

MPS magazine

Society for Mucopolysaccharide Diseases

Winter 2016 • www.mpsociety.org.uk

BOOKINGS BEING
TAKEN NOW
FOR UK MPS
CONFERENCE

Festive fun

Photos from all the
seasonal events

MPS VI MEETING

Maroteaux-Lamy
conference round up

MEET OUR MARATHONERS

Meet those going
the distance for MPS

NEW GLOSSARY

Do you know your
DOLS from your SALT?

MPS and related diseases

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

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

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

The MPS Society

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

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

Board of Trustees

Chair – Paul Moody

Vice Chair – Wilma Robins

Trustees – Tim Summerton, Judith Evans, Judy Holroyd, Bob Stevens, Bryan Winchester, Jessica Kafizas

Registered Address:

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

Registered as a Company
limited by guarantee in
England & Wales No. 7726882
Registered Charity No. 1143472
Charity registered in Scotland
SCO41012

The MPS Society Team

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

Group Finance Officer Gina Smith
g.smith@mpssociety.org.uk

Advocacy Team Manager Sophie Thomas
s.thomas@mpssociety.org.uk

PA to Group CEO/FIN Coordinator Toni Ellerton
t.ellerton@mpssociety.org.uk

Senior Advocacy Support Officer
Debbie Cavell d.cavell@mpssociety.org.uk

Advocacy Support Officer Steve Cotterell
steve.cotterell@mpssociety.org.uk

Advocacy Support Officer Rebecca
Brandon r.brandon@mpssociety.org.uk

Advocacy Support Officer Alison Wilson
a.wilson@mpssociety.org.uk

Advocacy Support Officer Louise Cleary
l.cleary@mpssociety.org.uk

Trust & Grant Fundraising Officer
Sue Cotterell s.cotterell@mpssociety.org.uk

Office/Finance Administrator Martine
Tilley m.tilley@mpssociety.org.uk

Fundraising & Information Officer Helen
Crawley h.crawley@mpssociety.org.uk

Magazine deadlines

Spring 1 March 2017

Summer 1 July 2017

Autumn 1 October 2017

To submit content email:
magazine@mpssociety.org.uk

The articles in this magazine do not necessarily reflect the opinions of the MPS Society or its Management Committee.

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

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Contact us

mps@mpssociety.org.uk
www.mpssociety.org.uk

T: 0345 389 9901

F: 0345 389 9902

**Out of Hours:
07712 653258**

Been struggling to
get through to us?
Make sure you've
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Mobile phone scam

We have had reports of a mobile phone scam from a company called Hot Prizes. If you are asked to click on any prizes for MPS Helpline then this is nothing to do with the MPS Society.

We advise you to ignore any phone messages like this and if you do have any concerns you should contact your mobile provider.



MPS VI MEETING

All the news from the patient and expert meeting devoted to MPS VI written by one of our trustees and an advocacy officer



FUTURE IN OUR HANDS

Support for teenagers and young people



OUR MARATHONERS

Meet those going the distance for MPS

WELCOME

Happy new year! This issue is full of your fantastic fundraising events and we are so grateful for the amazing work you have done at a time of year when you are no doubt extremely busy.

We also have plenty of photos from all the Christmas events from 2016 so if you attended any see if you can spot yourself.

There is a round up of the expert and patient meeting on Maroteaux Lamy disease (MPS VI) and plenty of new information about research and treatments, including NICE's final decision to approve Migalastat for Fabry.

With this issue you will find our programme for the MPS Society conference on 7th–9th July 2017. Have a look and book it in. We can't wait to see you there!

From the Group Chief Executive



*Christine Lavery
Group Chief Executive*

One of the highlights of the past three months is the first ever International MPS Consensus Conference for Cognitive Endpoints in MPS I, MPS II and MPS III held at Heathrow 1–3 December and co-organised by Shapiro and Delaney LLC, the UK MPS Society and the US National MPS Society. This three day meeting supported by 18 pharmaceutical companies brought together clinicians, psychologists, scientists and medical representatives of the pharma industry from across the globe along with Dr Patroula Smpokou and Dr Wen-Hung Chen from the FDA (Food and Drug Administration), Dr Caroline Auriche from the EMA (European Medicines Agency) and representative patient advocates from the USA, Canada and UK.

The goal was to explore the challenges and solutions in collecting cognitive endpoint data for MPS I, MPS II and MPS III. Reflecting on the challenges for families of bringing their MPS children for psychological testing for their clinical trial Kate Delaney spoke on how 'Every Data Point Counts'. Kate also spoke of the crucial input from parents in a cohort of patients where the child being tested may have sensory and or behavioural challenges. Hanneke van de Lee from the University of Amsterdam spoke on her research on new methodologies for clinical trials for small population groups that recognise that generic outcome measures are not usually responsive.

The FDA and EMA speakers set out how they respond to appraising new therapies for marketing approval in ultra-rare diseases. Many other eminent speakers continued to set the scene before the meeting moved on to a full day Delphi process of panel discussions on the Cognitive endpoints for MPS I, II, and III. Parallel workshops included Patient Reported Outcomes, Newborn Screening and Early Identification of MPS diseases and the Basics of Reliable Cognitive Assessment.

The MPS VI Expert Meeting for families and professionals held 15–16 October in Northampton was well received and hugely successful. It brought together parents, adults

with MPS VI, carers, clinicians, psychologists and scientists and was expertly chaired by Paul Moody (MPS VI dad and Chair of the MPS Society) and Dr Fiona Stewart. The panel discussion session was engaging for all present. The MPS Society has previously hosted an Expert Meeting for MPS III, MPS IVA and ML. Subject to funding we are planning Expert Meetings for MPS I and MPS II in the near future.

All these meetings are vital to achieving improved clinical and treatment outcomes but as the MPS Society we are also committed to putting a spark in the lives of our member families and affected adults. The MPS team have worked hard throughout 2016 to raise the funds for regional and national events. During this festive season some of you will have enjoyed the pantomime Dick Whittington in Manchester, Sunday lunch with Santa in Newcastle, a Christmas experience at Glenarm Castle, Northern Ireland and as I write families have just returned from a date with Santa Claus in his North Pole home in Arctic Lapland. The pictures later in this magazine are testament to the magical time had by all and thanks must go to the donors who made this possible; the Gosling Foundation and a gift from MPS Commercial.

For those not able to go to the North Pole, families enjoyed seeing Father Christmas during a day at Lapland UK in Windsor. Feedback tells us these events are important to our membership so if you have ideas on activities you would like to participate in during 2017/2018 please do email your thoughts to the events team: g.smith@mpssociety.org.uk

Finally, in the last MPS Magazine, on behalf of the Board of Trustees, Life Members (parents, adults with an MPS, Fabry or related disease or partners of) were invited to consider putting themselves forward for a position of Trustee to the MPS Society. We are pleased that we have had some level of interest but as we strengthen the Board of Trustees going forward, and when Christmas is over, we would really encourage our Life Members, particularly with an accountancy, financial, business or HR background, to consider exploring this further. Please do consider Trusteeship and feel free to email me if you would like to know more: c.lavery@mpssociety.org.uk

I wish you all a peaceful New Year

Christine Lavery MBE

For more information on the Delphi Process, which will play a very important role in defining cognitive endpoints across the MPS I, MPS II and MPS III clinical trials when evidence is presented for marketing authorisation and NICE appraisal see page 31.

News from the Board of Trustees

The Society's Board of Trustees meet regularly. Here is a summary of the main matters discussed and agreed at the Board meeting held on 1–2 July 2016 and 23–24 September 2016

Governance

The Trustees had an open discussion on Trustee recruitment and agreed to actively pursue new Trustees to serve the Board in 2017. Following the resignation of the MPS Society's Chair, Sue Peach, in September Paul Moody was appointed Acting Chair.

Personnel

The Board of Trustees agreed a way forward in respect of the Auto Enrolment to Pension Scheme. The Group Chief Executive advised Trustees that Emma Henry has moved on after a year in the Fundraising and Communications Team and Karen Minashi, would be leaving to be a full-time mother. Trustees wished both very well.

Financial Management

In July and September the Treasurer, Judith Evans, presented her report and this was followed by reports on the, Profit & Loss, MPS Cash flow and Financial Management Reports. Trustees were advised that the dormant accounts for Wicked Genes have been filed with Company House. The Trustees agreed the named persons for People with Significant

Control (PSC) at Company House in respect of The MPS Society and Wicked Genes. In July 2016 the MPS Consolidated Budget was reviewed and Trustees noted that £533,243 was still to be raised and sought reassurance from the Group Chief Executive of where these funds will come from. A paper on mortgage options for MPS House were considered and following the September meeting the mortgage papers were duly signed.

Risk Management

The proposed IT upgrades were approved. The Business Continuity Plan (BSP) and Health and Safety Report were noted. The Group Chief Executive confirmed provider changes to the BSP. Trustees reviewed the Risk Register and noted the arrangement for the Group Chief Executive's planned absence.

Income Generation

The generating income reports were noted and the Board of Trustees acknowledged the high percentage of success with Charitable Trust grant applications.

Policy Strategy

Trustees received a draft of the MPS Society's three year Strategic Plan and it was agreed one Trustee take the lead and consult with the Senior Leadership Team. The final draft needs to be ready by the November 2016 meeting. The following policies were reviewed and approved:

Server Back Up Policy; Home Working Policy; Conduct Policy for Trustees; Use of Electronic Equipment Away From the Office; Equality and Diversity Policy; Whistle Blower Policy; Flexible Working Policy

Research Grants

The Group Chief Executive appraised Trustees of the current research initiatives funded by the MPS Society. Trustees approved a grant of £46,650 to *Study the In-depth Characterisation of Fabry Patients with Cardiac Devices to Predict Risk of Malignant Arrhythmia and Sudden Cardiac Disease*.

Advocacy Support

The MPS VI Expert Meeting planned for 15–16 October was agreed subject to restricted funding being achieved. Trustees noted that Sophie Thomas has raised in her report each of her Advocacy Worker's caseloads exceeding recommended levels for Social workers. Trustees asked the Group Chief Executive to obtain recommendations as to how this can be managed and report back at the next Board of Trustees meeting in November.

International Collaboration

The Group Chief Executive spoke of the discussions around the Future of the MPS International Network that took place in July in Bonn and gave feedback on plans for the first meeting of the MPS European Network.

WHAT'S ON?

Regional Clinics

Great Ormond Street Hospital

MPS IV – 28th Mar, 25th Jul, 10th Oct

MPS I BMT – 28th Feb, 27th Jun, 12th Sep, 28th Nov

MPS III – 14th Mar, 11th Jul, 26th Sep, 12th Dec

Birmingham Children's Hospital

MPS IV – 20th Jan, 17th Mar, 15th Sep

MPS III – 24th Feb, 21st Jul, 18th Aug (afternoon)

Transition clinic – 24th Apr (afternoon), 29th Sep (afternoon)

Fabry – 24th Apr (morning), 19th May, 20th Oct (afternoon)

MPS I – 16th Jun (afternoon)

BMT – 16th Jun (morning)

Mixed clinic – 18th Aug (morning)

MPS II – 17th Nov

MPS VI – 29th Sep (morning)

Queen Elizabeth Hospital Birmingham

Adult Fabry – 10th Jan, 14th Feb, 14th Mar, 11th Apr, 9th May, 13th Jun, 11th Jul, 8th Aug, 12th Sep, 10th Oct, 14th Nov, 12th Dec

Manchester Children's Hospital

Post HSCT clinic (over 6 years) – 20th Jan, 28th Apr, 14th Jul, 13th Oct

Post HSCT clinic (under 6 years) – 27th Jan, 7th Apr, 7th Jul, 6th Oct

Conferences and Regional Events

MPS Awareness Day

Monday, 15th May 2017

5th Fabry International Network Meeting • Athens, Greece

19th–20th May 2017

MPS Weekend Conference 2017 • Hilton, Coventry

7th July–9th July 2017

15th Annual International Symposium on MPS and Related Diseases • San Diego, California

1–4 Aug 2018

Announcements

NEW MEMBERS

Naomi has recently been in contact with the Society. Her son Rupert has a diagnosis of Hunter Disease. Rupert is 2 years old. The family live in Suffolk.

Christine has recently been in contact with the Society. She has Fabry Disease. The family live in the Chester area.

Gemma and Gavin have recently been in contact with the Society. Their son Percy has a diagnosis of MPS II Hunter Disease. Percy is 2 years old. The family live in the West Midlands.

Marie Schulz has recently been in contact with the Society. Her son Cole has a diagnosis of MPS III Sanfilippo Disease. Cole is 8 years old. The family live in the north of England.

Mr Ali has recently been in contact with the Society. His son Mustafa has a diagnosis of MPS III Sanfilippo Disease. Mustafa is 7 years old. The family live in the South East of England.



Congratulations to Angela and Alan on the birth of their beautiful boy Miles Alan Cawkwell who was born on 22 November 2016 weighing 9lbs 2ozs.

Angela wrote to tell us that Miles is not effected by MPS but a healthy carrier like his mum and dad.

“He is little brother to Martino, Abi, Mia and our late son Max Cawkwell who had MPS 1 and passed away during a transplant operation in January 2015 at 13 months old.”

Congratulations



to our Group Chief Executive, Christine Lavery, who will be awarded the Patient Advocate Leader (PAL) Award 2017 by the WORLDSymposium.

13th Annual WORLDSymposium
13-17 February 2017 • www.worldsymposia.org

Bereavements

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

Katy King who suffered from MPS III Sanfilippo and passed away on 26 August 2016.

Luke Chapman who suffered from MPS III Sanfilippo and passed away on 5 October 2016.

We have been informed that Edward Allnutt who had Fabry has passed away.





First class!

Steve Cotterell, one of our advocacy support officers, has completed a BA Hons degree in Social Work through the Open University. He achieved a first class degree which was well deserved after the 5 years he spent studying. We are pleased to have him back in the office full time after finishing his placement at Buckinghamshire County Council which was part of the final year of the course. Steve will be joining HCPC as a registered qualified social worker.

New Chair of Trustees announced

Paul Moody was elected Chair of the MPS Society Board of Trustees on 26 November 2016. We are really pleased to welcome him to the role.

Paul and his wife, Dawn, and three children became involved with the MPS Society when his son Ollie was diagnosed with MPS VI. Paul phoned the Society for advice and his first contact was with Christine directed the family to Professor Wraith and the Manchester team.

Paul has a background in engineering, has run his own engineering automation company and now works as a business development consultant recovering and managing companies for a large group of manufacturing and automation outlets. He has been a member of the Board for a number of years and was chair of the MPS VI expert meeting earlier this year.



Thank you to Sue Peach

Sue Peach stepped down as Chair of Trustees in September after 16 years as a Trustee and 5 of those as Chair.

Over this period Sue has steered the MPS Society through a period of advancement that has put the MPS Society at the forefront of pioneering advocacy and campaigning for our members in the ultra-rare disease voluntary sector. We thank Sue for her time and commitment and wish her and her family the very best for the future.

Celebrating 35 years of the MPS Society

MPS research diagnosis support

Wicked GENES

MPS Society national conference

7-9 July 2017, Coventry, UK

save the date

Advocacy

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

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

Telephone helpline

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

Disability benefits

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

Housing and equipment

We take a major role in supporting and advocating appropriate housing and home adaptations to enable the needs of an individual with an MPS or related disease to be met. Where requested, we can provide comprehensive and detailed housing reports based on individual need.

