MPS magazine

Society for Mucopolysaccharide Diseases

Winter 2017 • www.mpssociety.org.uk

My good friend Christine

A TRIBUTE TO A SPECIAL WOMAN

MENTAL CAPACITY ACT

Part 2 of our series on understanding the Act

NATIONAL DRAW

Winners and prizes announced on page 8

Focus on clinical trials in this issue

wicked walks

our halloween local walk

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.**

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

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

Map on page 11: Scotland - Single Color by FreeVectorMaps.com

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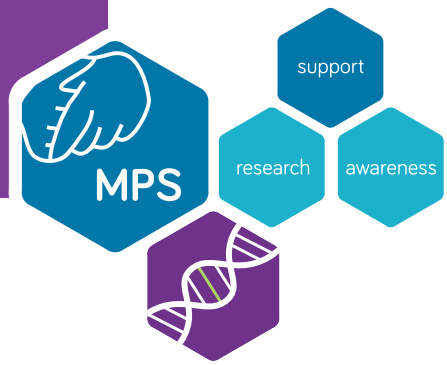

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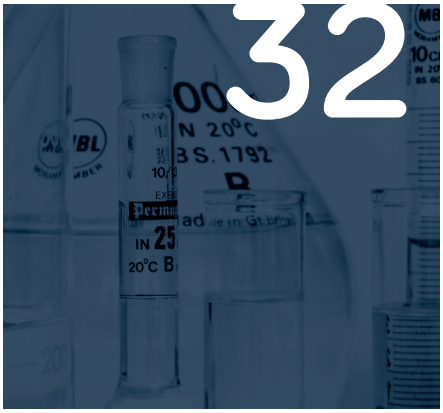

FOCUS ON CLINICAL TRIALS

A special round up of all the ways the MPS Society is involved with clinical trials and why they are important

ALL OUR EVENTS

News and photos from the regional events

LONDON MARATHON

Meet the runners braving 26.2 miles for MPS

WELCOME

This is a strange issue for us, as you can imagine. Production had started before Christine passed away so there are many photos, references and articles that mention her and we made the decision to leave them in. We wanted to carry on as normal as we believe that's what Christine would expect of us. So we have kept this issue of the magazine pretty much as Christine intended.

Therefore it includes our special feature on clinical trials, it includes news and announcements from fundraising, advocacy and MPS Commercial and it includes all the usual research news. It also includes a fitting tribute to a wonderful friend and colleague written by Wilma, who was with Christine from the beginning as she built the MPS Society into the charity and patient organisation it is today.

We hope it gives you the opportunity to remember Christine as we all do – a formidable and hard working woman who cared so much for all those who suffer at the hands of MPS and related diseases.

Please let us know if your contact details have changed.

We want to make sure we have your correct phone number, email and postal address so if you've moved house please tell us at: magazine@mpssociety.org.uk

Planting the saplings at Childhood Wood

My friend, Christine

Wilma, trustee of the MPS Society, mother to an MPS child and great friend to Christine pays a personal tribute to a special woman

By Wilma Robins

I was with Christine for the weekend at the beginning of December, as it turned out, for the last time. It was a very busy weekend, governance training all day on Friday at MPS House, the MPS staff and Trustees Christmas dinner on Friday evening, and the Board of Trustees meeting all day Saturday. As usual, I stayed with her for the remainder of the weekend. She was happy, on top of the world. A chest infection which had troubled her for months had finally gone, and, more importantly, the latest biopsy had shown she was clear of myeloma. She was well and full of energy, enthusiastically making plans for the MPS activities in the New Year. She was going to Tel Aviv for the first time in January, and, in particular, she was looking forward to the annual MPS WORLD Symposium in San Diego in California in February. She was very excited about the plans beginning to take shape for the celebrations to mark the 25th anniversary of the Childhood Wood, a fun packed, fundraising event at the end of June this year. She was at last content, in peace of mind about the future of the MPS Society for which she cared above all else.

> I became more of a personal friend to Christine during this time. . .and central to our lives was the MPS Society

My husband Peter and I first met Christine and her husband Robin in June 1982. Our young son, Gethin, was nearly three years old and had been diagnosed with MPSI, Hurler syndrome, in May 1980 at the age of ten months. We had never heard of such a thing, and no internet in those days! For the next two years we were desolate, we began to think we were the only family in the world with something called mucopolysaccharidosis. Then, by chance, in Guy's Hospital we met another MPS family who told us that a family in Little Chalfont whose son had MPS and had recently died, had set up a support group for MPS families. We phoned the telephone number given immediately on returning home and arranged to meet Robin and Christine Lavery. I shall always remember Christine's first words, she turned to Robin and said of our son Gethin, "he is a typical three year old Hurler", and we had thought there was no one else in the world with MPS. Their son Simon who had MPS II, Hunter syndrome, had recently passed away, aged seven. Not having had any contact with any other MPS families, and thinking there must be others, they set about finding other MPS families and eventually contact was made. At this point Robin and Christine held their first fundraising event, a coffee morning around their kitchen table, which realised £25. A big decision had to be made, should this princely sum be put in the church box or should they be brave and proceed to start a support group. Thank goodness, they began the support group, and the MPS Society was founded. Within weeks the first MPS Newsletter was sent out and in September 1982 the first family conference with 50 families attending was held in Birmingham, barely four months since the speck of an idea had emerged in the minds of Robin and Christine. It transformed the lives of MPS families, just to be together and share experiences relieved the isolation, we were no longer alone. Sadly, our son Gethin died in December 1984, aged five and a half. Peter and I knew we had to remain close to the MPS Society to contribute to its progress to support others.

The period since 1982 to the present time has witnessed the greatest technological development and the MPS Society has evolved accordingly. What started at the kitchen table is now known in very many countries worldwide. Enzyme replacement therapy has changed the lives of so many MPS children, many now young adults; and those with other related diseases. Christine devoted her life to develop the organisation, working closely with doctors and pharmaceutical companies all over the world. Sadly, tragedy struck Christine's life when Robin died suddenly in January 2008. He had been her staunch, unfailing support during a time when the MPS Society, of necessity, became ever more demanding to keep pace with the rapid progress of medical research. I became more of a personal friend to Christine during this time, and following the death of my husband Peter in 2009, we became closer, supporting one another, and central to our lives was the MPS Society. There was a further setback in Christine's life in December 2015 when she was diagnosed with myeloma. I say setback, because she refused to let it affect her life any more than that. She had to undergo major surgery to repair her broken back, the myeloma having damaged the skeleton, and she endured months of harsh chemotherapy and a stem cell transplant. She did not ever give in to this most debilitating condition, travelling the world whenever she could possibly do so, to establish and nurture the work of the MPS Society. As Trustees we pleaded with her to refrain from such pressure. The appointment of Bob Stevens as Director of Operations and Governance in April last year, and with whom she worked closely over the following months, finally satisfied her that she could begin to step back. At last she was content that the Society was in safe hands and she was looking forward to introducing Bob to all the important contacts in San Diego in February. She promised me that she would begin to do less, even to the extent of contemplating some form of retirement in the coming year! One big task remained, she said, the volumes of paper of the story and history of the organisation needed to be archived, to ensure her legacy.

