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Society for Mucopolysaccharide Diseases

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The MPS Society

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

Our Aims:

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

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

To bring about more **public awareness**
of MPS and Related Diseases

MPS and Related Diseases

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

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

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



Will you be
part of our
Wicked Walkabout?



We've seen a fantastic response
since we first launched our
Wicked Walkabout – people from
Derry to Dorset, Bucks to Bridport,
have donned their walking shoes
in the name of fundraising and
awareness for the MPS Society.

We would love to see more
people wending their Wicked
way across the UK, so if you would
like to hold a Wicked Walkabout
please drop us an email at
fundraising@mppsociety.org.uk
and we can send you out a Wicked
Walkabout Guide. Get your friends,
family and even your pet dog
involved and get walking, running
or cycling!

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

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

www.mppsociety.org.uk

Front cover photo:
'Wear it Blue' MPS Awareness day at Beauchamp

Welcome!

It's been a busy few months here at the MPS Society: the 15th of May heralded International MPS Awareness Day and our supporters did us proud by Wearing It Blue and undertaking all sorts of events in a bid to spread the word and raise funds. The 'Fundraising' section of our magazine highlights what some of our wonderful supporters did to mark the day, as well as articles from some of our 2014 Virgin London Marathon runners.



Christine's Chief Executive's report highlights various clinical trials, including an update on the Genistein trial. She also touches on Vincent Willink's incredible fundraising challenge – a full article of which can be found in our 'Fundraising' section.

As per usual, Advocacy are on hand to offer lots of advice, and we have an update on regional clinics and our All-Ireland Advocacy Service.

Anna Eaton writes about her experience when her son, Isaac, was fitted with a 'halo'. The article covers how Isaac coped with the operation, and the practicalities of living with the 'halo'.



We remembered Professor Ed Wraith at his memorial event (p7), and we celebrated the new marketing approval for Vimizin at the Palace of Westminster (p16), both emotionally charged events that we were proud to be part of.

Don't miss the latest news in our 'Research & Treatments' section, starting on p14.

The MPS Society team

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Chief Executive's Report



Christine Lavery, MPS Chief Executive

Summer 2014

The last few months have been one of frenetic activity for the MPS Society in pursuance of its aims and objectives. We have welcomed the European Medicines Agency awarding market approval for Elosulfase (Vimizin), the enzyme replacement therapy for MPS IVA with a reception at the House of Commons on 2nd June 2014 'Celebrating a better future for Morquio disease' hosted by Mark Pawsey MP.

We welcome the new clinical trial studies for MPS II, MPS IIIA and MPS IIIB and celebrate the end of the first year on study for three UK MPS I patients who received a haematopoietic stem cell transplant in their first two years of life and are now trialling the enzyme replacement therapy Aldurazyme.

Whilst recruitment for the MPS III Genistein clinical trial is nearly underway we have suffered a potentially detrimental set back.

The UK MPS Society is committed to raising £650,000 for the study and until the 6th May 2014 with the commitment of a grant of \$250,000 (£147,000) directly to Dr Brian Bigger by the American National MPS Society, the UK MPS Society had only £100,000 to raise.

The American National MPS Society made a payment of \$125,000 in May 2013 to Dr Bigger. Just as the final payment was due, Dr Bigger, the UK MPS Society and the MPS International Network received an email from the American National MPS Society saying they would not be making a second grant payment of \$125,000 (£73,600). A request to reconsider was rejected outright.

As I write, Dr Brian Bigger, Dr Simon Jones and members of my Board of Directors are in discussions on how we go forward. After the amazing efforts of our MPS III families to raise funds for this study and the support in small grants we have received from MPS Societies around the world, it may sadly just

be, that the number of patients recruited to this study will have to be decreased.

We have received feedback from some families who purchase synthetic Genistein from the internet that supplies appear to be dwindling. This is not a result of the trial however, it is not surprising that companies are unwilling to make further supplies until a clinical trial has proven efficacy and benefit and the drug is on a firmer regulatory footing.

On a more positive note I want to thank all our members who 'Wore It Blue' on MPS Awareness Day and raised funds for MPS. In the MPS Society every pound raised is highly valued as are donations of every size and these are acknowledged in the MPS Magazines. I must however thank Vincent Willink whose two sons have MPS II and his eight colleagues from London and the Netherlands who navigated themselves to Carlisle and cycled coast to coast along Hadrian's Wall towards Newcastle upon Tyne raising £50,000 for Hadrian for Hunter. It was an amazing sight for John and Barbara Arrowsmith and myself as we greeted the riders in the wet and cold at Tynemouth Castle. Whatever the level of your fundraising or donations, we say a huge thank you. Without you the Society could not continue to deliver professional support, advocacy, family events or research.

Christine Lavery MBE
Chief Executive
c.lavery@mpssociety.org.uk



Can you be part
of our
Wicked Walkabouts
and organise one
in your local area?

Check out
www.mpssociety.org.uk

News from the Board of Trustees

The Society's Trustees meet regularly. Here is a summary of the main matters that were discussed and agreed at the Trustee Board Meeting held on 24-25 January 2014.

Governance

The minutes of the Board of Trustee meeting were agreed as true and accurate notes. The minutes of the Finance Sub-Group meeting, Board of Trustee and company meetings from June 2013 were also signed off.

The Strategic Plan for 2014 was agreed and signed off.

The Business Continuity Plan is to be reviewed with Senior Management and brought back to the next meeting.

Risk Management / Health and Safety

The Chief Executive advised Trustees that no changes were necessary to the Risk Register at this time, but the Register would continue to be reviewed at every Trustee meeting.

Personnel

Trustees were advised that the MPS staff team continues to work well together.

Treasurer's Report

Trustees agreed that it was vital they are aware of and committed to understanding MPS financial management. A training session for Trustees is to take place prior to the next Trustee meeting.

The Treasurer presented her report.

Advocacy

Trustees were updated on the current caseload of the MPS advocacy team including a number of complex cases requiring considerable time and support.

Clinical Management

The Chief Executive advised Trustees that she had been alerted by NHS England that the whole Specialised Services drug budget is already 3 Million pounds overdrawn at the end of December 2013 and that this has the potential to cause problems for the Highly Specialised LSD service.

A discussion took place around access to carrier testing for young adult siblings of MPS brothers and sisters. The Chief Executive advised that the Society receive two requests a month in regard to support for carrier testing. In MPS II particularly it was seen as nonsense that obstacles are put in the way of female siblings securing carrier testing. The average cost of a carrier test is in the hundreds of pounds and ERT for an affected child is potentially £250,000 per year. It was agreed the Chief Executive to write an article for the MPS Magazine.

The Senior Advocacy Officer appraised Trustees on the current situation with Homecare and the development of the patient homecare charter.

Clinical Trials

The Chief Executive advised Trustees that all Clinical Trial and HTA studies have moved across to MPS Commercial.

Visit our online shop at www.mpssociety.org.uk.

Purchase our information resources and MPS merchandise including our **T-shirt!**



'Like' and 'Follow' to Raise Awareness

With Twitter's users reaching the 175 million mark, and Facebook climbing to a staggering one billion, it is undeniable that social media plays a huge role in modern communication. These platforms offer us a fantastic opportunity to raise awareness of Mucopolysaccharide and related diseases, as well as allowing us to keep all our supporters up to date with the work of the Society. With your help we can spread the word, so please support us with your 'likes', 'shares' and 're-tweets'!

 Find us on
Facebook
www.facebook.com/mpssociety


twitter.com/MPSSocietyUK

What's on 2014

CONFERENCES & REGIONAL EVENTS

Cadbury World Family Day
2 August

**13th International Symposium
on MPS Diseases, Brazil**
13 -17 August

Chessington Family Weekend
15-17 August

Kent MPS Family Day, Howletts Animal Park
30 August 2014

All Ireland MPS Conference
12-14 September

Childhood Wood Planting
26 October 2014

MPS REGIONAL CLINICS

MPS I - GOSH: 22 July

MPS III - GOSH: 8 July

MPS I Post HSCT (over 6's) - RMCH:
3 October

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

MPS Clinic - Birmingham:
14 November

Fabry Clinic - Birmingham:
24 October

Saturday 2nd August 2014

Join the Society for a fun-packed day
at **Cadbury World**.

Only £10.00 for a family of four.

For more information please phone
the MPS Office on 0845 389 9901.

Limited places available



SAVE THE DATE

BAHIA | BRAZIL | 13 - 17 AUGUST 2014

13TH INTERNATIONAL SYMPOSIUM ON MUCOPOLYSACCHARIDOSES AND RELATED DISEASES

LOOKING FORWARD TO SEEING YOU IN BRAZIL!

mps2014.com

For the first time in South America, the 13th International Symposium on Mucopolysaccharidoses and Related Diseases will take place in Brazil, in one of the most beautiful resorts in Bahia, Costa do Sauípe.

This meeting will be a unique opportunity to congregate families and professionals, aiming to share experiences and new information on the MPS disorders.

Your participation is very important. Save the date and spread the word!

Organized by

MAIN SPONSORS: **BiOMARIN** **genzyme** **Shire**

Genética Para Todos | ABRAMPS Associação Brasileira de Retardados Mentais

Remembering Professor Ed Wraith

On 5th April 2014 professionals and families were invited from around the world to join in a celebration of Professor Ed Wraith's tireless and selfless work over three decades to improve the diagnosis, understanding and treatment of lysosomal diseases.

The memorial meeting was held at The Point, a state-of-the-art venue, which boasts views of Old Trafford Cricket Pitch. Within this lovely setting we celebrated Ed's life and the work he did.

The people who attended formed a diverse group, with both Ed's former patients and his colleagues standing up to share their experiences of a man who was incredibly respected and

truly seen as the exceptional individual that he was. Many of those attending learned more about Ed's achievements, his unique perspectives and brilliance as a doctor - but all there were familiar with his warm and unassuming manner and his support of the children and families under his care.

In order for families to be able to participate in the programme of speakers, the MPS Society, along with some of our volunteers, were pleased to be able to offer care for both children and vulnerable young adults during the course of the day. Everyone then met up in the evening for a special gala dinner, where we drank a toast and listened to reminiscences about Ed - everyone had their own special memories, and it was hard not to think about this warm and amazing man without smiling.



As one of those attending said, "all in all it was a wonderful, insightful, provocative and moving event."

Jacob's Pedal Power Coast to Coast in memory of Ed Wraith

My name is Jacob Singleton. I have Hurlers (MPS I H) and was lucky enough to have been treated and looked after by Ed Wraith at Royal Manchester Children's Hospital from being 4 months old until I was transferred to adult services at Salford Royal Hospital a couple of years ago.

I'd been thinking for a while that I'd like to do something big to show that people with MPS conditions can take on a challenge too, we see lots of stories of friends and family doing things to raise money and I wanted to do something. When Ed died last year I was really sad about it and decided that whatever I did, I would do in his memory.

Well, I decided to cycle the coast to coast route across north England while on the summer break from college to raise funds for the MPS Society (something to help keep me occupied while I wait for my first year results!)

- I thought this would be a good way to make sure whatever I manage to raise would be put to good use. I thought it would be a big challenge but I wasn't too worried about it because I cycle a lot anyway.

My dad Andrew cycled with me, we made it a camping trip over several days and my mum Heather came along to drive the campervan and cook our suppers! I've called the challenge Jacob's Pedal Power Coast to Coast.

Well done to Jacob for completing this challenge! They did fantastically well, had a hard couple of days in the middle over the north Pennines but Jacob insisted on cycling every inch of the way - no short cuts and no getting off to walk any steep parts (which Andrew had to do occasionally!).

Jacob raised £605.16 on his Justgiving page!



New members

Louise and Paul have recently been in contact with the Society. Their son Blake has a diagnosis of ML II. Blake is 5 months old. The family live in the North Yorkshire area.

Ms Groves has recently been in contact with the Society. Her daughter Erin has a diagnosis of Hurler disease. Erin is 2 years old and the family live in the South East.