Education

We help members to access appropriate education and adequate provision for its implementation. This is achieved through providing educational reports used to help inform and educate professionals, and in many instances, to inform Statements of Special Educational Need. Where requested, we also provide information days/talks to schools and relevant professionals.

MPS careplans

We undertake a comprehensive assessment of the issues which

need to be addressed when caring and providing support to a specific individual diagnosed with an MPS or related disease, as well as other family members, by producing a careplan.

Respite care

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

Independent living/transition

We provide advice, information and support through the transition from child to adult services. This could include access to independent living, learning to drive, further education and employment.

Befriender service

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

Bereavement support

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

Advocacy Resources

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

- Life insurance
- Travel insurance
- Hospital travel costs
- Disabled access holidays
- Carers legal rights
- Carers allowance
- Wheelchairs and flights
- Guide to housing and disabilities facilities grant
- Benefits including, Personal Independent Payment, Benefit Cap, Council Tax Benefit and Universal Credit.

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



Sophie

Manages the MPS Advocacy Team



Debbie

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



Rebecca

Fabry, MPS II Hunter, ML III/ML IV, Mannotidosis, Fucosidosis



Louise

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



Alison

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



Steve

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

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

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

Our recent work

Wow! What a busy year 2016 has been for the Advocacy Support Service. In January we welcomed Louise to the team, Steve went on placement for 6 months and is now a qualified social worker (well done Steve!) and Alison became a mum and is currently on maternity leave. During this time we have seen a rise in support requests particularly relating to Personal Independent Payment (PIP), Employment Support Allowance (ESA) and the new Education Healthcare Plan (EHP).

The team not only has to have knowledge of both children and adult services and laws but also knowledge of all areas of need from medical to social care, housing, occupational therapy and education. There are not many professionals with this level of expertise and I am very fortunate to have such a dedicated professional team, who are willing to travel by any means necessary, sometimes involving early mornings, nights away and – given our location – many hours sat on the M25!

The advocacy team always tries its best to meet the needs of its members but as you can appreciate we are only a small team and although we try our best we do not always reach everyone.

This is where we need your help. Please! Please! Please! If you need any help or advice let us know. Most of our urgent work is taken up by crisis intervention which can sometimes be prevented with one phone call for advice or early support.

So do contact us. Either pick up the phone, email us, send us a message via Facebook or tell us if you would like to request that we periodically call you.

As manager for this service, I am immensely proud of my team for all their achievements and support to our members and for providing a professional service not only across the UK but Internationally too. 2017 is already looking to be a busy year but we will continue to go above and beyond to ensure the needs of our membership is met.

Sophie Thomas, Advocacy Support Team Manager



Areas of responsibility

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

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

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

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

☎ advocacy@mpsociety.org.uk

@ 0345 389 9901

f [facebook.com/mpsociety](https://www.facebook.com/mpsociety)

Clinics



1. Yousaf



2. Tia

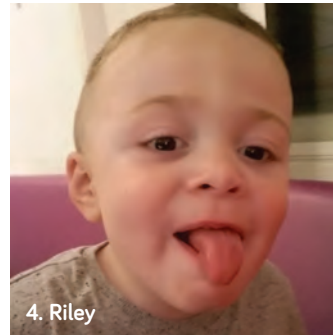
**BCH
MPS IV
16 SEP**
(1-3)



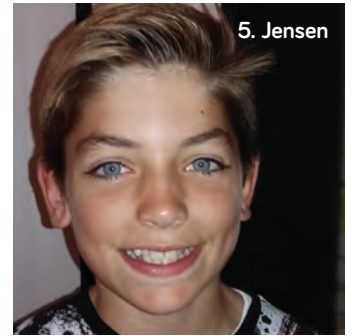
3. Juveira

I was glad to arrive in sunny Birmingham following the storms and flash flooding we experienced close to the MPS Society office in Buckinghamshire. It was good to meet with our members and see how they are getting on.

*Debbie Cavell
Senior Advocacy Support Officer*



4. Riley



5. Jensen



6. Jensen

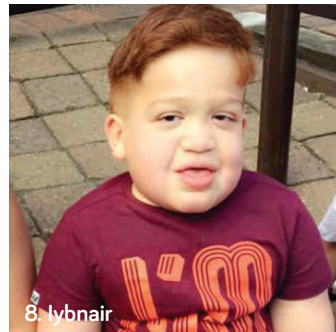


7. Joseph

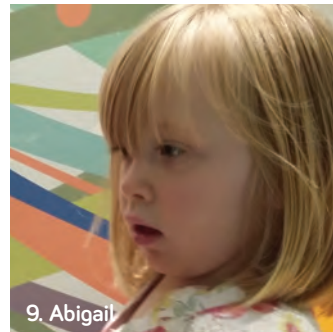
Well it was another busy clinic at BCH. Noisy and chaotic but in an organised manner, as always, from the lovely team at BCH. The families and children love to get together and have a catch up with all the news. For me it is scary on how much the children have grown especially if you have missed them at a clinic and it's a year since you have last seen the children.

Looking forward to seeing you all in 2017.

*Rebecca Brandon
Advocacy Support Officer*



8. lybnair



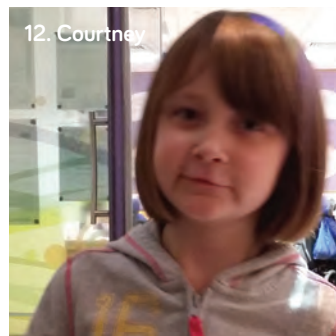
9. Abigail



10. MuQuadas



11. Zack



12. Courtney

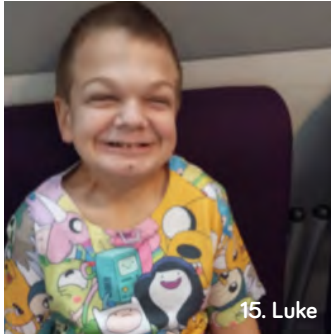
**BCH
MPS
14 OCT**
(4-12)



13. Alicia



14. Thomas and dad



15. Luke



16. Lyla

**RMCH
MPS I
7 & 14 OCT**
(13-19)



17. Steven



18. Cody

It was lovely to meet with all the families at the October Manchester Children's Hospital clinics and see so many smiling faces.
*Debbie Cavell
Senior Advocacy Support Officer*



19. Melissa and Rachel



20. Tabeer

**GOSH
MPS III
13 SEP**
(20-21)



21. Patryk



Elizabeth shares her experiences of starting high school and the determination it gave her to raise awareness of her condition

In September 2015, I moved into Year 9 at high school.

Now, moving to high school is challenge enough for the average 13 year old and making new friends is daunting at the best of times, but when you're trying to get your head around a new school layout, new teachers and a whole new range of subjects in addition to living with MPS, the challenge is even greater.

But, I've never been one to run from a challenge and to add a bit more pressure, I arranged, together with the support of my Head Teacher, to deliver a series of presentations on MPS, to my Year 9, 10 and 11 peers. In all, three presentations to approximately 900 students.

I wanted to give my friends and peers a little glimpse into my world. In talking candidly and honestly about living with MPS, I knew I had an opportunity to raise both awareness about this rare, incurable condition as well as much needed funds for the MPS Society.

I worked on my presentation during the summer weeks and I have to admit, I was a bit anxious about the task ahead. Then the big day came. Was I worried about their reaction? Yes. Was I concerned if they would support me? Yes. But as soon as I started my first presentation, I discovered I had absolutely no need to be worried or concerned about anything.

My three presentations went without a hitch. My audiences listened intently. You could hear a pin drop. When I had finished, the response and support I received from them was overwhelming and beyond anything I could ever have imagined or hoped for.

During my first year at High School, this fantastic support continued and within the first term, my friends, teachers and I, organised an inter-class Witch's Hat Design Competition in conjunction with a loose change collection. It was fantastic! We raised just under £200 for the MPS Society. I was so happy and so grateful to the number of friends who emptied their pockets and contributed to such a worthy cause – all because they had been made aware of MPS and wanted to help.

I might have known that this help and support wasn't a one-off. The support of my school was displayed once again, in June 2016, just as my time in Year 9 was coming to an end. The last day of term is given over to celebrating the school year and we usually finish off with a school fair. I came up with a brilliant fundraising plan and put it into action. I had gained my school's support to launch 1000 helium-filled, latex, blue balloons to retrospectively mark Global MPS Awareness Day which is held on May 15th every year. All the arrangements were in place and I had already attended several logistical meetings with my teachers. However, shortly before our magnificent event was due to happen, a range of environment problems regarding helium balloons were brought to our attention. Little did we know, latex balloons pose significant dangers to wildlife and rural animals, as not all the balloons break down into tiny pieces and animals can eat and choke on these pieces. Living in Northumberland, surrounded by open countryside and farms we knew the dangers were too great to take that risk. With great sadness, we had to abort our balloon launch. But, on balance, we knew we were making the right decision in order to protect our amazing countryside.

Disappointed, but never the less determined, we changed course and arranged a non-uniform and cake bake day. It was fantastic and was just as successful as the balloon release would have been – as well as being environmentally friendly! Together, with additional proceeds from my JustGiving page and a £250 donation from our local Rotary Club, we raised a total of £1957.89 for the MPS Society!

I could never have achieved all of this without the amazing help and support of my friends, peers, teachers, Head of Year, Pastoral Manager and Head Teacher. I had no idea that my first year at High School would be so brilliant! I am so grateful to have been given the opportunity to put a spotlight on MPS within my high school and to have been given the platform to raise awareness about such a devastating range of conditions. I know with full certainty that my school will continue to support my efforts to help those living with and dying from MPS. This condition is very close to my own heart and I am humbled by how much everyone cares.

Elizabeth

“ I had no idea that my first year at High School would be so brilliant!

Names and images in this article have been changed to protect the identity of the author.

Your stories

MPS VI at school

Mrs Sadaf Imran wrote to tell us about her two daughters, Wania and Manahil, who have MPS VI and how well they have coped their condition and school.

Wania had HALO Vest Surgery on 7 June 2016. It was a big major operation which took more than 6 hours. She then had to wear the HALO Vest for 4 months. She had a very tough time but she accepted the HALO and started going back to school as this summer was her last days in her primary school and she didn't want to miss them. After her summer holidays she started to go to her new secondary school which she was very excited to attend with her elder sister Manahil. She finally had her HALO removed but she has to wear her collar for at least 6 more months.

Manahil is 13 years old and enjoying her second year at secondary school. She is a brilliant student with highest praise points in every term and in the highest tutor group. She has lots of friends, some of which she shares with her sister, Wania.



A special retirement

When Linda Pack, whose daughter Lois has Hurler-Scheie, retired from the hospice where she worked she drew parallels with the work she did and that offered by our advocacy team.

"We had a wonderful retirement celebration and I gave a brief overview of MPS and how the society has helped us over the last 26 years since Lois was diagnosed. As I was writing some notes, I realised that what the MPS Society has done for us, runs parallel to the service we provide for our patients.

"From supporting families at diagnosis with a 24 hour helpline, to form filling, and helping us obtain information as we needed it, you enabled Lois to experience appropriate education and assisted her growth to adulthood.

"Without the support of the Society, our life with Lois would have taken a different and much more difficult path. Our family, want to thank the Society and the members who have given us so much help over the years."

Linda asked the rather than a retirement gift her colleagues donate to the MPS Society and raised £220. Linda told us it was the first time her and her colleagues had raised for a charity other than the hospice where they work. We are very grateful and honoured.

Did you know that in 2016 you opened 107 new fundraising pages, made over 2500 donations and raised £86,717 on online fundraising platforms? Thank you!

Did you know

ATTENTION TEENAGERS AND YOUNG ADULTS

“The Future is in Our Hands”: Advocacy Support Service for MPS teenagers and young people

We are delighted to have secured funding from several grant-making trusts including the Rayne Foundation, the Hobson Charity and the Shauna Gosling Trust, which is being put towards a really important area of our work: our Advocacy Support Service focusing on teenagers and young adults with MPS, Fabry and related conditions.

One aspect of this work will involve our young people who are developing the skills and confidence to live as independently as possible and working towards achieving their ambitions.

You are the future of the MPS Society – we want to ensure we are providing the services you want and need, and giving you the voice you deserve.

Last year we gathered some thoughts from some of our members in this age group. Comments about what they want to achieve and the challenges they see ahead included:

“Living on my own”

“Passing my driving test”

“Getting a job”

“Change the way people see disabled people”

“Getting my mum to accept the possibility of independent living [for me]”

“Money, financial independence”

“Lack of work opportunities”

“Getting adequate support from my university and getting tutors to listen”

“That I would be understood”



Aims

This is what we want to do:

- Review your current experiences of life as a teenager (13–17) or young adult (18–30) with MPS, Fabry or a related condition
- Seek to understand and act upon your needs and aspirations
- Provide advocacy support and advice in areas including secondary and further education; access to training, volunteering, employment; accessible accommodation and independent living, and encouraging you to reach your potential
- Develop information resources tailored to those at transition stage and young adulthood
- Provide social and confidence-building opportunities
- Engage with you and seek your views to shape our services for this age group and the future direction of the MPS Society and other services, through questionnaires and discussion groups

Outcomes

The outcomes we want to achieve in the next few years are for you to:

- Be on the path to achieving greater independence, with strategies and support to achieve your full potential – this might involve taking up educational, volunteering and employment opportunities and making positive choices about your life
- Have increased confidence and a support network of other young people with similar life experiences, reducing feelings of exclusion and isolation
- Have information resources focusing on the needs of your age group – to put what you need to know into your hands in a way you can relate to
- Have a voice to shape your future – sharing your views and ideas to influence the direction services take



So, if you are
a teen or
20-something
The Future is in
Your Hands

Your views

Sophie (Advocacy Support Team Manager) and her team want to hear from you:

Recently a brief “Survey Monkey” questionnaire was sent out to all member families by email – we really want to hear from our young people so please do take a few minutes to give us your views if you haven’t already. If you are under 18 it will have been sent to your parents’ email address, and if you are over 18 it will have been sent to your own email address if we have it. If you haven’t received an email about this, please make sure we’ve got your email address – call us on 0345 389 9901 or email mps@mpssociety.org.uk.

Or if you want to send your comments, thoughts and ideas directly to Sophie, email s.thomas@mpssociety.org.uk.

Your input will make a difference to the direction of our work for your age group in the next few years.

Just for you...

We are planning various activities specifically for our teenage and young adult members over the next couple of years so look out for information about these and get involved!

We are already organising sessions just for our young adult members at the 2017 National Conference in Coventry (7th–9th July) – make sure you come along for opportunities to learn, socialise together, discuss issues that matter to you, and share your views as a young person with MPS, Fabry or a related condition.

Young people who have attended previous MPS events and conferences say it made them “More confident in making friends” and that “Going away without a member of my family” was an important achievement.

We hope as many of you as possible will engage with this work and benefit from it in the next few years.

Childhood Wood



On 16th October we gathered on a rainy day for our annual tree planting ceremony at Sherwood Pines Forest Park but, as always seems to be the case for the Childhood Wood, the sun came out in time for the service.

We started the day with lunch at the Clumber Park Hotel which gave us a chance to come together and families enjoyed the opportunity to share memories of their loved ones with other families. After lunch we met at the woods and walked, with balloons wafting in the air, to the memory boards. Commander Judith Swann, a local dignitary who has been present at our planting ceremony for a number of years, and Christine Lavery (Group Chief Executive) welcomed everyone. Wilma Robins (MPS Society Trustee) then read the poem Remember by Christina Rossetti.