Her sudden death on Tuesday December 19th stunned us all. But rest Christine, and be assured that Bob and the team in MPS House, Paul and the Board of Trustees, will work tirelessly to ensure that your beloved MPS Society will endure and continue to thrive.

Families at the first MPS Conference in 1982

News from the Board of Trustees

The Chair, Paul Moody welcomed the Trustees and stated that this meeting was unusual as it was a one day meeting. Paul Moody welcomed Bob Stevens who had recently been appointed Head of Operations and Governance. Judy Holroyd and Tim Summerton had sent their apologies and no conflicts of interest were recorded.

In matters arising The Group Chief Executive was asked to arrange the next Governance training for Friday 1 December 2017.

Christine Lavery gave the Group Chief Executive Report confirming that Bob Stevens will be responsible for all internal matters and she for all external work and relationships.

The Group Chief Executive spoke about the GDPR regulations coming in from May 2018 and how this is a huge change to how to data is managed. In simple terms the regulations will affect how we store sensitive information on both paper and electronically. The MPS Society has met with Cassie Edmiston from the Prison Reform Charity on the GDPR to discuss areas of concern in relation to rolling out the GDPR.

Governance

There were no changes to the Business Continuity Plan and risk register.

Bob Stevens outlined the business review he had undertaken and took the trustees through the challenges, opportunities and new proposed structure.

Trustee Recruitment

Under Trustee Recruitment all Trustees were asked to consider the resolution that Daniella Vandepeer in accordance with the Company's constitution, be co-opted as a Director (Trustee) of the Company. This was agreed unanimously.

Financial Management

Bob Stevens reported to the Board the financial position of the MPS Society and a necessary change that had to be made to the Group Accounts.

Research Funding

Board member, Professor Brian Winchester gave an excellent presentation on gene editing, induced pluri-potent stem cells and the future of gene therapy.

The Board considered a grant application for £15,000 to study the value of portable technologies in recording day to day patient monitored information in children and young people with Fabry Disease: A Pilot Study proposal from Dr Uma Ramaswami at the Royal Free Hospital, London. This was agreed unanimously.

Support and Advocacy Service

The Support and Advocacy report was considered and Trustees asked for changes.

Events

It was reported that the Bereaved Parent Weekend at Thoresby Hall in October will be very well attended with 59 attendees. Christine Lavery and Wilma Robins will be attending and leading. It was suggested that a committee of bereaved parents might help with organising the event.

Bob Stevens gave all trustees the trustee programme of events and explained the schedule. Wilma Robins will be leading the trustees on this initiative at the MPS Weekend Conference in July.

Policies

Safeguarding Children, Young People & Adults at Risk Policy – to be reviewed after the MPS Weekend Conference

Volunteer Carers Policy & Procedures – Agreed unanimously

WHAT'S ON?

Regional Clinics

Great Ormond Street Hospital

MPS I – 27 February, 26 June, 11 September, 27 November
MPS III – 13 March, 10 July, 25 September, 11 December

Birmingham Children's Hospital

MPSIVA – 17 August
MPSIII – 20 July, 19 October
Fabry – 21 September
LAL D – 10 August
MPSII – 16 March
MPSVI – 16 November
MPSI – 18 May, 15 June
MPSVI – 16 November
Mixed clinic – 21 December

Queen Elizabeth Hospital Birmingham

Adult Fabry – 13 March, 10 April, 8 May, 12 June, 10 July, 14 August, 11 September, 9 October, 13 November, 11 December

Manchester Children's Hospital

Post HSCT 0–10 year – 20 April, 13 July, 12 October

Post HSCT 10 year plus – 26 January, 27 April, 20 July, 19 October

Regional events

25th anniversary celebration of the Childhood Wood
30 June 2018

Northern Ireland Family Information day • Hilton Hotel, Templepatrick
19 May 2018

Weekend for bereaved families • Warner Leisure Hotel • Thoresby Hall, Nottinghamshire
12–15 October 2018

Childhood Wood Planting
14 October 2018

Conferences and expert meetings

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

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

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

Announcements

FAREWELL

Gina Smith left the MPS Society in September after 17 years running the MPS Society's finances. Gina was here as the MPS Society developed into the established patient support organisation it is today and the MPS Society wish her well as she moves on to pastures new.

NEW MEMBERS

Mr and Mrs Fisal have recently been in contact with the Society. Their son, Abdulla, has a diagnoses of MPSIH. Abdulla is 14 years old. The family live in London.

Nic and Darren have recently been in contact with the Society. Their daughter has a diagnosis of Sanfilippo Disease. Isla is 4 years old. The family live in West Yorkshire.

Jo has recently been in contact with the Society. Her son has a diagnosis of MPS I Hurler. The family live in Essex.

Eleanor (always known as Cookie to friends and family) has recently been in contact with the Society. She has a diagnosis of Fabry disease. The family live in the Sussex area. She would be happy to help and support any fundraising for the Society in the Sussex area.

Mark has recently been in contact with the Society. He has Fabry Disease. The family live in the South East. Mark attends Royal Free and St Barts Cardiac Unit. He is married with two boys.

Alison and David have recently been in touch with the Society as Alison and their sons, Oscar and Alex, have Fabry. The family live in Glasgow and would be very happy to speak with other families that have been affected by Fabry.

A big welcome to our newest Trustee, Daniella Vandepeer

My name is Daniella but most people call me Dani! I live in Hertfordshire with my husband, Mark, and our boys, Caleb (10) who has MPS II and Gabriel (3) who is unaffected. We are currently expecting our third baby, a daughter, in March 2018. In the last five years I have worked for a local sexual health charity as an outreach worker supporting those living with HIV. I am passionate about advocacy and also help support local mums getting to grips with a difficult diagnosis.