Katrina has recently been in contact with the Society. She and her two sons Joshua and Joseph have Fabry disease. The family live in the Suffolk area.

Nadine has recently been in contact with the Society. Her daughter Evie has a diagnosis of Sanfilippo disease. Evie is 5 years old and the family live in Wales.

Mrs Zepciukiene has recently been in contact with the Society. Her son Robertas has a diagnosis of Sanfilippo disease. Robertas is 12 years old and the family live in the South East.

Angela and Alan have recently been in contact with the Society. Their son Max has a diagnosis of MPS I Hurler. Max is 6 months old and the family live in Scotland.

Gareth has recently been in contact with the Society. He has a diagnosis of Fabry. Gareth is 35 years old. The family live in the South.

Share your experiences...

Our grandson has been diagnosed with MPS II, Hunter disease and I have so many questions and mixed feelings and would love to be connected to other parents and grandparents who have experienced this situation. We are from Johannesburg, South Africa. Thank you. Mrs Christina Adams adamse@mtn.blackberry.com"

Isaac receives tablet to help at school



Isaac Eaton received this following a competition across several schools for a whole class to win a set of tablets for the school.

Each child in Isaac's class had to write a letter saying how they would benefit from the tablets and why they should win. Although they did not win as a school, the organisers decided to give Isaac a tablet on an individual basis as

they were very moved by what he had put in his letter.

Isaac had written about his difficulty with handwriting and using a conventional keyboard due to his condition making his hands get tired and also about having lots of time out of school for hospital appointments.

Anna Eaton

Remembering William at his Sister, Lindsay's Wedding

The wedding of our daughter, Lindsay Ferrier to Andrew Jones took place in February 2014 at Lainston House near Winchester. It was a very happy event and Lindsay was keen that her brother, William who had Sanfilippo Disease, should be remembered on this family occasion.

Lindsay organised the purchase of MPS pins and made her own favours for the wedding guests.

As well as helping to provide funds for the MPS Society, family and friends who had known William were able to share their memories of a special son and brother.

Many of the guests wore their pins and the grandparents of both Lindsay and Andrew were moved to send donations to the MPS Society.

Bill and Caroline Ferrier



Our halo experience

Isaac and his brother Archie have Morquio A Syndrome (MPS IVA). Just over a year ago we got the news we had successfully been avoiding for the last 10 years: Isaac's neck had deteriorated to the point where surgery was needed – he was having a halo!

A date in early June was set for the operation at the Royal Manchester Children's Hospital and we started to try to prepare Isaac, then aged 10, as best we could for what was going to happen.

Isaac was prepared for theatre and the cervical fusion procedure which would stabilise the bones in his neck and protect the spinal cord from damage. We had been warned that it would take several hours, as it would first take time to set up the anaesthetic and ensure his breathing was stable and then the halo would be fitted to hold everything still, before starting on the main surgery during which bone from his skull would be taken and used to bridge the top of his spine to the base of the skull.

It was a very different Isaac that we encountered afterwards on the recovery ward, a sobering sight to see him fixed in his strange new metal framed "halo" and with wires and tubes everywhere. He stayed silent and virtually asleep for the next two days.



However, over the next few days, things moved on steadily. He mastered sitting up comfortably in bed and progressed to sitting up out of bed, and it wasn't long before he was standing.

A week after the operation, we were free to go! Time to get back home, back to normal and back to school! And time to work out just what you can do with a halo on!

What we learnt...

Isaac soon learnt that with some adjustments and practice he could still camp out, ride his bike, play ball games, go on rides, go to school, and generally enjoy being active, but it wasn't just Isaac who had to learn to adjust to the halo – as parents we had a key role in managing the care of the halo. The areas where the pins holding the halo in place gripped Isaac's head needed daily care to avoid infection and the fleece liner under the hard jacket had to be changed regularly. We also had to learn the best way to wash his hair, and I had to make friends with my sewing-machine to produce some halo-friendly clothes. Thank goodness for Velcro!



The end of the halo!

Finally in October, it was time for the halo to come off. Although a huge relief, this was not the end of Isaac's recovery.

The removal process itself, whilst quick, was not a particularly pleasant experience for Isaac. A soft collar replaced the halo and Isaac had to get used to supporting the weight of his head for himself again. He was quite tired for a few weeks and suffered with headaches and pain in his neck and shoulders as his body re-adjusted again, a stark contrast to how active he had been in the last few weeks with the halo.

By Christmas though, his energy and stamina were returning and at his check-up in January he was declared fully fit, with just a few faint scars remaining to remind us all of the traumatic experience.



By Anna Eaton

(Anna & family are happy to be contacted with questions about any aspects of their experience of caring for a child in a halo!)

Please update your contact details

We like to keep you informed of news, events, information and opportunities. To minimise our costs we aim to contact you by email wherever possible rather than by letter so it is vital that you keep us informed of any changes to your contact details and let us have your current email address.

Please email mpps@mpssociety.org.uk to advise us of your email address and we can amend our records.

Sophie at One Direction!



I am Sally and my daughter Sophie has Sanfilippo.

I recently took Sophie to see One Direction at Wembley Stadium. She has shown an interest in the music and the group for a while now, and her face lights up when she sees them on the television! As Sophie has just turned 17, we decided that a ticket to the concert would be a good birthday present.

The first thing I had to do was get the tickets which didn't prove to be too difficult in the end. I telephoned

Wembley and spoke to their Disabled Team and they told me that there were spaces in the wheelchair area on all three days, but Friday had the most room. Due to Sophie's high needs she needs two people to look after her, I had to get medical evidence to prove that two carers were required, so the next step was to write to the GP and ask him to write a 'to whom it may concern ...' letter. I collected the letter the next day, scanned it and emailed it to the Wembley Team. The tickets were booked! I only had to pay for Sophie's ticket as both carers get in for free.

Sophie has been very tired and sleepy due to a new medication and I was quite anxious that she may sleep through the whole concert, which would be quite possible! Tim (my husband) thought that was probably the best way to see One Direction!

Sophie came home from school on the day of the concert quite alert and

happy. She had a rest on her bed, changed into her 1D t-shirt! and we set off at 6pm to the tube station. The trip to Wembley was very smooth and we were directed to various lifts to get on the Wembley walkway. There were hundreds of very excited young girls and their parents milling about and we made our way up to the stadium, Sophie appeared to enjoy the whole experience.

The Stadium staff were very helpful and they directed us to the wheelchair area which is situated centrally, opposite the stage and screens.

The concert eventually started at 8.30 and Sophie stayed awake for the whole event, and except when it got very loud with the screaming fans, she was very relaxed and animated.

We are so pleased she had a good night and would do a similar event again now we know it wasn't too traumatic!

Sally Summerton

Ben advises England cricket boss

"After a dismal winter in Australia, England's new cricket coach turned to young cricket genius Ben Conlin" lied dad.

Ben Conlin who suffers from MPS Hurler-Scheie was recently lucky enough to be invited to watch the England Cricket team train at Lords.

England were preparing for the test series against Sri Lanka and India.

The wish was organised by the Ray of Sunshine Charity, and was made possible by Anne Lynch, Nurse for Health Care at Home.



Ben had a brilliant time and was able to pass on lots of his expertise to all the players he spoke to. Ben's expert advice is probably the reason why Matt Prior's form has improved.

Before this, Ben had been invited to train with Middlesex County Cricket team in February, once again this was at Lords. During this visit Ben received bowling and batting advice from the Middlesex coaches and a tour of Lords Cricket Ground.

Finally on Ben and cricket, at a recent match for Stockton under 13's team Ben took his first ever wicket for Stockton. Stockton were playing Hartlepool when the Ginger Jim Laker struck. The ball was caught by Leon Kelly.

Ben's dad Peter tried to get a Mexican Wave going to celebrate but no-one

else realised how important this wicket was or is. Ben dedicated his wicket to Professor Ed Wraith who died last year.

We would like to thank:

Anne Lynch - Nurse, Health Care at Home

Claudia and everyone at Rays of Sunshine

Fletch and everyone at Middlesex County Cricket Club

Peter Moores and everyone to do with England Cricket

Peter Conlin

Ben with Peter Moores (left) and Alistair Cook (below)



The MPS Advocacy Service

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

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

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

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

Support provided by the team

- **Telephone Helpline**
0845 389 9901– the Society provides an active listening service, information and support. This includes an out of hours service
- **Disability Benefits** – in understanding the complexities and difficulties individuals and families have in completing claim forms for Personal Independent Payment, the Society continues to provide help and support

in completing these forms and, where needed, will take a representative role in appeals and tribunals

- **Housing and equipment** – the Society continues to take a major role in supporting and advocating appropriate housing and home adaptations to enable the needs of an individual with an MPS or related disease to be met. Where requested, we can provide comprehensive and detailed housing reports based on individual need
- **Education** – the Society helps members to access appropriate education and adequate provision for its implementation. This is achieved through providing educational reports used to help inform and educate professionals, and in many instances, to inform Statements of Special Educational Need. Where requested, we also provide information days/talks to schools and relevant professionals
- **Respite Care** – the Society continues to work closely with a number of respite providers and, where appropriate can make individual referrals
- **Independent Living/ Transition** – the Society provides advice, information and support through the transition from child to adult services. This could include access to independent living, learning to drive, further education and employment
- **MPS Careplans** – the Society undertakes a comprehensive assessment of the issues which need to be addressed when caring and providing support to a

specific individual diagnosed with an MPS or related disease, as well as other family members through the writing of a careplan

- **Befriender Service** – the Society links individuals and families affected by MPS and related diseases for mutual benefit and support

• Bereavement support.

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

Email:
advocacy@mpssociety.org.uk
Telephone: 0845 389 9901

Advocacy Resources

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

- **Life Insurance**
- **Travel Insurance**
- **Hospital Travel Costs**
- **Disabled Access Holidays**
- **Carers Legal Rights**
- **Carers Allowance**
- **Wheelchairs and Flights**
- **Guide to Housing and Disabilities Facilities Grant**
- **Benefits including Personal Independent Payment, Benefit Cap, Council Tax Benefit and Universal Credit**

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

Team members



SOPHIE
Manages the MPS Advocacy Team



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



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



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



ALISON
Supports members living in Ireland

All Ireland Advocacy Support Update

You will remember from my article in the last magazine that the first quarter of 2014 was crammed full of events and education sessions – the second quarter has been a little less hectic! Over the last few months I have travelled the length and breadth of Ireland visiting families in their homes and attending housing, school and medical meetings to provide support. Providing face-to-face support is at the heart of the Advocacy Support Service and we are always more than happy to pay a visit if you need us to.

If you live in Ireland (North or South) and have an unmet support need please do not hesitate to get in touch!

Alison Wilson

Telephone: 0044 77862 58336 or 0044 28950 47779

Email: a.wilson@mpssociety.org.uk

Northern Ireland Clinic

In May we had our N.Ireland MPS Specialist clinic. This was the first of our new one-stop-shop clinics where families were able to meeting with the Metabolic Team (from Belfast and Manchester) alongside a range of other professionals including an Orthopaedic Surgeon, Dentist, Respiratory Doctor and Nurse, and a Hospice Nurse. As with anything new, our first clinic was rather busy! We hope that all those who attended found the new format of the clinic beneficial. We look forward to receiving your feedback so that we can tailor the clinic to meet your needs.

The clinic has now moved from Antrim Hospital to the Royal Victoria Hospital For Sick Children which we hope will now become the Hub for all our MPS families.

International MPS Awareness Day



On the 15th May 2014 the MPS Society held a Northern Ireland 'Wear it Blue' party at the Dunsilly Hotel in Antrim. The aim of this party was to bring families together for a fun, social event to celebrate International MPS Awareness Day and to raise funds for the MPS Society. We had a fantastic night and we raised £130.75 for the MPS Society!

We were delighted to receive donations from some of our MPS families and a range of local crafts people for our raffle. We raffled off everything from bubble bath to National Trust family passes and no family left empty handed!



