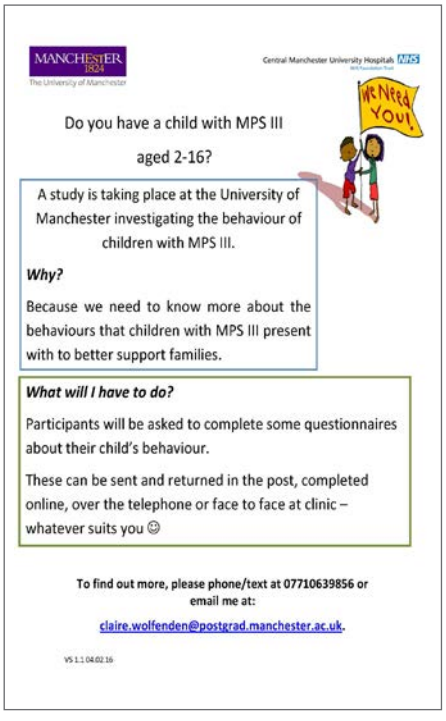
2. I can arrange a convenient date and time to complete the questionnaires over the telephone or

3. Questionnaires can be completed online.

Unfortunately, I am unable to offer any payments for participation but families will be sent a summary of the results and the research will be published in a peer reviewed journal. I also hope to present the research at the MPS Society annual conference and other scientific conferences.

All research in the NHS is looked at by an independent group of people, called a Research Ethics Committee. They protect the rights, safety, dignity and wellbeing of participants. This study has been reviewed and given a favourable opinion by the University of Manchester Research Ethics Committee and the Leicester Central Research Ethics Committee.

All data collected will be handled sensitively and in confidence, and further with adherence to legal and ethical guidelines.



Research & treatment

MPS III studies come to an end

It is with great disappointment that the Shire MPS IIIA HGT-SAN-055/HGT-SAN-067 and HGT-SAN-093/SHP-610-201 studies have stopped and the MPS Society would like to extend its sympathies to all patients and families this has affected.

At present, the only information we have is that the study has ended for efficacy reasons. We have requested further information from Shire which we will share as soon as it is made available. In the meantime, the Advocacy Team are on hand to lend support should you need it.

3 Minute Thesis in Fabry

Simon Heales at Great Ormond Street Hospital has been keeping us up to date with the news of his PhD student, Jonathan Lambert's progress in the 3 Minute Thesis (3MT) competition. He said:

> "I am pleased to let you know that Jonathan Lambert is now a finalist in the national 3-minute thesis competition... So far Jonathan has won the local competition, UCL faculty, UCL overall and then was selected from the national semi-finals. Excellent with regards to raising the profile of Fabry."

You can find out more about 3MT here:
http://threeminutethesis.org/

And watch Jonathan's presentation on YouTube:
https://youtu.be/poyot209O_k

Patients as key partners in rare disease drug development

An important paper by Max G Bronstein and Emil D Kakkis published in Nature in August 2016 explores how understanding disease burden from the patient perspective and early patient engagement in clinical trial development from pre Phase II to pre Phase III is crucial for developing a more comprehensive suite of end points beyond established measures as well as for understanding which patients are candidates for a particular clinical trial.

The authors suggest two types of effort should be considered. First the establishment of quantitative, direct measures of diseases that might be acceptable as primary clinical end points based on the patients' clinical function. Secondly the development of complementary patient-reported or clinician-reported outcome tools should be assessed either in clinical surveys or in Phase II learn studies to help assess the clinical meaningfulness of changes.

In rare diseases it is already it is already acknowledged by patients and MPS clinical experts that commonly used patient-reported outcomes often do not relate specifically enough to the rare disease in question resulting in data being diluted by lack of relevant measures and this was also the view of the authors.

This is an important paper in highlighting to the regulatory agencies that a framework involving very early patient engagement in rare disease drug development needs to have some flexibility and that there is a vital role for patients to play.

Christine Lavery

www.nature.com/nrd/journal/vaop/ncurrent/full/nrd.2016.133.html

Information & resources

Back To University

The 'back to school' frenzy has come and gone and the academic year is well underway. We hope that all students are well settled in. If university students are interested in supporting a good cause while being at campus, then choosing the MPS Society as your university's annual RAG would be something you might wish to get involved in. Some universities host their RAGs for one week in the year and others host them right throughout the year. Your student fundraising association would be able to let you know which one applies to your university.

If you choose to get involved, fundraising will be done throughout the RAG season to support the Society, usually involving putting on events and raising awareness which encourages as much fundraising as possible. If you require a fundraising pack to help you get started in your fundraising efforts, then please let us know at fundraising@mpssociety.org.uk and we will gladly send one to you.

If you're too busy with studies and don't have enough time to be involved in RAGs, why not buy your stationery, laptops or any other materials needed for university through Easyfundraising? You simply have to purchase the goods you wish for on easyfundraising.org.uk. Once online, you will be able to make your selection under the store which stocks the items you desire and it won't cost you anything extra. Once the purchase it made the Society automatically receives the donation.

Perhaps you're interested in building up learning skills whilst at university? Maybe you are in childcare, marketing, business, design, illustration, film, drama or photography? Let us know as we could find a volunteer opportunity to match the skills you wish to develop. In this way you will be gaining valuable experience whilst supporting a good cause.

We wish you all a prosperous academic year ahead!