National draw

Thank you to everyone who bought and sold tickets for the National Draw in 2017. The money you raised from the draw will provide support for individuals and families affected by MPS and related diseases as well as funding research into treatments and a cure. Here is a list of all the lucky winners. Congratulations, we hope you enjoy your prizes! We would love to hear about your experiences so get in touch if you want to share.

TOP PRIZE 3 night stay for two including breakfast at the Adina Hotel in either Berlin, Hamburg, Frankfurt, Nuremburg, Leipzig, Copenhagen or Budapest Adina Hotels – M McAllister (08262)

SECOND 2 first class return tickets with Virgin Trains West Coast Line – Amy Hongue (15524)

THIRD 1 night stay for two at The Staybridge Suites in Birmingham – A Greening (13785)

Anthony Joshua signed boxing glove – Alison Beales (09288)

Marks and Spencer £150 voucher – D Lee (21802)

Two Eurostar tickets – G Berry (23062)

John Lewis £50 voucher – Ben Deverell (15753)

Clarins gift box – D Gedge (13706)

Tesco £50 voucher – M Jones (11644)

Ocado £50 voucher – Michael Bond (15790)

Two tickets for West End musical '42nd Street' – Danielle Swain (00861)

Silver Wren and Oak bespoke silver oak leaf pendant – Tammy Didcock (00852)

Amazon £50 voucher donated by Snopake – Lisette Dupre (15692)

The Entertainer £50 voucher – Richard Zeronign (13937)

Galt Toys Dino Play nest – Russell Bywater (12821)

Clarks £25 voucher – Mrs K Pyke (20118)

Fired Works £20 online voucher – Gwen Williams (15615)

L'Oreal beauty products – Crispin John (08240)

Redbush Tea Company gift set and books – Jess Dicks (00849)

Thompson and Morgan Seeds £20 Voucher – Mel Harvey (12864)

Two tickets for Kew Gardens – Ray Franklin (24701)

Bluebeards Revenge men's grooming products – Rik Bhachu (13921)

Two Everyman Cinema tickets – Lee-Anne Lorimer (22377)

Thompson and Morgan solar lights – Mrs A McMulle (05300)

Boxclever Press family diary – Mr Witts (09146)

Bucks Leather colourful cotton pashmina – Mr Witts (09150)

Box of Liquorice Allsorts and box of Wine Gums – E Morrice (11641)

Congratulations to David Oulton who has MPSII and recently celebrated his 30th birthday and to Oliver Stevens who has MPSII and recently celebrated his 18th birthday.

We hope you both had a great day!

Miss Lily Cheeks

From Fabry's sufferer to a pinup in the making, Gemma also known as Miss Lily Cheeks, is pushing out the boundaries of her condition and not letting pain crisis and stomach flare-ups stop her.

Readers may remember Miss Lily Cheeks, from the Spring 2017 issue. Gemma took part in the Miss Pinup UK 2017 competition in October and finished in the top 10!

"The competition was amazing. The day before I had severe Fabry's issues – mostly stomach based which left me feeling a bit deflated and I was not my usual bubbly self (except in the stomach department) when I first got to the venue.

I have to say that every competitor was immensely talented, beautiful and amazingly supportive and the energy in the dressing room was so positive that I perked up within about 10 minutes of being around them.

I was lucky enough to get through to the final 10, (much to my sweary surprise). I got to perform my Musical number with Tap dancing at the Gala Dinner in the main hall for the 4 previous winners of Miss Pinup Uk which is a huge honour. Although I didn't get into the top 3, I still feel like I won a personal challenge that day! Not only did I prove to myself that Fabrys Disease will not stop me doing something I love but I came away with a larger Pinup family. Don't let Fabrys stop you doing something you love, just make sure you plan for every eventuality – spare pants is a good place to start!

For anyone interested I have very recently started a blog, beginning with my Pinup experience and will continue to update weekly on my adventures! http://misslilycheeks.blogspot.co.uk

Further information about Miss Pinup UK and Miss Pinup International can be found here: www.pinupuk.com

Advocacy

Advocacy team visit to Scotland

During the last week of October, myself and my colleague Debbie Cavell travelled to various locations in the Southern part of Scotland and particularly concentrated on meeting families in the wider Glasgow and Edinburgh areas, where we hoped we would meet a number of families and we did.

We planned the visit as both Debbie and I had contact with a number of families who needed our support, and were then fortunate to meet other families that we have not had contact with for a little while. As well and conducting individual visits we also set up meet and greet sessions in Glasgow and Edinburgh where we met families and had an opportunity to chat informally.

Steve Cotterell

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

s.cotterell@mpssociety.org.uk
07341 564185

Sally Briody

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

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Rebecca Brandon

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

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Meet the team

Our advocacy support service is at the core of everything we do at the MPS Society. We know how isolating and challenging it can be living with MPS or a related disease so we want you to know that you are not alone and we are here to help. We are always striving to improve the support we offer and to sure we respond to each individual need as best we can. For help at anytime contact us below or get in touch with someone from the team directly.

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@mpssociety.org.uk
- 0345 389 9901
- facebook.com/mpssociety

We learnt from the families that we met that your support needs in Scotland can vary, from getting the right care and respite care to getting the right benefits, access to medical services and treatments, housing and adaptations, befriending and even smaller support needs such as how to safely transport those that can escape their car seats. It was good to understand that some of the systems in Scotland are different and therefore some of the services that can be received are slightly different from those in the rest of the UK.

In the past in Scotland we have not always been able to put on events regularly or have had to cancel events when there has not been a great take up. We asked members what events they would like to see. It was interesting to start to understand what support and events our Scottish members would like to see, and some of the suggestions we had where to hold more events aimed at older children and young people. For those members in Scotland we did not see if there is any support you feel we could provide better we would like to hear from you.

As well as any comments on how you think our support and events could improve, it would be great to hear if we are doing well. One of the families we visited stated "We feel the MPS Society have given us a lot, which is why I am running the London marathon next year."

If anyone in Scotland ever needs our support no matter how big or small, please do get in contact with the advocacy team on 0345 389 9901.

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Debbie Cavell
MPS IVA Morquio, MPS I Hurler HSCT, Hurler Scheie, Scheie, MPS VI Maroteaux Lamy, MSD, ML II (shared with Sally)
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07881 343925

All Ireland Advocacy update

What a few month's it's been. We've had storm Ophelia and some pretty impressive snow over here in Ireland; but the All Ireland Advocacy Service has continued! Alison Wilson updates us on all things in Ireland.