Top prize: National Trust Family Pass and Custom-Made Blue Bear

2nd Prize: 'Apples' Painting by Bernie Drayne and an M&S gift voucher

3rd prize: M&S gift voucher

Those of you who visit the MPS facebook page or follow us on twitter will have been introduced to our custom made MPS Blue Bear. This beautiful bear was donated to the MPS Society by Jude from 'Very Poppy Adams'. Our Blue Bear was very aptly named 'Hope' by the family who won him on the night. I trust that Hope is settling in well to his new home!



We would also like to say a huge thank you to Erik from ProSound NI who provided DJ services for the evening. Our dance-off proved a big hit with the kids (and the big kids!).

Thank you to all who attended and I look forward to seeing you all at the All Ireland Conference in September!

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

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

Advocacy Focus on ...

Lifetime Community Nurse & Psychology Service – South West England

This needs-led service based in Bath provides a unique community nursing and psychology service for families across Bristol, South Gloucestershire, North Somerset, Wiltshire, BANES and Mendip areas. The service is for families with a child up to 18 years old with a life-limiting condition. Some MPS Society members already access the service which started in 1993. Families have found the nurses supportive and invaluable in helping them access the complexities of the health services.

Referrals can be made via a professional such as GP or health visitor and further information can be found on their website at <http://www.sirona-cic.org.uk/health-care-services/child-health/lifetime-childrens-community-nursing-and-psychology-service/>

The nursing services offered include:

- Assisting with the funding and provision of medical equipment
- Care of ports, lines and feeding tubes
- Creating healthcare plans
- Training for procedures
- First point of contact for signposting to other health services

- Support with transition to adult services
- Support with end of life care

The psychology service can offer support to children and young people, siblings and parents through individual, family or group sessions who have a child/children with a life limiting condition.

The contact details for the service are:
Lifetime Community Nurse and Psychology Service
NHS House, Newbridge Hill
Bath, BA1 3QE, Tel: 01225 731624

MPS Regional Specialist Clinics

Summer 2014

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

Since the last magazine we have travelled to Manchester for the post HSCT MPS I clinics and to the Birmingham Fabry clinic.

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

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

Sophie Thomas
s.thomas@mpssociety.org.uk



Photos left to right: Fabry Clinic at Birmingham Children's Hospital - Cameron and Connor Merrick. BMT Clinic at Royal Manchester Children's Hospital - Reeda, Hamza and Sumaya Sajid, Amina and Hafsa Zaid

Bereavements

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

Gracie Sims who suffered from ML II and passed away on 28 May 2014 aged 1 year.

Jackson Whittaker who suffered from Hunter disease and passed away on 12 May 2014 aged 16 years.

Richard Turner who suffered from Hunter disease and who passed away on 7 June 2014 aged 32 years.

Shannon Rose Mallon who suffered from GM1 Gangliosidosis and passed away on 16 May 2014 aged 5 years.

Thea Paterson who suffered from MPS I Hurler and passed away on 10 May 2014 aged under 1 year.

Healthcare at Home Fails LSD Patients

Healthcare at Home has been deluged by complaints at its failure to deliver vital medication to patients on time amid wider concerns about the commercial home delivery of medicines.

Many LSD patients and their families have suffered from the incompetence of Healthcare at Home in recent months with telephone calls and emails to MPS and clinical teams expressing their anger at Healthcare at Home, which describes itself as the UK's leading provider of hi-tech home healthcare.

The delivery problems emerged after Healthcare at Home moved in March from an in-house delivery service to

using an outside company, Movianto, a big logistics firm operating in Europe with its headquarters in Richmond, Virginia, in the US. Healthcare at Home said the move would improve the service, but some of Movianto's IT systems failed despite extensive testing. We understand an IT glitch has meant Movianto did not have full visibility over their delivery schedules and this led to a surge of calls from patients requesting delivery time updates, which have continued for longer than expected.

Amongst the problems are no show of deliveries and wrong contents. To add insult to injury Healthcare at Home does not answer the phone and our

members are running up huge phone bills waiting more than 30 minutes on the line. It would also seem they do not answer emails.

We are told Healthcare at Home are working hard to fix problems and are fully aware of the inconvenience and anxiety caused to any patient when an agreed delivery slot is missed. However if you are having problems with Healthcare at Home now or in the future please tell your MPS / Fabry centre immediately and also let the MPS Society know too.

Christine Lavery
c.lavery@mpsociety.org.uk

Investigational Next – Generation Approach for Lysosomal Storage Diseases



Enzyme Replacement Therapy (ERT) replaces a specific enzyme that is absent or deficient in individuals with certain lysosomal storage diseases (LSDs). An enzyme is manufactured for intravenous infusion into the circulation, increasing the amount of missing or deficient enzyme.

ERT is currently available for many LSDs. These therapies are generally well-tolerated and improve manifestations of disease in some patients. However, the infused enzymes may unfold and lose activity at any point in the process including in the infusion bag and the bloodstream. This loss of enzyme activity may affect treatment outcomes with ERT.

Proposed Mechanism of Action

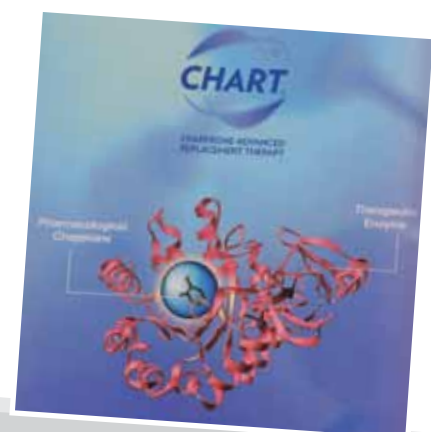
A specific pharmacological chaperone is designed to bind to a specific therapeutic enzyme, stabilising the enzyme in its properly folded and active form.

Potential impact

- Improved tissue uptake
- Increased lysosomal activity
- Greater reduction of storage material
- Improved levels of active enzyme in circulation

The CHART platform consists of a specific pharmacological chaperone combined with a specific ERT to each LSD. The pharmacological chaperone binds to the therapeutic enzyme, stabilising the enzyme in its properly folded and active form.

Amicus is investigating whether the CHART platform can improve ERT treatment outcomes for people living with LSDs.



Shire Update on Sanfilippo B program



In April 2012 Shire enrolled the first participant into a multi-centre observational, prospective natural history study of Sanfilippo Syndrome Type B (MPS IIIB) (HGT-SNB-088). The last patient enrolled in the study in September 2013, making a total of 19 patients. The period of observation in this study is 12 months for each patient.

The study was designed to evaluate the natural course of disease progression in individuals with MPS IIIB who are untreated, and to identify potential endpoints that may be utilised in future treatment trials of MPS IIIB using predefined assessments including

standardised clinical, biochemical, neurocognitive, developmental, and imaging measures.

Upon study completion, which is estimated to occur around the end of 2014, the data will be analysed and reported. A full publication is anticipated to occur as soon as possible after completion of the study, so that investigators and scientists engaged in developing treatments for MPS IIIB will be able to use this information.

The commitment of families to participate in such a study, with no guarantee of a successful therapeutic development, is deeply appreciated. Shire had a therapeutic development

program for MPS IIIB at the beginning of this natural history study. However, as a result of difficulties encountered in the development of the program, we are no longer pursuing the development program in MPS IIIB.

We remain committed to the MPS Society and patients with MPS IIIB in completing the natural history study and sharing the results from this study in the hope that this will provide a foundation to explore avenues for therapeutic intervention.

Philip J. Vickers, Ph.D.
Global Head of Research and Development
Shire

We may be small but we mean business

- please help us fundraise at work

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

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

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

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

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

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

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

What do I do now?

If you think you might be interested in raising the profile of MPS and related diseases at your place of work and getting colleagues fundraising then drop us an email at fundraising@mpssociety.org.uk

or call us on **0845 389 9901**. We can send you our super smart guide to fundraising, we can support you with ideas, posters, balloons and where possible guest speakers.

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



Marketing approval of Elosulfase (Vimizim) for Morquio disease

On 28th April 2014, the European Medicines Agency (EMA) gave marketing approval to Elosulfase (Vimizim) as an Enzyme Replacement Therapy for MPS IVA Morquio disease.

This is obviously very good news but is tempered with the concern that it may take some time to achieve reimbursement in the UK. The NICE process is already underway with the scoping meeting having taken place in May. Unfortunately NICE have advised that if Ministers agree to Elosulfase going through the NICE appraisal process a decision to reimburse may not be forthcoming for at least 18 months! The other option is for

Elosulfase to be appraised by the clinical priorities advisory group (CPAG) under NHS England and the MPS Society and clinicians are working towards this in parallel. We recognise that after all the anticipation of Elosulfase receiving marketing approval this is a real dampener.

The MPS Society is doing everything it can to work with commissioners and NHS England to achieve a resolution to timely reimbursement but recognises the uncertainty this is putting on our MPS IVA members and their families.

The reception hosted by Mark Pawsey MP held at the Palace of Westminster on Monday 2nd June 2014 'Celebrating a Better Future for Morquio disease' was a great success and it was

wonderful to see so many MPS IVA members and parents. The speaker programme included Mr Ali, father of Sultan Ali, the first child in the world to receive Elosulfase, Mary O'Toole whose daughter Helen lost her life to Morquio aged 33 years, as well as Dr Chris Hendriksz, Dr Paul Harmatz and Dr Edmund Jessop and told the story of Morquio disease from best palliative care to a licenced enzyme replacement therapy.

In order to keep the reimbursement issue at the top of NHS England's agenda as well as Wales and Scotland, we are encouraging families to meet with their Member of Parliament. Where appropriate we are also there to support individuals and families wishing to speak to the media.

Christine Lavery



Reception at the House of Commons, photos clockwise from top right: Dr Edmund Jessop, the MPS Staff team, Dr Paul Harmatz, Aiden Kearney and his mother Margaret Coyles

European Medicines Agency (EMA)

Reporting Side Effects – Guidance to Patients

Did you know that as a patient you now have the right to report unwanted side effects of medicines directly to the authorities. You can also report a side effect on behalf of someone you are caring for, such as a child or relative.

The EMA says:

- It is always learning more about medicines and although they are tested extensively before they are authorised, not everything can be known about their side effects until they have been used by many people and for some time. By reporting side effects, you will be helping to find out more information, which will ultimately lead to safer medicines.
- If you think a medicine you have taken has caused a side effect, please check the package leaflet that comes with your medicine for information on the ways to report it.
- You can report side effects to authorities by:

- reporting over the telephone
- filling in a form from your doctor or pharmacy
- filling in an online form accessed at www.yellowcard.mhra.gov.uk

What does the Medicines and Healthcare products Regulatory Agency (MHRA) say?

You can report suspected side effects (also known as adverse drug reactions) to a medicine, vaccine, herbal or complementary remedy by clicking “Report Side Effect”

The Yellow Card Scheme, run by the MHRA and the Commission on Human Medicines, is used to collect information from both healthcare professionals and the general public on suspected side effects.

All medicines can cause side effects. Some may not yet be known so that's why it's important for people to report to the Yellow Card Scheme. Many

side effects are mild, but some can be serious and even life-threatening. Others appear after taking a medicine for a long time or even after stopping a medicine.

It is important for people to report as these are used to identify side effects and other problems which might not have been known about before. If a new side effect is found, the MHRA will review the way that the medicine can be used, and the warnings that are given to people taking it to minimise risk and maximise benefit to the patient.

Please note the MHRA cannot provide medical advice. If you are worried about a symptom you think may be a side effect:

- Check the patient information leaflet supplied with the medicine. This lists the known side effects and advises you what to do.
- Talk to your doctor or pharmacist.

www.mpssociety.org.uk

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

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

If you are planning an event don't forget to visit our online shop for T-shirts, badges and bands.