Adaptive clothing from Tommy Hilfiger

The Daily Mail's magazine, *Femail*, has reported on a new venture of Tommy Hilfiger to make stylish clothing for children with special needs. They write:

"In February, designer Tommy Hilfiger teamed up with nonprofit Runway of Dreams to make a collection of 'adaptive clothing', which is specially designed to address issues that kids with special needs face while getting dressed."

Runway of Dreams founder, Mindy Scheier, decided to do something about the void in the market for adaptive clothing when her then 8 year old son who has Muscular Dystrophy had to wear leg braces and she struggled to find trousers to fit him.

The Tommy Hilfiger range has magnetic fasteners instead of buttons or zips, and stretchy trousers with adjustable leg openings. The modifications are invisible making the clothing as smart and stylish as you'd hope from Tommy Hilfiger.

Unfortunately the clothing range does not appear to be available in the UK yet but it's great that a large designer has drawn attention to the issue. Meanwhile, Marks and Spencer now offer the Easy Dressing range of school uniform which runs from age 2–16 and has been developed in collaboration with The National Autistic Society. Available to buy from their website now.

Read more:

Full article in *Femail*: www.dailymail.co.uk/femail/article-3681481/Tommy-Hilfiger-s-collection-adaptive-clothing-designed-children-special-needs-seller-fashion-brand.html#ixzz4L4ciFiwu

Tommy Hilfiger's UK site: http://uk.tommy.com/

M&S Easy Dressing range: www.marksandspencer.com/s/kids/school-uniform/help-and-inspiration/easy-dressing-school-uniform

Source: www.marksandspencer.com/s/kids/school-uniform/help-and-inspiration/easy-dressing-school-uniform

On 17 November 2016 there is a Kidz to Adultz Up North event

www.disabledliving.co.uk/Kidz/North

It is one of the largest, free UK exhibitions dedicated to children and young adults with disabilities and additional needs, their families, carers and the professionals who work with them.

80-150 exhibitors will be offering advice and information on funding, mobility, seating, beds, communication, access, education, toys, transport, style, sensory, sports and leisure and more...

Running alongside the event are free CPD and topical seminars for parents and professionals. Topics include: Moving & Handling, Sleep Issues, Continence Issues, Finance & Budgets, Parental Experiences, Transition, Legal Advice, Managing Behaviours that Challenge and more!

Disability Fair - round up of information and resources

Dragonmobility
www.dragonmobility.com
Build specialist, highly versatile power chairs for very active disabled people.

Aunty Agatha's Little Sensory Shop
www.auntyagathas.co.uk
Helps you to find the products and often the advice you need to support independent living to give you more time with your loved ones

Disability Horizons
www.disabilityhorizons.com
Disability lifestyle publication which publishes articles on a wide variety of topics, all to support the aim of a world where disabled people live exactly as they choose to.

Elbee
www.elbeemobility.com/uk/
Elbee is a vehicle you can drive directly from your wheelchair helping you to avoid complicated methods of entering the vehicle, folding the wheelchair into the trunk and other complications associated with the use of vehicles converted to manual control.

Made2Aid
www.Made2Aid.co.uk
A specialist product search engine.

For Sale – £3,500

Citroen Dispatch 1.9 diesel (2005)
Wheelchair accessible car.
51,000 miles
MOT June 2017

Three seats in the front and middle row of three seats (all removable). Wheelchair fits with middle seat removed. Also two fold-down rear seats.

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Guide for DSAs at university

Student Finance England, a service provided by the Student Loans Company, has published a guide to DSAs at university. Student Finance England provides financial support on behalf of the UK Government to students from England entering higher education in the UK. The guide gives information about Disabled Students' Allowances (DSAs) for new and continuing students in higher education. DSAs help pay the extra essential costs students may have as a direct result of a disability including a mental-health condition, or specific learning difficulty such as dyslexia or dyspraxia.

You can download the guide here: www.gov.uk/disabled-students-allowances-dsas/further-information

Fundraising

Alicia's wacky walkabout

Alicia Evans from Penparc School, near Cardigan in West Wales suffers from MPS I and her mum, Kelly, and support assistant, Julie, decided to organise a sponsored walk to raise money for the MPS Society as it's a cause very close to their hearts. The event raised £1858.98 for the MPS Society.

"The support we received from the locality was enormous. We had approx 40 walkers joining us including children." *Julie*

Tea anyone?

On Saturday 18th August myself and a small group of ladies from Acre Mill Baptist and some of my daughter Katie's friends held an afternoon tea at the church. This had been originally discussed to be held for MPS Awareness Day however I had to go into hospital so the event was postponed to the Queen's 90th birthday weekend, then again postponed due to an annual church event taking place. In the end the actual date fell on the 3rd anniversary weekend of Jack's passing so quite a fitting tribute to him.

The event was well attended and entertainment was provided by the church choir and a former Stars in their Eyes contender, Rachel, who provided lovely renditions by different performers including a special performance of Disney's Frozen 'Let It Go' supported by backing singers and dancers, Katie, Kaylee and Amy. The afternoon tea consisted of a selection of sandwiches, crisps, scones with raspberry jam and Cornish clotted cream (now available in the north west of England!), yet more cakes and of course an endless supply of tea or coffee. Further donations of jigsaws, books and good as new items were sold and there was also a cake stall. A good time was had by all, especially seeing the home-made produce being enjoyed and thanks must be passed to all who attended, donated, baked and sponsored the event – particularly my employer Mr Paul Keenan who commissioned a Victoria sponge complete with buttercream to be made by Katie for a phenomenal £250 – please note Katie was only 11 at the time and has never made buttercream before, a big ask – but enjoyed by his family!

We raised a fantastic £600 in all, which has been sent as a donation towards vital research for Hunter Syndrome.

