

MPS magazine

Society for Mucopolysaccharide Diseases

Summer 2017 • www.mpsociety.org.uk

Your spectacular
wear it blue
photos from MPS
Awareness Day

Recognising excellence

35 YEARS OF THE MPS SOCIETY



FAMILY EVENTS

Photos and fun from
the family weekends

CAMPAIGN UPDATE

Latest news on the
HST consultation

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

Chair – Paul Moody

Vice Chair – Wilma Robins and Jessica Kafizas

Trustees – Tim Summerton, Judith Evans, Judy Holroyd, Bob Stevens, Bryan Winchester, Sukhvinder Bhachu

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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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Been struggling to
get through to us?
Make sure you've got
the right number.



0345 389 9901

We changed to make sure you're charged less
when you call. Update your phone book now!

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SPORTY FUNDRAISERS

Our active fundraisers who have completed races all over the country tell us their motivation for running and raising awareness



MPS AWARENESS DAY

All the amazing things you did to mark the day

THE ZOO

Photos from a great fun day at Howletts Zoo

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

This bumper issue contains so many pages of your wonderful stories, photos and experiences.

We are pleased to bring you a round up of the MPS Society conference weekend which was really well attended and being our biggest conference to date there is plenty to read about. It's also great to give thanks to those who received an award recognising excellence over adversity in the last 35 years of MPS Society.

There is a double page spread of the wonderful fundraising and awareness raising around MPS Awareness Day and all your wear it blue activities. There were so many more we wanted to include and your efforts have been amazing.

It's great to see so many fundraising pages and just shows what fantastic things you do for the MPS Society. From head shaves to colour runs, spring balls to concert tickets, there's so many stories to read about.

From the Group Chief Executive



Christine Lavery
Group Chief Executive

With the MPS Society celebrating its 35th anniversary and the demands on the MPS Society increasing year on year I was grateful that in February the MPS Board of Trustees accepted my proposal to appoint a Head of Operations and Governance to help shape and future proof the MPS Society for the benefit of its members and all those affected by Mucopolysaccharide, Fabry and related lysosomal storage diseases. After a robust and open recruitment process I am absolutely delighted that Bob Stevens has been appointed and started in post on the 11 May.

“ We are both wholly committed to ensuring going forward that the MPS Society is even more proactive in reflecting the needs of our Members and their families

Bob, his wife Claire and their two sons Samuel and Oliver are well known to many of our MPS members as Sam and Ollie are both diagnosed with MPS II. Bob and Claire have been active members of the MPS Society, participating in MPS conferences, regional events and Bob has served as a Trustee of the MPS Society and continues to Chair the Board of Directors of MPS Commercial.

Bob writes personally in this MPS Magazine and we are both wholly committed to ensuring going forward that the MPS Society is even more proactive in reflecting the needs of our members and their families. During the recent MPS Conference those attending saw a very active team of Trustees meeting and consulting with our members to gather opinions that may inform the future direction of the MPS Society. For those who were unable to join us in July we will be reaching out to you in different ways and settings over the coming months. Equally we welcome our members taking the initiative and approaching Bob in confidence with your thoughts, concerns and ideas. The MPS Society is your 'Go To' support network and we need to know what is working, new ideas and what we might do better.

The MPS Conference was hugely successful and there was a tangible buzz the whole weekend. If you were unable to make the MPS Conference in Coventry we would dearly like to welcome you to other events planned for 2017. In 2018 we have planned two expert meetings on MPS I and MPS II with all the latest in clinical management, treatment options and outcomes and current research initiatives in these diseases. Please see the forthcoming events page for more information.

We look forward to hearing from you.

Christine
c.lavery@mpssociety.org.uk
b.stevens@mpssociety.org.uk

News from the Board of Trustees

The Society's Board of Trustees meet regularly. Here is a summary of the main matters discussed and agreed at the Board meeting held 31 March – 1 April 2017

Governance

The Governance training given by Dorothy Dalton has triggered new ideas and a policy review.

Paul Moody reported that he went into MPS House and spent some time talking to employees and what he heard was enlightening. Paul Moody spoke to Senior and non-Senior employees and he could see there is a huge workload all round. The Group Chief Executive spoke of the widening skill gap between herself and the Senior Leadership Team as the Society continues to grow and develop. The addition of a person to take away areas of work to allow CL to continue the higher end work was discussed at length. Prof Bryan Winchester proposed the MPS Society proceed with the recruitment of a Head of Operations and Governance. This was seconded by Wilma Robins and agreed unanimously.

After a discussion about the role of the second vice chair Jessica Kafizas was proposed by Tim Summerton and seconded by Wilma Robins. All agreed.

Under trustee recruitment Paul Moody spoke of strengthening the Board by bringing in new people. It was agreed

to host a Trustee surgery at the MPS Conference with the objective of engaging our members in the governance of the MPS Society.

Personnel

The Group Chief Executive reported that the Advocacy Support Manager has been successful in appointing another fulltime Advocacy Officer to her team to enable the advocacy team to meet the growing demand on the service.

Financial Management

The Group Finance Officer's reports were considered and approved without amendment.

Risk Management and Policies

The following policies were agreed:

No. 1 Data Protection policy; No. 9 Recruitment and Selection policy;

No. 10 Recruitment of office volunteers policy; No. 11 Policy on Recruitment of Ex-offenders; No. 16 Out of Hours Report Policy; No. 17 Lone Working Policy

No. 32 DBS and Disclosure Privacy Policy; No. 38 Sickness Absence Policy

No. 53 Office Key Holders Policy; No. 64 Volunteer Fundraisers Policy;

No. 77 Vulnerable Supporters Policy

The risk register was considered and approved without change.

Bob Stevens spoke of the meeting the previous day regarding the property schedule and policy and how this policy and schedule need further work. Tim Summerton agreed to go away to research more and update the current documents and bring back to the next meeting.

Research Grants

Prof Bryan Winchester spoke to an application received from Dr Uma Ramaswami at the Royal Free Hospital. The Board had some questions regarding the research initiative and agreed that a meeting should be organised to discuss the application in more depth. It was agreed that this application be brought back to the Board of Trustees meeting in June.

Welcome to our Newest Trustee

At the Board of Trustees Meeting held 23 June 2017 Daniella Vandeppeer was co-opted to the MPS Society Board of Trustees. The Board are delighted to have Daniella join them as a Trustee particularly at a time when the Society is actively addressing how it can improve communication and support to its members.

WHAT'S ON?

Regional Clinics

Great Ormond Street Hospital

MPS IVA – 25th Jul, 10th Oct

MPS I – 12th Sep, 28th Nov

MPS III – 26th Sep, 12th Dec

Birmingham Children's Hospital

MPS IVA – 15th Sep

MPS III – 21st Jul, 18th Aug (pm)

Transition clinic – 29th Sep (pm)

Fabry – 20th Oct (pm)

Mixed clinic – 18th Aug (am)

MPS II – 17th Nov

MPS VI – 29th Sep (am)

Queen Elizabeth Hospital Birmingham

Adult Fabry – 8th Aug, 12th Sep, 10th Oct, 14th Nov, 12th Dec

Manchester Children's Hospital

Post HSCT clinic (over 6 years) – 14th Jul, 13th Oct

Post HSCT clinic (under 6 years) – 6th Oct

Conferences and Regional Events

Weekend for bereaved families • Warner Leisure Hotel • Thoresby Hall, Nottinghamshire
13th–16th October 2017

Childhood Wood Planting

15th October 2017

MPS I Expert and Patient Meeting • Hilton, Northampton
28th–29th April 2018

MPS II Expert and Patient Meeting • Hilton, Northampton
28th–29th April 2018

15th Annual International Symposium on MPS and Related Diseases • San Diego, California
1–4 Aug 2018

Announcements

Welcoming Bob who has just joined the Society as Head of Operations & Governance



My name is Bob Stevens and I recently joined The MPS Society as Head of Operations & Governance

As I sit here at my computer contemplating how to write this article I am reminded of my start on a journey that has brought me to this point in my life.

Back in 2002 I was a Director of a construction company and enjoying a highly successful career. I had a lovely wife, Claire, two young sons Oliver (2 years) and Sam (10 weeks) and two cats Matey and Pilsbury. Life was great. We'd had some troubles with Oliver, our eldest, who was born with a serious brain haemorrhage but he was recovering and doing remarkably well. Claire and I were counting our lucky stars but then through a series of medical issues we had with Oliver came the one word diagnosis Mucopolysaccharidosis and in our case both boys had Hunter MPS II. Our world stopped! It was at that point the MPS Society stepped in and helped us to hang to on to what was really important in life. It was also then that my life took an unexpected turn and my journey to where I am now began.

Since those early days I have learnt a lot about life and people. I have worked with and alongside people of all ages with learning disabilities and complex needs. I became a Trustee of the MPS Society in 2006 and am also Chair of MPS Commercial. Previously to joining the MPS Society in this role I was the Managing Director of a Charity in Woking, Surrey.

What do I hope to bring to the role? A different perspective and a greater understanding of the differences and similarities between running a charity and a private sector business. Also an even greater empathy with all our members. As a father of two MPS II boys I understand the daily issues that we all face and the challenges constantly being put in front of us.

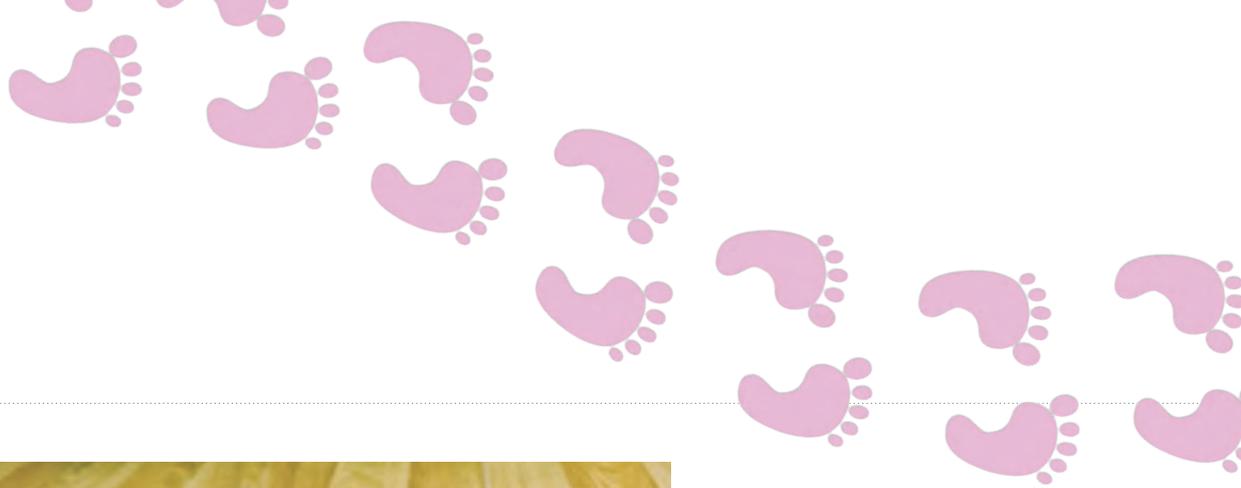
One thing is sure, although I am expecting to make a positive contribution to the MPS Society I will never be able to repay Christine and the team I find myself part of for guiding my family through those early dark days to today where now there is hope and a strong voice for those left most vulnerable in our society.

NEW MEMBERS

Lexi has recently been in contact with the Society. Her son **Reuben** has a diagnosis of Fabry Disease. Reuben is 1 year old. The family live in the Somerset area.

Jonathan has recently been in contact with the Society. He has a diagnosis of Fabry Disease. The family live in the Suffolk area.

Mr Kim has recently been in contact with the Society. His daughters, **Yedam** and **Yeshin** have a diagnosis of Fabry Disease. The family live in the West Midlands area.



We are very excited to announce the arrival of Lily Belle Sims, born 27th March 2017 and little sister to Nancy and our ML II angel Gracie.

Lauren Sims

We are so pleased to welcome three new starters who have joined in different departments across the Society.



Sophie

Sophie Dowsett is our new Fundraising and Information Officer. Sophie has previously worked in a care home organising fundraising and activities for the residents. Within the MPS Society Sophie looks after our community fundraisers, organises the challenges and events and makes sure our social media is always relevant. When she's not at MPS House Sophie enjoys playing with her two kittens and dying her hair crazy colours!



Sally

Sally Briody has joined the advocacy team as an Advocacy Support Officer. She has a background in social work in children's services and is quickly learning about MPS. She is looking forward to being able to support our members to navigate their way through the various services that they may need. Sally loves cats and has three tabby cats. She likes swimming and spending time with her grandchildren.



Barbara

Barbara Cotterell is our new Administrative Assistant to the Group CEO. She has previous experience as a medical secretary and also worked for many years in a local college, providing administration and support to students. When not at work Barbara loves spending time with her two young grandchildren.

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 here 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.

We look at needs and abilities independent of support and services. We then assess and advocate for the services taking on a needs-led attitude and approach to ensure services and support are tailored to individual needs and outcomes at all times.

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.mppsociety.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, 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 IVA Morquio, MPS I Hurler HSCT, Hurler Scheie, Scheie, MPS VI Maroteaux Lamy, MSD, ML II (shared with Sally)



Rebecca

Fabry, MPS II Hunter, ML III/ML IV, alpha mannosidosis, beta mannosidosis, Fucosidosis



Louise

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



Alison

Supports members living in Northern Ireland and the Republic of Ireland



Steve

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



Sally

MPS IVA Morquio, MPS I Hurler HSCT, Hurler Scheie, Scheie, MPS VI Maroteaux Lamy, MSD, ML II (shared with Debbie)

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:

advocacy@mppsociety.org.uk

0345 389 9901

[facebook.com/mppsociety](https://www.facebook.com/mppsociety)

**URGENT
ACTION
NEEDED BY
MPS MEMBERS**

LSD Survey update

A big thank you to everyone who has kindly completed the recent LSD survey.

This anonymous survey is the most important questionnaire we will ask you to complete this year as it informs NHS England on the performance of the LSD service at its 3 Paediatric and 5 Adult centres in England, the patient organisations and homecare companies. By taking a few moments to participate, you could help to enhance the future care of those affected by diseases for which the MPS Society offers support.

Our survey will be closing shortly so please take this opportunity to anonymously share your views on what is working well and where you think improvements can be made. You can do this in one of the following ways:

- Complete the survey online via Survey Monkey at www.surveymonkey.co.uk/r/LSDSurvey
- Email b.cotterell@mpssociety.org.uk for an electronic or paper copy. Please return completed forms to: MPS Society, MPS House, Repton Place, White Lion Road, Amersham HP7 9LP
- Provide your responses via a short telephone interview (to arrange a suitable time please email b.cotterell@mpssociety.org.uk)

For Scotland, Northern Ireland and Wales we are very pleased for our members to complete the survey so feedback can be given to the relevant health and social care authorities.