We are always looking for ways to improve our site, so if you have any suggestions or ideas please don't hesitate to contact us at info@mpssociety.org.uk



Gene therapy for MPS III Sanfilippo Type A shown to be safe and well tolerated, with promising results

New Rochelle NY, March 11, 2014 – Several young children suffering from a severe degenerative genetic disease received injections of therapeutic genes packaged with a non-infectious viral delivery vector. Safety, tolerability and efficacy results from this early stage clinical trial are reported in *Human Gene Therapy*, a peer-reviewed journal from Mary Ann Liebert, Inc., publishers. The article is available free on the Human Gene Therapy website.

Marc Tardieu, Universite Paris-Sud and INSERM, and a team of international researchers administered the adeno-associated viral (AAV) vector carrying a normal copy of the N-sulfoglycosamine sulfohydrolase (SHSH) gene into the

brains of four children affected by mucopolysaccharidosis type IIIA (MPS IIIA), an inherited lysosomal storage disease in which the SGSH gene is defective. The AAV vector also delivered sulfatase-modifying (SUMF1), needed to activate the SGSH protein.

In addition to measures of toxicity, adverse events and tolerability, the researchers evaluated the children for brain shrinkage (a characteristic of MPS IIIA) and for changes in behaviour, attention, sleep and cognitive benefit. They describe their findings in the article 'Intracerebral administration of AAV rh.10 carrying human SGSH and SUMF1 cDNAs in children with MPS IIIA disease; results of a phase I/II trial.'

"This is an important new approach for treating CNS manifestations of lysosomal storage diseases that could be applied across a wide array of disorders," says James M. Wilson, MD, PhD, Editor-In-Chief of *Human Gene Therapy*, and Director of the Gene Therapy Program, Department of Medicine, Philadelphia.

Enzyme Replacement Therapy for alpha Mannosidosis

12 month follow up of a single centre, randomised multiple dose study – Rigshospitalet, Copenhagen, Denmark

Alpha Mannosidosis is one of the rarest diseases of the 24 diseases supported by the MPS Society and presently the only available treatment option for alpha Mannosidosis is haemopoietic stem cell transplant (HSCT). This study is the first in humans and aimed to evaluate the safety and long term efficacy of enzyme replacement (rhLAMAN) therapy in patients with attenuated alpha Mannosidosis.

Ten patients aged 7 – 17 years were enrolled. The study was divided into Phase 1 and Phase 2a. The phase 1 was a 5-week single centre, open label, dose escalation study of the safety and pharmacokinetics (PK) of the enzyme. The patients were divided into five groups and each group received a different intravenous dose of enzyme.

The design of the Phase 2a was a 12 month single centre, randomised, open label, multiple-dose study of the efficacy and long term safety of the enzyme. The patients from phase 1 were randomised into two groups, receiving either 25 units per kilogram weight or 50 units per kilogram weight. This was to establish the lowest effective dose of enzyme. The patients were dosed weekly for 12 months. Clinical evaluations and functional assessments were carried out.

9 of the 10 patients completed the 12 months of treatment. 508 of a possible 512 infusions were completed. There were four serious adverse events (SAEs) in three patients but none occurred during the treatment or 1 hour observation period following infusion. Three were found unrelated and one possibly related to the rhLAMAN enzyme.

In this first randomised study investigating the efficacy of ERT in patients with alpha Mannosidosis, the authors of the paper reported improvements relating to somatic manifestations; in parameters of cerebral function and integrity and increased Forced Vital Capacity and conclude that preliminary data suggest the ERT with recombinant human alpha-mannosidase is safe and may be an encouraging new treatment for patients with alpha Mannosidosis.

The study is designed to continue for a total of 18 months with longer term follow up of patients in this study and the ongoing placebo controlled phase 3 trial required to give greater support for the findings in this study.

Enzyme Replacement Therapy for alpha-Mannosidosis; 12 months follow up of a single-centre, randomised, multiple dose study. J Inher Metab Dis (2013) 36:1015-1024

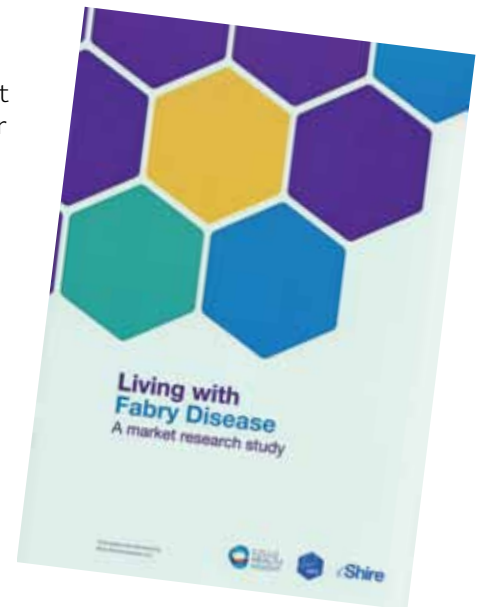
Living with Fabry disease

In 2013 the MPS Society funded with an unrestricted educational grant from Shire Pharmaceuticals and collaborating with an independent market research agency, Cello Health Insight, ran a study to gain a better understanding of people's experience of living with Fabry disease in the UK.

60 Fabry members of the UK MPS Society were interviewed. This included a mix of men and women of various ages, three quarters of whom were on treatment. These interviews were analysed in depth by Cello Health Insight who then collated the findings.

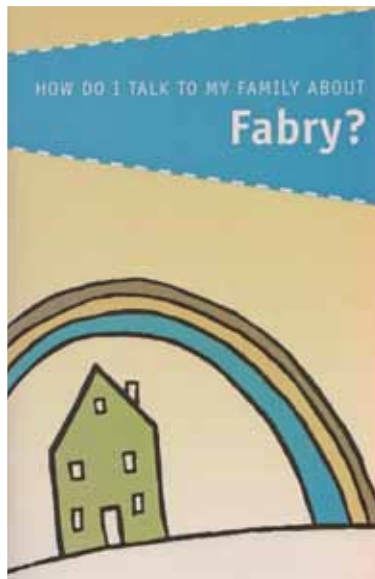
The study set out to explore the impact Fabry disease has on peoples' lives, their views on treatment and what support they feel they need. When the findings were presented to MPS the broad spectrum of experiences were striking and we felt it would be good to share the common themes identified with not just the study participants but all our Fabry members. We are therefore grateful to Shire for printing the enclosed report which we trust will be of interest to our Fabry members and their families.

Christine Lavery



How do I talk to my family about Fabry?

This booklet was developed with support and collaboration from Genzyme by the Genetic Alliance www.geneticalliance.org

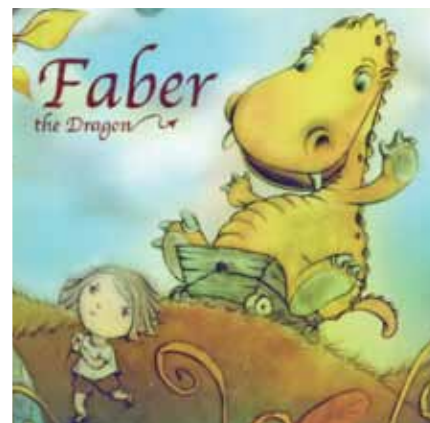


New Resources available

Faber the Dragon

New booklet for children helping to explain about living with Fabry disease.

This book was funded by Shire Australia on behalf of the Fabry Support Group Australia www.fabry.com.au



Challenging behaviour factsheets

The Challenging Behaviour Foundation has developed a series of three information sheets which look at the causes of challenging behaviour and explain how to make effective plans to reduce challenging behaviour.



The information is designed for families or professionals who support a child or adult with severe learning disabilities whose behaviour challenges. The sheets can be downloaded free of charge from www.challengingbehaviour.org.uk or phone 01634 838739 to request a hard copy (cost £1).

Rare Disease Day 2014

Instigated by a group of European countries back in 2008, Rare Disease Day on 28th February each year was launched with the express purpose of raising awareness of conditions that are not largely known to the general populace, but which are devastating to sufferers and their families, an aim that obviously runs parallel with the concerns of the MPS Society.

The campaign targets the general public and policy-makers, but everyone was welcome to join in; patients and their families, patient organisations, health professionals, researchers, drug developers, public health authorities.

This year the slogan of Rare Disease Day was 'Joining Together for Better Care', and its emphasis was firmly on the theme of care itself, highlighting the importance of improving the lives of people living with a rare disease and their families. Another of their key points within the concept of

'Joining Together for Better Care' was reminding sufferers and their families that they are not alone, which helps to dispel the sense of isolation that is bedfellow with rare diseases. With the day itself being observed on an international scale, with 84 countries participating and 410 events held worldwide, this idea of solidarity was particularly well illustrated, a point that is driven home even more by the growth of each successive Rare Disease Day as more and more countries recognize the need and join in. This year saw a further 9 countries joining in, including Cuba, Ecuador and Kenya. Each new country represents an advance of solidarity and a step forward for awareness.

Like the MPS Society, Rare Disease Day's chosen symbol depicts hands, and this is a very evocative reminder of solidarity, compassion, humanity and care, along with the assurance that each of us is not alone. This knowledge, heightened by the work of charities and research, is crucial to those affected and pivotal to keeping research going.

To recognize this event The Independent released a supplement entitled 'Rare Diseases' which covered some of the challenges faced by those living with a rare disease, as well as the difficulties encountered by their families. One of the MPS Society's Trustees, Bob Stevens and his wife Claire contributed to this article by writing about their two sons who suffer with Hunter syndrome and how this disease has affected their lives. The supplement also covered a range of topics within this area, including awareness of rare diseases for doctors, newborn screening, drug advancements and the UK Strategy for Rare Diseases.

If you would like to read these articles and more from 'Rare Diseases', please visit www.rarediseases.co.uk.



Students4RareDiseases



The Students4RareDiseases group is a committee that connects rare disease societies from different medical schools in order to share speaker information and advertise events.

3.5 million people in the UK suffer from a rare disease, 75% of which are children, and often these diseases are life threatening.

The Students4RareDiseases group aims to bring you into contact with both patients and clinicians in order to

educate you on rare diseases. By educating future doctors we can increase early recognition and diagnosis of rare diseases.

Students4RareDiseases is a collaborative across the five London medical schools although there are plans for it to become a nationwide event.

If you want to get involved with rare diseases at your own medical school, hold an event or even start your own Society please go to their website for more information.

students4rarediseases.org

European Fabry Working Group (EFWG)

Defining consensus of stop and start criteria in Fabry disease

**Amsterdam Medical Centre,
31 May 2014**

This meeting came out of an initiative by Professor Carla Hollak and Dr Gabor Linthorst, funded by the Dutch Ministry of Health.

Prior to the meeting, the team at Amsterdam Medical Centre (AMC) constructed an online survey that was sent to 41 clinicians. 27 completed the survey to establish consensus across Europe on start and stop treatment criteria for adults and children diagnosed with Fabry disease.

The consensus meeting was held under a modified delphi process and involved reviewing the responses from the online survey of 57 questions on criteria for starting and stopping enzyme replacement therapy. The 16 clinicians present then voted on each

question to reach a consensus. Starting with the first question it was soon clear that the clinicians were intent on putting all their energy into achieving the most appropriate pan-European start/stop Fabry guidelines for the Fabry community and took plenty of time to ensure the recommendations were consistent with the latest clinical knowledge.

For Lut de Baere and I representing FIN and the representatives from the Dutch patient organisation, we were not sure what to expect in our observing role. We need not have worried as being included in this process. Being observers offered us the reassurances that we needed to support and reassure the Fabry patient community that the discussions and recommendations that will likely result in a consensus document were transparent, fair

and ethical. Whilst non-voters in the consensus process, we were given ample opportunity to contribute to the discussions.

I should make it clear that Prof Hollak, Dr Linthorst and representatives of FIN and the Dutch patient organisation were non-voting and there was no interference from the Dutch Ministry of Health.