The winter months can often be a tough time for families living with medical conditions. Despite all the festive fun the cold weather can often bring with it difficulties in getting out and about, flare ups of pain and it can often worsen feelings of low mood – it's not all tinsel and fairy lights! We can all feel a bit down when the weather draws in but we know that for individuals dealing with MPS and Fabry this can mean so much more. Please don't suffer in silence. It's ok not to feel festive! As a member of the Advocacy Support Team I always see an increase in calls over the winter period and I would urge you all to pick up the phone if you need a pick-me-up! We are always hear to listen and will do our best to support you.

As always I can be contacted by phone or email, Alison

> "I always see an increase in calls over the winter period and I would urge you all to pick up the phone if you need a pick-me-up!

Welcome back Dr Stewart!

We are absolutely delighted to welcome Dr Fiona Stewart back after her extended period of leave. Fiona, it's wonderful to have you back and looking so fabulous! You have been missed!

Dr Stewart and I have been busy prepping adult clinics and we are looking forward to seeing you all in the New Year. As you would expect, Dr Stewart has been keeping up-to-date with everything that's gone on in her absence and I know that she's looking forward to catching up with each of you.

Save the date for the

NORTHERN IRELAND INFORMATION DAY

19 MAY 2018
HILTON HOTEL
TEMPLEPATRICK

Find out more on our website at www.mpssociety.org.uk/events-clinics

What's been going on in Ireland?

It seems like a long time has passed since our last Northern Ireland MPS Clinic. On the 20 October the Northern Ireland MPS Team assembled at the Royal Belfast Hospital for Sick Children for one of our three yearly MPS Clinics. This time we met mostly with MPS I families and it was lovely to see all the boys and girls doing so well. It was great to welcome Dr Alex Broomfield to our clinic for the first time and we hope to see him again in the future. Our next clinic is on the 26 January and at that clinic we are looking forward to seeing our MPS II and III families.

We've also been busy holding Fabry clinics at the Belfast City Hospital. This is a busy clinic but please do take the opportunity to speak with me and bring along any paperwork you would like me to have a look at. I'm more than happy to walk and talk while you make your way to the ECHO department.

On the 23 and 24 November I had the privilege of being invited to attend the Republic of Ireland Paediatric MPS I clinic in Our Lady's Children's Hospital in Crumlin. This is always a whirlwind clinic! With 30 families, some not previously known to the MPS Society, it's fair to say that my vocal chords had a good work out!

This year many families spoke with me about housing issues and it's clear that the Housing Crisis is Ireland is having a huge impact on families who already have a lot on their plates. Three families attending clinic are facing homelessness and are now receiving support to communicate their needs to local housing offices.

As well as dealing with difficulties in clinic it is also an absolute joy to share in successes. There were a number of children moving on to the adult clinic in the Mater Hospital and it was fantastic to see how they have grown into young adults with lots of ambition and the world at their feet. The Mater Hospital Adult Clinic staff will not know what's hit them next year – there are some real characters coming your way!

Update on access to Vimizim in the Republic of Ireland

As many of you will be aware, the Irish MPS Society have been campaigning on behalf of their members for access to Vimizim for all those affected by MPS IVa, Morquio Disease. On the 14 November 2017 the HSE wrote to the pharmaceutical Company (Biomarin) to advise them that 'due to lack of clinical data', the HSE had decided NOT to reimburse Vimizim for use in the Republic of Ireland. This was, as you might expect, a devastating blow for families affected by Morquio Disease across Ireland. That day two little girls, who were part of the clinical trial for Vimizim, were informed that after the 5 December they would no longer have access to this life changing treatment.

It is incomprehensible that the HSE would make this decision based on data that has been used to approve the treatment for use in 17 other European countries. The HSE drug approval system is flawed and is not designed to appropriately assess treatments for rare diseases. This is something that needs to be challenged urgently.

The Irish MPS Society are in the midst of a campaign for access and in recent weeks have spoken to TD's in Dail Eireann; met with the Health Minister (Simon Harris) and have sought legal advice. Families in Ireland have appeared on television, written newspaper articles and written countless letters to political representatives and individuals of influence. The UK MPS Society were delighted to be invited by the Irish MPS Society to speak to TD's in Dail Eireann about the experiences of our Northern Irish members who have been receiving Vimizim since 2016.

The UK MPS Society continue to be supportive of the Irish MPS Society's campaign for Vimizim. We know all too well what it's like to be in the midst of a political battle for fair treatment and access to medication. However, given our UK base we would recommend that any of our Southern Irish families contact The Irish MPS Society with any queries relating to the Vimizim access campaign. This is a battle that needs to be fought on the ground by local people.

We will continue to provide individual advocacy support to families in Ireland and are keeping up-to-date with progress in relation to Vimizim access.

> "The HSE drug approval system is flawed and is not designed to appropriately assess treatments for rare diseases

Remembrance

Jack Edmond
20 April 2015–15 June 2017

Jack was born on 20 April 2015 at Aberdeen Maternity Hospital. He was diagnosed with Mucolipidosis type II at four months old. Jack was a happy boy who loved playing with his toys, going out in his buggy and playing peek-a-boo! Jack became unwell and died on 15 June 2017, aged two years old. Below is a poem which was read at his funeral.

Our Boy Jack

A tiny new baby, we cuddled you and loved you
From that moment on our love for you just grew and grew

We got heart-breaking news that you would have a short life,
but you helped us stay strong
With your smiles and giggles, you were always so much fun

We were so lucky to be given a beautiful gift:
a wonderful boy, and precious time to make memories.
Amazing memories we will always treasure

You could move in your own special way,
you knew where you wanted to go and what you wanted to do
You loved to play: shaking a rattle, spinning a toy, kicking a ball.
We could play all day long

You babbled stories and songs,
that will always be our favourite noise

Our lovely cosy cuddles, reading books, singing songs
You fell asleep in our arms
We can still hear you breathe
We are still holding your hand
We wonder what you dream as a smile comes across your face

You were held, you were kissed, you were hugged,
you knew you were loved so much
And when you looked at us,
your eyes lit up and we knew you loved us too

We wouldn't have changed you for the world
Our perfect boy, in our hearts forever
Jack, we love you

Dr Alan Cooper, Retired Principal Bio-Chemist at the Willink Biochemical Genetics Unit, Manchester died unexpectedly at home on Monday 20 November 2017.

Alan joined the Willink unit in 1974. He worked alongside the late Prof Ed Wraith and was instrumental in setting up the Lysosomal Storage Disorders diagnostic service in Manchester. He directed and managed the LSD service until his retirement in 2010, which to date has diagnosed approximately 4330 patients with a storage disorder and performed 1566 prenatal diagnoses for families affected by LSDs.