Update from Ireland

It's been quite a while since I last put pen to paper (or fingers to keyboard) for the MPS magazine. As I write I've been back at my desk from maternity leave for 11 weeks – and what a busy 11 weeks they've been.

In the last few months my focus has been on reconnecting with our MPS families (North and South) – I haven't yet spoken to everyone but please know that I am making my way through my list of families and you have most definitely not been forgotten about! If you have an ongoing issue that you want to discuss please do pick up the phone. I am really enjoying reconnecting with you all, and as you know I am always up for a chat!

You can contact me on my usual numbers and email address:

0044 77862 58336 or 044 28950 47779
a.wilson@mpssociety.org.uk

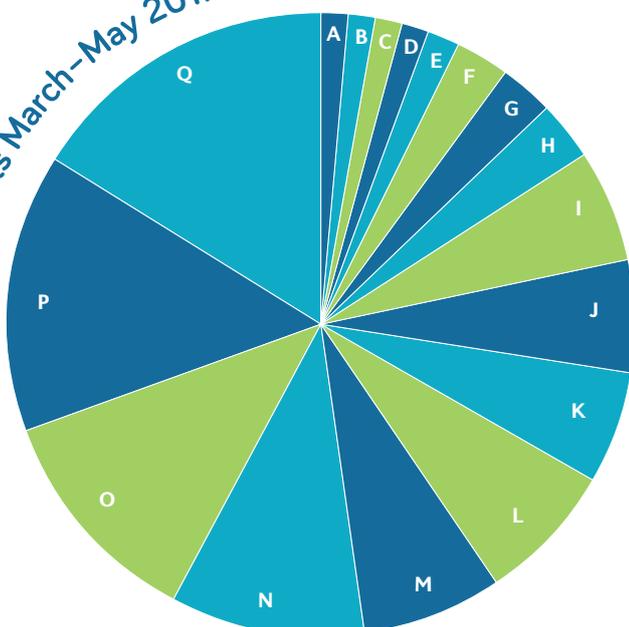
I'm sure that most of our Northern Irish families will now be aware that Dr Fiona Stewart is currently off for an extended period. Although she is off, she is keeping in touch and is up-to-speed with what's going on for each of her patients. I'm sure you will all want to join me in passing on our very best wishes. For those families in Northern Ireland who are managed by Dr Stewart, please contact me directly with any medical queries and I will do my best to point you in the right direction.

In the upcoming months I will be looking at our Ireland meetings and events. We want to tailor our activities as best as possible to meet your needs. Please let me know if there is anything that you need or would find helpful. We are also relooking at how our clinics are structured in Northern Ireland and would very much welcome your input. Please keep an eye out in the post for our short survey.

This update has been short and sweet! I look forward to bringing a more colourful update with a few photos in our next magazine once the dust has settled and the All Ireland service is back in full swing! It's great to be back!

Alison

Review of visits March – May 2017



- A Clinical management
- B Social care
- C Visa tribunal
- D Advocacy 1:1
- E Other
- F Continuing Healthcare/Deprivation of Liberty
- G Pharmaceutical/Research meetings
- H Funerals
- I Housing/Occupational Therapy/Disabled Facilities Grant
- J School talks
- K Benefits
- L Child in Need (CIN)
- M Education
- N Clinics
- O New member visits
- P Multi team meetings
- Q General visits

Remembrance



Photo: Genzyme Corporation

Henri Termeer who built Genzyme into a major biotech company has died

Henri Termeer, a biotechnology pioneer and the long-time CEO of Genzyme, has died at the age of 71 after collapsing at his home in the United States on Friday 12 May.

Henri was an important figure in the development of the biotech industry worldwide becoming an innovator in the field of rare disease drug development.

Having started his professional career at Baxter Healthcare in the 1970s Henri Termeer left to join a then small company, Genzyme, which was developing enzyme replacement therapies and was losing money in 1983. Termeer saw an opportunity to help turn Genzyme around and build a biotech company.

Henri was given a free rein to fulfil his vision in developing a rare disease drug development giant. He went

on to develop treatments for such conditions, among them Gaucher, MPSI, Fabry, and Pompe disease.

“We were also at GI [Genetics Institute] looking at Gaucher disease,” Schmergel recalls. “Where I saw only problems – small number of patients, insurance issues – Henri saw the opportunity. Henri just went for it, and that was the key to the magic kingdom for him. He was the guy who opened up the whole orphan drug field. So that’s the creativity he had, and vision.”

Henri Termeer had his share of setbacks, perhaps most notably a manufacturing crisis that led to a Fabrazyme shortage, a \$175 million FDA fine and the subsequent acquisition of Genzyme by Sanofi in 2011.

However, Henri Termeer’s impact has been felt worldwide with the rise

of the orphan drug industry and the accompanying increase in collaborative working and empowering of patient advocacy groups.

Our thoughts are with Henri’s family and friends at this sad time.

Christine Lavery
Group Chief Executive



Taken 2 weeks and 4 days before she passed away it's hard to believe with that special smile right to the end

In memory of Lisa Nurse 29th July 1971 to 28th April 2017

Our beautiful and loving daughter who suffered from Sanfilippo Disease Type MPSIII passed away very suddenly and unexpectedly on the 28th April it was such a shock, that our special girl had passed away in hospital, but we take comfort in that she is now at peace surrounded by those who love her in heaven. Our lives will not be the same without her. We had some very difficult years at first, not knowing what was wrong with Lisa until we got the diagnosis. Like all parents receiving such devastating news, we were left reeling and it took us some time to come to terms with. Our daughter certainly changed our lives. We were given the contact details for the MPS Society but we couldn't bring ourselves to phone them. My social worker did all the arrangements, however we were in complete denial and making that call would have meant admitting to ourselves that our daughter had a life limiting condition and a short time to live. I wasn't ready for that but Lisa proved everyone wrong and lived a long and happy life.

Christine Lavery and all at the MPS Society have been a very important part of our lives, and we thank you all.

We had Lisa for 45 years and 8 months and have so many happy memories of her. Lisa touched so many lives and was deeply loved by all of our family and friends, I hope that she knows how much she touched our hearts, and how much we all loved her. Lisa gave us unconditional love and made us appreciate the things we had, and that the simple things in life are the best. Everyone talks of Lisa's lovely smile, which could light up a room and her infectious laugh. These are the things we all miss about Lisa. Sleep tight our angel, we cannot wait to see you again, and feel the warmth of your touch and kiss as we share a big hug again. Forever in our thoughts and hearts you remain, never shall we be parted, God Bless you Lisa, we will love and miss you forever.

*Mum, Dad, Paul, Maria and Mollyann
and all your family and friends*

Luke Bown 16th August 2004 to 26th April 2017



Bereavements

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

John Corder who had Fabry disease and passed away on 3rd April 2017

Habib Mahmood who had LAL D and passed away on 4th June 2017

Jack Edmond who had ML II and passed away on 15th June 2017



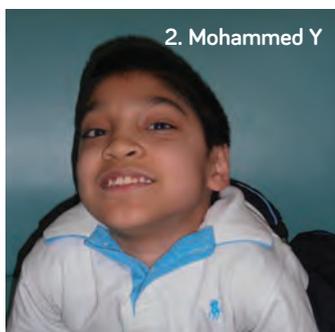
Clinics



1. Oliver

GOSH
MPS III
14 MARCH

(1)



2. Mohammed Y



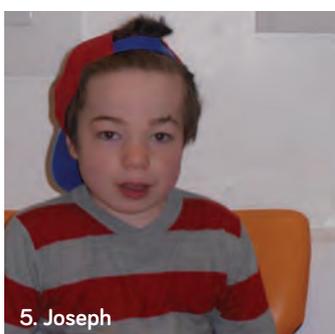
3. Muqadas



4. Mohammed G

BCH
MPS IV
17 MARCH

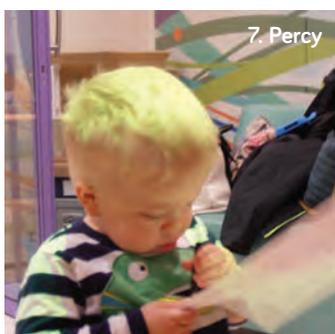
(2-8)



5. Joseph



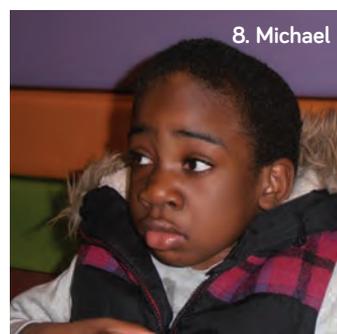
6. Zack



7. Percy

It was lovely to see the families at the Birmingham Children's Hospital MPS IV and Manchester MPS I clinics. It's amazing how much the children have grown up! And I look forward to seeing you all at clinic again soon.

Debbie



8. Michael



9. Ellie and Ethan

RMCH POST HSCT 7 APRIL

(9-10)



10. Leo

Download our BCH Feedback App or visit www.bch.nhs.uk/feedback



BirminghamChildrensHospital



@Bham

Please remember to complete the Friends and Family feedback



12. Elsie



11. Saskia and Keandra

BCH FABRY 19 MAY

(11-15)



13. Molly



14. Alfie



15. Freddie

It doesn't seem five minutes since the last Fabry clinic but time does fly and I realised that by the fact that the children and young people were all many inches taller, or is it me shrinking!

Well for some it is exam time and for others they do get some form of a treat because they have been at clinic and need something nice to look forward to. Fabry clinic's are always quieter than our MPS ones but it is lovely to catch up with the families and for some in the not too distant future I will be seeing them in the adult clinic at QE.

Thank you as always from the team for making us welcome.

See you again next time.

Rebecca



16. Rachel



17. Steven



18. Cody



19. Luke

**RMCH
MPS I
28 APRIL**
(16-24)



20. Charlie (centre) with brother and sister

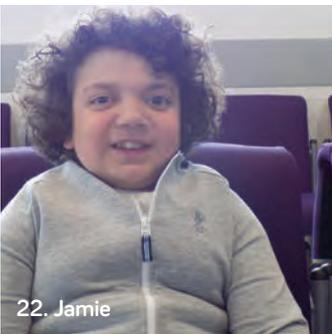


21. Melissa

I travelled to see the MPS I families at the Royal Manchester Children's Hospital Clinic. It was a very full clinic with lots of new faces to meet and greet. It was lovely to talk to all of the families and to see how supportive the families are to each other.

I enjoyed hearing about all the positive things the families were planning to do, such as holidaying in Cyprus, moving to secondary school, thinking of life beyond school and supporting Barcelona Football Club. A few families showed me pictures of new pets and pictures of their interests. Lastly but certainly not least I cannot wait to hear how Melissa's 13th birthday party turned out. Thank you to everyone who attended the clinic, it was a really lovely day, and I learnt a little bit more from each and every one of you.

Louise



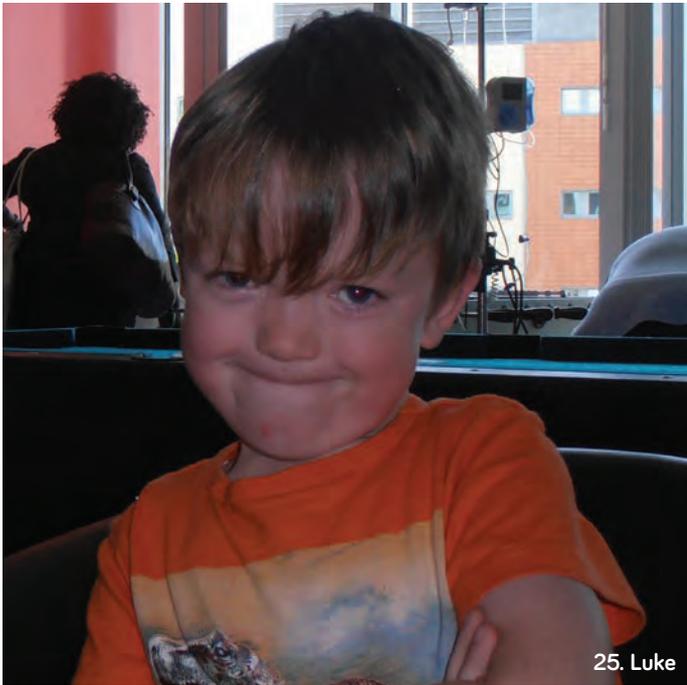
22. Jamie



23. Lyla and Luke



24. Alicia



25. Luke

MPS VI family clinic day 3 May

(25-28)

I would like to say that this family clinic day stemmed from a lot of strategic planning and organising...however it really was happenstance when I realised that I had managed to inadvertently book 7/10 of my MPS VI families into the same clinic!

Not to miss an opportunity to get folk together and mingle, the family day became a reality and all the plans were quickly put in place with the help of the play specialists, MPS Society and Willink gang.

A picnic lunch and the Giggle Doctors plus games and activities in the Teen Zone appeared to make the normal clinic appointment much more entertaining. Children played and parents nattered away whilst we ran around in circles collecting samples, directed them to physio and eventually sat them down in front of our very tolerant consultant Simon!

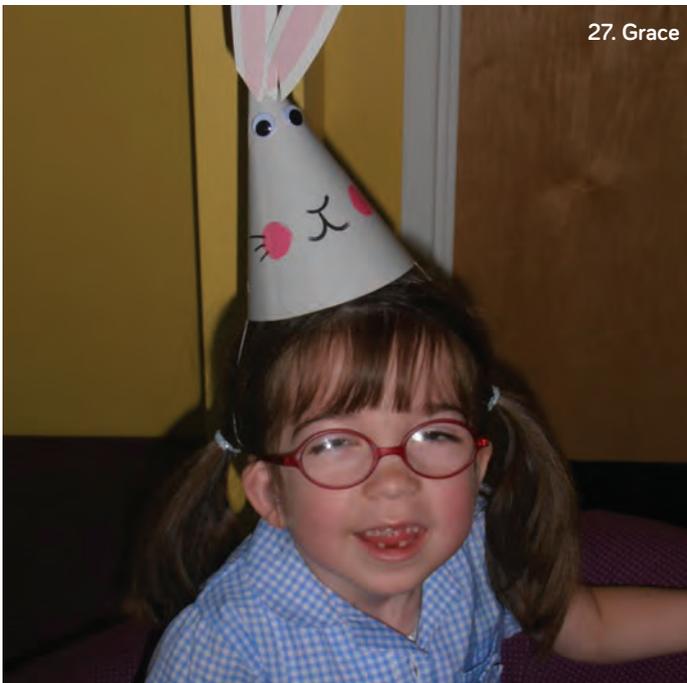
Again I would like to say it went without a hitch but, behind the scenes, we had a rather disastrous time with key members of staff ringing in sick and being unexpectedly, prematurely ejected from the Teen Zone. Nevertheless feedback was good except some felt that the day was a bit longer than they had expected. They felt it was informal and more like a social event or a reunion rather than just a generic clinic appointment. They all seemed to enjoy meeting all the other families and children and indeed one young lady reported how she felt 'tall' for the first time as she met the younger MPS VI children!