There is now a lot of work for the team at AMC to do following the meeting to pull together a first draft of consensus guidelines and I am pleased that we will continue to have a role on behalf of the Fabry patient organisations and their members.

Christine Lavery
President, Fabry International Network

7th European Conference on Rare Diseases and Orphan Products (ECRD)

**Berlin Germany
8 – 10 May 2014**

The European Conference on Rare Diseases is a unique forum across all rare diseases and all European countries, bringing together all stakeholders – patients' representatives, academics, healthcare professionals, industry, payers, regulators and policy makers - nearly 1000 attendees. It was therefore an enormous privilege to be invited to Chair a workshop and panel on the 'Role, risks and relevance of Registries in shaping therapy developments to 2020.'

Elfriede Sevinnen, Scientific Institute of Public Health, Belgium; Daniel Rosenberg, Senior Director, Actelion; and Tarek Hiwot, Queen Elizabeth Hospital, Birmingham, gave short presentations on Rare Disease Registries, where we are today and how they might look in 2020.

It was acknowledged that collecting data on rare diseases is a necessity of pre and post drug development stages to speed up clinical research, to provide data to regulatory and reimbursement bodies but the question that hangs over us is 'can these registries better serve the rare disease community in particular patients if not held by industry taking advantage of technology to share data repositories?'

There was a robust panel discussion with members including Jeremy Manuel, European Gaucher Alliance UK; Carla Hollack, Academic Medical Centre, Amsterdam; Micheline Wille, Shire, Switzerland; and Leeza Osipenko, Nice UK.

The overwhelming conclusion of the entire workshop was that patients, clinicians, researchers, regulators, payers and even industry would be much better served by the

establishment of 'independent' disease registries which allow transparency, dissemination of anonymised data and avoids duplication of effort. Independent rare disease registries also have the prospect of allowing researchers to 'grow the science' and equitable access to industry partners.

Christine Lavery



EU – North America Partners in Patient Health Council

13 – 14 May 2014
Paris, France



It was a privilege to be invited to be part of a unique 'Partners in Patient Health Council' by Sanofi. The Council comprised of representatives of large umbrella not for profit organisations from North America and Europe, Eric Lowe from Myeloma UK and myself. We were also joined by Sanofi and Genzyme senior leaders from Research and Development, Commercial, Public Affairs, Communications and Patient Advocacy covering Global, European and North American operations.

During our pre-meetings leading up to the Council, there was a clear voice that patient organisations need to be participants and partners in all processes and decisions that impact patient health. The objectives identified by Sanofi included:

- Learning about efforts in Europe and North America to enhance patient advocate participation in processes affected by research and development and patient access to therapies
- Identify opportunities to ensure patient advocates are participating partners in these processes
- Generate ideas and partnerships of mutual value for patients.

Being a member of this council was an amazing opportunity to gain a wider perspective of the challenges faced by patient organisations and industry of working together for the

best interest of the patients. As the only council member representing the ultra orphan rare diseases over the 24 hours together I came to appreciate that the role of rare disease patient organisations are in many ways light years ahead in their professional relationships with clinical experts and the pharma industry in both R & D and patient access to new therapies. I hope that the experience in the lysosomal disease arena can contribute going forward to a better experience for many more patients. **Christine Lavery**



FIN Fabry Expert meeting

11 - 12th April 2014

The Steigenberger Hotel, Amsterdam, was recently the location of the FIN Fabry expert meeting.

Spread across 11th and 12th April 2014, the meeting boasted a programme of talks by a range of eminent specialists, including Dr Gabor Linthorst's fascinating talk on 'New and Emerging Understandings of Fabry Disease', and Dr Maurizio Scarpa's exploration entitled 'Fabry Disease in Children: when and why should treatment start?'.

Further contributions from Fabry Patient Organisations from around the globe enriched the meeting with their discussions surrounding the support of Fabry patients worldwide.

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



Photo below: FIN Board Members



Fundraising

With MPS Awareness Day over for another year, we are able to stop and reflect on the dedication, inventiveness, energy, generosity and general wonderfulness of our supporters. We have come to expect nothing less, and we certainly were not disappointed with the fundraising events happening on or around 15th May – or indeed at any time.

People all over the country took our Wear It Blue fundraiser to school, college and work, they held stalls, threw parties, painted themselves blue, took selfies – all to show their support for the MPS Society! We would like to extend our appreciation to all who took part in our special day and made it such a success.

Our thanks also goes out to all those who have fundraised outside of MPS Awareness Day. We've had races, rides, festivals, quizzes, raffles, obstacle courses, and of course the excitement of the Virgin London Marathon. You all really pulled out all the stops to raise money and awareness!

Many of our supporters have already been planning fundraising events for the rest of the year, but if you are stuck for ideas why not organise a Wicked Walkabout and enjoy the summer weather? Or if you fancy something less strenuous why not try a coffee morning or a pub quiz. You could even nominate the MPS Society as the Charity of the Year at your workplace and get all your colleagues involved.

Whatever you would like to do, whether small or large, remember that every penny counts and any amount contributes to us being able to

continue doing what we do: supporting those affected by MPS and funding research into treatments, and by raising awareness you are helping us spread the word of these little known but devastating diseases and why we need to do something about them.

We do love to hear from our supporters, so if you need some help with your fundraisers, or some inspiration, or if you come up with any of your own ideas, please contact us at fundraising@mpssociety.org.uk.

We have highlighted some of your fantastic fundraising stories on the following pages. Our apologies if we have not included your story, but this section is intended as a snapshot only.

0845 389 9901

fundraising@mpssociety.org.uk

www.mpssociety.org.uk

A Cycling Challenge with a Difference



Headed by Lyn Mason, Newington Court Care Home in Kent organised a special event for MPS Society: they took on the challenge of cycling 412 miles – the equivalent of a cycle from their home town to Dublin – on an exercise bike!

Both staff and visitors alike joined in to pedal to raise money for the MPS Society; they managed to complete 150 just on the first day, and completed the distance over

the course of the next few days. They also held a book and jigsaw puzzle sale, a bake sale, and staff also paid to Wear It Blue!

Lyn and everyone involved had a brilliant day and were thrilled by the £442.25 they collected in sponsorship. The care home decided to raise money and support MPS Awareness Day as one of their residents, Lisa Nurse suffers from MPS IIIA, and Lisa and her parents have been supported by the Society for many years. Lisa is now in her early forties.

Many thanks to Lyn, and the staff and visitors at Newington Court Care Home for their excellent support and fundraising. We hope that your legs weren't aching too much afterward!





MPS Awareness Day , 15th May 2014

How you helped us mark International MPS Awareness Day

The **Northern Ireland Genetics Service** held an MPS Awareness Day coffee morning raising £142.

King Edward VI Academy held a Wear It Blue event raising £72.77

Tania Trimmer donated £10 to Wear It Blue for Ethan.

Barbara Penny collected £100 from family in lieu of Wearing It Blue. She donated this money to the MPS Society to say thank you for the support given to her Grandson, Merlin Penny-Smith, who suffers with MPS II.

Staff at MPS House raised £9 from a cupcake sale for MPS Awareness Day.

Hannah Cooper (MPS I, post BMT), the pupils and staff of **Raynsford Church of England Academy** raised £140 by wearing blue on MPS Awareness Day.

Luke Bown raised £75 by holding a

Wear It Blue day at his school, and by collecting contributions from friends and family.

Linda Bell and staff at **Lloyds Bank, Manchester** raised £127 by Wearing It Blue on MPS Awareness Day.

Louise Lucas, landlady of **The Hampden Arms**, raised £140 from a pub quiz on MPS Awareness Day.

St Mary's Catholic Primary School Wore It Blue on MPS Awareness Day and raised £200.

Barclays Bank joined in our Wear It Blue day and donated £280.92 – part of which was collected by Ian Baldwin who proudly donned a blue onesie for the day!

Susie Godfrey from **Genzyme**, Wore It Blue at work and raised £369

Jude Butler held a Wear It Blue event

and a cake sale at **Hertfordshire County Council's** Farnham House site raising £270.

Manor High School, Leicester raised £924.27 by holding a non-school uniform day for MPS Awareness Day.

Yewstock School donated £101 by Wearing It Blue.

Sukhi Bhachu and colleagues at **HSBC**, held a work event to celebrate MPS Awareness Day. The sweet-toothed bank workers sold cakes and cereal bars and managed to raise £104.88, an amount which HSBC agreed to double! Many thanks to Sukhi and HSBC Bank.

St Thomas's C.E. Primary School in Lancashire held a Wear It Blue day for their pupils and raised a fantastic £200. The children had a great time with their blue balloons and also enjoyed playing with blue plasticine!

Wear It Blue Selfies!

In light of several other highly successful selfie fundraisers, the MPS Society launched our very own Wear It Blue Selfie campaign in time for MPS Awareness Day – or if you are a Twitter user, #wearitblueselfie.

All we needed you to do was take a selfie while wearing blue and Text MPSS01 £2/£5/£10 to 70070 – couldn't be easier! Not only does it raise awareness and funds, but we also get to see all our lovely supporters!

We got a fantastic response, with people posting Wear It Blue Selfies, as well as other lovely photos, on facebook and Twitter – so a big thank you to all those who participated in our selfie campaign and for everyone who Wore It Blue.



'Wear it Blue' MPS Awareness day at Beauchamp

This year at Beauchamp College Leicester, Aisha Seedat along with the help of many students and staff held a 'Wear it Blue Day' that eventually turned into a week! The whole college was involved, aiming to raise both awareness and funding for research.

Aisha created a video interview about life with MPS that can be viewed on 'youtube' (search MPS awareness).

Posters were distributed to all tutor groups on the Monday along with collection boxes and stickers. On Wednesday of that week tutors read out a short information sheet about MPS followed by a viewing of Aisha's video. Finally on the Thursday, 'Wear it

Blue Day' arrived and our college was a sea of blue. Tutors kindly collected the donations for us, which so far have totalled £991.00! This has still to be added to with a dress down Friday in June so we hope to top the £1000 mark!

Aisha also has a Just Giving page www.justgiving.com/AishaSeedat which shows the link to her video on 'youtube'. This brought in another £360, bringing her total on Just Giving to £2,107.00!

Aisha suffers from Morquio Syndrome (MPS IV). In 2013 Aisha trialled for Enzyme Replacement Therapy (ERT) in Manchester until October, when Aisha had to stop the trial due to an anaphylactic reaction. Despite this Aisha continues with the trial but without the drug. Aisha has now been

asked to be a 'Young Ambassador' for the MPS Society and will be visiting Westminster Palace in June to support the licensing of ERT in the UK. Good luck Aisha! **Aisha Seedat & Paula Whalen**

Thank you to Aisha and Beauchamp College for their excellent support and help in raising awareness!



Hannah's Wear It Blue Bonanza

Hannah Brentnall, mum to Jacob (MPS III) and Samuel, really painted the town blue as she helped us to mark MPS Awareness Day, by involving a school, a supermarket, a Mother & Toddler group and a wedding! Phew!

Jacob's old primary school (and Samuel's current one!) allowed the children to Wear It Blue, and sold ice-creams to raise money for the MPS Society. On one of the hottest days of the year, this went down a treat and Hannah collected a cool £500!

Hannah didn't stop there, however, she then went on to set up a stand to collect donations, sell MPS merchandise

and raise awareness in her local Morrisons. The supermarket was fantastically supportive and were wonderful hosts, even allowing their staff to wear blue on our special day. Hannah and Samuel were overwhelmed by the donations and kind words they received from staff and customers.

Local shops and businesses also Wore It Blue and contributed, cakes and items made in blue were sold by a local Mother & Toddler group, and Hannah sold all of the merchandise that was given to her! Her friends Jayne and Nathan very kindly bought all the remaining MPS ribbons to adorn the favours at their forthcoming wedding.



Hannah pictured in the blue wig!

Money is still coming in, but so far Hannah has raised well over £1,500

We would like to send our sincerest thanks to Hannah for all her hard work, her incredible enthusiasm and dedication. A real inspiration!