After taking a moment to view the memory boards and appreciate the surroundings we released the balloons with their personal messages attached. This was followed by the planting where each family were given an oak sapling to plant and then spent time contemplating their loved ones. Trees were planted on behalf of the families who could not be present.

Thank you to the families who attended and made the day so special. Those being remembered were: Harry Brennan, Hayleigh Reynolds, Helen Skidmore, Jack Stuart, Lorraine Helen Rock, Marwan Al Obaidi and Thomas Edward Farwell.

Thank you as well to Christine, Wilma and Judith for being part of the day and to the forest rangers for all their hard work caring for this special place.

Steve Cotterell, Advocacy Support Officer



Spring Ball

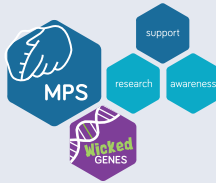
raising money for the
Society for Mucopolysaccharide Diseases (MPS Society)

Saturday 29th April 2017
The Crown Hotel, Harrogate, HG1 2RZ

Arrival at 7pm, music till midnight, bar open till 12.30am
Reception drink, 3 course dinner and dance, charity raffle & auction

Black tie/lounge suits

£40 per person (tables seat 8–10 guests)
To book tickets please email Katy at mpsspringball@gmail.com



PayPal Giving Fund

Have you ever considered fundraising for the MPS Society, but just can't find the time? **PayPal Giving Fund** makes it easy for you to support the MPS Society whilst at home or on the go. It offers a giving programme for some of the world's leading online shopping sites such as PayPal, eBay and Humble Bundle by collecting the donations made by online shoppers and distributing it to the selected charities on a monthly basis.

eBay for Charity makes it easy to support the MPS Society when buying and selling in one of the world's largest marketplaces. You can donate a portion of your sales or add a donation to purchases during checkout.

Humble Bundle, the leading retailer of digital games and eBooks, gives you the opportunity to support us as well. Through an innovative "pay what you want" pricing model, you will be able to choose not only what you want to pay, but also what portion of that purchase is donated through PayPal Giving Fund to the MPS Society.

Support the MPS Society the next time you do your shopping online. It's just a few clicks away!

What do you think?

There is still time to shape the future of the MPS Society and tell us what you think by filling in our member's survey.

We are constantly monitoring the services and support we offer to make sure they meet the needs of those accessing them and your feedback is essential in making sure we deliver what you need.

The survey will only take a few minutes to complete so please follow the link below and give us your feedback.

www.surveymonkey.co.uk/r/TFRPM37

Events

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

15–16 October 2016
Hilton, Northampton

The great advantage of a meeting devoted entirely to a single MPS disorder is that it can focus on the unusual or even unique clinical aspects of that particular disease. These symptoms may just be regarded as rare or not very important in the context of a meeting covering all of the MPS disorders. However, they may be common and very important in most patients with a particular form of MPS. This meeting on MPS VI proved this point. We heard from our clinical colleagues about the unusual ophthalmic presentation and cardiac complications in MPS VI and the varying progress of patients who had been on enzyme replacement therapy. Equally valuable were the presentations from patients and their carers about their experience of living with MPS VI. Often these talks revealed common practical problems or difficulties in obtaining the appropriate treatment or help. The recognition that other families have similar problems can be helpful and reassuring. Some answers to these problems were provided by talks on how to get the right support in education, the type of equipment available to help with mobility and

other everyday activities and the organisation of infusion of replacement enzyme at home. Excellent summaries in layman's language of the current knowledge of the biochemical and genetic basis, clinical presentation and potential forms of therapy for MPS VI provided the background to the meeting.

There was plenty of time for discussion in the formal sessions and opportunities for families to talk to professionals and other families between the sessions and during the informal reception and dinner in the evening.

The British MPS Society is uniquely placed to organise such specialised meetings because of its contacts in industry, close working relationship with health professionals, its own highly skilled staff and the willingness and desire of its family members to participate. Well done to everybody involved in the organisation of this meeting. I am sure that it was very useful to MPS VI patients, their families and carers.

Bryan Winchester, Trustee



“The talks were really interesting... It was great to learn about new treatments and to find out things about MPS that I hadn't known before... It was great to hear something aimed at issues which affect adult members as it often feels as if those things aren't touched upon very much.



“Excellent info
very informative and
practical.

“Interesting
aspects from the
nurses' view



The MPS VI conference weekend was a wonderful opportunity for the MPS Society, professionals and families to come together to share knowledge and experiences. The children, both those affected and siblings, also had a fun-packed, activity filled weekend making new friends as well. The children joined in for an evening meal and entertainment as we all celebrated 10 years of Naglazyme.

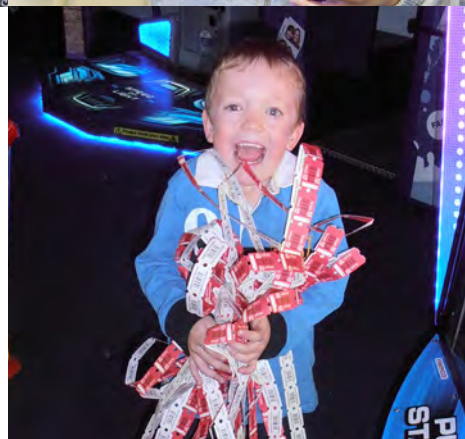
Many families and professionals commented that it was very beneficial to have a conference focussed on a particular disease type as all participants were able to gain something from the presentations. It was interesting to hear Peter Wolfson speak about the cardiac complications of MPS VI and to hear Jane Ashworth's experiences of treating patients with ophthalmic complications. Both families and professionals found the family experiences inspiring and we were very fortunate to have Paul Harmatz travel from the USA to join us and speak with Rob Wynn about treatments for MPS VI. The conference closed with Sunday lunch and it was good to see families have the opportunity to chat before heading home.

Deborah Cavell, Senior Advocacy Officer



“ The conference was wonderful and some of the puzzling questions I’ve had over the years have been answered

“ Both families and professionals found the family experiences inspiring



Thank you to all our speakers and volunteers who made the meeting such a success

CHRISTMAS EVENTS IN 2016

Lunch with Santa

On the 4th December 2016 Steve Cotterell and I from the Advocacy Support Team travelled up to the Holiday Inn, Jesmond, Newcastle for a lunch, disco, party games and activities. On this mild December day, we met a number of families and children who were looking forward to the merrymaking and meeting of Santa Claus.

It was delightful to meet everyone, and join in with the festivities. Once we had all eaten Christmas lunch, the DJ played lots of games and it was enjoyable watching who was best at hula hooping, musical statues and musical bumps. Adam certainly took the competition very seriously and won the most prizes for both winning and becoming runner up. Although Demi-Leigh gave Adam a run for his money when it came to musical statues.

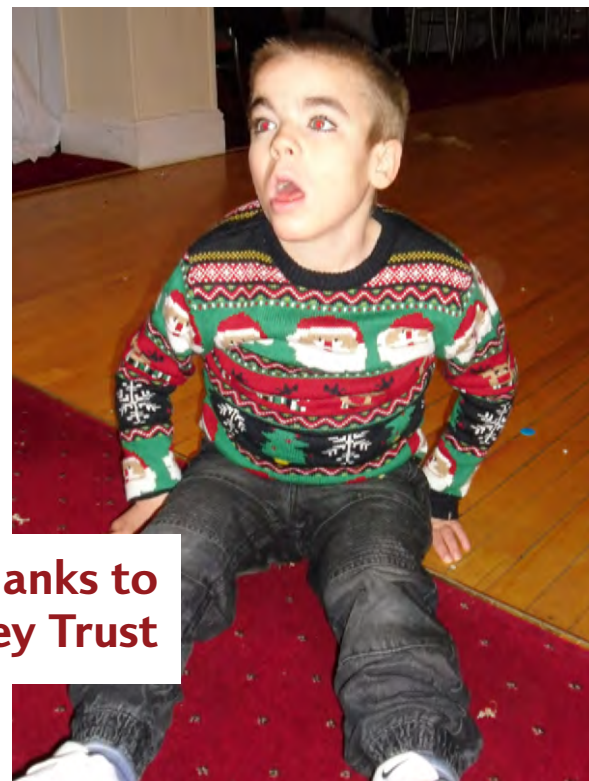
After the first set of games had concluded Santa Claus made his appearance, it was lovely to see the excitement on everyone's faces. The children and young people waited patiently as they each went up to see him, to talk to him about what they are doing at Christmas. Santa Claus in turn gave each of them a gift, which for most could not wait to be opened, let alone be left for Christmas Day!

As the afternoon went on and more games were played the parents had the opportunity to mingle and share stories and experiences. One of the most memorable moments of the afternoon occurred when the father's were invited to the dance floor, and their families decorated them as snowmen. They made fantastic snowmen!

By the end of the afternoon, everyone was exhausted from their dinner and from having lots and lots of fun.

Louise

“ We had a lovely day with you guys, Adam thoroughly enjoyed himself. .It was lovely to be with people who understand what we go through and we look forward to the next event we can get too.



**With thanks to
The Rothley Trust**





Manchester Family Day

Sunday morning came bright and early for our trip up to Manchester for the family Panto. Sophie and I felt like Santa's little helpers with all the presents that had been wrapped up to give to the children. It was lovely to see new members and old all coming together to start the Christmas celebrations. The hotel was decked out and looked very festive so it got everyone in the spirit.

First and most importantly was the food, we all sat down to a selection of hot food and of course cake! Then the main event was the Panto 'Dick Whittington'. The children were quite subdued at first but as time passed the noise level and the excitement grew. No more so than when the ghost was behind them! I know – where is there a ghost in the Dick Whittington story? – it is panto you know.

During the break the children opened their presents and then settled down to the second act. This time the noise level escalated...including us doing a rendition of happy birthday to some of the children. Time flew and before we knew it was time to go home. Sophie and I enjoyed ourselves and I believe everyone else did.

Until next time wishing you all a happy Christmas.

Rebecca and Sophie

**With thanks to
William Brake
Charitable Trust**



**With thanks to
Enkalon Foundation**



Glenarm Castle, Northern Ireland

Families also met Father Christmas at Glenarm Castle in Northern Ireland on Saturday 10 December. As well as meeting the main man, children enjoyed craft activities and fairground rides.





Lapland UK

On the 29th November 2016, we went to a special place called UK Lapland to meet some of our members. Here is what our day looked like.

The day started and ended with perfect weather. Although it was cold, the sun was shining and the frost was sparkling. There was a hive of activity as families waited to enter Lapland, and lots of excitement and anticipation to see Father Christmas and his busy elves.

Once everyone was registered in the husky group we were given the story of how the elves came to be Father Christmas' little helpers, and we were all taught how to say hello in Elvish. We promise we were not being rude! We also had to say a secret elf rhyme before we could enter and before we started our training to become elves.

The elves then showed us how they made some of the toys ready to be delivered on Christmas Eve, and we all had the chance to make a reindeer and a wooden teddy, before we visited Mother Christmas.

In Mother Christmas' kitchen, we listened to some stories and then we got to decorate some of the delicious gingerbread men she had baked. Though some of the decorations were irresistibly tempting and often ended up being sampled and were scrumptious!

Once we had finished decorating the gingerbread men, and had sung The Elf Song, we were given a stamp in our Elf passport. Next we visited the Elf Village, where we could ice skate with penguins, write a letter to Father Christmas and meet some adorable huskies.

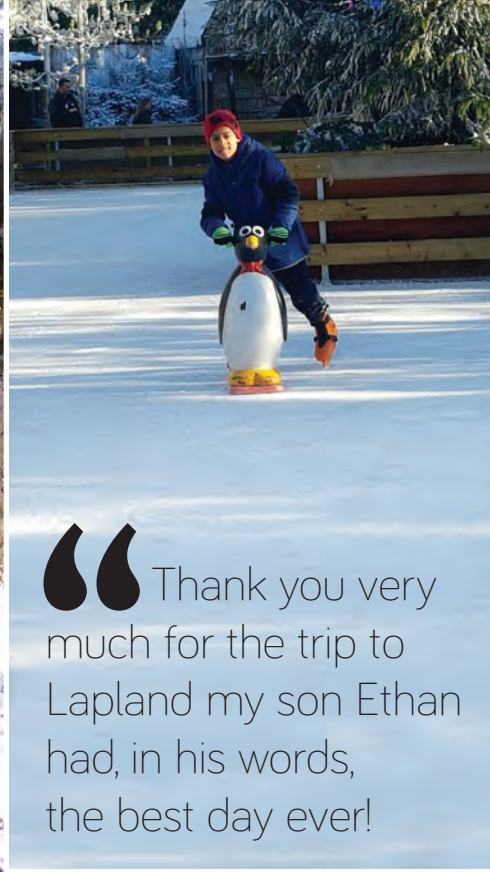
The special elf money (Jingles) we had been given allowed us to buy some food and drink or more importantly some Christmas goodies, before going on a secret and magical journey to meet Father Christmas.

When our elf passport was full with stamps, we had completed our elf training. As we had been very good and helped Father Christmas we were allowed to meet him. Meeting Father Christmas was magical, and he knew what I wanted for Christmas! Father Christmas gave me a very special gift to remember my time at Lapland, and at the end of such an amazing day we were all very tired and all went home.

*Louise Cleary
Advocacy Support Officer*

“Kamal and Amelia had a magical day! They got to help build toys, decorated some gingerbread men and listened to a story from Mrs Claus, played with huskies and last but not least, got to sit and talk to Father Christmas himself.





“ Thank you very much for the trip to Lapland my son Ethan had, in his words, the best day ever!



Lapland, Finland

“ We had a truly magical time and our memories will last forever.

Sophie and I arrived at Gatwick Airport South ready to check in at 4.30am. All families were met by Rudy Reindeer and Hal the Husky. There were opportunities for a photo shoot and the children received a goody bag from Monarch.

On the whole we were all sat together on the plane, a chance to get to know each other. We arrived at Kittilä Airport and took a bus to the terminal which was very small, with only two arrivals halls and baggage carousels. An Elf was keeping us entertained while waiting for the luggage to come through. We were met by our representative, Rapping Rachel who was with us for the duration of our stay, and yes she did give us a rap and a HUGE number of carols and jokes on every coach journey which the children just loved. Our bus was called the Bells Bus and yes there was a jingle to go with it – Jingle Bell, Jingle Bell, Jingle Bell Bus, come with us on the Jingle Bell Bus.

We travelled up the road ten minutes to a large shed which housed multiple boots and thermal suits of all shapes and sizes. Once kitted out we continued to our hotel in the resort of Levi. Arriving at the hotel Levi Spa, the temperature was a balmy -9 degrees. After a few room moves all the families settled in to their rooms. Our welcome meeting was at 4.30pm, remembering to put our clocks forward two hours, we arrived to some Berry Juice and Mulled Wine. This was the time to book any additional activities to those already on the schedule.