So in essence we are planning to do this again in the future and hopefully next time we will be fully staffed and firing on all cylinders!

Jane Roberts

MPS VI specialist nurse at Manchester Children's Hospital

26. The Giggle Doctor



27. Grace



28. Aliya



It is coming up to five years since Josh was diagnosed with MPS. Josh is now 8 years old and has Maroteaux Lamy Syndrome (MPS VI). Since Josh's diagnosis myself and my family have been constantly raising awareness and money for the MPS Society and research. Raising awareness is very important to us. It's so hard to explain a disease that is so rare, especially when no one knows about it. I set up an awareness page for Josh on Facebook and he has over 2000 followers, including other MPS mums and dads who I am now in contact with because of his page.

The power of social media is incredible, as we all know. I would be totally alone without it but instead I am in contact with parents and even patients with MPS from all over the world.

I have four children altogether, Jasmine is 14, Libby is 9, Josh is 8 and Annie is 4. I currently work at a local petrol station getting up at 4:30am four days a week! I also administer Josh's infusions myself. Along with all of the hospital appointments, infusions, not to mention the things we do with the

girls as well, it becomes difficult not to get caught up in a world that seems so different from everyone else's.

On the 28th February this year I took a big risk and started my own business. I wanted the freedom of being able to work from home, and to be able to raise even more awareness for Josh at the same time. I became an Independent Presenter for younique, a make up and skin care company where every product is cruelty free. It is all done through network marketing and is now the fastest growing direct sales company in the world!

I now get paid to promote and sell make up from my own home! Within two minutes of me joining, I had my own website, for free, which has Josh's story in the description and I have shared Josh's story with over 10,000 people, from all over the world!

I paid £70 and got more than £280 worth of products for me to start. I made my money back within a week. At first I thought it was too good to be true but I am living proof that this can work for anyone. I have created so much more awareness for MPS in so little time, and I am earning a living whilst doing it!

I wanted to share this with you all to let you know that you can do it too. In a couple of months time I will be able to quit my job at the petrol station and I am looking forward to an easier life with my family.

Find Josh's awareness page at:
www.facebook.com/joshhashope

Visit Sarah's makeup business at
www.youuniqueproducts.com/daretodreamdaretohope

Your stories

Sarah Cutler shares her story on how the power of social media stopped her feeling alone as a parent of someone with a rare disease and has given her the confidence to start her own business

Sarah started the craze of blue lips this MPS Awareness Day by offering a discount on her blue lipsticks

Our little hero

On the 19th May Sophia Scott was awarded the Our Little Hero award which was sponsored by Utilita as part of the Our Heroes Awards held in Glasgow. The awards are for outstanding children whose achievements, courage or bravery make your heart burst with pride.



On the night guests enjoyed a performance from Melanie C and the incredibly talented Voice 2017 semi-finalist Craig Ward. As well as a champagne reception and three course meal.

Sophia attended the event with her mum, dad and grandparents. Sophia's mum, Amanda, and dad, Darren, filled us in on what happened at the awards.

When and how did you hear about the award?

We had an email come through to us that said Sophia had been nominated because of her courage and inspiration. Three children were put up for the category, all three won the award.

How was the lead up to the event for Sophia?

The day before the awards we were taken to Braehead shopping centre and Sophia was bought a dress which you will see that she wore on the night. They also took all the children in the awards to play crazy golf.

We had recorded an interview before the event that they played on the night to raise awareness on Sanfilippo.

Where is Sophia's award now?

Sophia called her award "Trophy" and took it to school the following Monday. It now is hanging on our lounge mantelpiece. Sophia was also given an Aurora Doll and Princess house on the night and really enjoys playing with her.

The Bath Chronicle published this piece about Sarah Long, one of their 2016 Brave Heart Hero winners, in June 2017

Heart of Bath 2017: Our Braveheart hero has defied all the odds

Sarah Long, 45, continues to fight morquio syndrome after doctors told her parents she would not live past her teens



Sarah Long is not a woman that gives up easily.

The 2016 Brave Heart Hero is thought to be one of the oldest people living with Morquio, a disease that affects the major organ systems in the body.

Only 88 people in England – and 160 worldwide – are known to have the syndrome and barely a handful have made it into their thirties. But Sarah, now aged 45, continues to defy the odds.

In 2012 she signed up to a free trial of the drug Vimizim, and reported its ‘unquestionable’ benefits.

She described how almost immediately her health picked up, her breathing improved, she was able to sleep, she was less prone to infection and she was able to enjoy life by studying for a PhD at the University of Bath, where she also teaches.

“Pre-Vimizim, my body was very heavy, like moving through thick treacle, everything was a struggle and I was in acute respiratory failure,” she said.

“Being on the drug has changed me enormously. I realised that I was very trapped in a shell of myself...It really has been my little miracle.”

But she was devastated to learn that the free trial was to be stopped -

because an independent committee, which advises the National Institute for Health and Care Excellence (NICE), suggested the benefits of the drug had been overstated.

Sarah vowed to ‘fight until her last breath’ to keep the life-transforming drug available on the NHS.

She penned a powerful open letter to then-prime minister David Cameron, writing, “I am living proof that this drug prolongs life as well as providing a quality of life.

“If, as it seems increasingly likely, the UK will not provide this treatment, it will be my end.”

But thankfully, drugs manufacturer BioMarin decided to reinstate the drug following reports of clinical deterioration of Morquio patients since supply was stopped.

She continues to receive Vimizim, but this is subject to constant review by NICE. “It’s living with the unknown. When I had to come off the drug before I got so sick. If they do decide to pull the plug, that will be me gone,” she said starkly.

Fighting is something Sarah knows all about. Since her mother died of cancer in 1989 she has lived independently, despite her disability.

“This is when I had to become independent,” she said. “It was her battle with cancer . . .that inspired me to push myself. Education was important to my mother and she pushed for me to have one.

“I have forged my own way in the world, including getting an undergraduate offer from the University of Bath, learning to drive and running my own independent living scheme.”

Although now back on Vimizim, the period where Sarah was unable to access the drug was physically damaging, particularly to her lungs.

“I took a big hit,” she explained.

“I’ve had to have a very gentle return back to my life.”

Vital to her recovery has been her assistance dog, a Shitzu-Poodle cross. The bond between them is such that the puppy can detect a change in Sarah’s breathing and alert her carers.

“Last night at half four she picked up that I was struggling and woke my carer up to nebulise me,” she said.

Being named Brave Heart Hero 2016 meant a great deal to Sarah.

“I can’t believe it’s been a year!” she said.

“It was very touching to know that I wasn’t just on my own, writing emails - that people cared.

“It gave me validation, if you like. It was a confidence boost, knowing that I’m doing the right thing.”

This article, written by Holly Thatcher, has been reproduced with permission and was first published in June 2017 here: bathchronicle.co.uk/news/bath-news/heart-bath-2017-braveheart-hero-106239



Events



Gulliver's Land

for MPS Awareness Day





Howletts Zoo



Gina and I spent a glorious spring day at Howletts Zoo with families from the Kent area. It was great to meet families that I have spoken to and supported from the office, and it was good to hear how things were going for them and how the MPS Society may support some of them further.

It was amazing to see the tigers, lions, monkeys and other animals. However the best part about the day is that the families all appeared to enjoy themselves and had opportunities to meet up with both old and new acquaintances, and make special memories for their families all thanks to funds received to allow us to put together this great event.

Louise Cleary, Advocacy Support Officer

Even the giant anteater came out to see us, something he has never done before





The best part about the day is that the families all appeared to enjoy themselves



Thank you for an excellent day out at Howletts zoo in Kent. We arrived promptly and managed to meet up with our friends whose son also has Sanfilippo like our son. We had not managed to see them for a couple of years due to lots of obstacles but we spent most of the day catching up. We also managed to meet up with two other families we knew and compare notes and supply each other with ideas. The staff who accompanied us were helpful, kind and friendly and it was great putting faces to names. We live fairly locally to the zoo and know it well and have visited many times however we do not always manage to see many animals but everything must have been just right as most of the animals were out and about which was great. Even the giant anteater came out to see us, something he has never done before. We stayed for several hours relaxing and chatting (and viewing animals of course!). It was a lovely day out and thank you for facilitating it.

Jan and Rob Gremo



MPS Society weekend conference 2017

Conference A Saturday 8th July By Bob Stevens

In the first conference of the morning, which was chaired by Bob Stevens, James Davison kicked off with an overview of MPS diseases and the reminder that early diagnosis leads to earlier treatment which in many cases leads to better outcomes. After this Elaine Robinson talked about meeting the orthopaedic needs from the hips down and that bone cartilage does not form properly in MPS diseases meaning that knee valgus is typically evident in MPS I and II along with hip dysplasia and would usually indicate the need for early corrective surgery. We also heard from Andrew Jester on carpal tunnel and the hands, Stuart Wilkinson – who talked about respiratory complications, the need to ensure vaccinations are up to date and that improved airway management that results in better sleep could have significant benefits to a child's education and learning – and Eleanor McGovern who talked about dental care and early intervention.

Our own staff presented on their areas of expertise, including Bob Stevens on how benefits and access to services change when individuals become adults, Alison Wilson who gave practical advice about careful planning based on outcomes from the UK pregnancy project and Charlotte Roberts on how the Vimizim campaign resulted in the Managed Access Agreement and treatment being made available.

Hearing from families about their experiences was valuable, Claire Stevens spoke about how home infusions give back part of their family life as it allows flexibility to plan for holidays and days out. Katy Brown spoke about the fight for access to treatment which took her family and the MPS Society all the way to Downing Street and proved there is power in the collective voice.

As always the voice of the professionals was very welcome. Anupam Chahrapani gave an overview of current treatments, Simon Jones spoke on the importance of natural history studies to better understand disease progression and help with information needed in clinical trials and Rene Sonders gave an insight into the working life of homecare nurses.



Addressing the needs of young people is a key area in the future strategy of the MPS Society so it was helpful to hear from [Stewart Rust](#) who gave a fantastic talk on meeting the psychological challenges of young people with MPS conditions. Take one day at a time and share feelings with family and friends. It was also inspirational to hear from some of our younger members.

[Asma Seedat](#) gave an emotional presentation on behalf of [Aisha](#) who was unwell with regards to choices and achievements moving into further education. Despite many challenges Aisha has never let this stop her from reaching her goals and she is an inspiration to all.

One of our young adult members gave a memorable presentation and how she does not suffer from MPS I but lives with it.

“The Department of Health states that there is a two way relationship between health and well being: health, both physical and mental, influences well being and well being influences health. But health and well being are individual and tailor made. They're driven by who we are and formed and shaped by the hand of cards we are dealt at our conception. We have no control over those cards. And it is with those cards that we begin the game of life.

“Winning or losing that game is determined by ourselves. Recognising, accepting and working with the cards we have been dealt is vital – if we want to win. And I for one want to win. I want to win big!

“In order to truly have a great quality of life, we have to love our life. We almost have to love MPS. And most importantly, we have to love ourselves.”

“In order to truly have a great quality of life, we have to love our life



“ I wanted to send congratulations to you and the team on what was a really professional and very enjoyable 2017 conference. It seems to get better and better year by year!



Conference B Saturday 8 July 2017 By Jessica Kafizas

Myself and Dr Brian Bigger co-chaired the Saturday morning's conference with a very busy agenda.

Dr Julian Raiman from Birmingham Children's Hospital opened the conference giving an overview of MPS and related Diseases in a easier to follow format including a history of the diseases, and many photographs to illustrate his points including the different attenuations of each disease.

Michelle Wood, a specialist physiotherapist from Great Ormond Street Hospital gave a presentation on meeting the orthopaedic needs in individuals with neurological decline. This had a real focus on ensuring the quality of life of each sufferer is improved, with the aim of them being able to function independently. She emphasised a holistic assessment is required, and a multi-disciplinary approach. She shared a photograph of a card a young patient drew for her calling her the "No 1 Psycho!" – clearly some improvement is required on their spelling (!) to "No 1 Physio" – but this showed how grateful she was for the time that particular child took to say thanks and the significant improvement that the physio had on their life.

Dr Chris Hendriksz from Salford Royal gave a talk on respiratory complications and interventions, saying how very different the airways of MPS sufferers are and how important it is that immune systems are built up. He said that 4-8 infections a year are common in the general public, so MPS sufferers can expect many more than that. He also posed the question of whether fit to fly

assessments should be obtained by sufferers in good time before any flights they may take.

Dr Ashish Chikermane from Birmingham Children's Hospital gave a presentation on Cardiac manifestation in MPSIII and MPSII. The general message was that one needs to look far beyond murmurs and there is a real interplay in being pragmatic about what can be done for a patient including a multi-disciplinary approach because MRI scans can require anaesthetics etc.

Chris Hendriksz then gave a second presentation on disease manifestations in conditions with late onset.

After the short break, we heard from Dr Sanjeev Rajakulendran (Dr Raj) from The National Hospital for Neurology and Neurosurgery on Seizure Management including epilepsy. Lots of questions were asked by parents about what to do in an epileptic seizure situation.

Dr Julie Eisengart came all the way from the University of Minnesota and presented on how we measure cognitive function in individuals with progressive neurological disease. She discussed what she called "childhood dementia" which could be a slowing down or loss of skill and knowledge.

Shauna Kearney from Birmingham Children's Hospital gave some tips on behaviour management techniques, and drew comparisons that some children with more severe forms of MPSIII have autistic-like traits. She suggested that behaviour charts could be used to reward good behaviour.





“ It’s wonderful to hear about all the advances that have been made

Karen Robinson, the mother of Oliver with MPSIIIA gave an excellent insightful talk on useful hints and tips on how she and her husband Stuart manage their son’s behaviour. Her presentation was very useful to parents in the audience as she told them tips that worked for her family, such as producing a small laminated information card to give to members of the public who tutted at Oliver’s behaviour not knowing he suffered with an MPS disease, and the type of cups and chairs she uses for Oliver and twiddle pom-poms he has so he doesn’t hit out at others.

Jo Marks from the Royal Manchester Children’s Hospital then concluded the morning’s session by giving a talk on swallowing difficulties and MPS. This provoked many questions from the parents of MPS sufferers in the audience. The general message was how important it is to monitor mealtimes, as some meals may need to be modified including being blended more, or more sauce being added.

After lunch, my charring duties were over (and yes, we did break for lunch on time thankfully).

Judith Evans (former Trustee of the MPS Society) and Dr Chris Hendiksz took over in chairing the afternoon session. The first half of this session had an emphasis on advocacy.

Alison Wilson from the MPS Society stepped in and presented on continuing healthcare assessments, which are usually provided within the home.

Dr Dougal Hare from Cardiff University then presented on whether children with MPSIII have symptoms of Autistic Spectrum Disorder. He concluded that autism is more likely to occur in individuals with known genetic disorders than in the general population, although this has led to many a misdiagnosis within the MPS world.