Isle of Wight goes blue!

Gemma Gallagher helped to coordinate Wear It Blue events in two schools on the Isle of Wight for MPS Awareness Day. Avah Flynn's (MPS I) sister attends St Mary's Catholic Primary School, and they raised £200, while Dover Park raised £187.73.



RBS Sunderland goes blue!

Sarah Robson and the rest of the staff at the Royal Bank of Scotland in Sunderland, have been raising funds in support of Jack Watson (MPS III). Jack, who in 2012 won the "Child of Courage" award at the Sunderland Echo Pride of Wearside awards, inspired Sarah and the rest of the kindly RBS staff to start fundraising back in February – everything from Sarah's sponsored zip wire challenge to selling MPS ribbons and handmade bracelets and necklaces. Of course, on MPS Awareness Day all staff donned their blue wigs and even featured in the local paper, The Sunderland Echo! A great way to raise awareness!

To date, Sarah and the RBS team have raised £557.65, which is to be allocated to the Genestein trial – plus there is still more yet to come from her Just Giving page (www.justgiving.com/shareen-lancaster).

Thank you to Sarah and the rest of the staff at RBS, Sunderland for all their help in raising both funds and awareness.

Simon's 24 hour radio show



When my daughter Seren Rose was diagnosed with Hurler Syndrome, myself and Elouise had never heard of it. When we searched the internet, the MPS Society came up first, we found a wealth of information and support available, and had a home visit too. I wanted to help in some way, to help us and other families. I didn't really know what to do, but then had an idea of a 24 hour radio broadcast. I contacted Roger Davis, my radio station boss at Big L Radio and he thought it a great idea.

So on May 14th at 5pm I took to the airways. It was a fun 24 hours, the boss came to the studio at 11pm and stayed till 4am, and other presenters joined me throughout the day. I finished at 5pm on the 15th, to coincide with MPS Awareness Day. I look forward to doing another fundraiser soon. **Simon Davis**

Thank you Simon for putting on such a great show over MPS Awareness Day. Simon's Just Giving page has now reached £1,925 with Gift Aid – a fantastic achievement!

Arriva Wear It Blue on the buses!

Since the turn of the year, I have had many people at work wanting to know when next they could dress up or down for my son.

'MPS Awareness Day, do you mean?' was my reply. 'Yeah, that's it. Last year was great!'

So the ball started rolling again: people started offering their help and people in the town of Aylesbury and the surrounding area began donating prizes in preparation of the day. All I needed to do was get the relevant permission from Arriva the Shires and Essex to lift the uniform regulations for the day, and to allow collection boxes on the buses. Luckily, this was agreed straight away and I could get planning.

I was not short of help; one kind person even agreed to give up his day and spent it dressed as a pantomime dame,

talking to the public in Aylesbury bus station.

We all had a wonderful day not only talking to the public about the MPS Society and the support that they give to us families, but also collecting donations – and many of the bus drivers also paid to be out of uniform for the day, and most of them wore blue. Dennis and Janet Mauger were once again fantastic as Buddy Holly and Peggy Sue, and managed to raise an amazing amount; they did, however, get stopped by the VOSA (Ministry of Transport) on their school run...the usual checks were carried out and then the officer was asked if he wanted to dance with Peggy Sue – he declined, but did give a donation!

We even had a Community Support Officer ask us for a photo – it did of course cost him a donation to the MPS

Society before we agreed!

After the day at work, I went to another wonderfully well organised quiz at The Hampden Arms in Great Hampden, Buckinghamshire. It did make it a very long day, getting up at 4am and going to bed at 11:30pm, but this is nothing compared to the support, help and advice that are given by the Society to all of us.

We raised £1,100, which is fantastic, and I would like to congratulate anyone who organised an event on 15th May. I am already looking at ways to make next year's event bigger and better. Finally I would like to thank Arriva the Shires, Dennis and Janet Mauger and Phil Godleman, also all members of staff at the MPS Society for everything that you do for families like ours.

Ian Evans

– father of Harry Evans (MPS I)



Well done Ian for organising yet another amazingly successful MPS Awareness Day event – thank you for your incredible support of the MPS Society, always going above and beyond the call of duty!

We would also like to extend our thanks to Arriva and the staff at Arriva the Shires for supporting Ian's fundraising.



Genzyme employees Wear It Blue

On Thursday 15th May 2014, Genzyme UK and Ireland held an all employee conference and as part of the event, all attendees were asked to Wear It Blue to recognise MPS Awareness Day.

During the day, two patients told their stories of living with lifelong conditions. We were pleased to welcome an MPS I and a Multiple Sclerosis patient to speak to us about how their conditions have affected their lives.

Joanna Wilson Smale gave a very insightful talk on how she was eventually diagnosed with MPS I (Scheie) and what she has achieved since starting treatment.



A fantastic £119 was raised with a further £250 pledged by other external speakers. Thank you to all at Genzyme for their support.

Thank you Katrina

As was the case last year, Katrina Fanneran was very busy over MPS Awareness Day. Not only had she a newborn baby and young Ethan (MPS VI) to look after, but she also got involved in fundraising and raising awareness! Amazingly, Katrina managed to get all sorts to take part in our important day, from Juan Mata of Manchester United to Sky Sports News Team, Tom White and Rachel Wyse.

Building on the successes from the last two years, Katrina's Just Giving page (www.justgiving.com/ethansmps) has now reached £17,411.54. Many thanks for your kind support of the MPS Society, Katrina and well done for all your work raising awareness and funds.

Hadrian for Hunter



On 9th and 10th of May 2014, a group of property professionals undertook a challenge which called upon all their endurance, stamina, courage and good humour! This crack team, led by Vincent Willink, consisted of: James Wilkinson, Tom Walker, Simon Robson Brown, Hugo Machin, Dirk Jan Lucas, Anneke Hoijtink, Ingrid Prins and Jaap Jan Fit. These brave men and ladies took up the challenge of cycling the length of Hadrian's Wall...

This fiendishly difficult event was planned by Vincent, a veteran of the real estate business, who wished to raise money and awareness for Hunter Disease (MPS II); his twin sons, Teun and Wander, both were diagnosed with the condition in 2009, so the cause is very close to his and his family's heart. Both Teun and Wander were given walkie-talkies in order to stay in contact with their dad as he traversed the North of England, and must have been very proud to hear of the adventures he was having!

The team started in Bowness-on-Solway on the West coast. At breakfast on the morning of the 9th, all were quiet in anticipation of things to come. James had been so excited that he hadn't slept for more than an hour! The weather outside was miserable, but the team were still looking forward to their 9 hour ride on rusty bikes.

Soon after starting it became apparent that the bikes they were using would lift the challenge into new realms; Tom soon had to 'do a walker' as his bicycle (lovingly dubbed 'The Beast') was down

to only its 3rd gear, soon giving up the ghost entirely when its front wheel axel broke on the second day, and Hugo and Simon took turns risking their lives doing 50 miles an hour downhill on a Brompton folding bike. These were the kind of bikes for a 5 minute ride to a pub in Amsterdam, not for a 100 miler! Though, as Vincent said, 'It will be painful, but it has to and will be done'.



The scenery that flew past them was incredible, but the team had little time to take it in as all their concentration and energy was required to keep those pedals rotating! Luckily their support team, Amy and David Chapple were never far from the team, with a car full of spare parts, food and beverage supplies, as well as being on hand to provide physiotherapy, mechanical expertise and mental support.

By the time they reached Tynemouth Castle on the East coast, the team had done 95km in 9 hours on the Day 1, and another 80km in 6 hours on Day 2. After this mammoth ride, the challenge was finally over and their mission was completed. Spurred on by friends and fuelled by adrenaline, the team had yet another 15km to go to reach Newcastle where a cold beer and their hotel rooms waited.

That night the friends who had conquered Hadrian's Wall on completely unsuitable bikes, celebrated their success in style with a star-studded party, complete with champagne and VIPs – one of whom

was none other than our Chief Exec, Christine Lavery, who explained how the money they had raised will support over 170 families, such as the Willink's, affected by Hunter Disease and how vital it is to engage in further research to create better treatments

The whole Hadrian for Hunter project has been an enormous morale boost for the Willink family. The whole family got involved, with Sanneke (Vincent's eldest daughter), creating a beautiful 'thank you' banner. The attention and support have given them renewed strength to face the challenges ahead.

So far Vincent and the team have raised a staggering £51,365.71. Thank you to all for those who have donated to this event, including Corio, Hambourner Reit, British Land Company, CBRE Clarion Securities, Citycon and Sponda.

On behalf of the MPS Society, may we extend a massive thank you to Vincent, James, Tom, Simon, Hugo, Dirk, Anneke, Ingrid and Jaap Jan for completing this fantastic challenge and for raising money and awareness for Teun and Wander, and for all the other children and young adults who are affected by Hunter Disease.

Many thanks also to Vincent Willink for his assistance with this article.



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



Get active with a Wicked Walkabout?

The sun is out and the sky is blue (hopefully), so why not get out there and enjoy it?

The benefits of walking are well known to most of us: on a regular basis walking can help to tone muscles and strengthen the heart, lower risk of disease and aid weight loss, and can even make you happy! One other benefit we would like to add to this impressive list is that walking can also raise awareness for MPS and generate funds to help us to continue supporting those affected and working towards finding treatments. And to add the cherry on the cake, you can do this with all your family and friends, and even your pet dog – in fact the more the merrier!

If you would like to raise funds and awareness for the MPS Society, as well enjoy the great outdoors, kickstart a new healthier you and spend some quality time with loved ones, why not organise a Wicked Walkabout? Whether you choose a relaxed woodland stroll on a summer's day, or whether you scale the local hills on a crisp morning, the extent and location of your walk is entirely up to you. You might even decide to jog the route instead of walk, or even hold a 3-legged race – the choice is yours.

We've created a poster to help you on your way, but it couldn't be easier, you just need to promote your event and get as many people to join you or sponsor you as possible; then of course when you have completed

your Wicked Walkabout just send us your sponsorship money and please do include photos and let us know how you got on.

We are always on hand to offer guidance and help where we can: just drop us an email at fundraising@mpsociety.org.uk or phone **0845 389 9901**, and you can download anything from sponsorship forms to a Wicked Walkabout Organiser's Guide from the 'fundraising downloads' section on our website, or you can send us an email to request these materials by post.



Bridport Wicked Walkabout

The irrepressible Kath Hiller held her second Wicked Walkabout in May, and did it in style with a 'wear blue fancy dress if you dare!' challenge to all entrants, and of course this attracted much attention from passers-by en route, especially with a ballet dancer and spiderman in tow! A perfect way to raise awareness, we think!

Kath's daughter, Louise, also pulled out all the stops as she travelled all the way from London to Dorset to join in – and she didn't stop there! Louise then went on to connect with Emma Hiller's team in the Bridgnorth Walk in Shropshire. So all in all Louise walked almost an equivalent of a marathon for MPS!



Unfortunately Kath's party got rather soaked by a downpour on their way back, but as Kath said "spirits were not dampened!" This fortitude certainly seems to be a hallmark of all our members and supporters, whether in fundraising or in their daily lives.

Kath's wonderful fundraising events are held in response to the help and support the MPS Society have provided for her grandson, Joe (MPS II), and his family.

Many thanks to Kath, Louise and all the Wicked Walkabout team who have so far raised over £1000!



Virgin London Marathon 2014

On Sunday 13th April, Chief Executive of the MPS Society, Christine Lavery MBE, along with myself, went to see the London Marathon and to offer our support to all of those who ran for the MPS Society.

We could hear the crowds almost from Cannon Street station, so we needed no directions to where the action was. A mixture of unrelenting applause and shouts of approval and encouragement led us to Upper Thames Street, which was lined with spectators. Christine promptly leapt a railing for a better view, while I proceeded to get tangled up with my bag in an awkward attempt to follow in our Chief Exec's agile wake. Once I reached the side of road (aided by Christine) we had a great view of the runners, some waving, some focused, all lean and determined. And, of course, the always admirable fancy-dress runners made appearances to the delight of the crowd. Just within ten minutes or so I had seen an impressive menagerie: a man painted purple, a tiger, a man in an oversized business suit, and a very warm-looking Thunderbird. As if running a marathon was not hard enough.