Elizabeth A Heath

Sporting challenges day

At East Cliff Pre-School, Bournemouth (ages 2-4) we like to support charities close to our heart.

We are a small privately owned school that works with families that are often less fortunate than others, however we still take pride in supporting causes, showing both the children and their families that even a little can go towards helping others.

One of our families is affected by MPS III (Georgia Watts' big brother Sam has Sanfilippo) so this year we wanted to make The MPS Society our charity to support. On 14th July we did a 'sporting challenges day' which all the children took part in; egg & spoon race, running race, obstacle course and long jumping into our sand pit amongst others. They all got sponsorship from their friends and family. We are proud to say we raised £498 and had a lot of fun.

Beckie Winkley, Manager/Owner, East Cliff Pre-School

Sponsored bike ride

This is a picture of Ethan Greening's school presenting him with a cheque for a total of £1,771.50, from JustGiving, sponsorship and a collection from the six bells pub and other local businesses, teachers, colleagues and helpers who took part in a 55 mile bike ride and a sponsored walk organised by Deputy Head Mr Lambert. Thank you very much to everyone involved in this fundraiser!

Thank you to our cyclists, Paul Ashman and Elaine McLellan who rode in the Prudential RideLondon–Surrey 100 on Sunday 31 July. Elaine said:

"I had a blast! What an amazing bike ride. It was incredible to ride through the streets of London and Surrey with no cars and perfect weather. My bike was brilliant reaching speeds of 31 mph and believe it or not I don't like going fast!

I cycled on my own, chatting to other riders and enjoying the support by the crowds along the route. At the finish I collected my medal and met up with family and friends.

I woke the next morning with no aches or pains, how lucky am I! So far I've raised about £600."

...and here is Simon Greening after the Cardiff half marathon

Also thanks to our British 10K runners: Paula Sheridan, Ricky Brown, Heather Lloyd, Joe Flaherty and Daniel Goodge. Daniel contacted us to say:

"I managed it in 51 minutes and to date have raised £270 there is still some money to come in so will keep you updated hopefully this will go a little way to help the people that suffer from MPS."

Wear it Wicked! Getting ready for Halloween and looking for some spooky fun? Why not organise to wear it wicked in the office or at your school and get everyone in scary costumes to help fund research into treatments for MPS and related diseases.

Celebration of excellence

How a school in High Wycombe chose to support the MPS Society as their charity of their year and fitted in fundraising around their studies.

The Highcrest Academy have made an amazing difference to the MPS Society by supporting us as their charity of the year. Not only have the students at Highcrest worked hard on their studies, sporting achievements and fundraising for other charities, they have raised a very fitting £2016 in 2016!

I was honoured to visit the school at the end of last term on their celebration of excellence day and to have the chance to say thank you in person for choosing to support the MPS Society.

After a very inspiring photo montage of everything the school had achieved last year (and believe me when I say it was a lot) I had the chance to share with the school what a difference their fundraising makes to our members.

Thanks to the efforts of The Highcrest Academy, and others like them, we can organise events and conferences, visit clinics and give school talks, fund research into treatment and so much more.

It is inspiring to see how much the school has done towards supporting the charity, including bag packing at Sainsbury's, organising a Christmas Fayre and of course wearing blue on MPS Awareness Day amongst other things. Thank you Highcrest! We are truly grateful.

If you know a school that is looking to support a charity get in touch with our fundraising team at fundraising@mpssociety.org.uk

Main photo from left: Haleem Hussain (deputy Head boy), Helen Crawley (Fundraiser for MPS Society), Shena Moynihan (Headteacher) and Danni Childs (deputy Head girl) at The Highcrest Academy on the Celebration of Excellence day. Below: students at a bag packing event for Sainsbury's.

It is obviously the season of throwing oneself out of a plane for a good cause as we have received a number of skydiving stories from some very brave individuals who are willing to risk it all in the name of the fundraising.

Find out more about skydiving for MPS at www.tiny.cc/qqwzey

Amy Beanland has raised nearly £600 after deciding to jump for MPS when her colleague's son Harley was diagnosed with MPS III. On 3rd September, Accounts Administrator, Amy skydived from 15,000ft having received the skydive as a gift from her parents for her 21st birthday. Amy had been looking for a charity to support for a while.

"After hearing about Harley's condition, this seems like the perfect opportunity to help out a friend and raise awareness for a worthy cause at the same time."

On the 21st July Sydney Scott took part in a tandem skydive from 13,000ft! She said: "A family member was diagnosed with Sanfilippo so I wanted to raise money for the MPS Society. I paid for the jump myself so that all money raised would go to the charity, so far I have raised £736 on my just giving page". The construction company that Sydney works for also matched her funding so she raised a total of £1256, smashing her original target of £300!

Kim and Robert raised a combined total of £1760.

Kim Mills and her friend Robert Thompson jumped for MPS in memory of her son Andrew Parker who had MPS III. Kim told us:

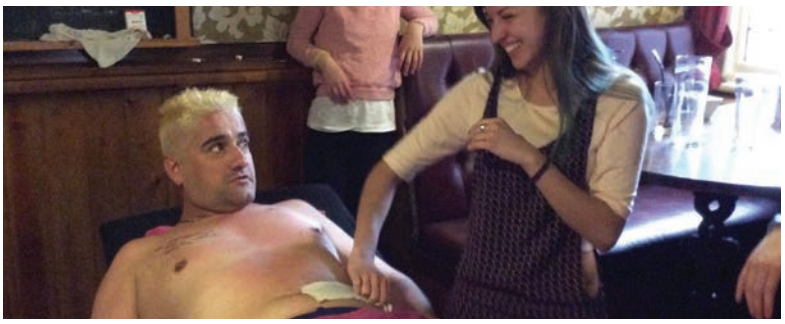
"My boy was my world and I was very blessed to be chosen to have him. I miss him beyond words. It was a pleasure to make a small contribution via my parachute jump towards the MPS society. I was nervous but I was very focused and all I could think about was my boy. The parachute jump was amazing, it was really really fabulous! I loved every minute of it."