Stewart Rust of Royal Manchester Children’s Hospital gave a fantastically enthusiastic presentation on sleep disturbance and how to manage it as a parent/carer. He told us all by way of a comparison, that all the “ingredients of a cake are important” which means that you have to try to keep the balance right between all the components to ensure your child gets a good



“ Thank you very much. The one thing in life that was no hassle, so relaxing and felt like a mini holiday!



night's sleep and, in turn, this means that you as a parent are well rested. He also reminded us all how important it is to pat yourselves on the back from time to time to tell yourself what a good job you are doing. He also recommended the website kidsleepdr.com.

Charlotte Starling of Kent County Council did her first presentation for the MPS Society on how to plan for the future including getting the right equipment and adaptations in place. She showed us how housing adaptations could really change an MPS sufferer's life, but that if adaptations to the home are needed it is important to get the council to approve the works before they are started because they cannot be reimbursed retrospectively. She illustrated her talk well by reference to a case study of Mr H and how the adaptations had significantly improved his life.

Sophie Thomas from the MPS Society gave a great overview of the complex area of the law that is the Mental Capacity Act 2005 in 5 minutes (as a lawyer myself, I was particularly impressed this could even be attempted because the Act is encyclopaedic).

Parent, Peter Hawkins presented on the court of protection and the difficulties he experienced, in particular in the area of “personal welfare” as opposed to “property and affairs” (the former being far harder to get) including the many, many forms he had to complete and useful tips including keeping a record of every person's name that you speak to along the way and if they are particularly helpful, making sure you keep that reliable contact on tap at the court of protection!

After the break the topic of the presentations was on clinical trials leading to market access and reimbursement.

Dr Maureen Cleary from Great Ormond Street Hospital gave a clear and brief overview of the

current treatments available, including ERT, bone marrow transplant, small molecule therapies, and gene therapy.

Dr Saikat Santra from Birmingham Children's Hospital presented on how natural history studies help us to better understand disease progression and how they are the important feeding group for clinical trials. He suggested that you should consider giving consent for DNA results to be stored, because it can be very helpful to document any correlation going forwards.

Dr Joseph Muenzer came all the way from the University of North Carolina and presented on gene therapy for MPS conditions with neurological decline. This was a thought provoking topic that the audience wanted to ask lots of questions on.

Jo Goodman from the MPS Commercial team presented on understanding the role of the Patient Access to Clinical trials and treatments team, including the practicalities for arranging the patient access including ensuring interpreters could be immediately available on the telephone and how this is a fast reactive team, reacting to each individual patient's specific needs wherever they are in the world.

Daniella Vandeppeer gave the final presentation of the day which was a really excellent talk on her family's experience of being enrolled on a clinical trial, and the ups and downs this entailed for her son Caleb (who suffers from MPSII) and the wider family in ensuring he got to the trial each time for the dose to be given to him before they could treat him to a McDonald's on the way back from Birmingham to Hertfordshire! She illustrated the talk with no text at all, but a series of photographs of Caleb and their family, especially his late paternal Grandmother, which made it a very powerful and emotive presentation.



Conference A - Sunday 9 July 2017

Chaired by Bryan Winchester and Paul Moody this Sunday conference drew together a variety of interesting and useful presentations about therapies for MPSI, MPSII, MPSIVA, MPSVI and LAL D (late onset). Highlights of the morning were Bryan Winchester's overview of enzyme replacement therapy (ERT) where he gave good, clear explanations about where we are with this treatment today as well as a helpful summary of the latest developments.

Chris Hendriksz and Alex Morrison's presentation on ERT for MPSIV and how the Managed Access Agreement is working 18 months after it started was well received. There has been a rapid sign up to the MAA following the issue of NICE guidance and currently 53 patients in England are being treated under the scheme. There is concern that the four monthly reviews are a burden to adults in employment but on the positive there is good data to answer the complex question of who will benefit. Time will tell if this is fair and will capture real benefit.

Other popular talks included an introduction to gene therapy by Joseph Muenzer from the University of North Carolina, whose research is focussed on the development of treatments for brain disease in MPS patients.

Also, from Royal Manchester Children's Hospital, Simon Jones' presentations on intra-cerebral and intravenous gene therapy for MPSI and MPSII were interesting and clearly presented.

All the speakers did a great job and provided an insight into the relevant therapies available to MPS patients.



Conference B - Sunday 9 July 2017 By Alison Wilson

Being at an MPS Society National Conference can sometimes be bittersweet. It's wonderful to hear about all the advances that have been made in the treatment and management of the MPS and related conditions. However, when your loved one is living with one of the MPS or related conditions where there is not yet a widely available treatment, those stories of success and scientific breakthrough can be hard to hear. In Conference B on Sunday the focus was on Emerging Therapies for MPS III, MLD and infantile LALD. These are some of the most life limiting conditions that the MPS Society support and for many years treatment seemed a long way off. However, in recent months and years there has been a flurry of scientific activity and we are now in an exciting time for these very rare conditions.

Dr Bigger kicked off the session with a concise and informative session on current and emerging therapies. He spoke about the pros and cons of existing treatments; and importantly explained why some of the existing therapies do not quite fit with some of the very complex neurological conditions represented. In reviewing the current therapies Dr Bigger pointed us to areas where research is now focusing on to break down some of the barriers in treating these conditions. During this session we also heard from Prof Simon Heales about the different approaches to gene therapy. He explained the difference between ex vivo (when cells are removed from the body before gene therapy) and in vivo (where the new gene is delivered directly to the target tissue). These presentations provided a sound basis for understanding the presentations that followed.

It was exciting to hear from Dr Arunabha Gosh about the development and clinical trial of Sebelipase Alpha for the treatment of infantile LALD. Without treatment it would be very unusual for an individual with infantile LALD (Wolmans Disease) to live beyond 12 months. It was fantastic to see trial data of 5 children living beyond 3 years and doing well with the help of this new treatment. It was particularly exciting to hear that these 5



children are all entering the education system and are doing well with only some additional support. This treatment received EMA and FDA approval in 2015 but was unfortunately not granted approval by NICE. An appeal decision is awaited.

We heard again from Dr Brian Bigger about learnings from the Genistein Trail. As we have come to expect, this topic generated a great deal of discussion in the room. Dr Bigger presented some background information about Genistein and the development of the clinical trial. It was interesting to get an insight in to the timeline of the trail from first results in mice in 2010 to the first patient being treated in 2014. We expect the full results of the trail in November 2018. Dr Bigger explained the possible outcomes of the trail and what the next steps would be. The biggest topic of discussion from the floor was the plan for continuing access to Genistein after the end of the trial period. Without the involvement of a large pharmaceutical company there are unique challenges in funding the production of Genistein in the absence of formal approval.

Dr Bigger kept hold of the mic and went on to present information on stem cell gene therapy for MPS IIIA/B delivered by Haematopoietic Stem Cell Transplant (HSCT). Dr Bigger explained the scientific basis of gene therapy and how using gene therapy on cells used in HSCT would result in the enzyme over-expression (particularly in the brain). It is this brain over-expression that is hoped will help address the very difficult to treat brain disease in MPS IIIA and B. This treatment will be trailed soon with Orchard Therapeutics.

Dr Suresh Vijay presented on intrathecal delivery of ERT from MPS III B. He explained that this involved the placement of a port directly into the spinal column to deliver ERT to the nervous system. The aim of this delivery of treatment is to overcome the difficulty of ERT not crossing the blood brain barrier. Dr Vijay presented data from the trail and reported that the treatment has been well tolerated and that participants have shown some stabilisation in both behaviour and sleep patterns. Full results are expected in 2018.

Finally we heard from Dr Maureen Clearly (in two separate presentations) about the use of intracerebral infusion therapy for MLD and MPSIII. Dr Clearly explained the mode of delivery - involving 6 reservoir devices placed in to the brains of affected individuals allowing 12 intracerebral injections of gene therapy. Although this sounded quite terrifying to those in the room, Dr Clearly reassured those listening that this is a commonly used mode of delivered for other treatments that neurologists are very familiar and comfortable with. Treatment on MPS III patients is due to start in 2018 following promising results in animal models; MLD studies are now in the human stage and the initial results are promising.



Conference C - Saturday 8 July 2017 and Sunday 9 July 2017

Thank you to the amazing presenters who ran the conferences focussing on Fabry on the Saturday and Sunday. Although this was a very full on programme with lots on information to take in we had some great feedback from the attendees who described it as a fantastic weekend, a professional, informative and interesting conference and a great opportunity to meet other Fabry patients.

In particular the speakers living with Fabry who presented on their experiences were very well received. Naomi Carter who spoke on life and living with Fabry was “fantastic” and Nicola Carnall, who shared what she would change after receiving a diagnosis in hindsight, was described as a “courageous lady with a very honest approach to Fabry’s”.

The Fabry conference also made great use of the sli.do polls, asking important questions and displaying the audience’s responses as a wordle where the size of the word represents the number of responses given. Understandably pain, and in particular, headaches, were a big issue for Fabry patients as shown below.



Gala Dinner & Recognising Excellence Awards

The 2017 Conference Gala dinner was not only an opportunity to come together and celebrate 35 years of the MPS Society it was also a chance to recognise excellence over adversity within our community.

It seemed fitting that the first surprise of the evening was reserved for our own Group Chief Executive, Christine Lavery who was presented with a book of memories of the last 35 years.

For the first of the Recognising Excellence over Adversity Awards it was impossible to separate the two top nominees so the Board agreed to jointly award two exceptional young ladies who have not only dedicated much of their life and time to supporting their sisters with MPS but serving the wider MPS Community by volunteering for the MPS Society and spreading awareness of MPS diseases.

Rhoswen McKnight (1) whose older sister, Sarah was diagnosed with MPSI Hurler at just 9 months old has been a devoted sister. Rhoswen came to the MPS conferences with her family from a very early age and for the last six years she has volunteered on the MPS childcare programme as well as travelling to Salvador in Brazil with a group of MPS young adults to support them to participate in the International MPS Symposium. Since then Rhoswen has become a cardiac intensive care nurse at the University Hospital of Wales.

Keshini Nonis (2) whose sister Roshani was diagnosed with MPSIIIB at the age of 8 years when Keshni was 14 years of age adapted to her little sister like a duck to water and found ways of managing Roshani's challenging behaviour. As a loving sister Keshni has always been able to see beyond Roshani's diagnosis and these skills have served Keshini well as a childcare volunteer for the MPS Society. In 2010 Keshini was chosen as a young ambassador to participate in the International Symposium in Adelaide, Australia.

The volunteer award went to **Helen Patterson (3)**, a very familiar face to many parents. Helen first started volunteering with the MPS Society in 1986 and has been a constant but certainly not quiet key member of the volunteering team! Helen's love for the MPS family community and her cheerful can-do attitude is a fine example for all other volunteers to aspire to.



The award for an adult living with MPS went to a young lady who has shown immense fortitude living with her MPS disease, whilst at the same time continuing with her further education and inspiring others to raise funds for the MPS Society and the wider community. **Aisha Seedat's (4)** incredible determination has allowed her to accomplish almost everything she has set her mind.

Katie Brown (5) was recognised as a parent whose child is living with MPS. Katie and her husband Simon became members of the MPS Society in 2010 following a diagnosis of MPSIVA for their eldest son, Sam. Katie became a very active member and led the 'Keep Sam Smiling' social media campaign to achieve access to Vimizim. Katie played a key role in the success of the campaign to secure access to Vimizim and continues to raise significant sums of money for the MPS Society.

The Recognising Excellence Award for a grandparent went to a truly inspirational grandmother who has never ceased to amaze. **Marina Foster (6)** was inspired to start fundraising in early 1996 after the diagnosis of her only grandchildren, twins, Francesca and Josephine Kembury with MPSIIIA. Since those early days Marina has raised a staggering £170,000 for Sanfilippo disease research. Marina's shop, known as Marina and Friends Fundraisers, is not only about fundraising it is a community gathering point. At 82 Marina continues to show tremendous fortitude and her

resolve to continue to raise money for Sanfilippo research goes undiminished. Brian Bigger present Marina with her Award and Marina presented Dr Brian Bigger with a cheque for £10,000 the recent proceeds of her shop.

We also recognised two professionals who have made an outstanding contribution to the MPS community. Dr **Brian Bigger (7)** has been at the forefront of research into MPS since 2006 when, funded by the MPS Society, Brian set up the MPS Stem Cell Group at the University of Manchester. Brian has also taken the development of Genistein Aglycone from the mouse model to a Phase III clinical trial in MPS III patients. He has also pioneered a stem cell gene therapy for MPS IIIA. This should enter clinical trial in the very near future. Brian was presented with a cheque for £5000 to be used at his discretion at the University of Manchester.

The final recognition of the evening went to Professor **Bryan Winchester (8)**. Bryan obtained a PhD in molecular enzymology in the Biochemistry Department at University College London after graduating from Cambridge University in Chemistry. For over 50 years Bryan has been dedicated to the field of Lysosomal Storage Diseases both in his professional career and since retiring. In 2006 Bryan was welcomed on to the board of Directors of the MPS Society where he continues to serve and lead on aspects of the Society's research grant programme.



Research & treatment



NHS England/NICE Consultation on Highly Specialised Therapies

I want to thank all our members who lobbied their Members of Parliament following the publication of NHS England/NICE's response to their public consultation. Your efforts were phenomenal and your concerns were certainly heard in the highest echelons of Government.

Many of you will have received through your MPs or directly a stock letter defending the introduction of Cost per QALYs for evaluating highly specialised therapies and arguing that where the new therapy is transformational the higher level of the Cost per QALY is extended to £300,000.

Currently it is suggested that enzyme replacement therapies for MPSI, MPSII, MPSIVA, MPSVI and Fabry if evaluated through the NICE process now would have a Cost per QALY in excess of £450,000 and would not be reimbursed under the latest rules. Whilst in the immediate future we do not see reimbursement of ERT for MPSI, MPSII, MPSVI and Fabry being at risk we have no idea what the Secretaries of State for Health going forward may decide to save money for the NHS.

In respect of MPSIVA the Managed Access Agreement for Vimizim is in place for a further 3 and a half years but then Vimizim will go back to NICE for appraisal and we can only presume

the reimbursement decision will be based on the consultation outcomes.

We did not want to lose the momentum in our Campaign during the General Election but there were considerable constraints on what charities can be seen to do during purdah and indeed for 8 weeks there were no MPs to lobby! Thank you

to everyone that raised the matter of funding on the doorstep or via telephone or email. This really helped and some new MPs seem to be taking an interest in access to highly specialised therapies for ultra-rare diseases.

Christine Lavery

Breaking News

The Association of British Pharmaceutical Industry (ABPI) applies for Judicial Review over new procedures that restrict access to cost-effective medicines for Ultra Rare Diseases

The UK drugs industry is going to court in an attempt to stop the country's taxpayer-funded National Health Service from imposing new limits on the price it will pay for medicines.

The highly unusual action comes amid fears that a crackdown introduced in April may prevent patients from securing cutting-edge medicines for the most serious diseases.