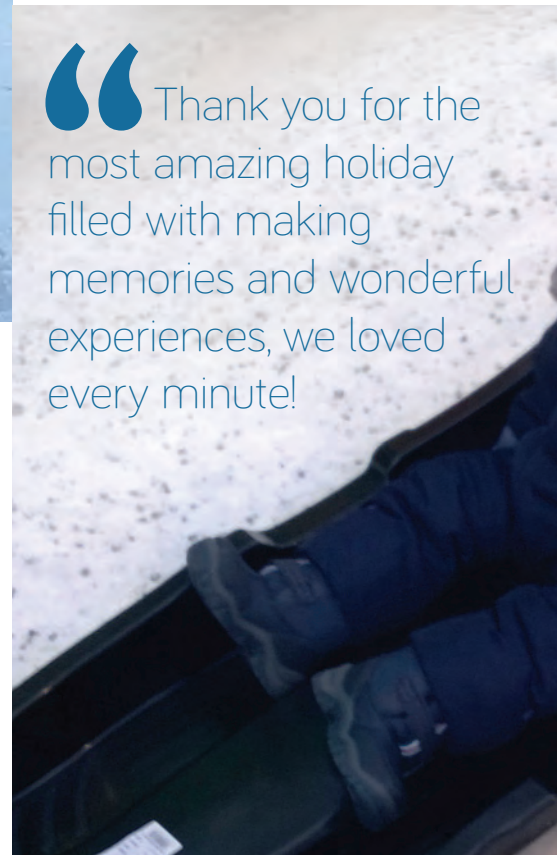


The next morning there was time to look round the resort, where there was a few shops, a swimming pool, bowling, skiing and a children's land that included a toboggan slope and low roped lift to the top of the hill, offering free sledges.

At 12.50 we all gathered on the coach for the main event, the temperature was dropping at this point and by the end of the day it had reached -22 degrees! On the Bells Bus, we sang the jingle bus jingle along with multiple carols of the children's choice.

We started our afternoon with tobogganing, with all the children and some adults coming down the slope, while others just played in the deep snow. We then split in to two groups, one went to the Arctic ceremony while others went on the reindeer sledges and then we swapped.

“ Thank you for the most amazing holiday filled with making memories and wonderful experiences, we loved every minute!



A warm up by the hot log fires outside was then followed by a husky sleigh ride, by this time some of the younger children were getting very cold. A warm up in one of the huts with some berry juice was just what was needed.

We then moved on to the snow mobile rides – I think this was the adults favourite – crash helmets on and off we went, adults two by two and children in sleighs. An amazing day but very cold.

“ We had a brilliant time and didn't want to come home!





“ We also met some fantastic families who I’m sure we will have lifelong friendships with as we all have so much in common.



and outside area. The experience of swimming outside in -19 with the snow coming down on your hair is a once in a life time experience

Monday warmed up a little (only -13 degrees!) and we set off to find Santa singing along to the Bells Bus jingle, looking in Santa’s post office, the elf school, bakery, elf bedroom and finally there was the great man, there was a stampede of children running towards him and Santa agreed to come to our party that evening.

Monday night at 6pm was feast night, a three course meal of roast turkey, not forgetting chips for the kids. Each family met Santa and everyone was given a present. A mini disco followed for the children.

Tuesday it was time to go home, -26 this morning the coldest of them all, many new friends made and magical experiences had. With huge thanks to MPS Commercial and the Gosling Foundation for supporting this event, we could not have done it without you.

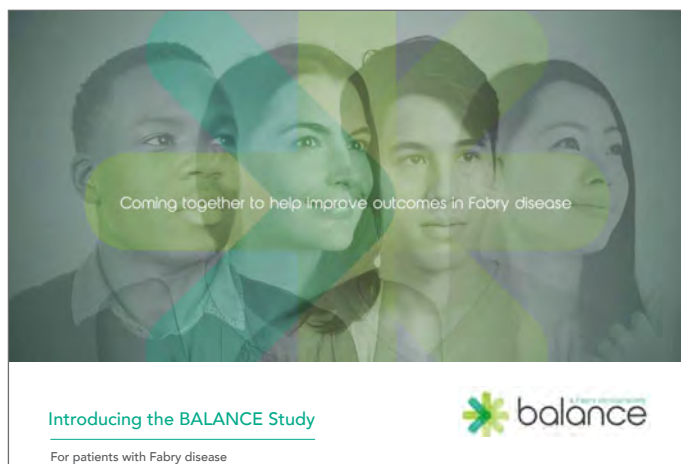
Gina Smith, Group Finance Officer



Families then had time before dinner, to take in the surroundings or take a nap. At 8pm we grouped together and made our way up past the children’s land to an outdoor fire and sang carols, the two wheel chairs even made it up with lots of help from others. I think the most popular carol was jingle bells and the twelve days of Christmas.

Sunday morning we woke up to snow, snow and even more snow which did not stop until early afternoon. Many of the families took to the swimming pool, which was amazing with various heats, depths, a Jacuzzi or two, a slide

Introducing the BALANCE Study



This is a new randomised double-blind, active-control study of the safety and efficacy of pegunigalsidase alfa compared to agalsidase beta on renal function in patients with Fabry disease previously treated with agalsidase beta (Fabrazyme).

This is a Phase 3 clinical trial examining a novel enzyme replacement therapy called pegunigalsidase alfa (PRX-102), in men and women with Fabry disease who have impaired renal function and currently use the ERT, agalsidase beta (Fabrazyme).

The BALANCE study will include around 80 patients who have been using Fabrazyme for at least a year and who have been on a stable dose for at least six months. Participants will be randomised in a 2:1 ratio to receive either pegunigalsidase alfa at 1mg/kg or continue on Fabrazyme at 1mg/kg every two weeks for 24 months. The participant can receive their infusion at the clinical trial centre or as part of a homecare set-up.

What are the Key Findings to date in previous clinical studies with pegunigalsidase alfa?

The interim report from Phase ½ studies with pegunigalsidase alfa (approx. 15 patient-years) includes the following preliminary data.

- Available enzyme throughout the 2 week infusion interval
 - Half-life of approximately 70 hours
- Safety and immunogenicity
 - Low antibody formation (19%)
 - 98% of adverse events (such as vomiting, chest tightening, rash, lightheadedness, shortness of breath and facial flushing) were mild to moderate
- Effectiveness demonstrated in various disease parameters
 - 86.5% reduction of globotriaosylceramide (Gb3) over 6 months in kidney peritubular Capillaries quantitative BLISS score
 - Stable kidney function

Key eligibility criteria

We are looking for men and women who:

- are 18-60 years of age with a diagnosis of Fabry disease
- experience neuropathic pain, cornea verticillata, and/or clustered angiokeratoma as a result of Fabry disease
- have been treated with agalsidase beta (1mg/kg per infusion) every 2 weeks for at least 1 year, with stable dosing over the past 6 months
- have no history of anaphylaxis or severe hypersensitivity reactions to agalsidase beta or alfa or gadolinium contrast agent
- have declining renal function based on at least three serum creatinine values over approximately 1 year (range, 9-18 months, including at screening visit)
- are not renal dialysis or transplant recipients
- do not have a history of acute kidney injury (including specific kidney diseases [e.g., acute interstitial nephritis, acute glomerular and vasulitic renal diseases]; non-specific conditions [e.g., ischemia, toxic injury]; as well as extrarenal pathology [e.g., prerenal azotemia, acute postrenal obstructive nephropathy] in the 12 months prior to screening)
- are free from cardiovascular or cerebrovascular events in the past 6 months
- do not have a urine protein-to-creatinine ratio (UPCR) >0.5 g/g and are not treated with an angiotensin-converting enzyme (ACE) inhibitor or angiotensin receptor blocker (ARB)
- have not had ACE inhibitor or ARB therapy initiated or dose changed in the 4 weeks prior to screening
- are not female subjects who are pregnant, breastfeeding, or planning to become pregnant during the study period.

Research & treatment



Abeona Therapeutics

Abeona Therapeutics presented its top-line data of low-dose cohort for ABO-102 in phase I/II Clinical Trial for MPS IIIA patients at the Orphan Drugs and Rare Disease Congress in October 2016. On the 18th October ABO-102 was granted Orphan Designation from the European Medicines Agency (EMA) for potential gene therapy for MPS IIIA.

Abeona highlighted that it completed enrolment of the low-dose cohort for ABO-102 in phase I/II Clinical Trial for MPS IIIA on 29th August 2016. On 5th October 2016 Abeona announced that the Data Safety Monitoring Board approved ABO-102 dose escalation for second cohort in its phase I/II clinical trial for MPS IIIA.

ABO-102 is Abeona Therapeutics' first in-human, intravenously – administrated AAV gene therapy. Abeona has reported that ABO-102 has been well tolerated through the 30-day post-injections in patients injected with the low-dose (3 patents). Encouraging signs of early biopotency have been observed in urinary and CSF GAG (glycosaminoglycan, specifically, heparin sulfate) measurements, as well as a potential disease-modifying effects in the liver and spleen.

For more visit: <https://goo.gl/DZl2jl>

AVROBIO raises \$25m for clinical programs

AVROBIO, Inc., a clinical-stage biotechnology company developing transformative, life changing gene therapies for rare diseases and cancer, announced that it has raised \$25 million in a Series A financing co-led by Atlas Venture, Clarus and SV life Sciences. AVROBIO will use the proceeds from this financing to accelerate development of its clinical stage programs in Fabry disease and acute myeloid leukemia (AML), and to expand its pipeline in rare disease and solid and liquid cancers.

“AVROBIO’s highly innovative therapies offer potentially life-altering impact for patients following a single infusion of genetically-modified cells,”

said Geoff MacKay, AVROBIO’s President and Chief Executive officer.

“We are grateful for the funding and support we have received from our investors, as we continue to focus on displacing the standard care for patients with Fabry disease or AML through the development of these disruptive gene therapies.”

Gene therapies represent a new paradigm in human health, with the potential to deliver dramatic disease-modifying effects with long lasting, durable impact. Underlying these advances are a deeper understanding of cell biology, immunology and a newer generation of vector designs enabling safe and effective delivery of therapeutic genes targeted to specific cells. AVROBIO’s initial two programs are leveraging the established safety and effectiveness of ex-vivo gene therapy to provide Fabry and AML patients with new therapies that have the potential to significantly improve both their quality of life and lifespan.

AVROBIO’s phase 1 gene therapy to treat Fabry disease seeks to deliver lasting and meaningful benefits for Fabry patients. The patient’s stem cells are extracted and genetically modified by adding a new, functional copy of the faulty gene. The modified cells are then infused back into the patient via a one-time procedure. A durable elevation of endogenous enzyme is expected, with the potential to significantly improve patient outcomes and eliminate costly lifetime biweekly intravenous infusions of enzyme replacement therapy.

Printed with kind permission of the Fabry Support and Information Group in the USA.



From left: Diettian Diane Green, Sub Investigator Dr Karolina Stepien, Data Manager Andrea Hill, Principal Investigator Dr Reena Sharma

New research into bone health

A new research study is starting at Salford Royal to find out more about bone health in adults with Mucopolysaccharidosis (MPS).

People affected by MPS often suffer pain, fractures due to reduced bone density and bone deformities. At present scientists don't really understand much about the process of the bone disease and how to assess it accurately in clinical practice. The hope is that the study will develop their knowledge to help identify and monitor thinning of the bone, manage it appropriately and so reduce the risk of bone fractures in the future.

The researchers, led by Principal Investigator Dr Reena Sharma will also be comparing different types of scans – a DXA scan and a QCT scan, both of which check bone mineral density – to see which is most accurate. DXA (dual energy X-ray absorptiometry) scans are a special type of

X-ray while QCT (quantitative computerised tomography) uses a scanner and computer software to assess bone density. They will also measure markers of bone turnover in blood and urine.

The Mark Holland Metabolic Unit at Salford Royal is a specialist centre for MPS disorders. Patients travel from across the UK and this study will link into their regular six-monthly or annual checks – they will not need to make extra visits to take part.

People aged 16 and over with MPS and the capacity to consent could be eligible to take part in the study which starts in December 2016 and will run for two years.

To find out more about the research, please contact reena.sharma@srft.nhs.uk or marie.meehan@srft.nhs.uk or karolina.stepien@srft.nhs.uk.



Charlotte was invited to speak at a press conference by David Taylor, Emeritus Professor at UCL, about the future of funding of drugs in our healthcare system. Charlotte alongside Jayne Spink CEO at Tuberous Sclerosis Association (TSA) spoke about access to medicine and process failure. The MPS Society and TSA joined forces last year in the fundourdrugsNOW campaign but sadly the TSA another year on are sadly still waiting for NHS England to make a decision. Professor Taylor highlighted the need that England and the other UK nations should seek to achieve flexible and humane approaches to valuing and supplying innovative treatments which promote equity and foster high levels of public and patient confidence without generating unaffordable costs.

The report can be found here: <https://goo.gl/Ty5sTz>

Thank you also to the trustees who attended the event.

News on Fabry treatment

NICE recommend Migalastat for the treatment of Fabry disease for individuals over the age of 16 years with an amenable mutation

The National Institute for Health and Care Excellence (NICE) has today released their final decision (FED) to recommend Migalastat for the treatment of Fabry in people over 16 years of age with an amenable mutation. This is on the proviso that the discount agreed in the patient access scheme is applied and that only those who would be offered Enzyme Replacement Therapy (ERT) be considered.

Consultees have until 5pm on the 18 January 2017 to notify NICE of any factual errors or to lodge an appeal against it.

NHS England now has 3 months to ensure that Migalastat is available for use in line with NICE's recommendations.

To read the full reports, please follow on this link: <https://goo.gl/xgcFfs>

What is the Delphi Process?

The name Delphi comes from the 'Oracle of Delphi' and is based on the assumption that group judgements are more valid than individual judgements.

The Delphi process was developed at the beginning of the Cold War to forecast the impact of technology on warfare. Different approaches were tried, but the shortcomings of traditional forecasting methods, for example theoretical approach, quantitative models or trend extrapolation but were quickly shown not to work. To combat these shortcomings, the Delphi process was developed during the nineteen fifties and used to ask experts to give their opinion on the probability, frequency and intensity of possible enemy attack. Other experts could anonymously give feedback and this process was repeated several times until a consensus was reached.

First applications of the Delphi process were in the field of science and technology forecasting. The objective of the method was to combine expert opinions on likelihood and expected development time, of the particular technology, in a single indicator.

Later the Delphi method was applied in other places, especially those related to public policy issues, such as economic trends, health and education. It was also applied successfully and with high accuracy in business forecasting.

The key characteristics of the Delphi Process help participants to focus on the issues at hand and separate Delphi from other methodologies. Using the Delphi technique experts are drawn from both inside and outside the organisation with the panel consisting of experts having knowledge of the subject requiring decision making. Each expert is required to make anonymous predictions.

Anonymity of the participants

Usually all the participants remain anonymous even after the completion of the report. This maximises the possibility of freeing participants from their biases and allow free expression of opinions, encourages critique and facilitates taking ownership of errors when revising earlier judgements.

Structuring of information flow

The initial contributions from the experts are collected in the form of answers to questionnaires and their comments to these answers. The panel-director controls the interactions among the participants by processing the information and filtering out irrelevant content avoiding the negative effects of face-to-face panel discussions and solves problems with group dynamics.

Regular feedback

Participants comment on their own forecasts, the responses of others and on the progress of the panel as a whole. At any time they can revise their earlier statements.