Family fundraisers

Two of our newest families have already been hard at work fundraising for the MPS Society

Harley Bond's

family have been raising awareness at Glastonbury, as well as fundraising with friends and family at a funday, the Cheltenham half marathon and with a colleague's sky dive and even a friend's sponsored chest wax! They've raised a superb £3050 and counting!



Ronnie Kirkley's

family have been fundraising for the MPS Society since they learned of his diagnosis. His mum, Chloe has done a tremendous job on her JustGiving page, raising an astonishing £1000 in the first 10 days of opening it. With so many events on the go, including an open day and summer fair at Ronnie's nursery and "Ronnie's family fun day", it's not surprising that they have raised £4,549 already.

Kids Planet Prestwich raised over £900 from their Summer Fair

Kids Planet Prestwich raised over £900 for The Society for Mucopolysaccharide Diseases. The nursery held a summer fair, in aid of a child who attends the nursery, who was recently diagnosed with Hunters Syndrome. The staff and families felt very passionate about raising money and awareness for this condition.

The Summer Fair and open day was held on Saturday 30th July with lots of activities for children including bouncy castle, arts & crafts, face painting, tombola, raffles, stalls, refreshments and more. The total raised was an incredible £977.13!

Kirsty Powell, Nursery Manager also said "Following the recent diagnosis for one of our children, we thought it would be a fantastic opportunity to support this family and raise the additional awareness we felt this syndrome deserved. We had a great time at our summer fair, we know it will help Ronnie and his family, as well as other families. A big thank you to all of our families at nursery for making the event such a success"

From www.kidsplanetdaynurseries.co.uk

Coast to Coast Charity Walk

When you're first diagnosed with a genetic illness it's very overwhelming and a lot to take in, but when your child is also diagnosed it becomes life changing for the whole family. For my family, coming to terms with Fabry Disease and the impact it has on our lives has been extremely difficult and the MPS Society has shown support and guidance throughout.

When we received a letter advising of the Government's intention to review funding for our medication, I felt angry, worried and that I had to do something. The MPS Society's work can only continue in helping to represent patients like us if they have enough funding.

We decided to walk the South West Coastal Path from St Ives, around Lands End, ending in Marazion (44 miles, over 3 days) hopefully encouraging as many friends and family as possible to join us. Little did we know the amount of support we would have. Over the 3 days, people joined us at different parts of the walk, offering a huge amount of support, sharing lots of laughter and making our aches and pains more bearable.

Reaching Marazion beach was an amazing feeling and we were all extremely proud of ourselves.

On behalf of my family, I am delighted to say that we raised £5579.76..What an unbelievable experience, fantastic support and I'm sure the Society will put this money to great use.

Julie Baker

Garden Party

On Bank Holiday Monday, 30th May 2016 we held a fundraising garden party at the home of Kathryn Wallis, Granny to Archie and Isaac Eaton (MPS IVA). Kathryn has an amazing, interesting and diverse garden and has long had ideas of opening it up to visitors to raise funds for the MPS Society but the timing has never quite been right! However, following all the support the family had received from the society during the long Vimizim campaign and with MPS Awareness Day falling very close to a visit by Archie and Isaac to Granny's it suddenly seemed like the right time.

What started out as inviting a few friends round to see the garden grew rapidly and we were overwhelmed with donations of cakes and raffle prizes. The weather was fantastic and there were loads of visitors to see the varied areas of the garden all looking their best.

With the help of social media, Kathryn's Garden Party became an online event too, with those unable to come in person joining in a "virtual garden party" online, with pictures of the beautiful settings and the chance to donate via JustGiving. Through a combination of donations on the day and online, the Garden Party raised an amazing £1795 for the MPS Society. Thanks go to Granny Kathryn for hosting the party and everyone who visited or supported the event.

Anna Eaton

Orwell Walk for MPS

On Sunday 26 June a few LV= people participated in the Orwell Walk in aid of the MPS Society. Stuart Chapman, Sarah Fournier, Darryl Brook and Lucretia Lindsay were aiming to complete the whole 25 miles, with and Tim Buttle and Laura Allen putting in a healthy 18 miles.

By 8 am we had endured a fairly awful first hour and half as the rain rolled in up the river and pretty much soaked us to the bone! Possibly the worst start you could have. Underfoot the terrain was tricky. Whether it was seaweed on the shoreline, bog in the wetlands or the puddles and mud we encountered in the countryside; it did not dampen our spirits and we were determined to power through.

We dried off soon and as the morning progressed, it came to our group's parting point. We said good-bye to Laura and Tim, wrung out our socks and re-applied our plasters, opened the jelly babies and set off on the last leg before the mid-way point.

As we strolled along the Trimley marshes, it was becoming apparent that not many people had chosen to do the 25 mile walk - as we hadn't seen a soul for some time. This was possibly due to the poor weather first thing or just because they were chicken (or just really sensible). We made it to Trimley around 1pm only to find out we were the last group through and in danger of being caught up by the "Sweepers" - which basically meant we would not be able to finish the walk if they caught us up.