The Association of the British Pharmaceutical Industry confirmed to the Financial Times that it had applied for judicial review of a decision by NHS England and the National Institute for Health and Clinical Excellence (NICE), that medicines would no longer automatically be funded if they were set to cost the NHS more than £20m a year in any of their first three years of use. The Judicial Review also concerns drugs for very rare diseases, which often affect children as in future, they will face a cost per QALY bar of £300,000, in demonstrating that they will significantly extend patients' quality of life.

Gene Encoding for MPSI and MPSII

MPSI and MPSII are caused by different faulty mutations in the gene encoding. Sangamo is using its zinc finger nuclease (ZFN) genome editing technology designed as a single treatment strategy intended to provide stable, continuous production of the missing enzyme for the lifetime of the patient.

Sangamo is conducting a Phase I/II clinical trial evaluating SB-913 in 2017. This study is an in vivo genome editing treatment for MPSII (Hunter). Sangamo is also planning to conduct a Phase I/II study this year evaluating in vivo genome editing treatments SB-318 for MPSI

Sangamo's ZFN – mediated in vivo genome editing approach makes use of the endogenous albumin gene locus, a highly expressing and liver-specific site that can be edited with ZFNs to accept and express therapeutic genes. The approach is designed to enable the patient's liver to permanently produce circulating therapeutic levels of corrective protein. The ability to permanently integrate the therapeutic gene in a highly specific, targeted fashion significantly differentiates Sangamo's in vivo genome editing approach from conventional AAV cDNA gene therapy. Ultimately the target population for these programs will include paediatric patients, and it will be important in this population to be able to produce stable levels of therapeutic protein for the lifetime of the patient.

For more information search SB-913 (for MPSII) or SB-318 (for MPSI) at clinicaltrials.gov

An update on the BioMarin MPS IIIB clinical development programme

April 2017



BioMarin have received a number of enquiries regarding the status of their clinical development program in Sanfilippo Syndrome Type B (MPS IIIB). The program, which involves multiple centres around the world, remains open and is actively enrolling. The program is enrolling up to 30 children between the ages of 1 and 10 and consists of two studies, described below.

The first study is an observational study of children with Sanfilippo Type B and includes testing of cognitive and adaptive function. The observational study lasts for 48 weeks. These tests explore how the child thinks and acquires new information as well as how the child deals with daily living. There are also assessments of behaviour and quality of life. This study is intended to provide baseline information about how Sanfilippo Type B progresses in the absence of treatment. This baseline information can then be compared to disease progression information from BioMarin's subsequent treatment study (both for individual children and in aggregate).

The observational study is enrolling at sites in Australia, Colombia, Germany, Spain, Taiwan, Turkey, UK and USA.

First study information

The second study is a treatment study in which children with Sanfilippo Type B will receive an investigational enzyme replacement therapy, known as BMN 250. The enzyme is administered directly to the brain as an infusion via a surgically implanted port. This study will be run at centres which are also part of the observational study. To enroll in the second phase of the treatment study, a child must have completed the observational study as outlined above, or have completed the first part of the treatment study.

Second study information

Any questions or concerns you may have should be directed to your child's doctor, who remains the best source of information about the care of your child. For more information about active clinical studies, including Sanfilippo Type B, please visit www.clinicaltrials.gov

Vimizim in adults

According to a new study, the long-term use of Vimizim (elosulfase afa) in adults with MPSIVA Morquio is effective and provides those patients with increased strength and endurance to tackle their daily activities. The research by Hughes et al. indicates that while adults with Morquio may have more disabilities, as the disease has taken its toll on the body, the use of enzyme replacement therapy still provides great benefit to these patients.

Read more: www.bit.ly/OJRDmorquioarticle

Update from Abeona Therapeutics on the ongoing clinical trial, ABO-102, for MPSIIIA

I wanted to take the opportunity to share about Abeona Therapeutics and the recent updates from the ongoing clinical trial, ABO-102, for Mucopolysaccharidosis IIIA (MPSIIIA), occurring at Nationwide Children's Hospital in Columbus, OH.

First, let me start by sharing some background about Abeona.

Abeona Therapeutics is focused on developing therapies for rare, genetic diseases, particularly those impacting children. The company was created in 2013 through a close collaboration with a dozen Sanfilippo foundations from around the globe. All involved aimed to progress the promising gene therapy research in MPS IIIA and MPS IIIB being done at the time by Drs. Haiyan Fu and Douglas McCarty from Nationwide Children's Hospital in Columbus, Ohio. In May of 2015, the company merged with PlasmaTech Biopharmaceuticals, a publicly traded organization, also focused on treating rare diseases through a proprietary purification technology that selected therapeutic proteins from human blood plasma. The newly formed company chose to return to the Abeona Therapeutics name as it better reflected the commitment to rare or underserved diseases impacting kids. Abeona is the name of the Roman Goddess who watches over children as they start their journey from home; a fitting name for our organization and mission.

And now, about the Natural History Study and ABO-102 for MPS IIIA.

It was fortunate to have the involvement of 25 families who participated in the MPS III Natural History Study, also conducted at Nationwide Children's Hospital, where children were followed and evaluated for twelve months. This allowed the clinical team to not only understand what assessments or tests would be helpful and informative but also gave a way to compare the results for the potential gene therapy to the natural progression of Sanfilippo. In addition to those families who gave time and traveled to contribute to this important study, funding to support it was provided by The Children's Medical Research Foundation, The Sanfilippo Children's Research Foundation, and Ben's Dream.

ABO-102 is the name given to the potential gene therapy that involves delivery to an individual's cells by a vehicle called adeno-associated virus (AAV) containing the SGSH gene. The SGSH gene in children with MPS IIIA is not properly creating the enzyme that is so badly needed for break-down of the sugars building up in the kids' cells throughout the brain and body. The AAV was chosen to send the correct SGSH gene because it can be given by a single IV injection and make its way through across blood-brain barrier and to other areas of the body.

The initiation of the Phase 1/2 clinical study was at Nationwide Children's Hospital with the focus primarily on safety and then signs for results or efficacy. To date we have announced that 3 individuals have been enrolled at the low dose and one at the high dose. It was important to start with the low dose, as again, the priority is safety and less is thought to be safer than more. So far, this has been well tolerated by all four participants and we are starting to see encouraging early results though more time and participation are needed. Enrollment is expected to continue to a total of 6 to 9 at Nationwide, with another 6 to 9 each in Spain and Australia. Both the Spain and Australia sites are anticipated to begin enrollment yet this year.

Much of what is available for information beyond safety comes from the first two individuals in the low dose cohort or group through the first 6 months of participation. What is seen includes:

- Reduction of heparan sulfate in the CNS and urine
- Reduction of liver and spleen size•
- Early indication of stabilization of adaptive behavior as determined by the Vineland, a tool that has been used for 30 years to assess social and personal skills needed for everyday living•
- Stabilization in several areas or subdomains of the Mullen, a tool to determine early learning•

- Improved ability to complete the Leiter-R, a non-verbal assessment for intelligence

We remain inspired and amazed by the multiple decades of effort and time that the Sanfilippo families have put forth to bring the global community to this point. It is our commitment to continue to move these clinical trials forward by exploring and achieving accelerated or expedited regulatory review opportunities, continuing to collect and understand the important information or data from the clinical trials, and ultimately focus on the goal for swifter and more broad availability of an approved gene therapy.

As Vice President, Patient Advocacy, I am here to serve the impacted children, their families, Patient Advocacy Groups, and physicians by further informing and educating, translating the science to a more readily digestible language, by driving awareness for these diseases, and to advocate for the movement of therapies through clinical trial and into the hands of patient populations. I'll continue to share updates and information with the community when possible. Thank you to all who are interested in what we are doing, those who support our efforts and the greater Sanfilippo community!

Michelle Berg
VP, Patient Advocacy
Abeona Therapeutics

Migalastat has been recommended for use in Southern Ireland

Cognitive endpoints for therapy development for neuronopathic mucopolysaccharidoses: Results of a consensus procedure

The design and conduct of clinical studies to evaluate the effects of novel therapies on central nervous system manifestations in children with neuronopathic mucopolysaccharidoses is challenging. Owing to the rarity of these disorders, multinational studies are often needed to recruit enough patients to provide meaningful data and statistical power. This can make the consistent collection of reliable data across study sites difficult. To address these challenges, an International MPS Consensus Conference for Cognitive Endpoints was convened to discuss approaches for evaluating cognitive and adaptive function in patients with mucopolysaccharidoses. The goal was to develop a consensus on best practice for the design and conduct of clinical studies investigating novel therapies for these conditions, with particular focus on the most appropriate outcome measures for cognitive function and adaptive behavior. The outcomes from the consensus panel discussion are reported on line in Molecular Genetics and Metabolism.

www.sciencedirect.com/science/article/pii/S1096719217302111

The NCPE has issued a recommendation regarding the cost-effectiveness of Migalastat (Galafold®). Following NCPE assessment of the applicant's submission, Migalastat (Galafold®) is considered cost-effective for the treatment of patients with Fabry disease who have an amenable mutation. Migalastat is recommended for reimbursement subject to continuing availability of a patient access scheme (PAS).

In December 2016 Amicus Therapeutics submitted an economic dossier on the cost-effectiveness of Migalastat (Galafold®) for the treatment of Fabry disease in patients with an amenable mutation. The product obtained European marketing approval on the 26th May 2016. Migalastat acts as a pharmacological chaperone that is designed to selectively and reversibly bind with high affinity to the active sites of certain mutant forms of the lysosomal enzyme α -galactosidase A (α -Gal A), the genotypes which are referred to as amenable mutations. Migalastat binding stabilises these mutant forms of α -Gal A in the endoplasmic reticulum and facilitates their proper trafficking to lysosomes where dissociation of migalastat restores α -Gal A activity, leading to breakdown of globotriaosylceramide (GL3) and related substrates thereby preventing accumulation of same in the tissues.

Migalastat is licensed for the treatment of patients with a confirmed diagnosis of Fabry disease who have an amenable mutation. Approximately 30% of patients currently receiving enzyme replacement therapy (ERT) will be eligible for therapy and the clinical data suggests that migalastat has similar efficacy to ERT. The manufacturer has proposed a patient access scheme which satisfies the current cost-effectiveness thresholds for the HSE. In view of this the NCPE is in a position to recommend the reimbursement of migalastat for the treatment of patients with a confirmed diagnosis of Fabry disease who have an amenable mutation.

Protocol NGLU-CL01-T and NGLU-CL02: SBC-103 Program Decision

Alexion has made the difficult decision to prematurely terminate the SBC-103 Program and all studies evaluating SBC-103 effective immediately, in respect of MPSIIIB.

Alexion understand that this decision may be disappointing to the families of the clinical trial patients, however, it was reached after a review of the data from the NGLU-CL02 study.

The data from this study showed that:

- SBC-103 was safe and biologically active;
- Changes in cerebral spinal fluid heparan sulfate (CSF HS) were minimal at 24 weeks in Part C, and not maintained at 52 weeks Part C;
- There was also no clear evidence of a clinically relevant improvement in neurocognitive function.

The MPS Society is doing all it can to support its members affected by this decision and appreciate how difficult this must be for them. Whilst we are in touch individually with each of the member families we appreciate this may have an impact on the wider MPSIII community. If you wasn't to discuss this announcement further please do talk to your child's clinical expert or call the MPS Advocacy team.

Information & resources

Fabry International Network 5th Fabry Expert Meeting Athens, Greece

The FIN Board of Directors were delighted to welcome so many members to the Royal Olympic Hotel in Athens for the FIN Expert Meeting. Whilst the spectacular views from the roof top bar were not lost on any of us, the main focus of attention was on the cutting-edge programme of talks on all aspects of living with and treating Fabry disease. Feedback from the participants was very encouraging with the rating for the programme content scoring over 90% and overall meeting organisation scoring 100%.

In the two days immediately before the start of the FIN Expert Meeting the FIN Board of Directors had individual face to face business meetings with Shire, Sanofi Genzyme, Amicus as well as Protalix, Actelion and Freeline who joined us for the first time this year. Whilst most of our discussions were conducted under Confidentiality Disclosure Agreements (CDAs) FIN is delighted to have been able to connect with three companies relatively new to the Fabry scene and to understand their Fabry programmes. Once any information is in the public domain FIN will indeed share it with its members.

Toni Ellerton, FIN Coordinator

“Lots of innovative presentations and discussion on the heart and kidneys and outcomes from registries for Fabry patients”

The Gold Standards Framework (GSF) believe in giving the right person the right care, in the right place at the right time, every time. They have created five videos giving showing simple steps to take in order to help plan future care.

See the videos here:
vimeo.com/channels/acp5steps

Ellie's Haven holidays

Ellie's Haven are a charity providing holidays to families caring for children with long-term and life-limiting illnesses. Their goal is to help such families spend quality time together away from their normal surroundings, in a specially adapted property in the Looe Valley in Cornwall.

Their website states that their “special place for special children” has been designed to help families with disabled children have a stress-free holiday. It is in a peaceful, secluded location and is equipped with built-in hoists, an assisted bath and other facilities. They also offer a sensory studio, which is an Aladdin's cave of specialised equipment aimed at helping children with special needs develop a wide range of skills.

www.ellies-haven.org.uk

Fundraising



Kemsley Community Angling Preservation Society held a charity fishing match on Sunday 25th June in aid of the MPS society. A fantastic amount of £205 was raised by the match participants as well as through donations. This was then matched by the club itself raising a total amount of £410.

We were asked to attend the weighing at the end of the day with Lily to have her picture taken with the winners and other fishermen.

1st place was taken by Dan Parfitt with 27lbs worth of fish caught, 2nd place was taken by Peter Dormendy with 16lbs worth of fish caught, and in 3rd place was Lily's grandad, Denis Brogden, with 12lbs 8oz of fish caught.

Michelle and Scott



My twin sisters had a very special birthday in April and asked people to make donations to the MPS Society to celebrate their big 40.

The theme was 'around the world' (they are pictured wearing suitcases) and I'm their proud big sister in the middle.

Knowing how hard it is for our family (my husband, his mum and our daughter all have Fabry disease) this provided a wonderful way to support the MPS Society and in turn know, that we continue to be supported really well by the work that you do.

I have put £260 in the bank for you. They continue to raise money for you through their birthday and sponsorship for the Great North Run on a Just Giving Site which is nearly at £1000 now.

Thank you for all the work you do. I can't tell you the difference your support means to us as a family living with this condition.

Rachel Matthews x

Daniel's charity fete

Rash and Sandra Singh raised £1820.50 at Daniel's Charity Fete on 14th May held every year, with grand stalls, music and food.

21st with a difference

Christian Natrella's 21st birthday party raised £1523! Instead of presents he asked everyone to spare anything they could and donate to the MPS Society. It was a very special day for Christian and he wanted to hold this fundraiser in the memory of his dearest best friend Jack Stuart. Jack who had MPS, unfortunately passed away two years ago. Amanda, Christian's mum, told us that "Jack, like Christian was such an inspiring individual and he is forever missed and always in our memories".