Alan was well known for his commitment to the field of Lysosomal Storage Diseases both within the UK and internationally, travelling to both Egypt and Turkey to help set up local diagnostic services in those countries. Alan was a frequent contributor at the MPS Society conferences over the years and took a great interest in the MPS community over several decades.

He very much enjoyed attending patient-orientated meetings and loved seeing the children.

On behalf of the Trustees, staff and members of the MPS Society I send our deepest condolences to Alan's family.

Christine Lavery

We want to say thank you to Alan's friends and family who made contributions at his funeral to two charities, the GEM appeal and the MPS Society. They raised £150 for each charity and Alan's son, James, wrote: "I would be ever so grateful if you would accept this donation and continue to provide hope for the patients diagnosed with these cruel diseases."

Michael Burke
22 December 1951–17 October 2017

My father was diagnosed in, what we have recorded, 1971. He was on the first Replagal trial in London. We've read many early reports from the medical profession, some of which have helped change the way Fabry was viewed, and unfortunately some reports that were very wrong and mistaken assessments.

Fabry disease shaped my dad's life, and so hopefully his life will have helped shape other sufferers lives for the better. As the last sufferer in my family, I will continue to support the MPS Society.

Ellen Nicholson

Bereavements

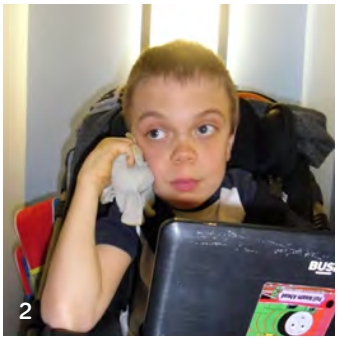
We also wish to extend our deepest sympathies to the family and friends of:

Raja Nadeem Khan who had MPS III Sanfilippo and passed away on 4 October 2017 at the age of 16.

Weronika Martyniw who had GM I Gangliosidosis and passed away on 9 October 2017 at the age of 2.

Clinics


1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3

GREAT ORMOND STREET HOSPITAL
MPS I CLINIC
12 SEPTEMBER 2017
(1-3)

I had the privilege of representing MPS Society at the GOSH clinic again. As usual it was extremely busy with lots of children attending clinics for various reasons, and as I am still not familiar with all of our families I spent time trying to figure out who was who and then when I was reasonably confident that I identified our children and their families I went and introduced myself. Three young men were happy to oblige for pictures – so a big thank you to them! I love to come back to the office with pictures for all the staff to go "awww" at!! *Sally*

4

BIRMINGHAM CHILDREN'S HOSPITAL
MPS IV CLINIC
15 SEPTEMBER 2017
(4-7)

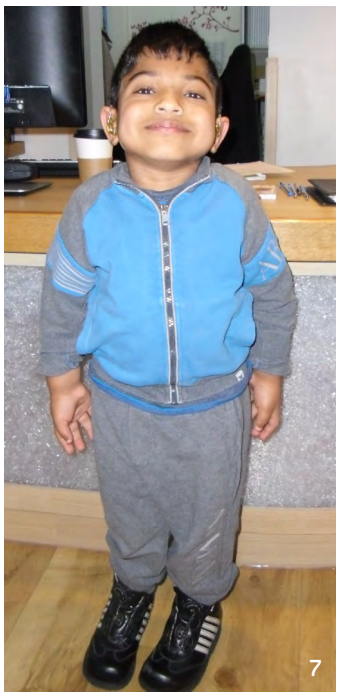

5


6

The clinic was really busy but you were happy to pose for pics for the magazine, albeit a little shy in one case but thanks to some encouragement from Dad and a bit of time we got one and it was worth it – look how cute you are!

It was lovely to catch up with all the children and their families and just have a chat and see how everyone was and of course, remind them that we are always there to support in any way we can.

Sally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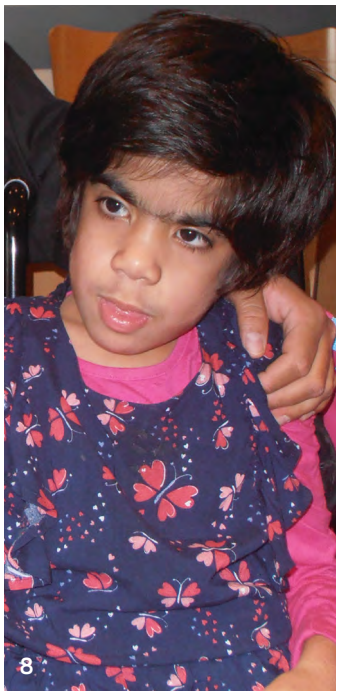
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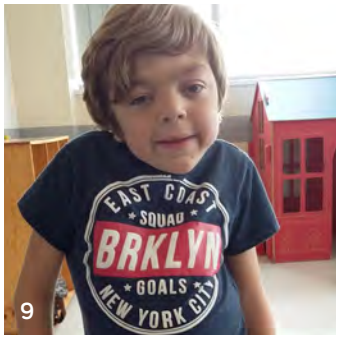
GREAT ORMOND STREET HOSPITAL
MPS III CLINIC
26 SEPTEMBER 2017
(8)

I attended the MPS III clinic on 26th September at Great Ormond Street Hospital to see and offer support to all the families that were there. The clinic waiting room was very busy but I managed to talk to everyone, although I didn't get much of an opportunity to take photos. It was still good to see everyone and to take the opportunity to catch up with the specialist nurses. Thanks to the team at GOSH for arranging the clinic.

Steve Cotterell



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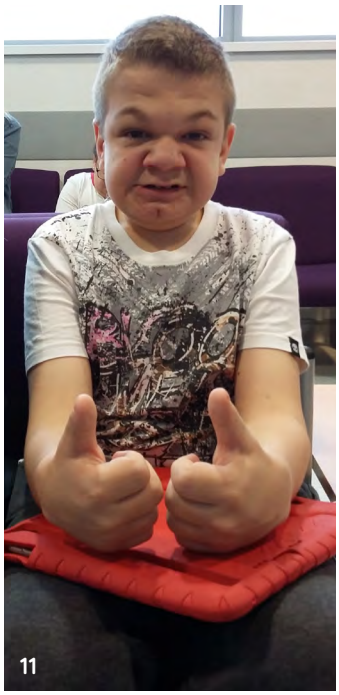
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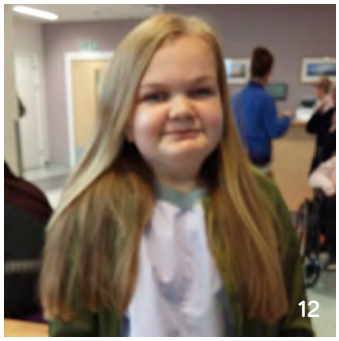
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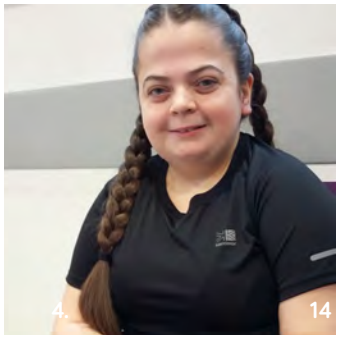
ROYAL MANCHESTER CHILDREN'S HOSPITAL
POST HSCT CLINIC
29 SEPTEMBER 2017
(9-14)