We decided that we didn't want to be caught by the sweepers, so we upped our pace and managed to pull our average minutes per mile down from around 24 minutes to 18 minutes. We finally found a good pace in the afternoon heat and our spirits were high - probably due to the vast quantities of jelly babies and jelly beans consumed!

By the time we reached checkpoint 7 of 10 and we knew we weren't going to be caught by the "Sweepers", we were able to slow our pace down. This is where it started to hurt. Our legs felt like dead weights but our determination pulled us through. Around 16:45 Gainsborough sports centre in sight and we knew that the end was close.

We crossed the finish line around 5pm to a round of applause by the organizers and the president of the local Rotary Club. Our final time was just under 9 hours. Had the weather been better, we might have shaved an hour off our time, but we were still immensely proud of our efforts and it was an enjoyable experience.

Darrly Brook

Online fundraising

If you're planning to fundraise and gain sponsorship or donations online, bear in mind that BT MyDonate is the only online fundraising site that takes zero commission and has no set up charges.

So where Virgin Money Giving charges 2% commission and JustGiving charges 5% commission, BTMyDonate charges 0%. As well as this, the MPS Society pays £15 a month to use JustGiving and had to pay a one-off fee of £150 to set up Virgin Money Giving.

What this actually means is you could be earning an extra 63p for every £10 donated with BTMyDonate. It doesn't sound like a lot but it soon adds up.

It's not as well known as JustGiving or Virgin Money Giving but it's just as easy to use and you'll find the MPS Society on there already.

The Weather Lottery

We would like to thank all of The Weather Lottery players who have chosen the MPS Society as the charity they would like to support whilst having a chance to win huge prizes in the weekly draw. They are: Mrs L Hiller; Mrs O Megoran;
Mrs J Edwards; Mrs C Lavery; Mrs G Plummer; Mr McCawille; Mr A Selwood; Mrs T Brown; Mr A Dickerson; Mrs M Crespin; Miss D Halleron; Ms C Halleron; Mrs D Bown; Mr M Hughes and Mrs J Speed.

We are so grateful that you have chosen to support the MPS Society and wish you good luck for The Weather Lottery.

For those who are interested in playing – The Weather Lottery is the UK's leading fundraising lottery where players are given the chance to support a good cause close to their hearts (be it big or small) as well as having a chance to win prizes in a weekly lottery draw. A minimum of 37% will go to the chosen cause. The remainder is split between the prize fund and the cost of operating the lottery. The cost of the weekly lottery draw is £1.00 per entry per player and a there is a guaranteed jackpot of £25,000 every week.

If you would like to add the Society for Mucopolysaccharide Diseases as the charity you would like to support, then you can log onto www.theweatherlottery.com and select 'Society for Mucopolysaccharide Diseases'.

The Weather Lottery

4C For Charity
CHARITY & GREETING CARDS

If you're interested in e-cards this year, or want to choose your own design, look at 4C For Charity at www.charitycards.org/charities/

They produce charity cards, calendars, e-cards and more to help raise many hundreds of thousands of pounds for charities each year. Ten pence from every card sold is contributed to the charity you select and 10% of the cost of e-cards or calendars is returned to the nominated charity. If you opt to support more than one charity, the contribution is divided equally between those chosen.

Yorkshire Three Peaks Challenge

It was a very wet and windy weekend, with a few short lived spells of sunshine. I had to be dressed and at the start line by 6am, so I had to be prepared for the day ahead well in advance. I was slightly nervous about the walk because I got a blister on the back of my foot the week before, so I was hoping that the various blister plasters I had stuck on would hold out.

I was in group ten, the second to last group to leave, and our group set off at 06:45. The initial climb up the first mountain was slow going, as all the groups were queuing to get up, once it started to move, I decided that I wanted to go a slightly quicker pace, and before I knew it was in the third group from the front.

On the journey there were some real highs and lows (excuse the pun), the lows being long stretches of walking before meeting another person, and the heavy downpours and the highs being the stunning views, and the excitement of some of the more challenging parts of the walk.

Whilst walking I talked to various people who asked me about my MPS t-shirt and the charity I was supporting, all who asked found it an interesting cause, even if they and a few paediatricians had never heard of Mucopolysaccharide Diseases.

It was great to be part of an organised event, and when I achieved the 24 mile walk in 9 hours and 39 minutes I was chuffed (well within the 12 hour time limit). I went straight to the pub next to where the walk finished and had a very refreshing pint to celebrate and a well deserved relax.

I enjoyed the experience so much that I may walk the Welsh 3000's (a series of mountains) in the future.

Darren Cleary

Step by step for the Merricks

In the last issue of the MPS Magazine we published a snippet about Chris Merrick raising £110 for walking from London to Brighton. In fact we were way off the mark as the walk was completed by both Chris and his sister in law, Alison Merrick, with a team name of "Step by step for the Merricks". Alison and her two boys (Connor, 8 and Cameron, 6) have Fabry so it was an amazing achievement for Alison to complete the 100K walk and she told us that the boys and her husband, Sean "supported us through the night at every stop making drinks and giving hugs when needed."

We also failed to credit the Step by step for the Merricks team with the full total they raised which was actually an incredible £1220, most of which was raised by Alison and family and her husband Sean.

This is an amazing achievement and we're very sorry not to have given it the recognition it deserves first time round.

Feeling fit and festive?

Why not look for your local Santa Dash and jingle all the way with hundreds of other Father Christmas lookalikes. All you need to do is:

Find a course • Grab a suit • Get dashing!