We were due to be at Green Park in just over an hour, so Christine suggested we take a walk to Westminster station and go from there. I gamely agreed, and Christine disappeared in a cloud of dust, clearly setting the pace (which would have given Mo Farah a run for his money). The crowds of supporters grew thicker as we barrelled along beside the Thames, and the air was filled with the sounds of banger sticks and the exuberant hootings of air-horns, which created something akin to a party atmosphere. People were for once united in their support for those running – friends, family, work colleagues, as well as total strangers, were offered encouragement as they ran. Water and energy drinks were proffered from the sidelines, somewhere drums beat out a lively rhythm, people sat and cheered from the paws of a great stone Sphinx that lay by the Thames, everywhere was movement and sound, but amongst it

all the runners ran, intent and focused in what must have felt like the eye of a storm.

The Holiday Inn at Mayfair kindly supplied a space for us to use as a hospitality area for our runners, and shortly after we had arrived Christine and I were pleased to welcome and congratulate some of our supporters, helped by an energetic Oliver Gosling (MPS I), his mum and grandparents.



I stopped to speak to one of our runners, Mary Fullalove, who after running 6 marathons previously had managed to run her personal best - an incredible time of 3 hours, 15 minutes. She was accompanied

by her husband and two children, who were rightfully very proud, and who informed me that they had initially been concerned that Mary would be unprepared as a foot injury had prevented some of her training. Obviously they need not have worried, and Mary, who works as a Pilates teacher, looked in amazing shape, and completely unruffled from her 26.2 mile run. Mary chose to run for the MPS Society in memory of Libby Fullalove, and 2014 marked her final marathon.



Leon Batchelor arrived soon after, and settled down with a cold, well-deserved beer. Leon ran a very respectable time of 4 hours 48 minutes,

and although he was disappointed with this time, we certainly were not! Leon was inspired to complete the marathon by Oliver Gosling – and in his typical sparky fashion Oliver was close at hand to offer both congratulations and some friendly teasing.

All our entrants did amazingly well. No one can say that a marathon is easy; it is a huge challenge, which takes commitment as well as endurance, and we at the MPS Society are very grateful for the time and effort this took, and our runners' dedication to raising money for vital research and support, as well as raising awareness of these devastating diseases.

Spending the day with our tireless Chief Executive and meeting our marathon runners, as well as Oliver, made me reflect on the focused determination of the Society, and its supporters and members, a determination that has endured for 31 years with the aim of changing the lives of those suffering with MPS, Fabry or a related disease, as well as their families. **Elkie Riches**

James's Marathon

Whilst I was watching the London Marathon 2013 on TV, BBC News presenter Sophie Rayworth ran across the finish line in 3 hours and 45 minutes. As she did so I said to my wife Jules "if Sophie can run it in that time, then so can I." Thus started my third London Marathon.

The first London Marathon was 3 months after our first son Timothy died on Christmas Day in 1999 following a bone marrow transplant. Timothy was born with MPS I Hurlers in June 1998. Jules and I had decided to run the "London" to raise money for the Anthony Nolan Trust and were accepted in the October. When Timothy died the training for me was a real focus and helped with the grieving process. We ran the marathon together which was a very emotional time, holding hands as we crossed the finish line with thoughts of Timothy and the blessing that he had been in our minds.

The second was in 2007. Again Jules and I ran the marathon together this time raising money for the MPS Society in recognition of the fantastic support they gave us when Timothy died. Family, friends and colleagues were fantastic and we raised over £13,000.



Training went really well and I was running faster than I had done for years. I was consistently running 8 minute miles and the distances were getting longer. I was very confident of achieving my goal. However, whilst on

an 18 mile run in mid-February my body told me that I was getting old. I managed to tear my left calf about 12 miles into the run. A week later I was told it was a grade 2 tear and that it was very doubtful that I'd be in a position to run the marathon let alone get any sort of time. The rest of February and all of March was all about rest and treatment. I couldn't run which was so frustrating.

However, towards the end of March with lots of treatment and loads of prayer I was able to get out and run a mile very slowly. I built this up still with my physio saying I would struggle to get round. However he knew that I was determined so we started talking about a pain management plan and me wearing compression socks!! I built up my training very slowly, conscious that the marathon was only a few weeks away. The week before the big day I entered the Sheffield Half Marathon purely to see how my calf would cope. I ran slowly, prayed a lot and completed the race without any pain. I was now ready for London.

The 13th April 2014 was a beautiful sunny day, a little bit too hot but still glorious. Setting off with the thousands of other runners was exhilarating. I felt really good and maintained a pace that would see me to the finish in about 3 hours 55 minutes. The crowds were brilliant. Even louder and more encouraging than before. Cutty Sark and Tower Bridge came and went and at 20 miles I felt good. I'd run it just

under 3 hours and there was only 6 miles to go. The next three miles saw a bit of fatigue creep in and I dropped the pace to 10 minute miles still confident of finishing in under 4 hours. However, at 23 miles, just having seen our son Luke in the crowd with my brother, I started to get cramp in both thighs. Suddenly I had to stop and stretch as the pain was too much. I started to walk, run, stop, walk, run, stop which saw the miles go by far too slowly.

Reaching Buckingham Palace and turning down the Mall with 365 metres to go gave me the adrenalin rush I needed and I "sprinted" down the home straight crossing the line in 4 hours and 26 minutes. Was I bothered about the time? Not at all. I'd finished and raised over £3,500 for the MPS Society.

My entry for the London Marathon 2015 is in.....next time I'll definitely do it in sub 4 hours. **James Hope-Gill**

Nick's Marathon

In all honesty I did not prepare and train enough for what I was about to take on, it was not a daunting thing at first but as the weeks got closer I was slightly apprehensive of what was to come although excited as this was the biggest run I have ever and may ever do in my life.

The preparation did not seem to be progressing as much as I hoped and I did not want to push myself too hard in case of pre-race injuries.

Put it this way 5 weeks prep is not enough!!!

So I prepared my race bag with sweets and lucozade the night before the biggest event of my life, and then I think to myself "I need to get to bed early", but I couldn't sleep as I was looking forward to what was to come.

Waking up nice and early for a high energy brekkie and we're ready to go!

Arrived at Blackheath at 8am, and it was a nice warm morning, the Heath was already busy with runners from a Rhino to a Womble to the normal runners like me.

Stretching and waiting for the start seemed like forever and it took me 20 mins to get to the Start line from the official start time. At last I had begun the 2014 Virgin London Marathon and I was buzzing!. My aim was to get to half way without stopping which was Tower Bridge.

Going through Greenwich and the Cutty Sark, over Tower Bridge and passing through near Canary Wharf was like a wall of noise with people shouting my name with encouragement nearly every few hundred metres was great.

The spectators and family who lined the streets were fantastic and helped me push on, and I thank them for that!

The marathon is such an amazing thing to do and I would recommend it to anyone. It is a great thing to do but it does hurt, as I was about to find out at about mile 17/18, the muscles and joints in my legs and feet became sore and tight and actually slowing to a walk was more painful than trying to run.

Reaching the last few miles was very hard and seemed to take forever but getting to the last few hundred yards knowing around that last corner near Buckingham Palace was the finish line gave me that boost to pick up the pace, and finally getting to that finish line and receiving my medal was a great feeling full of enjoyment and relief.

Along the way the thought of not finishing never crossed my mind but the struggles that the families of those I am running for did, and this inspired me to finish - if Lily and other poorly children can battle everyday then this Marathon is nothing, and I will complete this.

I thank all those who sponsored me and the public who helped me along the way and I thank the MPS Society for allowing me to take part in the world's best marathon, the 2014 Virgin London Marathon.

If you think you can do it then do it, but please prepare for it! **Nick Brooker**

Hannah & Heather's Quiz Night



Hannah McGhin, along with her friend Heather Trev Scott (ML III), pictured left, hosted a quiz night in aid of the MPS Society, which included a raffle boasting some great donated prizes, such as an iPod docking station, makeup, hair and beauty vouchers and massage vouchers, to name a few.

Hannah was inspired to organise this wonderful event by Heather, having been close friends for 19 years, and managed to get her friends and family all involved. Both Hannah and Heather were overwhelmed and rightly proud of the final amount raised, which came to a spectacular £1145.32. Well done to both of you!

The Power of Pie

Susan Wilkinson from Northumberland held a fundraising event on 29th March, which consisted of a pie, pea and pudding supper and disco.

Susan, who suffers from Fabry, along with two of her best friends, Theresa Hartill and Tracey Robson, also did a raffle, tombola and curry challenge. 250 people attended and the final amount raised was an amazing £3230.

"People were so kind," said Susan. "Individuals, shops and businesses were very generous with donations, and very interested about the disease. It was a very worthwhile night that not only raised money to help the MPS Society, but it also raised awareness of the disease".

Thank you to Susan and her friends, pictured right.



Tell Your Story

As you all know, we at the MPS Society are constantly striving to make more people aware of MPS, Fabry and related diseases. I'm sure you are all familiar with the blank or puzzled expressions engendered by mention of these little known diseases, and this can certainly make fundraising more of a challenge. Any amount of fundraising, whether it be a coffee morning or a marathon raises awareness and causes people to take notice of the work of the Society, and all our supporters do an amazing job of spreading the word. But what more can we do to strengthen the message?

One of the most powerful tools in the fundraising armoury is that of the case study – in other terms, your stories. Far from being a cynical PR stunt, this is all about making people understand what these diseases are from a human point of view; it brings the effects of living with, or caring for someone with MPS, Fabry or a related disease, closer to home and easier to relate to. It will allow someone who has no prior knowledge of these diseases to

immediately understand why the work of the MPS Society is so important and why we need support.

If you feel you would like to help us by writing a bit about how these diseases affect you and your life and how the MPS Society has helped, please do get in contact with us. Perhaps you could describe a typical day as a carer or a sufferer. How has your journey been from diagnosis to the present day? Or maybe you could write about the challenges you face and the help you have received. However you decide to write your story, keep in mind how best to relate your message – imagine you are speaking to a person with no knowledge of these diseases.

If you would like to tell your story or if you have any questions, please email e.riches@mpssociety.org.uk or call 0845 389 9901. Anything we do receive will be used both online and in printed materials to help raise awareness for the MPS Society. We would always respect a request for confidentiality, so names could be changed if required.



Great Run events

We currently still have places available in the BUPA Great Birmingham Run on 19 October 2014. Please email us to register your interest.

Although we have now filled our other 2014 Great Run Events, please get in touch if you would like to register your interest for 2015 - the Great Manchester Run, Great South Run, Great North Run or the London 10k.

Cycle events

Doitforcharity London - Brighton Cycle 2013 - 7 September 2014. Please email us to register your interest.



Other ways to support MPS Society:

If you find yourself short of time and unable to organise any fundraising, but would still like to contribute to the work of the MPS Society, or if perhaps you are already a fundraiser but would like to do more, please see the ideas below that are easy to set up but which are vitally important to us as a charity.

Regular Giving

One of the best ways you can support the MPS Society, without having to actually fundraise, is by setting up a direct debit which allows you to donate automatically once a month. The amount you choose is entirely up to you, but even £3 a month would make an enormous difference to the children and adults who have been affected by MPS, Fabry or a related disease.

With a regular gift of just £3 a month, you could help fund much of our work, including:

- flasks to grow cells for testing the effectiveness of therapies for neurological diseases
- a tree planting and inscription in memory for one bereaved family at the MPS Childhood Wood
- a home visit from a member of the Advocacy Team to an affected family providing practical and emotional support
- a regional family event, such as a trip to Cadbury World, to have fun with other MPS families
- specialist MPS clinics
- groundbreaking research into treatments

Regular giving provides the ideal way to support your charity on an ongoing basis, with minimal interruption to your life, and you can increase or decrease or even cancel your monthly donation at any time.