13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Sally and I went to a very busy clinic at Manchester Children's Hospital. The clinics are usually split over two clinics of younger and older children but this time it was one big clinic! *Debbie*

BIRMINGHAM CHILDREN'S HOSPITAL
TRANSITION CLINIC
29 SEPTEMBER 2017
(15-16)


15


16

It seems a long time since I last went to Birmingham children's clinic and I was not the usual face at the MPS VI clinic, Debbie, who normally goes could not split herself in half and had already committed to go to the Manchester Clinic.

I arrived a bit windswept and soggy, it always seems to rain when I go to clinic...

As usual the team make you feel welcome and Catherine made me a lovely cup of tea and all was right with the world.

It was lovely to meet the new families and catch up with the old ones (parents, that's the length of time you have been coming not your biological age!)

Clinics are not quiet affairs with many of the children bouncing around like Tigger!

Some had just started school and were loving it.

Well it was soon time to make tracks back to Bucks, so until the next time....

Rebecca

GREAT ORMOND STREET HOSPITAL
MPS IV CLINIC
10 OCTOBER 2017
(17)

It was lovely to snap a picture and meet this young man and his Mum at the GOSH clinic as they are avid fundraisers for the Society and their efforts have helped to make sure the Society can keep supporting and providing events for members. I met two children that were not members and was able to talk to their families about the Society and give them a magazine to read to help them decide if they would like to become part of our MPS family in the future.

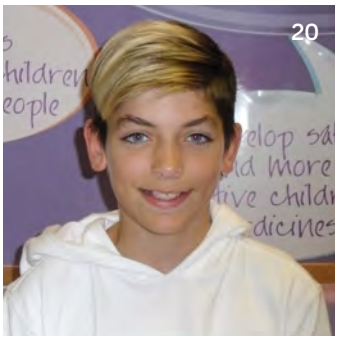
Sally


17


18

BIRMINGHAM CHILDREN'S HOSPITAL
FABRY CLINIC
20 OCTOBER 2017
(18–21)


19


20

On the 20 October I attended an afternoon Fabry clinic in place of Rebecca who was on her holidays. The clinic was short and sweet and as always it was a pleasure to meet everyone and hear their stories. There were some families that travelled a very long way to attend their clinical appointment, and I hope everyone made their way home safely.

Louise

21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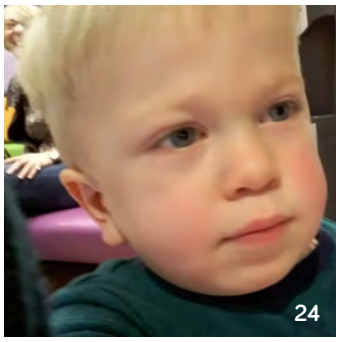
BIRMINGHAM CHILDREN'S HOSPITAL
MPS II AND VI CLINIC
17 NOVEMBER 2017

(22-24)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Starting the day on a cold frosty morning was not what I expected so it was a mad dash to get the train with four minutes to spare, the ice was so thick that the scrapper didn't work and the heater was sadly lacking in power!

The MPS II clinic is always lively as the children are always on the go. The children don't get a chance to sit down and play before the next appointment and sometimes it's a struggle to get them into their next appointment. One lad didn't want to have a photo taken so he did a selfie instead with the help of dad.

You are all super stars for agreeing to have your photos taken and always make our magazine so lovely. We hope you enjoy seeing your pics in print.

Thanks to the team for looking after me and I will see you all again soon

Rebecca


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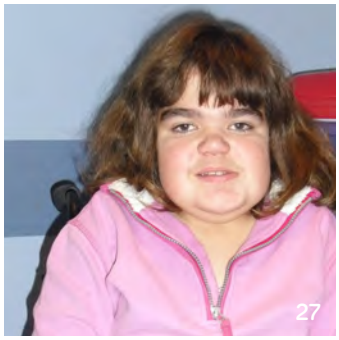
26

GREAT ORMOND STREET HOSPITAL
MPS I CLINIC
28 NOVEMBER 2017

(25-29)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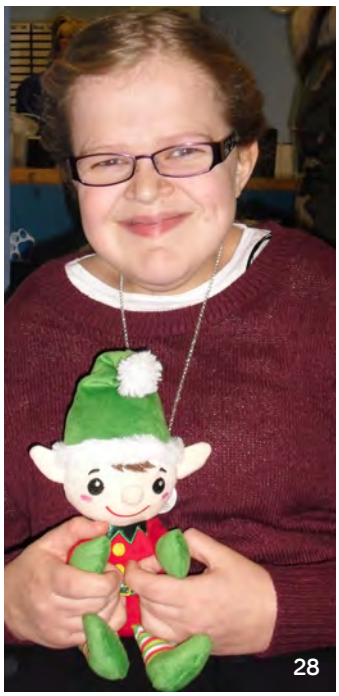
I took the MPS elf to the GOSH clinic and everybody was happy to see him. Everyone wanted to know why we had an elf and I explained it's for the MPS Society's Christmas campaign. One boy even bought himself a GOSH elf to take home. It was lovely to see everybody at clinic.

Debbie and the MPS Society elf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Your stories

Our journey to Rome to meet families affected by Mucolipidosis and other rare diseases

We've just returned from Rome where we attended the 5th Glycoproteinoses International Conference held by the ISMRD.

Conditions covered included Mucolipidosis, Fucosidosis, Sialadosis, Aspartylglucosaminuria, Alpha & Beta-Mannosidosis, Galactosialidosis and Schindler disease.

I was asked to do a presentation on our life as a family living with Mucolipidosis III. Sam is 15 now, and his health has deteriorated quickly over the past year. I tried to keep it positive, but sometimes it all just gets to you and brings you down. Sam still keeps smiling, singing and drumming. If he's happy, we're happy.