You can even get your little elves involved but leave your reindeer at home. For more information search for "Santa Dash near me" or visit www.santadash.co.uk to search by date.

To Paris and back

and a few other fundraising stories

It now seems a distant memory – the moment I said 'yes' to the crazy suggestion of cycling Paris to London in 24 hours in aid of the MPS society and my son Archie who has MPS VI. Worst still, I do recall stating a clear 'no' to running the Paris half marathon the next day...

During one of our sunny rides across Somerset, my next door neighbour, Jason Smith, started a casual chat about an endurance challenge – given that he had a captured audience at the time I could do little but listen. The idea of a cycling challenge seemed a great idea and to Paris sounded even better.

However, I started to get more than a little worried when the numbers joining our challenge started to grow rapidly. After a few more rides I plucked up enough courage to ask about the adventure and heard number '24' mentioned a number of times. I had unexpectedly signed myself up to cycle to Paris in 24 hours – starting in London, Marble Arch - and ending up under the Eiffel Tower the following day.

It also happened by 'pure coincidence' that the Paris half marathon was being run the same weekend!

After a few weeks of training I found myself hurtling through inner city London with a fellow team of riders - trying hard to avoid the rush hour traffic on the 4th March. After nearly being run over by a Lorry we made it to our first scheduled drink station. At this point, I pondered what lie ahead – a 225 mile journey including channel crossing, poor weather conditions and the possibility of losing 9lbs in weight (always a bonus).

After a long night of riding in all conditions, I finally made it to the ferry with 20 minutes to spare and after a bit of sleep, stew and plenty of water we set off again. The hardest part of the ride was probably the hill just outside of Dieppe – on the crossing the legs naturally relaxed and were most annoyed being woken up in chilly and wet conditions to start all over again. We cycled constantly only stopping for coffee and fuel breaks and chatting with our support team who did an amazing job in motivating us throughout.

During the final leg of the ride towards Paris, our team split up – bike damage and minor crashes took their toll and before long it was just two of us cycling into the capital. This was by the far the hardest time for us. At every intersection I felt in danger of losing my life or worst causing damage to my bike – but again the thought of completing the biggest personal challenge in my life and thinking of Archie kept me going.

So it was with tired legs but with a massive grin on my face that we finally entered the city centre and made all speed to the Eiffel tower. We attracted quite a fan base – a number of Parisians took it upon themselves to have 'selfies' alongside Jason and I and a lot of back slapping ensued.

The following morning, I found myself alongside the team in a holding pen wearing a black dustbin bag to keep me warm at the starting line of the half marathon. The atmosphere in Paris was something to admired – live bands, street artists and school children lined the streets to motivate all the runners. After recent terrible events in Paris, the spirit of the Parisians was something to truly behold. We managed to complete the half marathon in a reasonable time – all things considered – and had a few beers on the way home.

I cannot thank my fellow riders enough...Jason Smith, Clive Wheatley, Matt Rihan, Adam Holland, Jon Rawson, James Pendleton, Paddy White, Rodi Greene and of course all the fundraisers – especially my colleagues at Computacenter that generated so generously. We raised £3,740 in total.

However not long after the ride and run completed, I was drafted into my next fundraising event. This time, another neighbour, Allan Carchie, invited me to attend an 'informal, casual no preparation needed' pub quiz hosting by his fantastic company Ernst and Young. Allan is normally such an 'honest' character so when I walked into a rather classy wine bar and into an audience of senior E&Y partners, you could perhaps understand my surprise. Allan had 'forgotten' to tell me that a full blown PowerPoint presentation was needed so armed with a pencil and a till roll, I planned my presentation. After delivering an impromptu presentation on MPS and the wonderful work the MPS society does, I made my rather ungraceful exit. However, this 'pub quiz' raised in excess of £2k for the charity which was a remarkable achievement and I was truly humbled by Allan's idea.

Last of all, one of my close Computacenter colleagues recently decided to complete the legendary 'Rat Race' in aid of the society. Nathaniel Ives is well renowned within our company of being somewhat 'fit' so it came as no surprise that he decided to run 20 miles, over obstacles, in aid of MPS. Raising a significant amount of money in the process, Nat proved himself a legendary athlete!

This article has given me the opportunity to reflect on a crazy 2016 – a year that my friends and family truly showed what can be achieved with passion and commitment and just showed how highly thought of the work the MPS Society does. I was truly humbled by the pain people went through to raise money for a little lad only a few of them actually know. For me, it has motivated me to do more crazy things for the charity and my little boy Archie.

Philip Pearson

I do recall stating a clear 'no' to running the Paris half marathon the next day. . .

Thank you to all our donors and fundraisers – you inspire us!

Rachel Healy and Family from Calmore in Southampton have raised £200 for the Society.

Mark Hughes Grandfather to Zack held a stall at their local carnival and raised £241.

St Catherine's School in Guildford Russell Baker House have raised an amazing £1,000 holding various fundraising activities.

Iris Hitter held an Open Day at her house for Jacqueline her daughter who suffered from MPS III, friends were asking Iris when the day would be as it is a regular event as friends get the chance to catch up and have a chat. Some businesses donated gifts for a raffle and Iris and friends managed to raise £240.80.

Elizabeth Mee donated £30 that she received from the ladies of Nostell WI who enjoyed listening about her visit to Buckingham Palace.

PricewaterhouseCooper LLP in Bristol held a Quiz and a raffle and raised an amazing £2,100.