If you would like the flexibility and the peace of mind that you are making a difference to the MPS Society on a regular basis, you can set up regular giving using the following options:

Online: Set up Direct Debit via the MPS page on Justgiving.com

By post: Download our regular giving form from our website and post it to us

By phone: 0845 389 9901

And don't forget you can make your donation worth almost a third more if you sign up to Gift Aid.

Legacies

Many people wish to include a charity in their Will after they have provided for their families and friends, but according to www.charitychoice.co.uk only 7% actually do. Legacy gifts make a huge difference to charities – in fact the single biggest source of unrestricted gifts to charities in the UK comes through legacies, and despite the low percentage of people who leave a charitable bequest in their Will, legacy gifts are worth almost £2 billion to the sector.

If you would like to know more about legacies, and how to go about leaving one, please visit our website for more information, or send an email to fundraising@mpsociety.org.uk and we will post out one of our 'Leaving a Legacy' leaflets.

Leaving a legacy to the MPS Society would allow us to look to the future with confidence, knowing that we will be able to continue supporting those affected with these devastating diseases, funding projects to help improve the quality of life of sufferers, and keep pressing forward and being a part of the research into treatments.

Please help us to give hope for tomorrow by leaving a legacy to the MPS Society.

Where does your money go?

A donation of £3 per month could help us to offer so much more support in so many ways:

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

Please donate **online** at www.mpsociety.org.uk

phone 0845 389 9901

text MPSS01 £2/£5/£10 to 70070

or **post** your donation to our office, MPS House.

Thank you to all our donors including . . .

Pat Isaac gave a talk to her local WI and collected a donation of £25 in return.

Roz McKenzie held a coffee morning at the Department of Genetics in the University of Cambridge, including a raffle, and raised £128 for the MPS Society.

Thomas Reuters donated £300 as part of their Matching Gifts Program.

Ann Parsons donated £91 after selling MPS Society trolley key rings in her local ASDA supermarket.

Gordon and Mary Mellor set up a donation point at the Horsforth Grove Methodist Church and collected £200 for MPS.

Aisha Seedat and her family raised £635 by fundraising at her 16th birthday party.

Following the loss of her father, **Ceri Coleman** was given 2 donations totalling £45. Ceri's son, Joseph, suffers with AGU.

Monica Bowen sent in an annual donation of £60 in memory of Christine Lavery's son.

Admiral Charity Cards presented the MPS Society with a cheque for £44.60, which was raised by individuals and companies who had ordered personalised charity Christmas cards and had chosen to support us.

Larne High School donated £400 which they raised from some general fundraising. In addition to this, Larne Skills Centre also managed to raise £75, and the Hughes family raised £80.

TwoCan Associates kindly donated £25 to the MPS Society following Christine Lavery's support to their NICE project.

Michael and Ann Haywood donated £20 in support of Leon Batchelor running the 2014 London Marathon for the MPS Society.

Michael Winson donated £10 in lieu of a friend's birthday present.

The members of **St. James the Less Church Wives group** donated £200 to the MPS Society in support of one of their members who has a Grandson with MPS.

Hertfordshire County Council's Central SEN Team held a Bake-Off and raised £270 in support of Jude Butler's grandson, Jacob Carter.

St Nicholas Men's Breakfasts learnt of the MPS Society from two of our fundraisers who cycled from their home town to Paris. This encounter inspired them to raise £140 for MPS.

We received donations of £149.98 in memory of **Mrs Ruth Thirkettle**. We would like to extend our sympathies to Mrs Thirkettle's family.

Louise Lucas raised £185 for the MPS Society when she asked friends and family to donate in lieu of presents for her 40th birthday.

The MPS Society were once again the chosen charity for **Savvy Financial Planning** in Dorset, who wanted to raise money for a local girl, Ella Balestri, who has Sanfilippo type B. This year they completed a 10k run along the Dorset Trailway and donated £225 in sponsorship.

Sheila and Peter Ireland recently celebrated their Golden Wedding anniversary and received a donation to the MPS Society in lieu of a present. They also kindly added further to this amount themselves, raising a total of £100.

Jane, Martin and Ellen Bate took part in the Better Health fun run at Carterton and raised £50 in memory of Ben Illingworth.

William Neil Reid donated £15 in appreciation of a talk given by Christine Lavery.

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

Kate Leatham donated £20 for Ella Bella.

John O'Connor raised an amazing £1025.56 in sponsorship for running the London Marathon for the MPS Society.

Katy Brown raised £900 for the MPS Society from a cake sale and a Girls' Night In. Katy's son, Samuel, suffers from MPS IV.

Clare Brady and Nicola Carr donated £400 to the MPS Society. They are friends with Jordan Mount, who suffers from Hurlers, and they wanted to help after seeing Jordan's daily struggle.

Mr & Mrs Priest donated £50 on behalf of Luke Bown (MPS III).

Luke Bown's (MPS III) Grandmother, **Sue Hollidge**, recently celebrated her 60th birthday and asked her friends and family to donate to the MPS Society in lieu of presents. Altogether Sue managed to raise £1230.

Elizabeth Mee held a coffee morning at her village Parish Hall and raised £250 by selling coffee and cake, and holding a raffle, with prizes donated by local businesses and friends.

Yeoman Park School donated £47.31, which they raised from a cake sale.

The Amersham Trefoil Guild supported the MPS Society for their Annual County Banner Service, and collected donations amounting to £300.20, as well as giving representatives from the MPS Society a stall at the event.

Sandford St Martin's Primary School held a cake sale and managed to raise £167.03 for research into MPS VI research.

Dorothy Robinson and her friends held their 6th Card Workshop and raised £160 for the MPS Society. Dorothy is nanny to Hannah Robinson (MPS III).

Phil Powell donated £65.80 from the collection tin in his haberdashery shop, Bids & Pieces.

Emma Stuart did a sponsored tandem skydive for the MPS Society and so far raised £75, with more yet to come!

Richard Paterson from Thea's Trust kindly donated £390, and **Shirley Brown** donated £10 towards Samuel Brown's (MPS IV) family fundraising.

Eamonn, Nathan (MPS II) and Michelle Worsford completed a sponsored walk with family and friends and raised £460 for the MPS Society.

The Royal Bank of Scotland, Sunderland Branch have raised £557.65 from various fundraising activities for the Genistein Trial.

The ASDA Foundation donated £500, which was thanks to Katy Brown's fundraising at ASDA House.

Kirsty White took part in the Tough Mudder raising £800 on her justgiving.com page. Her company kindly sponsored an extra £75.

Thank you to Marina and Friends

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



 Find us on
Facebook
www.facebook.com/mpssociety

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Give As You Live

Shop online with Give As You Live. Thousands of retailers have signed up to donate to the MPS Society a percentage of every online purchase you make, without adding to the cost of your shopping. For more information and to start shopping head to <http://www.giveasyoulive.com/join/mps-society/63954/150009>



Wear it Wicked for some Fangtastic fundraising for the MPS Society

Halloween is a fantastic time of year to have fun and raise money and awareness for worthy causes. So we have lots of gruesome ideas for you...

You could hold a small ghoulish gathering with apple bobbing and pumpkin carving at home or perhaps you feel brave enough to hold a broomstick ball, or a deadly disco. You can even get Wicked at work or school and get everyone to donate a £1 and get fiendish with their wardrobe.

Here are some ideas...

- Donate a £1 and Wear it Wicked to work or school
- Hold a dusk Wicked Walkabout taking in local spooky haunts
- Guess the weight of the pumpkin
- Hold a fright night film evening and ask your audience to 'pay per view'
- Hold a Wicked cake sale with morose morsels

Email us at fundraising@mpssociety.org.uk if you would like a Wear It Wicked poster. Don't forget to send us your fright night pictures and stories so we can share them.



Donations

Mrs A Baker; Mr S Michael Brecker; J.J. Berry; The Shaheelan family; Valerie Firth; Mrs Anne Palmer; Holly Nowell; Mrs D.McLeod; VL Warrington; Mrs P. Mitchem; A J Green; James Stewart; Norton Rose Fullbright LLP; Ipsos Mori; Etonbury Academy; Dr C J Osbourne & Partners

In Memory

Edward Nowell; Steven Harriss; Alan Mitchem; Roy Parkinson; Mr Deryck Crespin; Alec Evans; Roy Parkinson; Mrs Maureen Patricia Fuller; William Ferrier

Collection Boxes

Ian Evans and his local post office; Frutin Travel

Stamps, foreign coins, mobile phones, ink cartridges; jewellery

Mrs D. Bowen; Your Derwent and Solway Housing Association; Ian Evans and Arriva The Shires; Glyn Goodman; Mrs A Baker; Lynne Grandidge of CSC Ltd; Sue Hollidge; Mrs Jan Smith

The Society would like to thank the following donors for their regular contributions by either Standing Order or Give As You Earn.

W Cavanagh; A Sabin; B Harriss; L Brodie; A Ephraim; J Dalligan; M Malcolm; E Mee; S & D Greening; K Brown; Mr Hahner; Z Gul; E Brock; M Fullalove; G Ferrier; R Taylor; R Gregory; C Pierce; K Brown;

E Moody; S & J Home; V Little; J Casey; E M Lee; D Palmer; J & V Hastings; C Gibbs; A Cock; A Dickerson; N Saville; S Bhachu; I & A Hedgecock; C Cullen; R Dunn; M Tosland; S Littledyke; J P Martin; M Kalsi; D Holmes; D Forbes; P & R Shrimpton; G Simpson; A Weston; E White; C L Hume; E M Brock; R & E Parkinson; R & K Henshell; K Bown; M Reeves; R & K Dunn; K Seeber; D Forbes

We would like to thank all our fundraisers and supporters who kindly set up online pages to collect donations via **Justgiving.com** and **Virginmoneygiving**. Once your event is over and you have finalised the amount you have raised, we would love to feature articles and photos about your wonderful fundraising. Please email them to fundraising@mpssociety.org.uk.

Wicked Walkabout

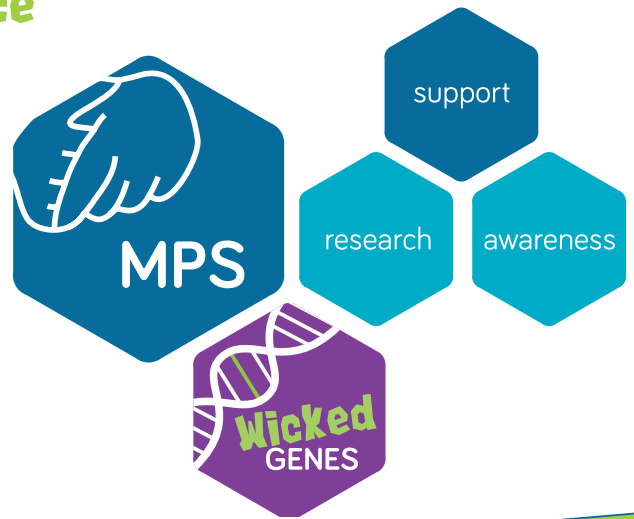
Join our Wicked Walkabout in aid of the **MPS Society**

- together we can make a difference

Get your trainers, walking boots and wellies on...

We are holding a Wicked Walkabout to raise awareness of Mucopolysaccharide (MPS) and related diseases. These are rare, genetic conditions which are devastating to the families they affect.

Have fun, get fit and help the MPS Society to support affected families, fund vital research and raise awareness of these diseases.



Date and time of our Wicked Walkabout:

Location:

Distance:

Our Wicked Walkabout is a Walk/Run/Cycle/Wellie Waddle



To take part, contact your Wicked Walkabout organiser:



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

www.mpssociety.org.uk

Registered office at MPS House, Repton Place, White Lion Road, Amersham, Buckinghamshire, HP7 9LP, UK
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