Dancing in the street

Thanks as always to the wonderful support from the Towersey Morris side who organised a tour for their 50th anniversary. Gina and Sophie from the Society attended and had a marvellous time.

Twilight Runway Challenge



Hello everyone, my name is James and I am here to tell you all why I am fundraising for the MPS Society, and why the charity means so much to me and is so dear to my heart.

I have two very close friends called Oliver and Samuel, though I regard them more as family, of whom I have known my entire life and have shared many wonderful memories with. Oliver and Samuel are both extremely upbeat, cheerful and undeniably loveable boys who are never seen without a huge smile painted across their faces. They are both amazing in their own rights, are two of the most interesting characters you could ever meet and are completely unique in every way apart from sharing one major similarity - they both have Hunters Syndrome.

Now I am sure that some of you already know about Hunters Syndrome and what this condition entails, but just in case you are unaware of what this condition is like here is a description to give you a better insight: Hunters Syndrome, otherwise known as mucopolysaccharidosis II, is a life limiting MPS II condition. Hunters Syndrome affects many aspects of a person's life, and both Oliver and Samuel have suffered from this since 2002 when they were diagnosed. Hunters Syndrome affects the; liver, spleen, soft tissue, bones and the heart, and is caused by a missing enzyme within the body which helps with the natural growth and development of the body. The boys have had a combined total of over 30 surgeries since their diagnosis 15 years ago, and both require weekly infusions of a drug called elaprase which is infused through a porta cath and takes over 5 hours from start to finish, and is repeated every week for 52 weeks a year.

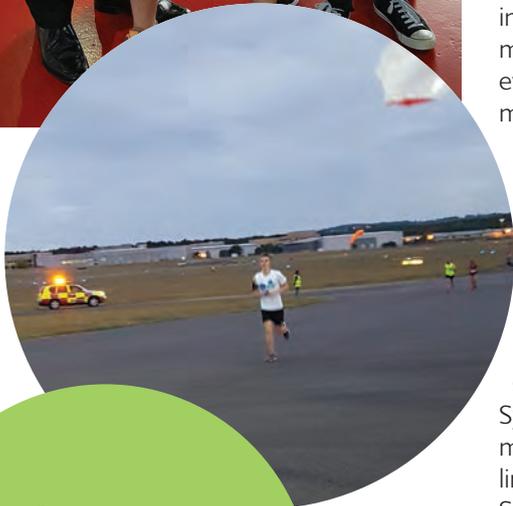
So far, like with many other MPS conditions, there is no definitive cure for Hunters Syndrome.

However, the MPS Society has been and is completely devoted to making a change to that, and is dedicated to giving my best friends a fighting chance at living happy and full lives despite the restrictions that Hunters can impose on those who suffer from this rare but destructive condition. For the past 17 years of my life I have been able to witness first-hand the positive impacts that the work of the MPS Society has had on both Oliver and Samuel, and have witnessed their lives developing in ways which, without the help and support of the MPS Society, would otherwise be impossible. Despite suffering from such a devastating condition, it is remarkable being able to see the boys so care free and joyous, and as their best friend nothing makes me happier than seeing them progress through life with the help of the MPS Society guiding and supporting them.

Like I have previously said, I have known the boys my entire life and have seen just how much of an impact that the MPS Society has had on their lives, and so would love to be able to give something back to the charity that are making a massive change to both their lives as well as to many others across the UK.

I am competing in the Twilight Runway Challenge on the first of July where I will be running 10K, the equivalent of two laps around the TAG Farnborough Airport runway and taxiway. This will be a struggle as I suffer from asthma which can inhibit me from running longer distances, but I am committed to completing this so that I can show my support for the MPS Society, and all the families that they help on a daily basis.

My ambition for my fundraising is to raise £250 to support this amazing charity, and I cannot wait to get my running shoes on and do my part in assisting the charity that ensures my best friends get the best treatments possible so that they can live their lives to the full!



James
raised
£330





Mark and Jamie Hughes dressed up as Mario and Luigi and had a stall at Arbury Carnival raising £280 for the Society. Mark brought the cheque to the conference where we got to witness his superb dance skills!



We loved this fundraising idea from Harriet on JustGiving.

As part of my Bronze Duke of Edinburgh award this year, I decided to do a challenging sponsored ride around my home area on my pony. It will take around 4-5 hours and I am hoping to cover the distance of a half-marathon (13.1 miles). The longest ride that I have previously done was approximately 4 miles.

I chose to raise money for MPS because it is a charity close to my heart. My cousins were diagnosed with Hunters Syndrome (one of a family of mucopolysaccharide diseases) over 20 years ago and the MPS Society has supported them ever since. My family also have a long history in supporting the charity which I would like to participate in and continue.

Harriet raised £235 in total.

Sarah Cutler (featured in Your Stories) organised a Wear it Wicked fundraiser for Josh raised



MPS AWARENESS DAY 2017

The Hampden Arms organised a pub quiz and raised £150 including the winner's money, which they donated back to the cause.

Simon Bradley donated £20.00 for awareness day



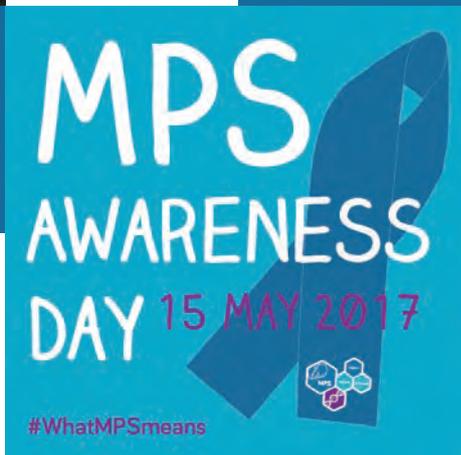
Porterhouse Medical Cake sale and Wear it Blue raised £50.00



Rupert Wright's mum, Naomi Watts, and his reception class at Gislingham Primary School raised £500.00 with a wear it blue day, raffle and cake sale.

Sally Mitcham held a bake sale and raised £26.15

Our first ever thunderclap was a huge success with a social reach of 62,415



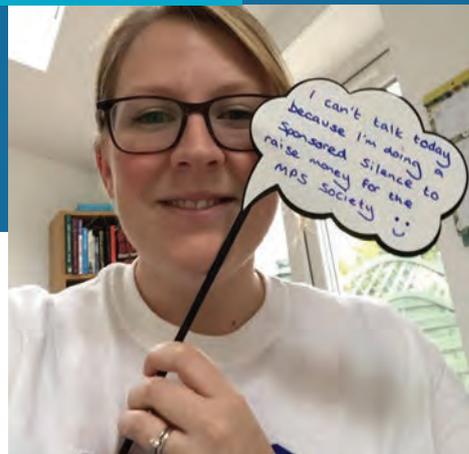
Safe Haven Day Nursery in Notts raised £48.28 through blue activities



Sarah Lock had a cake sale at work and raised £10.00



Rachael Jones held a sponsored Silence – thank you for the £884.90 raised



Lily Brooker and Meadowfield School had a Wear it Blue day raising £104.50

Pondhu Primary School Awareness Day in Cornwall raised £212.10



St Josephs Primary School Coffee Morning brought in £62.20

Kerry Siddall, aunt to Harley Bond, arranged a wear it blue event at her work at Bluestone Credit Management and raise £250 which was matched by the company.

Our traditional wear it blue photo. Highlight of the day was “blue music bingo” organised by Sophie D and the amazing cakes baked by Sophie T.



East Cliff Pre-School and Sam Watts raised £78.75

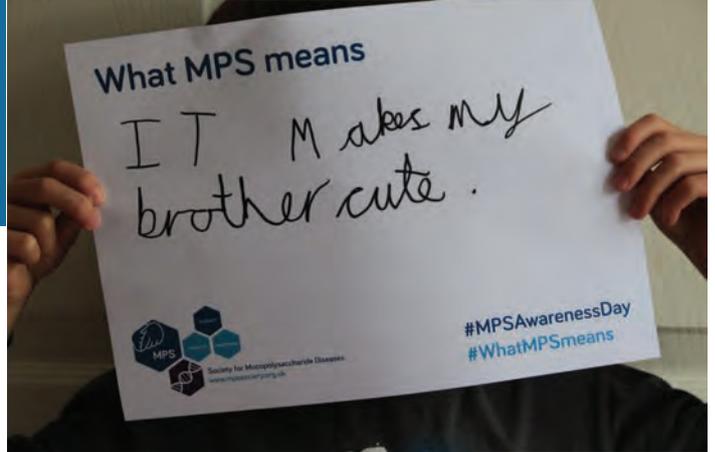


Amicus Therapeutics had a wear it blue day and bake sale raising £1188.70



Raymond Webber raised £200 at a car boot sale

Regenxbio had a gathering to commemorate the day as a company



Our neighbours at MPS House, Medical Billing & Collection, raised £73.95



We had fun at MPS House with blue nails, glitter tattoos and blue hair dye!

Clark & Partners wore blue and raised £855.00 to support Wayne Bond and family

Afternoon tea – Elizabeth Heath £515.00 raised to put towards research into breaking the blood brain barrier



From Abeona Therapeutics: We recognize your enormous collective efforts and are committed to moving these potential gene therapies forward to help realize success. We are motivated by you and the families who don't yet know they are a part of this community.

Here's to MPS Awareness Day 2017 and the 364 days until May 15th, 2018 and the advances made in between!

Spring Ball

We had two goals when we decided to organise a Spring Ball to raise money for the MPS Society. The first was simply to raise as much money (and awareness) as possible, and the second was to create an evening where everyone had a brilliant time. We think we achieved both!

From the start, we were completely humbled by the generosity of so many people. There was the kindness of those attending who knew how important it was and wanted to be there for us – and of course wanted to have a great night out too! We welcomed complete strangers who had heard about Sam's story during the Vimizim campaign who wanted to do something to help, alongside friends and family who've stood with us every step of the way. Looking around the room seeing all 190 guests that night was incredible. We are very, very lucky.

Then there was the unbelievable generosity of local and national companies who donated some amazing raffle and auction prizes – ranging from a meal in a Michelin starred restaurant, to camper van and open top bus hire. We had table centre pieces and chair covers donated for free, which made the room look absolutely stunning. Michael Sheen and The Brownlee Brothers donated signed sports shirts, a local framing company framed one of them for us – again for free. The list goes on and on but the theme is the same – people wanted to help and support, and for that we are so incredibly grateful.

The venue – the Crown Hotel in Harrogate, was brilliant, and all the guests looked fabulous in their best bib and tucker. Everyone commented on the quality of the food (especially the chocolate brownies) and nothing was too much trouble. Entertainment included a lucky Prosecco giveaway, a game of heads and tails, a disco, photo booth, and half way through the meal – a special surprise. Suddenly a waiter tripped and fell on the dance floor and everyone turned around in shock. But he was not a waiter – he was part

of a dance mob who treated us all to a fabulous dance routine, and really made the evening come alive. Dancing continued till 1am, with many of us ending the evening with sore feet and discarded shoes, and in the morning, a few sore heads over breakfast too!

Once we'd done all the adding up, we were amazed by how much money the ball had raised. Proceeds from the ball itself were £11,425.09. This was generously match funded by the wonderful Thea's Trust, who donated an unbelievable £10,000, bringing our grand total to £21,425.09. This was beyond our wildest dreams!

Organising the ball was very hard work, but it was great fun too, not least filling our bath the night before with 2,500 expanding bright blue aqua beads to fill the table centre pieces with, and sourcing an inflatable male bust from eBay to put Michael Sheen's shirt on!

Thank you so much to everyone who came, supported, bought raffle tickets, bid in the auction and donated. We really couldn't have done it without you.

Katie and Simon Brown



Thea's
TRUST

Woolaston primary and Gloucester troopers

Louise Penny let us know about their wear it blue day for Merlin which raised £894.16



The run was 5k round Glasgow green. There was a colour stop roughly every 1km where they either threw a colour bomb, sprayed colour on or painted you using a roller. Was really fun! I had a sore back so tried to jog a little but mostly hobbled, ha! Took me just over an hour to complete the course. At the end of the run they gave you a colour bomb and after a countdown everyone threw their colour bombs into the air for a colour explosion. It was a great atmosphere despite the miserable weather.

Stacey Loudon took part in a colour run and raised £162.50

“I thought if I did something extreme it would catch people’s attention”

Eleanor Taraborelli on shaving her hair for MPS



What made you want to pick this particular challenge?

I picked shaving my hair off because I thought if I did something extreme it would catch people’s attention and raise money plus raise awareness of my disability.

Why did you choose MPS Society to fundraise for?

I picked the MPS Society to fundraise for because the disabilities are so rare and I rarely hear people fundraise for the MPS Society, I have also always been told I’m an inspiration to people because I have such a positive personality so I thought I would actually like to do something inspiring rather than be an inspiration just because of my disability if that makes sense. I have always wanted to help people so I thought this would be a good way to make a difference.

How did you feel after it was completed?

I honestly felt like I was on cloud nine and immediately wanted to do it all over again. I didn’t feel nervous at all, my family were nervous enough for me. The support I had off friends and family was amazing, I went for a hospital check up and started talking to this lovely lady called Margery and I mentioned I was shaving my hair off for the MPS Society and she donated £10 – so even strangers were so supportive.

Would you ever do it again?

I would definitely do it again or maybe a different challenge in the future, it’s such a good feeling knowing this money could help a person or a family with an MPS disability.

What would you say to others thinking of taking on this themselves?

If others are going to do it, just do it because fundraising for a charity is the best thing you could do and it’s quite fun fundraising and you get amazing support off people. Just get the word out!

How much did you raise?

I have raised £2,100 and people are still donating on my JustGiving page.

Hair raisers

Jasmine Clayton had been growing her hair since she was three years old and now, after 10 years and waist length locks, she decided it was time to cut her hair in the name of charity.

She asked her best friend Katie Heath to nominate a worthy cause and as Katie's brother, Jack, who had MPSII Hunter Syndrome, died almost four years ago she suggested the MPS Society.

Jasmine's mum, Kathryn, told us "The plait went to the Little Princess Trust. Our hairdresser cut her hair for free and Kate came around for tea and to watch the proceedings so we had a little party."

Jasmine has raised £282 through sponsorship for the Society.

Katie also raised £300 with her mum by putting together an afternoon tea event.

Jasmine told her local paper: "I had only ever had my hair trimmed before at the hairdresser and only ever had the smallest amount taken off it.



"I was a bit shocked when I first had it cut"

"I was a bit shocked when I first had it cut and it was a bit scary, but now it is shorter it is easier to manage, however I can't do as many things with it as I could when it was longer.