Currencies Direct had a dress down Friday, all employees pay to wear casual clothes and money is given to charity, we received £185.60.

Spire Academy students raised £68.40 on behalf of Ethan Burley.

Mrs LE Davies' End of Term Medal Presentation Evening raised by Dance Students and from ticket sales.

Alf Hubbard saved £17.90 in 10 pence pieces in an MPS Society collection pot which was given to us after he passed this year.

Andy Hardy on behalf of Towersey Morris donated £600 from performing their Mummers Play at various pubs in December. Appearing in their play this year - Camila Batmanghelidjh, Sepp Blatter and the President of China! A cheque was made out to MPS and the Multiple Sclerosis Trust at a 'dance out' at The Three Horse Shoes in Towersey.

Mrs **Carol Westland** and her friend **Joan Phippord** sold "upcycled" bits and pieces and handmade crafts to friends and family locally. They made £175 and generously donated this. They plan on taking part in a few more events before Christmas and hope to make another donation at some point.

Fulmer Day was held on a sunny Saturday, 2nd July. Stalls and activities were hosted for young and old alike. The Fulmer Day fund managed to raise a substantial amount of funds (circa £3000) which they are generously distributing to various local charities and good causes. They have donated £500 to MPS society.

Mrs **Jean H Davy** won £100 in a raffle and decided to donate the money to the MPS Society.

SCA Hygiene Products had a Wear it Blue Day and the company matched the amounts raised. They donated £250 – Sent in by Rachel Summer.

Georgie's Dance School held a 'Back to the 80's' event at the Elgiva Theatre in Chesham on 16 July. £ 1905.42 was raised.

£125 was donated courtesy of Capgemini UK PLC via a cheque awarded to **Richard Beet** for his community engagement in 5-a-side football tournament at Heathrow airport.

Thank you also to all those who donated anonymously – we don't know who you are, but we think you're great!

In Memory

Alf Hubbard, Connor McDougall, Alévique Brooks-Daw, Hazel Amelia Jean Hill; Helen Nicola Skidmore; Pauline Anne Sharpe.

Donations

The Borrows Charitable Trust; The Hobson Charity, Linda Smart, Elegant Cuisine Ltd; Pamela Booty; Laura Croft; Mrs A Baker; Ann Cock, Roberto Perrelli, The D'Oyly Carte Charitable Trust; Revd George Wood; Mr Mervin Roberts; Sam & Yvonne Allen; Marilyn & Nick Sheppard; Pam Phenna; Peter & Jaqueline Chew; Helen G Hesford; Peter & Jane Joyce; Philip & Jenny Kitchin; Gill Jowett; Ruth Buller; Rachel & Timothy Venn; Mrs M L Heney; Philip Blatchy and Son Funeral Directors; Mrs T Ramsbottom; Mrs M Hodgkinson; Pauline Jordan; Janet Burgess; Lisa Norman; Keith Smallwood; PayPal Giving Fund; Michael Withington; Anne Fry; Alicia Hyde; Lawrence Burr; Lynne and Roger Nash; J May; P C Walley; Ernest Heal and Sons Funeral Directors, Jill and David Rhys Jones; Mrs T Adams; Bill and Pam Johnston; Lyndon and Joan Arscott; Rachael Dart; Sarah Wayte; Robert Thompson; Simon Greening; Robert McBride Ltd, BBC Public and Wor, Joanne Sahal; Old Steine Lodge; Mrs Baker; Sally Cartwright; Mr T Lavelle; Rebecca Brooks-Daw; Shirley Jamil; Julie Hedges; Samantha Hunt; Ana Leonor Jordao; Hollingworth Academy; Paul Mahmood; Cora Mc Neill; Charlie Dodd; Sarah Wolfe; Julie Baker; Daniel Goodge; Guy Horner; Sophie Ficco; Katrina Fanneran; Chris Merrick; Sydney Scott; Louise Hiller; Steve Ollington; Hannah Watts; Aaryanna Lever; Josie Pugh; Gary Harlock; Sam Worden; Ruth Sanders; Paul Williamson; Valentina Carminati; Chloe Kirkley; Kane Nelson; Sarah Cutler; Neil Baines; Robyn & Amy; Pete Norris; Claire Conway; Maureen Ryles; Darren Cleary; S Farrow; Emma Bebington; Sarah Bond; Elizabeth Hull; Deborah Burniston; Dan Conway; Stephen McCawille; Lucy Rembert; Matthew Lamb; Beth Chambers; Lucretia Lindsay; Helen Tandy; Rachel Matthews; Richard Cooke; Jonathan Freeman; Joanne Haines; Katriona Balfour; Reece Barlow.

Donations via collection boxes, stamps, foreign coins, mobile phones, ink cartridges, jewellery

Sandra Silcock; Marcia Burnett; Joan Crespin; Rachel Todd; Mrs Eggleton; The Croft Family in memory of Christopher Croft.

Regular contributions by Standing Order or Give As You Earn

J Winzar; Daniel Winzar; K & J Hudson; E Lee; R & K Dunn; Saville Norman; M Newell; M Tosland; C Cullen; S Bhachu; S Brown; L Twaddle; V Lucas; D Forbes; S Winzar; G Simpson; William Cavanagh; Barbara Harriss; L Brodie; A Sabin; A Ephraim; Andrew Cock; Alan Dickerson; M Kalsi; P Summerton; A Weston; E White; C Hume; A Sullivan; A Byrne; Dorothy Robinson; N & S Cadman; J Wilson; A & A Tresidder; Molly Rigby; K Osborne; Stuart Robinson; Nicholas Thompson; E Cox; M Peach; C Garthwaite; Raymond Arnold; J Ellis; I & V Pearson; D & S Peach; C Gibbs; A Sullivan; A Byrne; J Wilson; A Tresidder; K Osborne; E Cox; M Rigby; S Robinson; N Thompson; D Robinson; B Weston; M Peach; R Arnold; J Ellis; I & V Pearson; D & A Gunary; C Dalligan; M Malcolm; E Mee; E Moody; M Hahner; K Brown; M Fullalove; M Leask; G Ferrier; E Brock; E Parkinson; R Taylor; R Gregory; L Stillwell; R Henshell; K Bown; S Home; V Little; S Greening; Z Gul; M Reeves.