We thought it was important for us all to attend as a family this time. Shamim could speak to other fathers and share thoughts, fears and dreams for the future of our kids. It's not just the mums who care and suffer.

It was important for younger brother Edward to get to meet other siblings who were also silent carers for their affected siblings. To be able to share possible feelings of confusion or resentment at being either a carer, or how it could have been if they too were affected.

Families travelled in from Australia, New Zealand, USA, Norway, Lithuania, Spain, Slovenia, Jordan and UK to find out what if any progress was being made on links to finding cures or ways to improve the lives of those affected.

The scientific talks were way over our heads at times, but it showed how much research was being done in the background. Progress has been moving along at a quicker pace than previously. But never quick enough for the families I suppose.

The great news for us is that research has started on the gene therapy for ML II which in turn will help those with ML III.

> "You can never underestimate the power of unity when all you want to do is to stop your child from suffering.

The family breakout sessions were much more comfortable and intimate with geneticist, doctors and researchers answering our many questions on how, why and, importantly, when?

We spoke with many other families about their experiences. When did they get the diagnosis? What were the symptoms? What surgeries? What helps?

Shamim and myself discussed about how we were dipping in and out of people's lives, asking personal questions, supporting other families by just talking. People who in other ways had no common day to day similarities to ourselves, apart from having a child with a rare disease and feeling isolated.

Subconsciously, you were looking at the other children and possibly comparing and thinking "they're worse than ours", "we recognize those symptoms", and daring to dream when you see another and she/he doesn't look that badly affected and they're older.

The kids had a brilliant time getting to know each other at Gladiator school, clown class and the museum. Naturally, some kids were on phones a lot, but looking closer, they were translating their languages to talk to each other.

What shines out through the tears constantly welling, choked throats and biting of lips of parents trying to keep it together as they spoke about their children was the hope we all hold on to. The hope that a cure might be found soon.

We had smiling faces, heard sounds of laughter and tears, and shared hugs. Emotions that are the same across all continents, races, religion and gender.

Rare diseases don't discriminate. They can affect anyone. But together, we will help and support each other. You can never underestimate the power of unity when all you want to do is to stop your child from suffering.

Shirley Jamil

I Love Me Project

Aisha Seedat was invited as a guest speaker at Leicester's I Love Me Project: disable stereotypes of disabled people.

I have never spoken in a front of a sea of people of what my condition is and what the term "disability" means to me. Well that is one thing, to tick off my bucket list. I would not have had the opportunity without the special invite from Efaz Ahmed (Inspirational Speaker and Young Ambassador at the Prince's Trust).

The I Love me founder; Lydia Unsudimi launched this project in January 2015 after experiencing low self-esteem which resulted in bullying in 2012. Lydia gained a passion for helping young people overcome their feelings and embark on the journey of self-love.

The night consisted of many talents like, music, dance, fashion and top inspirationalist speakers. It taught me a lot. I may be in a wheelchair but I have many talents in me that I have yet to discover.

Lydia Unsudimi who founded the I Love Me Project

You can see the video of my talk on my youtube channel, Aish Inspires.

Don't forget to like, comment and subscribe. More inspirational talks and vlogs coming soon to your screen.

My speech

Welcome all, I am Aisha, currently studying health studies in year 2 at DMU. DMU is an excellent university that supports me through my challenges. Now please bear with me as I talk about the challenges I face with my condition. My voice does trail off, so forgive me in advance.

I suffer from a rare genetic disorder called Morquio Syndrome type IVA. It has a long medical name, Mucopolysaccharidosis, for short it is MPS. This means I lack an enzyme in my body which causes progressive damage. It is an ultra-rare condition. There is no cure for this disease but there is enzyme replacement therapy available, which I was on but had to come off it due to anaphylactic reaction. I have many complications related to my condition. One of the most challenging for me was when I lost the ability to walk at the age of 10. But with the support of my parents, sister and those close to me I have been able to remain positive despite this. The one good thing about my condition is that I get away with child tickets at times!!!

I have a passion for learning and fundraising for charities close to my heart. I am an ambassador for the MPS Society. I have actively fundraised for them since my childhood and have so far raised over £10,000. I fundraise for hospitals abroad and Drop of Compassion. I have recently started volunteering for Get Healthy Leicester.

My aim in life is to succeed in everything I set my goal on. Disability has many abilities in my dictionary.

New data protection regulation (GDPR)

This comes into effect on 25 May 2018 and has major time and cost implications for the MPS Society, so please help us to achieve compliance as effectively as possible

We are busy reviewing the data we hold for members and their families, how and where it is held and the authority we are required to have from you with regard to us holding data.

Once this is clearly assessed we will need to contact every member to advise them further and obtain the appropriate permissions to continue to hold this data. This will replace the membership forms and other authorities we already hold from you. It would be really helpful if the MPS Society could communicate electronically with as many families we support as possible to keep costs down.

If you have an email address, please help us by letting the MPS Society have it – simply drop an email to gdpr@mpssociety.org.uk with your name and postcode in the subject line and we will update our records from that.

We never share your information with anyone else without specific authority from you and our records are securely held, so you will not suddenly start receiving spam emails from MPS or anyone else. We will use your email to communicate with you regarding GDPR and your options regarding the information we hold about you and your family, which we will only use to post or email the MPS Magazine, information on meetings and support events, potential befrienders (we always ask you first), information on clinical trials and research and fundraising news. The MPS Registry data is only used for statistical purposes and data shared is always anonymised.

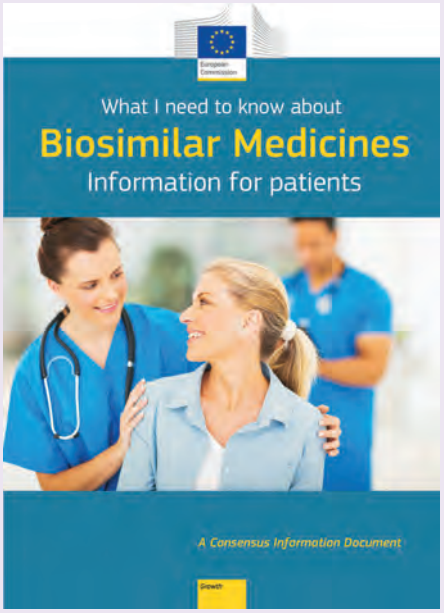
If you have any concerns or questions, please do contact Bob at b.stevens@mpssociety.org.uk

Information about biosimilar medicines for patients

The European Commission has announced that the Question and Answer document on biosimilar medicines for patients is now available in 23 languages.