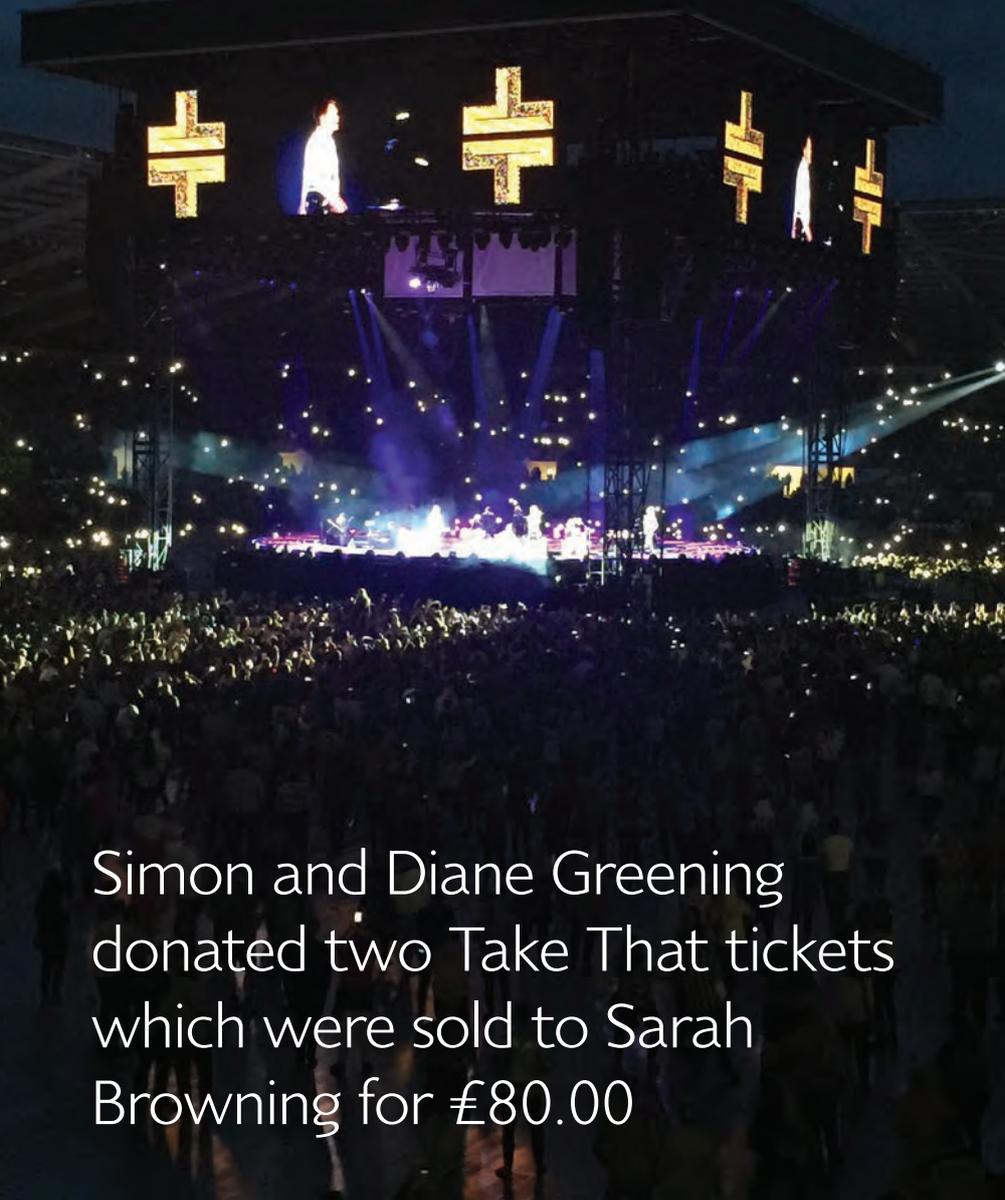
"When I went into school everyone said that they really liked it shorter, but I think I will grow it long again."



Mel Saint had her hair shaved for MPS on 24th February.

She raised over £500 in total!

"All for a truly amazing charity, in honour of a very handsome (and cuddly) boy, Corey"



Simon and Diane Greening donated two Take That tickets which were sold to Sarah Browning for £80.00



Just wanted to say we had an amazing time seeing Take That! It was a long way to travel but so worth it. Thank you to the people who donated the tickets, you made two youngish ladies very happy!

Glasgow Women's 10k

What a great day Sunday, the weather was fantastic for the woman's 10k. I did better than I thought I would have done – I completed the course in 1 hour 17 mins. I am so pleased with myself as I only did one week's training! I saw parts of Glasgow that I have never seen – Kelvingrove Park was a great place to start and finish the run. It was great to see so many woman running and walking for many charities. My niece, Nadine Murty, who suffers from Hurlers 1 was at the finishing line waiting for me it made me so proud. As you will see in the photos Nadine is a big Celtic fan as she had her football top on. We all can't believe how well she has been doing, she made her first holy Communion on the 6th May it was a happy time for all the family and also a sad time as Nadine's uncle had passed away the following day with cancer but he made it to her communion as that's what was keeping him going to see her on her special day. Nadine is now 9 and is full of beans she has a great social life going to all her clubs and her football club that she plays in. She meets up with Jordan Mount, he's 16 and also suffers from Hurlers 1, he loves Celtic too. He's the wee boy on the back of the t-shirt with Nadine.

Sandra Irvine – who raised £528 for the MPS Society



Harley Bond's family have been busy fundraising and raising awareness. Here's some of their photos and snippets as they have been keeping us updated on their journey

We have had a good run in raising funds already this month. Harley's school had a coffee morning and we had someone in the Sheffield marathon. Also my auntie is doing a virtual 5k run next month and we have had some good responses.



Fundraising this month has been fantastic too. My company held a wear it blue day and offered to match any donations collected on the day. We collected £426.71 so the company will be sending £855 your way plus they ordered £100 worth of ribbons from your store to hand out.

My sister in law did a wear it blue day where she works and raised £500. We are doing the virtual 5k run/walk all through May and we have over 90 people signed up at a fiver an entry. Also looks like I have sold my signed Def Leppard artwork for £200 so a good month really.



Flags are here and ready for Glastonbury.

We also made some new friends while there. A sister of a girl with Sanfilippo and a lovely family who are spreading awareness and hopefully fundraising soon. Also while I've been away the lads at work did a sponsored bike ride and have raised some money.



Countrywide Care Network had their fundraising event yesterday for the Society so hoping for a good amount from that.

Just off to promote MPS and the Society in our local paper. I will fill you in with the story later...



Making precious memories with our little boy as well as spreading awareness of his condition and the society that supports us

Great news! Sold our signed Def Leppard picture to a happy fan. The picture was donated by lead singer Joe Elliot to raise money for the Society – £200 coming your way as soon as I get 2 mins as we're glasto bound on Saturday to spread awareness!



Charity of the year with Oulsnam Lettings

This year we are fortunate to be the 'Charity of the Year' for Oulsnam Lettings. They have arranged a number of fundraising events throughout the year where MPS will be the beneficiary or shared beneficiary of any monies they raise. Examples of the types of events they have arranged are cake baking, marathon running, wolf walking and even holding sweepstakes for the Grand National and Wimbledon.

One of the more strenuous events that Laura Fiddes-Baron and Stephen Hill of the Oulsnam team recently undertook was the Lake District 24 Peaks Challenge in June. This challenge involved scaling 24 of England's largest mountains including Scafell Pike, Helvellyn and Fairfield to name but a few in 24 hours. The first day started in Buttermere and covered 10 peaks over

20 miles. The second day covered 15 miles and 14 mountains which started near Ambleside.

Branch manager Laura commented:

"Day 1 was a complete mental challenge as well as being crucifying for the knees. There was a very long hike between peaks 3 and 4 which was when the challenge became a bit of a mental game because you still had all the biggest peaks yet to do. The final peak was Bowfell which overlooks the Langdale region and from there we had to walk an exhausting 1.5 hour distance back to the car downhill on weary legs."

The challenge was far harder than Laura and Stephen anticipated and took a lot of grit and determination to get through. Most people who

complete the challenge use a professional guide but Laura and Stephen decided to do plan and navigate it all themselves to raise as much money as possible. They are extremely proud to have so far raised £800 to be shared between the MPS Society and Macmillan. Well done Oulsnam what a fantastic achievement!



“It is our most successful charity day to date”

Charity of the year with Country Wide Care Network



On the 13th June 2017 CCN hosted a 'Team Charity Driving Day' at Adventure Sport in Warwickshire. The event brought a record number of key industry players together from across the mobility and care sectors to participate in a number of highly competitive activities aimed at testing drivers' skills. Activities included an intense 4x4 course, blindfolded driving snooker and Segway riding! The event raised a total of £2,000 for the MPS Society.

'We are really happy with this, it is our most successful charity day to date, and hopefully it can go towards making a difference.' Cassie Barber-Jones CCN

We need to be your charity of the year!

'Charity of the Year' is the method many companies adopt to channel their fundraising efforts throughout the year to one or more charities. It forms a great opportunity for us to raise money and awareness. It also provides a chance to skill share and recruit volunteers.

HOW can we become the chosen charity....?

Many companies will turn to their employees for nominations and then hold a vote, the one with the most votes wins

Simple? NO!

Firstly the MPS Society need to be nominated!

Nominations often come from employees

So, who do you all work for? Who do your family members work for? Who do your friends work for?

Let me know this information and I will then be able to provide the background research, advice and support necessary in order to proceed with an application.

PLEASE CONTACT ME

a.minett@mpssociety.org.uk

Sporty fundraisers

Great Edinburgh



On Sunday 23 April 2017, I took part in the Simplyhealth Great Edinburgh 10 mile run to raise money for MPS Society. I live in Fife so this is a fairly local race for me and it was too good an opportunity to miss when I noticed that MPS Society had some charity places available for this event.

It is always good to be in Edinburgh, especially on such a nice sunny spring morning. There is always a lively atmosphere in the city, particularly when an event like this is being held. Crowds lined the streets to cheer on the runners and there was loud music at various points which helped create a lively uplifting atmosphere and enthuse weary runners.

The route was nice and scenic with a few tough climbs. Starting and finishing in front of Arthur's Seat, the route took you through/past some well known landmarks such as The Scottish Parliament, The Royal Mile, The Mound, The Meadows, Greyfriar's Bobby, Edinburgh Castle, Grassmarket and Duddingston Loch.

Here is a photo of me taken before the start of the race with Arthur's Seat in the background. I can tell you that I didn't look as fresh/energetic at the end of the race!

I finished in 1hr 23 mins and managed to raise a total of £250.00 - Lee Cessford



Great Manchester 10K

I decided to enter the Manchester 10k for the MPS Society because my sister Emma suffers from Sanfilippo syndrome (MPSIII A). Emma who is 23 now and in the final stage (stage 3) of the condition is unable to do anything for herself and relies on 24 hour care. I am also a carrier of the condition.

The MPS Society's advocacy team have helped my mum and my sister over the years and I felt that it was time that I did something to give back to them for the support that they have given us. So I thought if I could help raise some money to support this fantastic charity that would just be amazing and I thought running the great Manchester 10k would be good way to do it. I have currently raised over £400 but I am still collecting donations so the total is still rising. Thank you so much to the MPS Society for helping me to enter the race so I could support your wonderful charity - Ashlea Marie

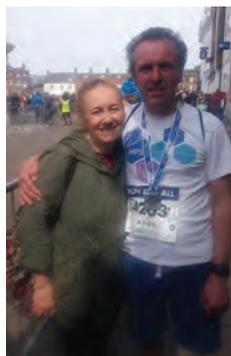
Great Manchester Half



The run went really well, It was a very emotional day with the tragic events in Manchester. We finished in 2 hours 26 minutes it was quite a humid day we only took one photo on the start line! - Deborah Burniston

Grand East Anglia

I had a fantastic day. The weather was brilliant, apart from a bit windy. I was determined to improve on last year's time of 54 minutes 30 seconds, by getting under 54. I'm glad to say I did 53:19 and my wife, daughters and 3 of our grandchildren were there to see me finish - Mick Yates



Asda Leeds 10K

Very happy with my time of 1:11:21 to say it was the first 10k I have ever done (especially in the heat), and it was all definitely worth it for the money I raised - Emily Moody

Orchard Therapeutics ran Tough Mudder

On Saturday May 6th, the Orchard team ran the full 12 miles Tough Mudder that took place West London (Henley on Thames), "Probably the Toughest Event on the planet" as the organisers like to call it. After pledging to the Tough Mudder oath "I understand that Tough Mudder is not a race but a challenge. I put teamwork and camaraderie before my course time" our Orchard team started the run, electrified by the great atmosphere and our goal: raising awareness on MPS diseases and supporting the MPS Society. From crawling through the Kiss of Mud to running the hilly Killa Gorilla, making it through the Mud Mile, surviving Artic Enema 3.0 the rebirth and jumping head first in the Block Ness Monster, the team worked together to vanquish the Hero Walls and the Pyramid Scheme, holding breath in the Augustus Loop before finally making it to the end after a good dose of Electroshock Therapy 2.0.

"It was a lot of fun ... but really tough", everyone felt happy and relieved to make it to the finish line! We were also very proud of being able to raise more than £2,000 for the MPS Society helping the association to raise awareness on MPS diseases and support families. A day everyone will remember!"

Orchard Therapeutics is a clinical-stage biotechnology company dedicated to bringing transformative ex-vivo gene therapies to patients with serious and life-threatening orphan diseases.



From left to right (standing): John Lavarino, Laetitia Schwab, Denise Carbonaro-Sarracino, Sylvie Blanchier, Adrien Lemoine, Charles Skinner, Tammy



Virgin Sport London 10K

I only had a few pictures with the MPS t-shirt on with the heat, it was too hot to wear while running on the weekend. I am doing another event in September to continue to raise more funds, so hopefully it will be a good sum by the end. I will be doing Richmond Runfest half marathon in September, so I will continue raising funds through the online page and hopefully get a nice big total. Thank you for the opportunity – Paula Sheridan

Plusnet Yorkshire Half



The half marathon went great, It was my first one and I managed to finish in 2hrs 27 minutes. I really wanted to do it to raise money for MPS for my friend's son who was diagnosed last year. It was such a great atmosphere with lots of people lining the streets to cheer the runners on. I managed to raise just over £200 – Joy Arnott who ran with her sister Alison

Bath Half

It was such a great atmosphere (the crowd really keep you going!) and it's inspiring to see so many people representing charities loved ones. Also, I would recommend a half marathon as an achievable goal for most people regardless of age...it's far enough to be a push but not so far you need weeks to recover! Finish time 2:35.24 - Annette Hales-Owen

Andy, Suzy, Jeff & Friends also took part in running the Bath Half Marathon this March. In 2013 many members of the family were diagnosed with a rare degenerative genetic condition called Fabry disease. It was great to hear they would be taking part in raising for the Society with a target of £2500 which was achieved and over





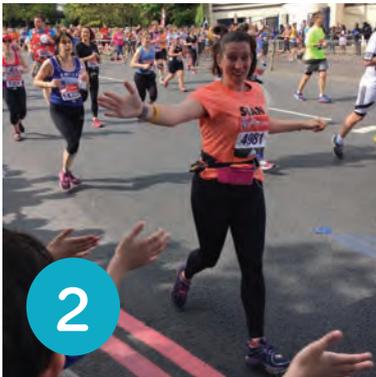
1

Daniel Goodge
Time:
4hr 27mins
Amount raised:
£1,300.00

Scott Bennett
Time:
4hr 34mins
Amount raised:
£253.25

London Marathon 2017

Amy Wright
Time:
5hr 27mins
Amount raised:
£1,730.00



2

1



Jemma Sibson

Time: 5hr 42mins
Amount raised: £2,126.28

For anyone yet to take on a marathon I'd say the training isn't something to be taken lightly, but the rewards are unbelievable.