Thank you to

Guy Horner and Harriet Benison who were married on 9 July and David Brooks-Daw and Naomi van Dyk who were married on 6 August, as they very kindly asked for donations in lieu of wedding gifts raising a significant amount for the MPS Society.

MPS Commercial

MPS Commercial

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Board
Jessica Kafizas
Sophie Thomas
Georgina Smith
David Patterson

Secretary
Christine Lavery

MPS Commercial is a Private Limited Company Registered No. 08621283.

MPS Commercial trades as Patient Access to Clinical Trials (MPS PACT), and is a wholly owned, not for profit subsidiary of the Society for Mucopolysaccharide Diseases (the MPS Society), Registered Charity in England and Wales No. 1143472.

MPS Commercial's social objectives are to reinvest any profits for the purposes of education, enhancing needs-led advocacy support, quality of life research and scientific research to the MPS community.

MPS Commercial Employees

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For more information about MPS Commercial and clinical trials visit our website:

www.mpssociety.org.uk/commercial

Patient Access to Clinical trials and Treatment

In 2013 the MPS Society set up MPS Commercial; a wholly owned subsidiary of the MPS Society whose social objectives are to reinvest any profit into the MPS Society so that the MPS Society can offer additional support and activities to its members.

Last year MPS Commercial's donation to the MPS Society included €20,000 to take members and their families to Bonn for the International Conference in July 2016 and €20,000, which was matched funding from the Gosling Foundation, to take families to Lapland in December 2016!

The MPS Commercial team are really excited by the opportunities that their hard work has provided.

Meet the team

Christine is the Group Chief Executive for the MPS Society and its commercial subsidiary

Gina is the Group Finance Officer for both the MPS Society and MPS Commercial

Charlotte manages the patient access clinical trials team who provide tailored logistical support to patients and their families

Jo is Lead Clinical Trial & Patient Access Officer and supports families participating in clinical trials across the world

Benedicta provides a logistical service for individuals participating in clinical trials.

Alex supports the newly introduced Managed Access Programme for Vimizim

Jackie is a Vimizim Managed Access Programme (MAP) Support Officer, for those with MPSIVA, Morquio.

Pauline joined MPS Commercial in February 2016 as a part-time accounts assistant.

Vimizim Managed Access Agreement (MAA)

Following the decision by NICE to make Vimizim (elosulfase alfa) available in England for the treatment of MPS IVA back in December 2015, we are pleased to report that sign up to the Managed Access Agreement has been rapid and both adults and children attending the specialist centres in England are receiving treatment under the scheme.

Northern Ireland announced in July that they will also be funding Vimizim under an MAA and a number of individuals with MPS IVA living in Scotland and Wales are also receiving treatment.

Review of the MAA

In June, MPS Commercial hosted a 6-month review meeting, bringing together clinicians, NICE, NHS England, the MPS Society and the Market Authorisation Holder, BioMarin. Clinical experts presented on their experience of the MAA in its first 6 months and the processes for collecting and reviewing the clinical and quality of life information were discussed.

Importance of Quality of Life information

The Quality of Life information that we collect is an essential element of the MAA and holds equal weight with the clinical measures made in the clinic that will determine decisions around the continuation of treatment.

Many of you will have completed your baseline, 4 month and 8 month assessments with us and we have been pleased to be able to offer a flexible system of telephone interviews, with an interpreter if needed and face-to-face interviews where necessary.

MPS Commercial is progressively becoming more involved in the collection, analysis and publishing of quality of life data and the design of surveys. These activities are aimed at quantifying the diagnostic journey, burden of disease and treatment value from the individual and family perspective. This type of information is important in the design of clinical trials and to quantify the benefits of treatment during the drug approval and reimbursement processes. Patients and their families play an essential role in sharing their experience in terms of priorities, risk/benefit assessment, and meaningfulness of treatment outcomes and acknowledgement of the value of this is increasing

“We are pleased to report that sign up to the MAA has been rapid and both adults and children attending the specialist centres in England are receiving treatment under the scheme.

Ultra-rare disease partnerships

The MPS Commercial team was asked to present at a policy engagement workshop in Edinburgh hosted by the University of Edinburgh to discuss Rare Disease, Patient-Private Partnerships and Access to Treatments. The select group of patient organisations, consultants in the biotechnology industry, policy makers and academics discussed the power of partnerships and the necessity of it in the ultra-rare disease field.

From left: Oliver Timmis (CEO of AKU Society), Lesley Greene (European Medicines Agency) and Charlotte Roberts (MPS Society)

Walkabouts

Get your boots on and walk your way for MPS

A great motivation to get fit whilst having fun with friends and family. Make it what you want, a midnight stroll through town, a hike in the forest or even a coast to coast trek. You can do a one off sponsored walk or set up a regular club. Just walk your way for MPS.

Find out more at www.mpssociety.org.uk/walkabouts