It would appear that the track record of the Delphi process is mixed however Chia-Chien Hsu of Ohio State University and Brian A Sandford of Oklahoma State University in their peer reviewed paper published in Practical Assessment, Research and Evaluation Volume 12, No 10, August 2007 – The Delphi Technique: Making Sense of Consensus concludes:

'The Delphi technique provides those involved or interested in engaging in research, evaluation, fact-finding, issue exploration, or discovering what is actually known or not known about a specific topic a flexible and adaptable tool to gather and analyse the needed data. Subject selection and the time frames for conducting and completing a Delphi study are two areas which should be considered carefully prior to initiating the study. The additional precautions concerning low response rates, unintentionally guiding feedback, and surveying panelists about their limited knowledge of the topic rather than soliciting their expert judgments should also be built into the design and implementation of the study. The Delphi technique has and will continue to be an important data collection methodology with a wide variety of applications and uses for people who want to gather information from those who are immersed and imbedded in the topic of interest and can provide real-time and real-world knowledge.'

Christine Lavery, Group Chief Executive

International

Charlotte Roberts spoke on behalf of Christine Lavery at the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) in Vienna about the Managed Access Agreement and the patient voice when it comes to reimbursement decisions. The panel alongside Charlotte included Sheela Updadhayaya, Associate Director of Highly Specialised Technologies at NICE and Samantha Parker from Lysogene, who are developing a gene therapy for MPS IIIA. With an audience of over 80 it was a well-received session with positive feedback from a delegate who emailed to say

“your presentation was very interesting and extremely informative, one of the best I went to!”



Rare disease in Rio

US Olympian, and rare disease patient, Cody Miller won a bronze medal at the 2016 Paralympics in the Men's 100 meter breaststroke. With a time of 58.87 seconds, Cody finished just behind Adam Peaty of Great Britain and Cameron van der Burgh of South Africa. That was

the only individual race Cody is scheduled to be in but look for him to compete later in the 400m relay. Congratulations, Cody.

Miller was diagnosed with Pectus excavatum at a young age. The hallmark of the condition is a sunken appearance of the chest. The condition also limits the person's lung capacity forcing them to take more breaths than an average person. Though not all bad, Cody Miller actually has his diagnosis to thank for starting his career in swimming. At age 8 he started swimming because of his condition to monitor his heart and breathing- now he is a 2016 Olympic swimmer.

The shocking part is he is not just a swimmer, but a breastroker. The world's best breaststrokers are 6'3" to 6'5", and he is 5'10". Yet Miller takes longer and fewer strokes than anyone. Miller never let his condition define who he was or stop him from going the distance. He became a junior national champion, set national age group records (15-16) in the 100- and 200-yard breaststrokes and was chosen for the national junior team. 2016 will be his first Olympic appearance. In tests at the U.S. Olympic Training Centre in Colorado Springs, Colorado, he was the most symmetrical swimmer that the judges have ever seen. Possibly due to his caved-in chest.

Pectus excavatum, also known as sunken or funnel chest, is a congenital chest wall deformity in which several ribs and the sternum grow abnormally, producing a concave, or caved-in, appearance in the anterior chest wall with an incidence of 1 in every 400 to 1000 live births. It causes respiratory issues and deficient lung capacity. Severe cases of pectus excavatum can eventually interfere with the function of the heart and lungs. The cause of pectus excavatum is not known.

Bonn is not Brazil

But who says Bonn must be Brazil? A great World Congress with great impressions, many beautiful moments with wonderful people and very interesting and instructive lectures from all over the world.

Of the lectures I have heard only from narratives. Above all, the workshops on physiotherapy and speech therapy were highly praised. Also, people always congratulated me on my great, strong sister after hearing Maria's talk.

When I write about my impressions, I can write about tears at the entrance to child care, which were dried again in the twinkling of an eye, and a dancing performance at the tram station to prepare us for the great performance.

Children are simply great – especially children with MPS. They are so incredibly strong and cheerful; they go out into the world and are simply there. Although they have a very hard way, they radiate so much strength and give so much strength and energy with their laughter. I love to be with these special children and their siblings and to enjoy the indescribable atmosphere. That was why I was so pleased when Carmen asked me if I would like to accompany the children's program with Manuel and Martina. Of course I wanted to! As an Austrian, I was still quite a long way away and followed the preparations mainly by email. The program was super-thought-out and I knew immediately that the kids would love it and the parents would give their treasures without any worry.

After a journey asleep in the night train we reached Bonn right for breakfast. We, that was my big little brother Paul, who cannot be missed as a childcare assistant at any event – the children love him. Paul and I really liked the child

care room. Everything was thought of. They had a ball pit, a lot of space for games, and a “youth corner” with lots of beanbags. In front of the hotel there were three large tents where the organised program was to take place. This program was very varied and entertaining for the children.

The many volunteers were assigned to Manuel, Martina and me, and we formed teams for the next four days.

Robin and Madelin provided entertainment in their theatre tent where my team spent the very first day. Many interactive games were played and I can assure you it wasn't only had the kids who had a lot of fun! Paul was also a good motivator for the more quiet kids. He never considers himself too good for anything and put the kids in good mood.

Tent number three was probably the most moving tent. Kelechi quickly transformed a group of children and adolescents into passionate dancers. It was wonderful seeing the development and especially what Kelechi got out of the children. Anyone who thinks that dancing is only possible for people without impairment is enormously wrong. What I saw and felt in this tent caused constant goose bumps.

An incredibly beautiful stage scenery was designed for the last evening. The motto for the days in Bonn was the Nibelungen, so it was worked hard on painting castle walls over which even ivy grew. The kids created bows and arrows and beautiful robes for the castle maid. The highlight was for sure the long and of course extremely dangerous dragon that had to be overcome on stage by the knights and maids.

In the sweat of our brow, we learned a dance to Justin Timberlake's “Can't stop the feeling“. The group

included some MPS patients who have difficulties to move, but who did not give up and did not stop fighting. And so there were a lot of children (and childcarers) on the stage to perform during the gala night.

The nice thing about the team was that a lot of older siblings have done a job as volunteers. Each of us knows what MPS is, how we have to respond on individual needs and, above all, that MPS children and adolescents are extraordinary people. I was also particularly pleased with the internationality of the Volunteer team. For example, I met Thea from Norway, Rhoswen from the UK, whom I met in Brazil two years ago, and Julie from Taiwan, who was like a friend who had always been there even though we've only met in Bonn. Maybe this is because our mothers, both MPS presidents, are very similar. When I think of the UK, I remember Nathan, who kept his carers busy too. He felt very comfortable in the dance tent and impressed everyone with his hip swing.

The gala evening also brings a unique atmosphere every time. One would

think that at such a congress with over 1,000 participants one would find the scientists in one corner, the families in the other, MPS specialists would sit together and talk incomprehensible stuff. Whoever thinks of an MPS meeting like that should take the next chance to join a meeting and correct his ideas. Already during the dinner, families sit with scientists and do not talk exclusively about MPS, because it is hardly believed that this disease is part of our lives, but we also have enough other things to tell or discuss. And when the band starts to play, there is no one to hold. Time to dance! Everyone storms the dance floor: MPS patients, MPS presidents, MPS scientists, pharmacist leaders ... children, adults ... all dance together, laugh and enjoy the atmosphere. This is a moment you really have to experience yourself, which cannot be written down in black or white or captured in a photograph. It's just wonderful.

Anna Prähofer



Information & resources

Glossary

Do you know your PP from your TAC or your DOLS from your SALT? No? Then it's time to take a look at our new glossary page. The list pulls together all the acronyms, initials and abbreviations you might ever come across with simple explanations for what they are and when you might need them.

Go to www.mppsociety.org.uk/glossary to see it now.

We will keep this up to date and add more definitions but if there's anything you want to see on the list please let us know at magazine@mppsociety.org.uk.

Education

EHC Plan – Education Health and Care Plan
SEN – Special Educational Need
SEND – Special Educational Needs and Disabilities
SENCo – Special Educational Needs Coordinator
ASDAN – Award Scheme Development and Accreditation Network
IPSEA – Independent Parental Special Education Advice
SENDIST – Special Educational Needs and Disability Tribunal
LEA – Local Education Authority
PP – Parent Partnership
IEP – Individual Education Plan
DSA – Disabled Students Allowance
AEN – Additional Education Needs
PMLD – Profound and Multiple Learning Disabilities
DFE – Department for Education
EYS – Early Years Service
FE – Further Education
HE – Higher Education
EWO – Education Welfare Officer
SLD – Severe Learning Disability
MLD – Moderate Learning Disability
ALD – Adult with Learning Disability
PSHE – Personal Social Health Education
BESD – Behavioural Emotional and Social Difficulties

Social Care and Mental Health

CIN – Children in Need
TAC – Team Around the Child
C.A 1989, C.A 2014, S.47 – Children Act 1989 updated 2014, Section 47
CAF – Common Assessment Framework
CWDT – Children with Disabilities Team
DCT – Disabled Children's Team
CP – Child Protection

LAC – Looked After Child
CIC – Children in Care
CSC – Children's Social Care
JARF – Joint Agency Referral Form
DOLS – The Deprivation of Liberty Safeguards
COP – Court of Protection
IMCA – Independent Mental Capacity Advocate
SSD – Social Services Department
CMHT – Community Mental Health Team
SAB – Safeguarding Adults Board
SCB – Safeguarding Children's Board
DBS – Disclosure Barring Service
SW – Social Worker
ASW – Approved Social Worker
CYPS – Children and Young People's Service
CAFCASS – Children and Family Court Advisory and Support Service
LA – Local Authority

Health

CAMHS – Child and adolescent mental health service
CQC – Care Quality Commission
DST – Decision Support Tool
NHS CHC – Continuing Health Care
CLDT – Community learning disability team
SALT – Speech and Language Therapy
CPN – Community Psychiatric Nurse
OT – Occupational Therapist
ENT – Ear Nose and Throat
MDT – Multi-Disciplinary Team
IMD – Inherited Metabolic Disease
GAG – Glycosaminoglycans
CNS – Central Nervous System
ADHD – Attention Deficit Hyperactivity Disorder
ASD – Autistic Spectrum Disorder
COPD – Chronic Obstructive Pulmonary Disease
ECG – Electrocardiogram
ECHO – Echocardiogram
MRI – Magnetic Resonance Imaging

CPAP – Continuous Positive Airway Pressure
BIPAP – Bilevel Positive Airway Pressure
ERT – Enzyme Replacement Therapy
HSCT – Hematopoietic Stem Cell Transplant
BMT – Bone Marrow Transplant
EEG – Electroencephalogram
LSD – Lysosomal Storage Disorder
LHB – Local Health Board
CNS – Clinical Nurse Specialist
MAU – Medical Admission Unit
IFR – Individual Funding Request
NSF – National Service Framework

Hospitals

Salford – Salford Foundation Hospital
UHB – University Hospital Birmingham (sometimes known as the Queen Elizabeth Hospital)
Addenbrookes – Addenbrookes Hospital
Royal Free – Royal Free Hospital
The National – National Hospital for Neurological Diseases
MCH – Manchester Children's Hospital
BCH – Birmingham Children's Hospital
GOSH – Great Ormond Street Hospital
University Hospital of Wales (sometimes referred to as Cardiff hospital)
Royal Hospital for Sick Children (sometimes known as Glasgow Paediatric)
City Hospital (sometimes referred to as Belfast Hospital)

Medical Approval Agencies

EMA – European Medicines Agency
SMC – Scottish Medicines Consortium
FDA – American Food and Drug Administration

NICE – The National Institute for Health and Care Excellence
WHSSC – Welsh Health Specialised Services Committee
NHSE – NHS England

Housing

DFG – Disabled Facilities Grant
AP – Allocations Policy
AST – Assured shorthold tenancy
AT – Assured Tenancy
TA – Tenancy Agreement
HR – Housing Register
CBL – Choice Based Letting
SH – Social Housing
DHP – Discretionary Housing Payment
DHR – Disability Housing Register
HA – Housing Association
HSO – Housing Support Officer
HO – Housing Officer
LHC – Local Housing Companies
ME – Mutual Exchange
NTQ – Notice to Quit
PRP – Private Registered Provider of Social Housing, also known as **RSL** – Registered Social Landlord
PSH – Private Sector Housing
TAROE – Tenants and Residents Associations of England
TA – Tenants Association – also known as Residents Association
UO – Under Occupation

Benefits

DLA – Disability Living Allowance
ESA – Employment Support Allowance
PIP – Personal Independence Payment
JSA – Jobseekers Allowance
IS – Income Support
UC – Universal Credit
CA – Carers Allowance
HB – Housing Benefit

Bupa Home Healthcare now owned by Celesio

Celesio, a leading healthcare services company, announced in July the completion of its agreement with Bupa to purchase Bupa Home Healthcare, a clinical home healthcare provider. This follows Competition and Market Authority (CMA) approval on 28th June.

The addition of Bupa Home Healthcare to Celesio UK complements existing services and strengthens the company's national healthcare infrastructure, enabling patients to benefit from greater flexibility and more choice.

Bupa Home Healthcare provides clinical care in patients' homes for a number of conditions including those that require specialty medicines such as Intestinal Failure and Lysosomal Storage Disorder, Cancer, Multiple Sclerosis and Rheumatoid Arthritis. The company has a national nursing team and its own aseptic manufacturing facilities. Around 1,000 employees are based across the UK.

Celesio press release – 1 July 2016
<https://goo.gl/Nr3Vpa>

Bupa Home Healthcare website – 20 October 2016
<https://goo.gl/0guxui>



BiOMARIN

One resource with the answers to your Morquio A questions

Morquiosity.eu is a helpful new website designed especially for the Morquio A community. Learn about the diagnosis, signs and symptoms, and management of Morquio A.

Visit morquiosity.eu to find out more.

EU/MPSIV/1016/0017 October 2016

MORQUIOSITY.eu

What is anxiety?

BBC's Newsround has released a guide about anxiety aimed at a young audience. The guide looks at depression and anxiety, what it is and ways to cope with it. It demonstrates what a normal feeling this is through the voices of various celebrities who suffer with similar feelings and struggles. The guide can be found on the BBC website here:
<https://goo.gl/ri46W2>



Pharmaceutical Directors Club

Christine Lavery and Charlotte Roberts attended the Pharmaceutical Directors Club (PDC) on 24 November 2016.

Christine spoke at the Club, which is an independent issue driven forum for Senior Executives, on the topic of high-value, high-cost drugs and how to pay for them as new funding mechanisms are required if patients are actually to be able to benefit from drug developments.

Geoff Frew, Chairman of the organisers of PDC said: "the notion of early and continuing patient involvement in the development and delivery of medicines was the mission of the meeting". He also said of Christine "the world is a better place because of you...you certainly inspired your audience".

Fundraising



On 2nd July, the Great Hampden Shoot held their annual Clay Shoot and BBQ. They always have a raffle and decided this year that the proceeds would go to the MPS Society. Pictured: Gamekeeper, Eric Cross, and his wife Ann with Harry Evans and his mum Lucy.



Halloween fundraising

Bournemouth Jobcentre had a charity dress down day for Halloween, where everyone wore black and orange. This was in aid of the MPS Society which was nominated by our colleague, Josie Hooper, whose nephew has Sanfilippo.

Josie (whose husband is a farmer and is holding the hamper in the photo below) donated a Harvest Festival Hamper to be raffled off as well.

We raised £60.00 for the charity!



Snow sports at Bath University

James Garthwaite organised a stall at his snow and ski club open day to encourage fellow students to fundraise for MPS. It was a great opportunity to raise awareness for the society.