I'm so proud to have raised all the money I have for the MPSI and equally as important I've increased awareness. I was out running in my charity vest one day and was stopped

by a lady who's daughter lives with MPSI. She was overwhelmed to see me supporting this cause and it really did give me the boost I needed.

I'm very proud of my achievements in all respects and I'll look back fondly at this experience.

2

Sian Young

Time: 5hr 3mins
Amount raised:
£2,399.50

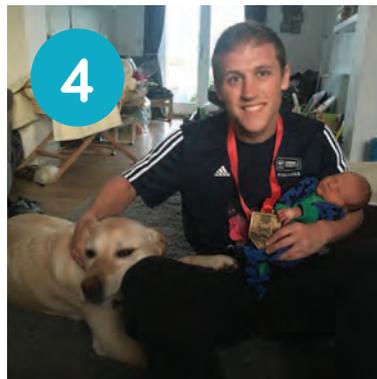
It was absolutely amazing - the support along the route was fantastic and my friends and family were a life saver at some points. I finished in 5hrs 3mins - was aiming for five hours and am so pleased with the time, I really couldn't have pushed myself any further.





James and
Nicholas Ball
Amount raised:
£1,008.81

Gary Phillips
Amount raised:
£1,857.94



3 Lizzy Farwell
Time: 6 hr 26mins
Amount raised: £2,800.51

The whole marathon experience has just been one of the best things I have ever done. From the training, fundraising, hours of dedication to race day. The crowds were AMAZING! They cheered you on and shouted your name when you needed it the most. My mantra throughout was don't set off too fast, stick to the pace I was comfortable going, keep clear in my head why I was doing this and most importantly just to enjoy it and I most certainly did. It was never about a time or a pb for me, all I wanted to do was honour the memory of my Brother and Sister, raise as much money as possible for MPS and finish! I would happily do it all over again.



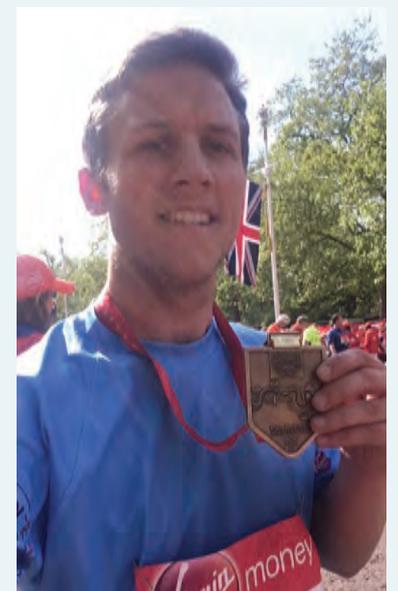
4

Ricky Brown

Time: 5hrs 16mins
Amount raised: £1,988.40

I did it, I actually completed the 26.2 mile course of London. What an awesome day, with a great atmosphere. Also, I have achieved at the minute, well above the target I had planned to raise for MPS Society and am immensely proud of everyone who has helped support such a great cause.

I want to thank the charity (plus other staff) for all the support you have offered and given throughout this whole regime. Without your hard work the charity would be completely lost and you are and the staff are a credit to the team. I'm grateful for the lifetime opportunity you have given me, to not only help the charity but also to achieve something that I thought I could never do. Sunday will definitely be a day to remember, or should I say the week. We had the swift arrival of my newborn son on Wednesday and with over 10 days spent in hospital with hardly any sleep I still managed to complete the marathon. Proud moment.





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

£260 plus £62.20 in gift aid was raised at St Joseph's Catholic Primary School in Sheffield through coffee mornings.

Mark Bayliss held an Easter egg raffle in his local pub and raised £158.

Tim and Sally Summerton sent in £4.66 from their collection box.

Karen and Tony Holland raised £125 in a tombola.

Robert McBride donated £431.22.

Hannah Watts raised £33 on her Etsy online shop selling handmade glass jewellery.

KM Charity Team sent us a 70% share of the sponsorship monies raised at 2016 KM Colour Run which was £302.75.

A further £250 was sent in from offline donations for Andy Battle and friends who ran the Bath Half in March.

Amelia and Lily, Guides at Huddersfield Golcar, fundraised for the Baden Powell Challenge award (highest award within Guides). A cousin of one of the girls has MPS and decided that it would be a good idea to let all the Guides know about MPS. They raised £89.51.

Grace Lodge care home raised £170 through funding events in memory of Helen Skidmore.

Marina & Friends Fundraisers in Bristol raised a further £3,335.53 selling preloved items at this unique charity shop. This makes their total raised so far £173,356.49. Marina received an award at this year's conference for her outstanding achievements.

Joyce Parkes made three cakes and sold them at middle Temple Society where her husband works. 67.88

Charles Read who has Fabry set up an ongoing book sale at work and raised £128.50 so far.

Megan Rennoldson held a bake sale at her local community centre and raised £175.

Weston Way Nursery School donated £70.60.

Sarah Schachhuber, a relative of the Bond family, raised £189.53 from collection boxes.

Elizabeth Mee raised £115 in her coffee morning and donated £65 to the MPS Society and £50 to emergency services.

Laura Fox raised £128 at her work's charity day in Bolton.

Monkton Pre Prep held a colour run in Bath and raised just over £260.



Are you or any family and friends starting University or at University?

RAG stands for "Raise & Give" and has become the focal point of student fundraising in the UK. Most Universities have their own RAG societies which work around the year to raise money for causes in need – some with incredible success. For example Brunel students last year raised over £100,000 for their chosen charities.

Students see RAG as their way of making a difference. It's could be a great opportunity to raise money and awareness for the MPS Society. Due to the rarity of these MPS diseases, as a charity we do not benefit from the widespread public awareness which larger charities enjoy. **MPS Society need to be nominated!**

So are you going to University? Are any of your family at Uni? Are any of your friends at Uni? Let me know where and I will provide the support necessary in order to proceed with an application to be a chosen charity .

Please contact [Amanda](mailto:a.minett@mpssociety.org.uk) at a.minett@mpssociety.org.uk

Donations

The Martin Connell Charitable Trust; Mary D M Andrew Charitable Trust; Jennifer Turner; Simon Down; The JTH Charitable Trust; Eva Annelie Hollstenius; Andrew Hackett; Nadia Browning; Robert Boyd; Mrs V Dawson; Richard Newson; Janet Hillier, Joanne Christie, Michelle Sharpe, Norma McMullan, Maggie Scott, Heather McAuley, Diane Wilmont; Lisa Gallyer; Simon Bradley; Bonita Tapp; Sara Elg; Ray Franklin; Mrs J Robertson; Janet Crosland; the Wixamtree Trust; Baron Davenport's Charity; Mr R L Speare; Patrick and Gillian Kimber; Ron and Pam Stevens; Miss M E Swinton Paterson's Charitable Trust; Pharmaco Foundation; Kim Forsyth; Mrs A Baker; Judith Tayler, Kieran Shevlin; Geraldine Linley; Robert Stephens; Christian Torres; Annette Brown; Kenneth Daniels; Chris Williams; Vicky Dowsett; Aileen Gibson; David Welsh; Anna Parkinson; Naomi Scale; John Silcock; Robert Potter; Chris and Daphne Allen; Kath Val and Paul

Golder; Newington Court Care Home; The Happy Wanderers; Mr and Mrs Brown; Mr and Mrs B Wells; Mr and Mrs A Wells; Mr and Mrs R Wells; Mrs L Lineham; Mr and Mrs T Gailbraithe; Gina Gayle Keith; Mr M Muldown; Mrs E Sweet; Mr and Mrs P Carey; Mr and Mrs T Holdcroft; Mr and Mrs Putick; Mrs J Short; Mr and Mrs J Bony; Mr and Mrs H Nupe; John and Meriel Barry; Mrs A Baker; Selva Selvaranjan; Brian and Mary Chenapa

Regular contributions by Standing Order or Give As You Earn

P J Martin; Norman Saville; M Newell; J Winzar; D Winzar; K & J Hudson; E Lee; R & K Dunn; M Tosland; C Cullen; S Bhachu; S Brown; L Brown; V Lucas; D Forbes; G Simpson; Williams Cavanagh; Barbara Harriss, L Brodie; A Sabin; A Ephraim, J Dalligan, M Malcolm, E Mee; Elliot Moody, S & D Greening; M Hahner; K Brown; Z Gull; Sarah Winzar; M Fullalove; E Brock; Margaret Leask; G Reeves; E Parkinson; G Ferrier; R Taylor; R Gregory; L Stillwell; R & K Henshell;

K & S Bown; S & J Home; V Little; J & V Hastings; K Seeber; D Forbes; G Simpson; A Sullivan; A Byrne; A Weston; E White; C Hume; J Casey; J & V Hastings; A & N Bansal; C & M Gibbs; Mr & Mrs Cock; A Dickerson; M Kalsi; P Summerton; John Scott; A Weston;

Donations via collection boxes, stamps, foreign coins, mobile phones, ink cartridges, jewellery, PayPal Giving, eBay for charities

Ellen Nicholson, Donna Bown, Marie Thomas, Mrs M Brock

In memory

Shujah Altaf, Paul Franklin, Sophie Summerton, Dean Lewis, Luke Bown, Katherine and Thomas Farwell, George Dennis Stevens, William Todd, Robert Henry Palmer, Yvonne Halls, Luke Bown, Molly Alice Rigby, Helen Skidmore; Luke Bown; Mary Agnes Moulding; Watty Kelly; Bob Silcock; Lisa Nurse; Shivahram Selvaranjan

Charity Currency Exchange

If you've ever been abroad for your holidays you'll know only too well that there's always foreign coins, and sometimes notes, that you're left with and can't exchange. Well now you can put them to good use and help raise vital funds for the MPS Society.

We work with Recycling for Good Causes, who can turn unwanted currency (even old, out of circulation currencies from the UK and other countries) into funds for us.

And it's not just currency we can recycle...

- Jewellery (any material, broken or wearable)
- Watches (broken or working)
- Used stamps (UK and foreign)
- Gadgets (mobile phones, cameras, sat navs, game consoles, laptops/tablets, MP3 players etc.)

Starting your own recycling project for the MPS Society couldn't be easier. It's completely free and requires no long-term commitment.

Simply contact Recycling for Good Causes for your free recycling sack now:

- info@recyclingforgoodcauses.org
- 0800 633 5323

You'll be sent everything you need and it won't cost you a penny.

Once your sack is full, call the free phone number and collection will be arranged free of charge at your convenience.

How simple is that?

Thank you to those who donated via the Weather Lottery:

Mrs T Brown
Mr A Selwood
Mrs G Plummer
Mrs J Speed
Mrs C Lavery
Mrs J Edwards
Mrs O Megoran
Mrs D Bown
Ms C Halleron
Miss L Lorimer
Mrs M Crespin
Mr A Dickerson
Mr M Hughes
Miss D Halleron

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



Patient Access to Clinical Trials and Treatment

Patient Access to Clinical Trials and Treatment (PACT) registered as MPS Commercial

Registered Address:

MPS House, Repton Place, White Lion Road, Amersham, Bucks, HP7 9LP, UK

mps@mpspact.com

www.mpsociety.org.uk/commercial

T: 0345 389 9901

F: 0345 389 9902

Board of Directors

Chair

Bob Stevens

Board

Derek Beards

Jessica Kafizas

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.

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Sam Wiseman – Clinical Project Administrator
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Sophie Henry – Clinical Trial and Finance Administrator
s.henry@mpspact.com

European MPS III survey update

We've had a great response to our call for volunteers to complete the MPS III survey so far. Thank you again to all those who have given their time to complete it with us.

Our colleagues in the European MPS Societies are just starting to interview their MPS III families and we will be meeting with them in July to share the initial results of the survey and plan the next stages of the project.

We still need around 30 more families to take part, so if you haven't had the chance to reply to our invitation yet please do get in contact with us and send in your consent form.

Email Alex at a.morrison@mpspact.com for more information

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 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 is Clinical Data Lead. She coordinates the collection, management and analysis of data from research surveys and the Vimizim Managed Access Programme.



Jackie is Clinical Communications Lead. She coordinates drafting internal and external reports, research surveys and medical/clinical communications.



Pauline is the MPS Commercial accounts assistant.



New to MPS Commercial



Sam Wiseman

I joined MPS Commercial in March 2017 as a Clinical Project Administrator. I work closely alongside Alex and Jackie working on the MAA for Vimizin and now more recently I have been involved with the European MPS III Survey.

Prior to joining MPS Commercial, I worked within the Clinical Research Industry for 9 years, working on Clinical trials for various diseases. I started within the Data Management, collecting and reviewing clinical data and then moved to Drug Safety becoming a Project Manager and reporting Serious Adverse Events to the appropriate worldwide authorities.

In 2015 I had my little girl and I decided change my career path. Joining MPS Commercial has been really interesting for me. By talking to sufferers and their families affected by the disease I am learning more and more about MPS and associated diseases every day.

As a mummy, I do not have much spare time, but when I do, I like to spend it with my husband and my little girl going for days out and about.

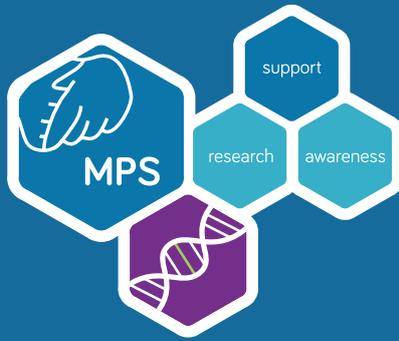


Sophie Henry

I joined MPS Commercial in May as a Clinical Trial and Finance Administrator. I will be assisting the Commercial team with the processing of patient expenses, invoicing and other financial tasks associated with supporting patients to attend on-going clinical trials across the world. In my short time in the role I am already amazed by the wonderful work of the MPS Society and MPS Commercial and I look forward to learning more over the coming months.

I have a research background in Developmental Neuroscience which led to a move into research administration at King's College London university. I have a young family and have spent the past couple of years at home raising my two children. Outside of work I have a passion for travel, enjoy playing tennis and love to attend major sporting events such as Wimbledon, Silverstone and following Arsenal FC.

You can now reach MPS Commercial on their
own dedicated number: 0345 2601087



7-9 July 2017
Coventry, UK



Celebrating 35 years
of the MPS Society



Thank you for
making the
conference
so special



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