If you're interested in fundraising at visit our uni page for more info: www.mppsociety.org.uk/Osi0W



Marina & Friends Fundraisers

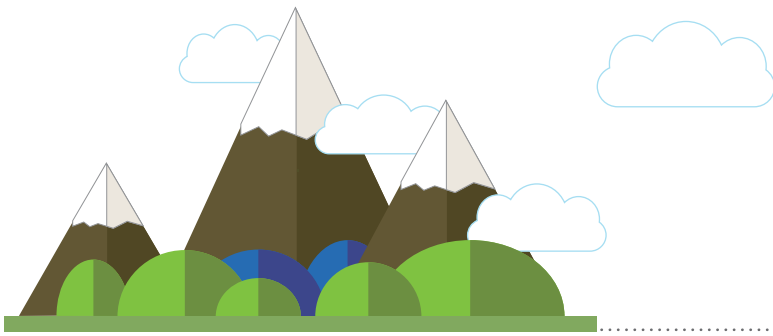
Regular readers of the MPS Magazine will know all about Marina & Friends Fundraisers, a charity shop based in the south east of Bristol. Described as "more than just a shop! It's the hub of Brislington!", this is clearly true as Marina & Friends have raised £8,313.13 since August this year.

We are so grateful to Marina and her team for running the shop and for donating all the proceeds to the MPS Society which have now reached a terrific £167,249.47 since it opened!

If you can't get to Brislington soon have a look at Marina & Friends Fundraisers on Facebook: www.facebook.com/MarinaAndFriendsFundraisers

Friday 4th November was our 'Blue Day' for Chloe in Year 6. The children and staff alike wore something blue which is Chloe's favourite colour and we made a donation to the MPS Society. An advocacy support worker from the MPS Society gave a talk in Assembly about Chloe's condition. We raised a fantastic £160.85! Thank you everyone!

Churchwood Primary Academy, Hastings



Three peaks in three days

On 24th June, a group of friends climbed the Three Peaks for the MPS Society because: “the Pearsons are an amazing family!”. What an inspirational statement.

The team members were Paul Stanton, Matt Worden, James Thomas, Kris Rich and Sam Worden. They chose to raise money for their close friend’s son, Archie. Archie has MPS VI and experiences symptoms such as curvature of the spine, enlarged body organs and cornea clouding.

Sam Worden writes that this has “a corresponding impact on Archie’s ability to walk for long periods and generally be a ‘normal’ kid. Every week Archie has Enzyme Replacement Therapy and is normally in hospital every two weeks for examinations. The money raised will directly benefit the MPS Society, a Society dedicated to the treatment and palliative care of MPS sufferers. It is a small Society but without their support for Archie and his family life could be very different.”

In total the team raised a brilliant £627.71 including gift aid.



Dorothy and Colin Robinson very kindly donated £105 which was a combination of donations for cards made for friends and the prize money from this beautiful wooden communion set which Colin carved. The set won first prize at the Timsbury Flower Show. Thank you both and congratulations to Colin.

We were invited by the Yorkshire Building Society Foundation to collect a cheque for £100. Members are asked to donate their interest received on their savings to charities and get the chance to nominate local charities in their area.

We were delighted to be nominated and would love to acknowledge the individual who put us forward. If you nominated us please get in touch so we can thank you.



In Memory of Chloe Walker

Leona, Kayleigh and Jess, proud friends of Chloe and Sharon Walker, raise money in their memory.

Our friend Chloe passed away on May 24 2010 on her 16th birthday when the bus she was travelling home from Keswick School on was involved in a collision. Chloe’s brother died in 2007 from MPS III, he was diagnosed when he was seven years old. In 2014 Chloe’s Mum, Sharon, sadly passed away after a short battle with cancer.

Chloe’s friends celebrated her birthday this year by holding another charity night in celebration of Chloe’s life. In 2015 they raised £600, this year they have raised an amazing £800.

We would like to thank Leona, Kayleigh, Jess and all of Chloe’s family and friends for their support.



Ronnie's family fun day

Despite only setting up their fundraising page in July 2016 the Kirkleys have already raised an astonishing £12,000

Their most recent fundraiser was "Ronnie's family fun day" held at a church hall in their home town of Whitefield where Ronnie lives with his parents, Chloe and Mike, and his older sister Laila.

The day looked to have been run with precision as family and friends brought together all the stalls you would expect to see at a village fayre including cake stalls, a tombola, lucky dip, children's games, face painting, nail painting, hair braiding and a bouncy castle. Ronnie's aunt, Natalie, said: "everyone just pitched in to help make the day a success."

As well as the fun of the fayre, Ronnie had the opportunity to meet Chase and Skye, his favourite characters from the children's TV series Paw Patrol. According to his aunt "he absolutely loves the Paw Patrol characters and, when they walked in the room, he was overjoyed. All the kids who were there loved meeting them too. It was the best part of their day."

The organisers also wore MPS Society t-shirts to raise awareness of the charity and of the condition and invited local press to the event to spread the word even further.

The fun day was a massive success and raised £5,810 in total.

When they tipped the £11,000 mark Chloe thanked everyone who had donated to their JustGiving page saying "Your support is incredible. Great cause and it will help the MPS Society so much."





Erin-Beth Robson is 10 years old and goes to Preston Primary School with Corey Jeffery aged 8, who has MPS Type 1-Hurlers. She decided to get her hair cut shorter to raise money for the MPS Society and to donate the hair (10 inches) to the Little Princess Trust.

She started with a target of £50 and was amazed when she exceeded this in days and raised it to £100 – needless to say she exceeded this too and the next and the next!

She had her beautiful hair cut and styled on Tuesday 25th October by Chloe Grace Dickison, free of charge at Kaboodles Hairdressers in Torquay.

She eventually raised a total of £716! She was over the moon and we, her family and friends especially Corey and family, are extremely proud of her!



Helen Skidmore's parents, Pat and Michael, organised a pop up charity shop to raise money in her memory. They have so far raised £2,031.50 which included the shop and a raffle. They were even visited by the mayor and local newspaper at the opening event.

Steve Ollington hiked up Mount Kilimanjaro in October to raise money for a close friend of his whose 1 year old daughter was diagnosed with MPS I. He raised a very impressive £2485.08 including gift aid.

Donations amounting £3070.93 have been donated in memory of the late Andrew Leslie Harris. Thank you to Samantha Harris for organising the Memory Giving page and contacting the Society.

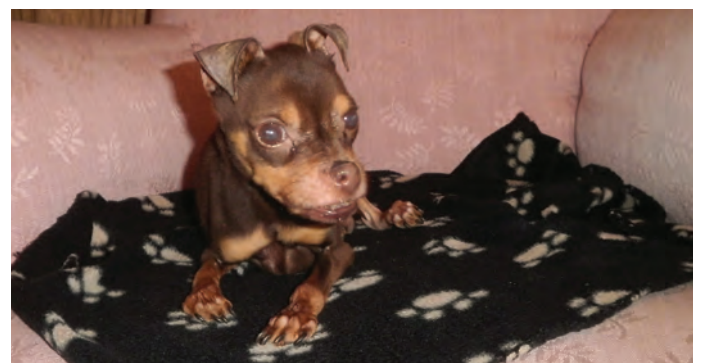


A dog named Dukey

We featured Elaine McLellan in the Autumn 2016 magazine as she raised an amazing £1013.75 in sponsorship from the Prudential RideLondon–Surrey 100 on 31 July. Elaine very kindly brought her cheque into MPS House in person and revealed over a cuppa that the reason she decided to donate her sponsorship money to the MPS Society was because of a dog named Dukey.

Elaine regularly cycles with her friend, Alex, who is part of the MPS Commercial team and when Alex mentioned her new job she was surprised to discover that Elaine had already heard of mucopolysaccharide diseases as Dukey, who she used to care for, had MPS.

Dukey has sadly passed away but as Elaine had very fond memories of the dog she felt ours was the right charity to donate her sponsorship to help humans with MPS.



A Season of Strides

Charlie and Paul Dodd are avid fundraisers for the MPS Society and always have a huge number of fundraising events in their calendar each year. Here Charlie tells us about their year and what they have planned.

On 22 May, the whole family, including our two boys, Alfie and Archie, aged 8 and 5, climbed [Mount Snowdon](#). Alfie and Archie raised £47 at school. As we always have a medal at the end of our events, we thought it only right they had one too. We had them engraved with their names and date of their tremendous achievement. They took them into school for show and tell in front of the whole school!

On Saturday 6 August, we successfully completed a 3 hour [spinathon](#) with the help of our local gym, [EvoFit](#) and 20 of its owners/members! It was so amazing as we paid for everyone to have and wear an MPS training top so every time they wear it to train, they are spreading awareness! We've had pictures sent to us from two participants who have been on holiday to Turkey and have taken and worn their training tops whilst away. MPS awareness is spreading far and wide!

Taking into account all the money raised by individuals taking part, money collected on the day from the cake sale and other [JustGiving](#) page donations we raised the total sum of £1950.02. We are so immensely proud and have now doubled our target amount for the year already!

We had huge success with the [golf event](#) which we held on Saturday 8 October at the [Asbury Golf Course](#). The weather was extremely kind to us and it saw 55 players heading off on the 18 hole course all in aid of MPS Society!

At the end of the game we raffled off all of the prizes we had managed to have donated. We auctioned off the 2 week [Villa stay](#) and that alone raised £1000! We haven't got a final figure yet but it is in excess of £3000 which is absolutely fantastic! To say we are chuffed is an understatement.



We are hoping to add a further event, [Ben Nevis Climb](#) with our two sons. They have combated [Mount Snowdon](#) so I'm sure they will take on [Ben Nevis](#) with the same success!



The 16th October saw myself, Paul and our special friend Liz take part in the [Great Birmingham Half Marathon](#). The day started wet but then as we began running the sun began to shine! We did fantastic and took on 'jelly baby' hill and beat it!! What an amazing event! Thank you to all that sponsored us!



To date Charlie and Paul's [JustGiving](#) page has raised

£6731.83

including gift aid since April 2016. Amazing stuff!



NATIONAL DRAW RESULTS

Thank you to everyone who bought and sold tickets for this year's national draw and congratulations to all the winners. We hope you enjoy your prizes!

FIRST 3 night family stay at the Adina Hotel, Hamburg – Mike Starkey, Trowbridge (18443)

SECOND €250 M&S voucher – A Marquis, Liverpool (12528)

THIRD 3 night stay for 2 at the Adina Hotel, Hamburg – Sally Mitcham, York (02036)

- €100 Love to shop voucher – S Bown, Bexhill on Sea (26373)
- €110 Love to shop voucher – A Wray, Bolton (12571)
- Family ticket to The Making of Harry Potter – M O'Hagan, Perth (18498)
- 2 New Year's Day tickets for Cheltenham racecourse – Mr & Mrs S Holt, Bristol (11862)
- Spotify ROCKI device – Wilma Robins, Hornchurch (15740)
- Ryman's stationery set – M Borrett, Portsmouth (13553)
- €50 M&S Vouchers – C Vivier, Tunbridge Wells (15427)
- €50 Ocado voucher – M McAllister (19460)
- 6 Tickets to Circus Starr Winter Tour – M Loveday, Gerrards Cross (01834)
- Love 2 Shop vouchers – N Worsford, Newry (08579)
- Love 2 Shop vouchers – Rachel Cable, Cardiff (15321)
- Online mindfulness course from Be Mindful Online – William Beards, Hornchurch (15732)
- 4 Empire cinema passes – Jayne Sutherland, Cardiff (15305)
- Culti Décor Assolato Scented Candle – Sarah McKnight, Carmarthen (01125)
- €25 Clarks Voucher – Russell Bywater, London (19229)
- €20 Boots Voucher – G Kershaw, Frodsham (09650)
- Pebble Smartstick phone charger and portable speakers – Sam Walls, Norwich (15677)
- Deluxe Body Pilates Set – J Bates, Templecombe (00532)
- €15 The Entertainer voucher – Jeremy Lowe, East Clandon (20582)
- Pronto! 'Let's cook Italian' cookbook by Gino D'Acampo – G Sidlow, London EC2 (19509)
- 2 Cream tea vouchers for Van Hage – Miss L Hiller, London E3 (20871)
- 5 Top Wash vouchers from IMO car wash – Barbara Newbury, Cambridge (19972)
- Tin of Quality Street and Cuddly Teddy Bears – D Allen, Birmingham (13380)
- 2 boxes of Quality Street and cuddly teddy bear – Julia Thompson, Mexborough (12413)
- 7 Nero VIP drinks vouchers – Innes Hall, London EC2 (19496)
- €10 Next voucher – Nick Rose, Woodford Green (19276)
- Star Wars USB memory stick, USB fan and earphones – M Mury, Glasgow (22226)
- 'It Can't Be True' book – T Malik, Liverpool (00485)

We heard from Mr and Mrs Clarke who won hotel vouchers in the 2015 National Draw and used them for two city breaks to Birmingham and Sheffield, two cities they longed to see. They told us how pleased they were at winning, especially as it was the first time they had won anything in a raffle.

Pom-poms



Daisy Mitcham-Harding decided to sell pom-poms at her school to raise money for the MPS Society because her little cousin has Hunters.

Daisy advertised the stall in her school newsletter by writing:

“It all started when I got a pom-pom making set over the summer and decided to raise money for charity making pom-poms. I’m selling pom-poms to raise money for The MPS Society because my cousin Danny and his family are being supported by that charity. Also, I love Danny so much and I want him to make the most of his life however long he lives. Danny has got severe Hunters. In a boy’s body there is an x and a y chromosome, to get the condition that Danny has, the x in a boy’s body goes



wrong and because there isn’t another x to help out, the boy will get Hunters. In a girl’s body there are two x’s so if one x in a girl’s body goes wrong there is another x to help out so girls cannot get Hunters. I want to help Danny and other boys like him. I am making pom-poms of all colours, shapes and sizes for children to hang on their bags because this is very fashionable. Most of the pom-poms will be just £1 to buy.”

Daisy and friends made well over 300 pom-poms to sell over the course of the week and they sold out (and ran out of a wool). She also designed and made a flock of pom-pom sheep which she sold to friends and family outside of school. Daisy even went on to make Christmas pom-poms which have sold well over the festive period.

Thanks to everyone who came to buy, who passed on generous donations and who donated much needed supplies of wool!

In total, Daisy and her team have raised £777.39 for charity.



Mucopolipidosis – a cure starts with awareness



Sam's school SENCO (special needs co-ordinator), Angela Kearney, noticed the reminders coming up on my Facebook page for Rare Diseases Day on May 15th. She asked me if it would be OK if the school purchased wristbands with the message 'Mucopolipidosis Awareness' on them and sell them in school to raise awareness and funds. The school ordered 500 and charged £1 for each wristband. We were quite touched by the suggestion and the wristbands sold very quickly.

Our local church, Sacred Heart-Rochdale, where Sam is an altar server, also asked if they could help raise awareness by selling the wristbands. Approximately 100 were sold in the space of 24 hours and donations reached £200.

In total, around £420 was raised after costs, and split between the MPS and the ISMRD (The International Advocate for Glycoprotein Storage Diseases) who both support our family.

Over the years, people have seen Sam limping or in his wheelchair. They sometimes ask: 'what is 'wrong' with

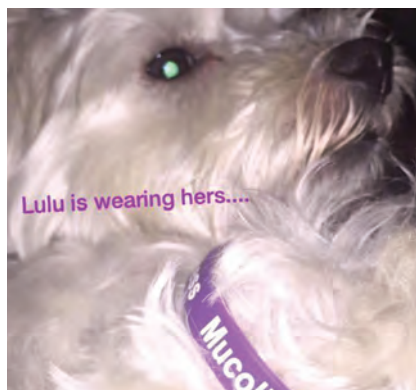
him?' To be honest, we can't always be bothered to tell them the full story as you lose them when you say 'muco'. When they see the wristband, they can look it up. I'm not saying they'll understand it, but at least they will have seen the name.

The aim is to raise awareness, and hopefully gain some support and maybe research into a cure for Mucopolipidosis. Dare to dream!

As an added bit of fun, we decided to send the wristbands to interesting places around the world, and places such as the theatre, seaside, pub. Anywhere where you'd be proud to take a photo of it and share it on social media – therefore spreading the awareness even further.

We used the strapline – 'Mucopolipidosis – a cure starts with awareness'

So far the wristbands have been tagged in Barbados, Dubai, Malaysia, Honduras, Hollywood, Rome, Spain, Venice, New Zealand, Wales, London plus many more cities. They've been abseiling and skydiving, and also seem to like visiting bars, music and sports events too.



Meet our marathoners

Thank you to everyone who applied for a marathon place running for the MPS Society – whether through the ballot or a charity space.

With 26.2 miles to cover these runners will be training hard till 23 April.

Jemma Sibson is 32 and will be running the London Marathon which is her first full marathon having only run the Liverpool Rock n Roll half marathon previously.

“It’s an honour to run for such an amazing charity who clearly make a huge difference to so many people. The MPS Society are very dear to certain friends and family of mine and I only wish they could have been around many years ago to support my close family the way they do for others today.

“This is a huge challenge for me but certainly one I have aspired to since I was young. Training has already started and I am beyond excited about tackling not only an incredible personal challenge but more importantly helping The MPS Society raise funds to help find a cure.

“I would be so grateful for any donation, no amount too small. Every single penny donated will spur me on in training through the harsh winter months and keep me remembering what’s really important. Thank you.”

Jemma’s fundraising page:
<https://goo.gl/vfoSCz>



Amy Wright is 24 and is running in memory of her brother.

“My extraordinary brother Matty passed away in August 2010, two days before his 23rd birthday. He was diagnosed with MPS II Hunter Disease when he was four and throughout his life instilled so much happiness into everyone he met with his contagious laugh, cheeky smile and generally being a bit of a lad. He was THE best brother I could ever have asked for, causing mischief wherever we went, spending many hours racing our peddle go-karts around the garden when we were younger and forming unforgettable memories on our family holidays to Disneyland with my incredibly supportive parents.

“In memory of my brother, I wanted to challenge myself. This led to the formation of ‘MegaMarathon4Matty’. My best friend Robyn and I decided to spend 2016 running several events including the Weymouth, Stratford-Upon-Avon and Hackney Half Marathons and a Tough 10k. This was to raise as much money for the MPS Society as we could, but most importantly in memory of Matty. Now having been given the opportunity to run the London Marathon for this charity that is so close to my heart, it is an amazing opportunity that will be the most challenging event yet, but for the best cause and for the best brother.”

You can keep up to date with Amy and Robyn’s progress here: <https://www.facebook.com/marathon4matty/>

Amy’s fundraising page:
<https://goo.gl/v7WxP7>



Ricky Brown will be taking on the 2017 London Marathon because of Harry Evans.

Harry, a friend of the family, has MPS I. Ricky has run a few events over the last few years and has recently completed the London Vitality 10km Run for the Society. With this charity being the only UK charity to provide support for Harry’s needs, Ricky’s target of £1,500 could go a long way to help.

Ricky is a local Play Leader at the Bearbrook & Elmhurst Early Years Excellence Hub, who has a passion for enriching the lives of young people. He has worked within the Education sector around Aylesbury, over the last 10 years, working at Quarrendon School, Aylesbury Vale Academy and most recently at Elmhurst Primary School as a Learning Support Assistant.

With a 17 Week training plan already underway, Ricky knows that this is going to need a lot of commitment. A few 10km runs along the way and the possibility of a half marathon will all go towards giving him the start he needs to complete the London Marathon in a reasonable time.

“Running the London Marathon has been top of the priority list for me and to finally have this chance and run it for a great cause only makes me want to work hard and support this great charity. Not only for Harry, but others who could also benefit from the research.”

Ricky’s fundraising page:
<https://goo.gl/7ycM1Z>

Barry Hammond is a friend of Ricky Browns and is running for the same cause.

“Following a couple of 10k runs, myself and Ricky Brown thought we needed a bigger challenge, the marathon was the obvious choice! After further discussions (mostly at the pub, where all best plans are made!) we agreed to enter, and run for charity. A close family friend of Ricky’s suffers with MPS, so this was the obvious choice.”



Elizabeth Farwell is running in memory of her brother and sister who both had Sanfilippo.

“I am so excited to have been chosen to run the London Marathon this year in aid of the MPS Society, not just because it will be a chance to take part in such an iconic event, but for personal reasons, it is a cause very close to my heart.

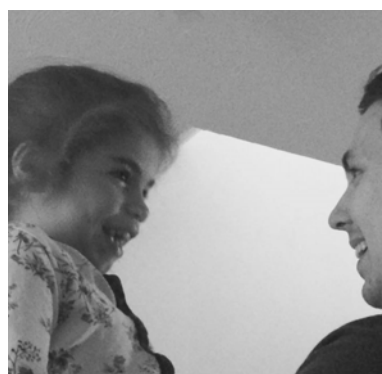
“My elder Brother and Sister were diagnosed at an early age with MPS, Sanfilippo Syndrome. I was only 5 years old when my Sister Katie died, being so young I have only a few memories of her – her love of music, sitting on her lap telling her stories and soft skin. Katie was only 11 when she passed away, but I can still remember that day so clearly.

“My Brother Tom was just 15 years old when he died, and I 12. He was just the kindest most gentle soul you could ever meet. Despite the Syndrome taking control of his body as he grew older I still loved helping him draw, singing to him, or simply just getting into bed with him and chatting to him, even though he couldn’t talk back I knew he was listening. When it came to his funeral I wanted to speak about how amazing he was and remember him in a positive way.

“For those that know, Sanfilippo Syndrome takes over the body gradually, and prevented eventually my Sister and Brother from being able to walk, talk, eat for themselves (needing a gastronomy tube to have food), swallow (needing suction), regular fits (I remember helping my Mother when my Brother would have them and the importance of chatting to him during), and needing 24 hour care. I am so thankful for those night nurses who helped my Sister, Brother, Mother and Father.

“I am doing the Marathon not only to raise vital funds to help families and other children with MPS, but to also keep alive the memory of my beautiful Sister and Brother. They taught me so much without ever even having to say anything. They taught me the importance of caring for those who need it the most and surrounding yourself with positive people. I have entered many 5km, 10km, Obstacle and Half Marathon races over the years and love the challenge of a new distance. The London Marathon will definitely be one to remember!”

Elizabeth’s fundraising page:
<https://goo.gl/eLNxK1>



Daniel Goodge is running for the girl he helps to care for.

“In 2017 I will be running the London marathon in support of the MPS Society. My involvement and support for the MPS Society has developed from being a carer for a beautiful 9 year old girl called Tillie Mae Mawdsley who suffers from Sanfillippo Syndrome. Tillie has faced more challenges in her 9 years of life than most of us will ever face, and despite her limitations she never gives up and never fails to put a smile on people’s faces. I want to keep a smile on Tillie’s

face, which is why I am running the London marathon to raise money to support MPS Society so that important research and support can be given to Tillie and all her friends and their families.

Over the last few years I have been involved in many activities to raise money for the MPS Society however, the marathon is my biggest challenge yet, any donations would be hugely appreciated and will make an enormous difference to kids like Tillie! Thank you!”

Daniel’s fundraising page:
<https://goo.gl/fTrZIX>

Damian Vigus is from Cornwall and will be running the London Marathon in 2017 after securing a ballot place.

Damian is aware of the MPS Society as his sister had Sanfilippo.

Damian’s fundraising page:
<https://goo.gl/aliJUPs>

Sian Young knows a family with MPS and wanted to support our charity when she received her place via the ballot.

If you are running the London Marathon for the MPS Society, or know someone who is, please get in touch. We would love to hear from you.

Wish you could be part of an event for MPS? Just turn the page to see how you can challenge yourself in 2017.

Challenge event opportunities 2017

What will your challenge be?

We have some great charity places and events available in 2017 for those wanting to support the MPS Society in the most challenging way. Spaces are offered on a first come, first served basis. Please register your interest at fundraising@mpssociety.org.uk for any of these events or let us know if there's something else you're interested in.

10K races

Great Birmingham 10k

Date: 30 April 2017
Distance: 10 km

This run's first 6km is generally flat, allowing plenty of time to warm up the legs in preparation for the slightly more challenging uphill section between 7km and 8km as the route heads back towards the city centre. The final flat and fast section is topped off with a downhill finish into Broad Street. Drinks stations, bands, cheering supporters and buses will be found along the route with a lovely meet up at Centenary Square surrounded by iconic buildings.

Great Manchester Run 10k

Date: 28 May 2017
Distance: 10k

The Great Manchester Run starts on Portland Street and runners will end of by crossing the finishing line at Deansgate. Runners will get to see the Manchester United's Old Trafford stadium, Imperial War Museum and The Lowry and famous cobbles of Coronation Street. Close to the end, runners will pass the 47-storey Beetham Tower skyscraper. Plenty of cheer as well as music will be provided by DJ's and live bands right throughout the run.

Great Women's 10k Glasgow

Date: 4 June 2017
Distance: 10k

Last year saw a brand new course for the Great Women's 10K – Glasgow which started on Kelvin Way. The runners pass Glasgow's famous Kelvingrove Art Gallery, Glasgow University, The Riverside Museum and the SSE Hydro. Post-

event, the party atmosphere will continue in Kelvingrove Park with lots to enjoy.

Great Newham London Run

Date: 2 July 2017
Distance: 10k

Not only will runners of this 10k route pass the sights of Lee Valley VeloPark and London Aquatics Centre, but they will also finish their run inside the Queen Elizabeth Olympic Park. Fancy running in the path of Olympic greats such as Usain Bolt, Jessica Ennis-Hill or Mo Farah? Then this race is perfect for you.

London 10k

Date: 9 July 2017
Distance: 10k

This is the UK's most prestigious 10km road race where 25 000 runners will fill the streets of London. Runners will get to see Big Ben, London Eye, St Paul's Cathedral, Trafalgar Square and Westminster Abbey.

Great Yorkshire Run – Harrogate

Date: July 2017 (exact day to be confirmed)
Distance: 10k

The Great Yorkshire Run – Harrogate is quite a unique run all set within a beautiful town. Amongst other beautiful sights, this 10k route starts and finishes on Montpellier Hill in the Montpellier Quarter. Runners will also pass the beautiful English Heritage Grade II Listed Valley Gardens alongside blooming shrubs and herbaceous beds as well as the Royal Horticultural Society's Harlow Carr gardens.

10 mile races

Great Edinburgh Run

Date: 23 April 2017
Distance: 10 miles

Participants will have the opportunity to take part in the only 10 mile run that goes through the heart of Edinburgh! This run starts and finishes in Holyrood Park. Runners will get to pass famous landmarks such as Edinburgh Castle, Greyfriars Bobby, Scott Monument and the beautiful Arthur's Seat.

Great South Run

Date: 22 October 2017
Distance: 10 miles

This is the world's leading 10 mile race. Runners will pass the iconic sights of Southsea and Portsmouth, including the Portsmouth Historic Dockyard, home of HMS Victory, past Spinnaker Tower, and the beautiful views of the Isle of Wight.

All runners are subject to a minimum pledge per run and will receive a fundraising pack, MPS running vest as well as ongoing support from the fundraising team.

If you have managed to secure a place on your own in any of the above runs, then please do let us know.

If, however, you have not been contacted and your application was unsuccessful then you are still more than welcome to join in any other challenge events in support of the Society, which we would love to hear about.

We value our supporters and appreciate the various challenges you undertake in support of the Society.

Great Manchester Run Half Marathon

Date: 28 May 2017
Distance: 13.1 miles

This run is a great tour of the city. It passes by UK's iconic football grounds, the Etihad Stadium and Old Trafford. It also passes the Imperial War Museum and Albert Square. Live music and cheering will keep you motivated throughout the run.

Great North Run

Date: 10 September 2017
Distance: 13.1 miles

This run starts at Newcastle upon Tyne with the route taking runners through the city centre towards the River Tyne. Whilst passing Gateshead runners will be entertained by music bands. The route will also lead runners to South Shields where at the top of Prince Edward Road, the view of the sea is one of the most welcoming.

Great Bristol Half Marathon

Date: 17 September 2017
Distance: 13.1 miles

This run is filled with history. It was first staged in 1989 and has become a long-held tradition with over 10,000 runners joining this historic run.

The course starts and finishes in Bristol's historic Harbourside. A scenic sea route takes runners past the Harbourside, along the Portway, through Avon Gorge, and under Brunel's famous Clifton Suspension Bridge. Finally runners will go around the old city before returning to Anchor Road.

Virgin Money London Marathon

Date: 23 April 2017
Distance: 26.2 miles

The Virgin Money London Marathon runs just south of the Thames through Greenwich and Blackheath. Thereafter runners will cross the river on Tower Bridge and pass some of the capital's famous landmarks, including the Coca Cola London Eye and the Tower of London, before finishing in front of Buckingham Palace.

Although our places for the 2017 London Marathon has now been filled, there is always an option to partake in this event in years to come. Make sure to register your interest with us early as this event is extremely popular and spaces are filled fast.

Running not your thing? Perhaps you would enjoy getting your adrenaline pumping by trying out a skydive, a trek or cycling challenge.

UK Skydiving Adventures

Skydiving is considered an extreme sport, not something for the faint hearted, but an absolutely wonderful experience and quite a bold way of raising funds! If you fancy a Tandem Skydive, Static Line Parachute Jump or Accelerated Freefall Skydive let the MPS fundraising team know and we will forward on all of the information you may need to take that daring jump in support of the Society.

Discover Adventure

Discover Adventure offers various trekking and cycling challenges that help to fulfil your personal ambitions as well as help support a charity. Challenges are available worldwide and with so many fantastic adventures available there is bound to be one to interest you. You could try an epic 10 day trek through Madagascar, a cycle ride from London to Paris or even an incredible Inca Trail Trek!

Find out more at
www.discoveradventure.com

Challenge Northumberland

The 3rd Mad March Mare event is taking place on Saturday 4 March 2017. This is a 10km multi-terrain obstacle race at Hexham Racecourse. To complete the challenges you will need to overcome 35 obstacles, battling through 10km of multi-terrain surfaces, including waterfalls and steep ravines.

To sign up visit
www.madmarchmare.co.uk

The Events Series**The Isle of Wight Challenge
29–30 April 2017**

Great challenges need great settings, and none are better than this stunning coastal path. 2,000 adventurers will take on the Island, past the famous Needles, to Cowes at half way, then around back to base camp after a spectacular 106km with full support and great hospitality all the way. You can choose the full island coastal path or do a half or quarter option.

www.isleofwightchallenge.com

**The London 2 Brighton Challenge
27–28 May 2017**

Over the May Bank Holiday 2,000 or so will take this classic capital to coast challenge. Most will walk it at a pace that suits them, quite a few will jog parts, and some will run the whole course. You can join as an individual or a team with full, half or quarter options available and more than 24 hours to complete the challenge you can set your own pace.

www.london2brightonchallenge.com

**The Cotswold Way Challenge
1–2 July 2017**

From Bath to Cheltenham, the magical Cotswold Way crosses areas of outstanding natural beauty, with fantastic panoramic views. It's a great setting for a new Ultra Challenge which offers 100km, half and quarter options.

www.cotswoldwaychallenge.com



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

Helen Tandy has Fabry and took part in Tough Mudder and has raised £250 so far via her JustGiving page. She has also written a blog about it.



The Old Steine Lodge 7875 in Brighton held a charity raffle and donated £65 to the Society. We were nominated by their Worshipful Master, Dennis Fiore, and his partner, Linda Windsor.

Karen Botterill from Northampton held a coffee morning with family and friends. Karen's nephew, Corey, has MPS I. Karen describes Corey as such a character and an inspiration to all. They also sold phoenix cards and a percentage was donated to the Society, £150 was raised including donations.

Jane, Martine and Ella from Oxon held a coffee and cake morning at the local Methodist church in the High Street in Witney and raised £50.

Cissbury Lodge in Worthing donated £305 raised by holding a Ladies' Night. The donation was made on behalf of Lyn Windsor.

Mytham Primary School in Bolton held a Summer Fayre and raised £52 for the Society.

Sarah Cairns from Sheffield raised £264 in recognition of families like Harley Bond and other MPS families at Templa Spa through pampering and raffles.

Karen Anderson from Bournemouth raised £60 by holding a dress down day and a harvest festival raffle, Karen supported the MPS Society as a colleague has a nephew with MPS III.

The girls of Digby House at Woldingham School in Surrey aged between 11 and 18 have been running sales of various descriptions and also were lucky to receive the schools prize day collection. The girls donated £2,463.32. They chose the Society due to one of the girls having family members who are suffering and have suffered with MPS.

Andy Bell from Manchester raised £438.80 by doing a skydive.

Rachel Yaxley from Ipswich, donated £72.92 in aid of a colleague's son.

Hemel Hempstead Methodist Church in Herts held a coffee morning and raised £51.55 for the Society.

Julie Hedges from Plymouth raised £3641 completing a charity walk.

Walkers News raised £100 from a collection box organised by Barbara Penny.

Chi Chi London donated £1500 on behalf of James Stuart.

Abaco Systems donated £408.19 for Employee Recognition to their customer support team.

Matthew Lamb raised £126.65 by holding a Rustic Crust event.

Monique Hirshman from Plymouth donated £100 instead of sending Christmas cards.

Louise Hiller donated £20 in lieu of sending Christmas Cards.

Jonathan Freeman ran the Glasgow 10k and raised £1217.47 on JustGiving on behalf of his nephew who has MPS.

Genzyme held a Bake it Blue sale and raised £20.36.



Donations

Eamonn Drayne; John Heath and Sons; Hannah Watts; Sarah Vickery; F G Robinson; V Dawson; J Brierley; Margaret Millar Deas; Lucy Brock; Mrs R J Scott; N Saville; N Belam; Mrs Eira March; B C Tilbury; Tina Hamilton; Samantha Matthews; Phillip Matthews; Julia Thompson; Oscar and Ruby Mitcham; Alan Marshall; Ian MacIntyre; Kevin Heap; Sarah Winzar; Mrs V Lazenby; Emily Mason; Andrew Baker; Mr T Brown; Kay Todd; Casse Edmiston; Mrs S Swayne; Mrs Y Puddy; Mrs D Duckett; Mrs A J Gunary; Valerie Zaldua; Ellen Louise Nicholson; Jenny Quant; Joan Ingram; Susan E Birks; P Rock; Christine Hume; Amicus Therapeutics UK Ltd; Moira Shaw; Jenny Mitcham; Katy Burson; Elizabeth Mullen; Frances Allenby; Enkalon Foundation; Carlo Copsey; Nathan Worsford; Barbara and Craig Newson; Ricky Brown; John Michael Brown; Joseph Lee; Mr A W Hall; Ian Mahoney; RN Dunn and Mrs K Dunn; Mark Hughes; Howard and Linda Matthews; Mr and Mrs Vockins; Tara Symonds; M Briggs; Chris and Julie Kembrey; Mrs A Baker; Viv Culley; Marlene Murty; Emma Morrice; Maureen Loveday; Elizabeth Hickman; George Vogt; Helen Clayton; Rachael Jones; Shaista Yaqoob; Pam

Hope; Teresa McGinn; Glen McKee; SG Clarke; Mrs M Haigh; Carol Copsey; Edna Morrison; Debra Horsley; Karen Botterill; Dr P Gordon; Katrina Gedge; Sarah Borrett; Jennifer Johnston; Moira Darke; Catriona Ogilvie; Pauline Selwood; G Berry; Pauline Headland; E Nally; Dee Baker; Gill Sutcliffe; Mrs D Kelly; Margaret Lyon; Michael Skidmore Estate Agents; Elizabeth Powell; JB & AV Kennett; Mellina Aston; Jeremy Lowe; Mrs M E Horsley; R JL Byrom; Adele Jones; Christopher Stanley; Shirley Jamil; Lynn Austin; Peter & Jean Hawkins; Wendy Turner; J C Sumner; Wendy McGinn; Janey & Errol Jones; Rachael Jones; Aron Greenidge; Shirley Jamil; Mrs V Dawson; Colin & Dorothy; Sue & Vic Lowry; Abigail Jones.

[Donations via collection boxes, stamps, foreign coins, mobile phones, ink cartridges, jewellery, PayPal Giving, eBay for charities](#)

Judith Evans, Andy and Jenny Hardy, Elizabeth Heath, Mr & Mrs Hunt, James Garthwaite, Kathryn Wilson, Melvyn Wilcox.

[Regular contributions by Standing Order or Give As You Earn](#)

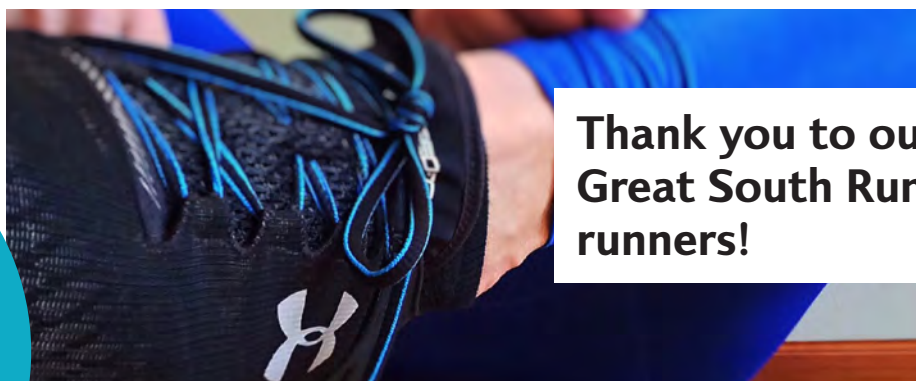
M Newell; J Casey; D Palmer; J Hastings; J Winzar; E Lee; K Hudson; D Winzar; R

& K Dunn; Saville Norman; M Newell; Helen Robinson; Marcia Tosland; C Cullen; S Bhachu; S Brown; V Lucas; D Forbes; Manjit Kalsi; J Richards; P Summerton; A Sullivan; A Weston; E White; C Hume; Anthony Lockyer; S Winzar; G Simpson; W Cavanagh; Barbara Harris; L Brodie; A Byrne; Dorothy Robinson; N Cadman; J Wilson; A Tresidder; Joyce Arnold; K Osborne; E Cox; M Rigby; N Thompson; Stuart Robinson; M Peach; C Garthwaite; J Ellis; I & V Pearson; A Sabin; A Ephraim; J Daligan; M Malcolm; E Mee; E Moody; S & D Greening; M Hahner; K Brown; Z Gul; M Fullalove; M Leask; E Brock; M Reeves; E Parkinson; G Ferrier; R Taylor; R Gregory; L Stillwell; R Henshell; K Bown; S & J Home; V Little; Neil Upton; J & J Heritage; D Forbes; C Gibbs; A Cock; A Dickerson; B Harriss; D Jowett; Arif Moledina; Frances Gee; Jonathan Croft; B M Giles.

In memory

Andrew Leslie Harris; Luke Edwards; Sophie Elizabeth Summerton; Chloe Walker; Jack Stuart; Katie Devine, Helen Skidmore; Evelyn Jarvie; Andrew Millar; Gethin Robins; Gracie Bella Sims; Denis Rowan; Betty McConnell; Daniel Allen; Anabelle Shepherd.

Thank you to everyone who donated anonymously in 2016 - we don't know who you are, but we think you're great!



Thank you to our Great South Run runners!

Emma Bebington from Croydon ran in the Great South Run on 23 October in Portsmouth and raised £1,405 including £500 from Legal and General as matched giving.

Anne Golborne ran the Great South Run and raised £220.40 including gift aid. She said: "I work with some of the children affected and their families. Amazing children!"

Terrie Brown ran the Great South Run in memory of Anabelle Shepherd and raised £291.25 including gift aid. This was her fourth Great South Run and when she trained for her first one four years ago it was also for Anabelle who Terrie described as "a very special little girl...who always had a big smile."

Jodie Costello ran the Great South Run in memory of her little brother, Lewis Cato, and to help raise awareness for other children with MPS. She raised £80 including gift aid.



Patient Access to Clinical Trials and Treatment

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

Registered Address:

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

mps@mpsact.com

www.mpsociety.org.uk/commercial

T: 0345 389 9901

F: 0345 389 9902

Board of Directors

Chair

Bob Stevens

Board

Jessica Kafzas

Sophie Thomas

Georgina Smith

David Patterson

Secretary

Christine Lavery

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

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

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

MPS Commercial Employees

Christine Lavery – Group Chief Executive
c.lavery@mpsact.com

Gina Smith – Group Finance Officer
g.smith@mpsact.com

Charlotte Roberts – Business Development Manager
c.roberts@mpsact.com

Joanne Goodman – Clinical Trial & Patient Access Officer
j.goodman@mpsact.com

Benedicta Marshall-Andrew – Clinical Trial & Patient Access Officer
b.marshall-andrew@mpsact.com

Alexandra Morrison – Clinical Data Lead
a.morrison@mpsact.com

Jacqueline Adam – Clinical Communications Lead
j.adam@mpsact.com

Pauline Walker – Finance Assistant to MPS Commercial
p.walker@mpsact.com

Vimizim Managed Access Agreement (MAA) update

It is now a year since Vimizim (elosulfase alfa) treatment was made available in England for the treatment of MPS IVA. Around 50 individuals have received treatment under the scheme and many are approaching their 1 year MAA anniversary when decisions around the continuation of treatment will be made. We have written to everyone involved in the programme with the details of this process.

Clinicians will be reviewing response to treatment against the MAA criteria to determine whether individuals are benefiting from treatment and will remain on the MAA. If there is any doubt as to whether an individual should remain on treatment or not, their case will be referred to a clinical review meeting, the first of which is to be held in March 2017. At this time the MAA stakeholders will also meet to discuss and evaluate the scheme and the experience gained over the 1st year of the programme.

Since our last update, a number of individuals with MPS IVA in Northern Ireland have started Vimizim treatment following the introduction of the MAA there in July 2016.

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

www.mpsociety.org.uk/commercial

Meet the team

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

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

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

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

Benedicta provides a logistical service for individuals participating in clinical trials

Alex supports the newly introduced Managed Access Programme for Vimizim

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

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



Co Tyrone man 'handed back his life' after drug approval

A Co Tyrone man has said he feels like he has been 'handed back his life' after a medicinal drug was approved for the first time.

Dermot Devlin was speaking after Health Minister Michelle O'Neill announced the news that Vimizin would be made available to patients in Northern Ireland.

The 36-year-old is one of only 15 people in NI who suffers from the extremely rare and life-threatening Morquio Syndrome (MPS), which restricts growth.

Read the full story at: <https://goo.gl/n0qpR9>

European study of MPS III

A major project for the MPS Commercial team over the last few months has been the development of a piece of research to extend the knowledge and understanding of MPS III across Europe.

Although two clinical trials for enzyme replacement therapy in MPS III sadly ended in 2016, international research continues with a number of companies involved in the development of gene, enzyme replacement and substrate reduction therapies.

Our study will provide vital information to support companies in their treatment development programmes. It will also benefit the wider MPS III community by exploring the patterns of symptoms that lead to a diagnosis of MPS III and the routes families take through the medical system and specialists involved in reaching a definitive diagnosis. It is hoped that the information gathered will support earlier diagnosis of MPS III.

Industry collaboration

A unique feature of the study will be the collaboration of MPS Commercial with a number of pharmaceutical companies who will contribute to the design of the study and share equally its results and findings.

The benefits of a collaboration of this nature is that it both reduces the number of times that individual families are contacted to take part in research on behalf of individual companies and the combined industry support allows us to

extend the project much wider than we would normally be able to.

One of the largest studies of it's type

The project will be a questionnaire based survey that seeks to fill some of the current knowledge gaps in MPS III. We are currently working closely with the sponsors to ensure our specifically designed questionnaire meets their needs.

Our intention is to conduct 200 questionnaires across a number of European countries with the help of the European MPS Network. Questionnaires will be completed over the telephone and administered by a member of the local MPS Society. Plans are in place to hold a training day with our European MPS Society colleagues in January and we hope to launch the survey in February 2017.

Get involved

If you would like to take part in the survey our find out more about the study please contact Alex Morrison at a.morrison@mpspact.com.

World symposium

Representatives from MPS Commercial will be attending the World Symposium being held in San Diego in February 2017. The WORLD Symposium is often cited as the most important scientific meeting on lysosomal molecular biology, disorders and treatment. Researchers, clinicians, patient organisations and pharmaceutical companies from around the world will be attending and presenting.

We are pleased to announce that the two abstracts we submitted for the meeting have been accepted and we will be presenting these at the poster sessions being held on the 14th and 16th February. The first poster describes the development of the Vimizim (elosulfase alfa) Managed Access Agreement, and the second looks at individuals with MPS IVA's experience of enzyme replacement therapy.

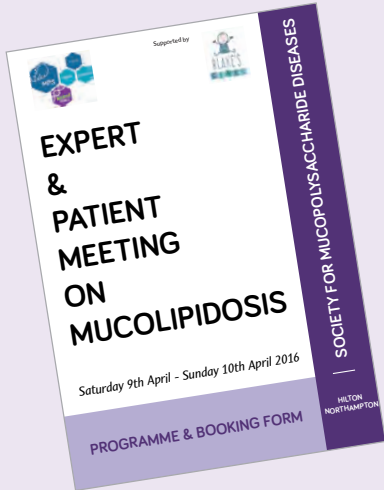
We'll also be available to meet up and discuss current and future projects so please do get in touch if you are attending World and would like to meet us. Contact Charlotte Roberts at c.roberts@mpspact.com.



Welsh Information Day was held on 16 January



Lysogene sponsor first scientific workshop on gangliosidosis



Wear It Blue!
FOR MPS AWARENESS DAY
15th May 2016

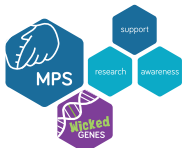


Drayton Manor family weekend was held from 5th to 7th August

8 of you ran the Great North Run raising money and awareness for MPS

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

15 - 16 October 2016
Hilton, Northampton



First Morquio patients receive Vimizim in Northern Ireland