The document aims to provide answers to some questions patients may have on biosimilar medicines and explains the role of the European Medicines Agency (EMA) in their approval and safety monitoring. It also includes references for further information at the end of the document for patients who wish to know more about biosimilar medicines. A similar information guide for healthcare professionals prepared by the Commission and EMA is expected to be available in six additional languages in the first half of 2018.

You can download the document here:
http://ec.europa.eu/docsroom/documents/26632



Information & resources

Ask the experts

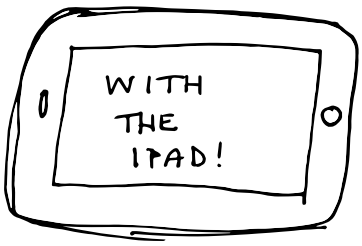
We have been asking a series of questions over social media to get an insight from you, the experts, as to the best approach to the unique situations you find yourselves in. We will include the answers in each magazine. If you have a question you want to ask the experts let us know at magazine@mpssociety.org.uk.

Haircuts - how do you do it?

I'm lucky, Danny has curly hair. It means I can chop it off a bit at a time while he's watching TV and no one will ever notice!

Mostly I've been soaked through and my clothes have been removed to dry. One time my top was put of the heater and by end of my appointment it was still wet as the heater wasn't even switched on!! Sometimes I don't go back to a salon for these reasons. I now take my car booster seat in when I get my hair washed.

I did both mine myself, my daughter didn't mind and just watched her DVD's my son had to be on someone's knee then he let me do it!



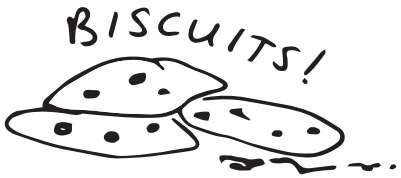
As a mum of a child with MPS and a hairdresser I take the soft approach. Normally I leave the child to decide where they want to sit or stand, it's been me on the floor before. If it takes five trips for one haircut then so be it - better than a distressed child. We all know our kids are very wary.

I have a mobile hairdresser who comes to the house Debbie, she is brilliant, patient and caring, always does a great job with my boys who both have/had Sanfilippo (Jake passed away in 2013). If you know anyone who does mobile hairdressing that's the best option!

Jon has four lovely ladies helping him. I sit on the floor and feed him crisps, lovely Lucy cuts his hair while Anna and Freya talk to him and comfort him. Most times he's fine but we have all ended up on the floor. The staff are so amazing, we're so lucky.

My friend Janine is a hairdresser, she cuts Will's hair at our house. He has always disliked it but at the end he will still give her a kiss!!

I bought a very quiet cutter for home and am taking care of his haircut. It's much better than going to the hairdresser!



The Mental Capacity Act 2005

"Best Interests" (England and Wales)

The Mental Capacity Act 2005 Section 1 (5) introduces the principle of "Best Interests" as a safeguarding principle to protect those without capacity. The Mental Capacity Code of Practice states that any act that is done or any decision made for a person that lacks capacity must be considered to be in the person's "best interests", this is regardless of who is making the decision and irrespective of the nature of the decision required.

Decision Makers

Many different people can be decision makers under the Mental Capacity Act and this will vary according to the various decisions that are to be made. Decisions will be required for tasks of daily living; in this instance those caring for the individual are likely to be the decision makers, where more complex decisions are required it is necessary to seek a decision maker with appropriate experience and qualifications.

Making Decisions

As long as the person/ people making the decision can justify their decision they will be protected from liability. The Act introduces a checklist that decision makers must follow when considering what is perceived to be in a person's best interests, this requires for decision makers to:

- Consider all the relevant circumstances
- Consider a delay until the person regains capacity
- Involve the person
- Consider the individual's own past and present wishes and feelings.
- Not be motivated to bring about death
- Consider any advance statements made (at the time when they had capacity to do so)
- Consider the beliefs and values of the individual
- Take into account views of family and informal carers
- Take into account views of Independent Mental Capacity Advocate or other key people
- Show it is the least restrictive option

It is important for those making decisions to show their workings and to document any decisions made.

Decisions can be short or longer term and can be reviewed according to need or changes in circumstance.

It is important to understand that if an advance decision has been made by an individual at a time when they had capacity to make decisions their wishes must be adhered to. It is also necessary to consider the individual's past and current wishes, as well as likely beliefs and values and any other factors that may influence a decision, examples can include religion, culture and political values.

Consultation with all those involved in the care or welfare of an individual should be consulted about decisions required such people include family members, friends, social care workers and health professionals.

Managing Conflict

As with any decision where people are required to make an opinion there is a risk of contention. It is usually possible to settle these disputes informally through discussion and a weighing up of possible options. However when there is no resolution found and interested parties wish to contest a decision there are several options available:

- Involve an advocate to act on behalf of the person who lacks capacity to make the decision

- Get a second opinion
- Hold a formal or informal 'best interests' case conference.
- Attempt some form of mediation
- Pursue a complaint through the organisation's formal procedures.
- If all else fails the decision can be made through the Court of Protection

What happens in an emergency?

Sometimes those who lack capacity will require urgent medical care. Unless advance decisions have been made it is likely to be considered reasonable for medical professionals to give urgent treatment without delay.

For more information visit: www.gov.uk/government/publications/mental-capacity-act-code-of-practice

Each of the provisions will be explained in further detail in future editions. Should you have any questions or if you need to talk about any aspect of this article please contact the advocacy service and we will be happy to help.

Contact, a charity for families with disabled children, have published the results of an inquiry carried out in February 2017 which investigated school transport for disabled children.

Contact found that most calls to their helpline were concerned with school transport. They write:

"School transport is an integral part of a child's education. If a child can't get to school or has a stressful experience getting to school, they are not able to learn and take part in the school day like other children."

The inquiry, which brought together evidence from 2500 parents, found that:

- 48% and mostly mums said that school travel arrangements for their disabled child meant that they can't work or have had to decrease working hours.
- 23% said their child's journey to school is stressful which makes it harder for their child to learn.
- 51% of local school transport policies in England include unlawful statements.

As a result of the inquiry the Secretary of State for Education has announced plans to review school transport statutory guidance to make sure all local authorities are providing school travel for eligible disabled children.

You can read the full report of the inquiry here: http://bit.ly/schooltransportinquiry

Advice and information about school transport is available from the Contact website here: http://bit.ly/schooltransportadvice

Research & treatment

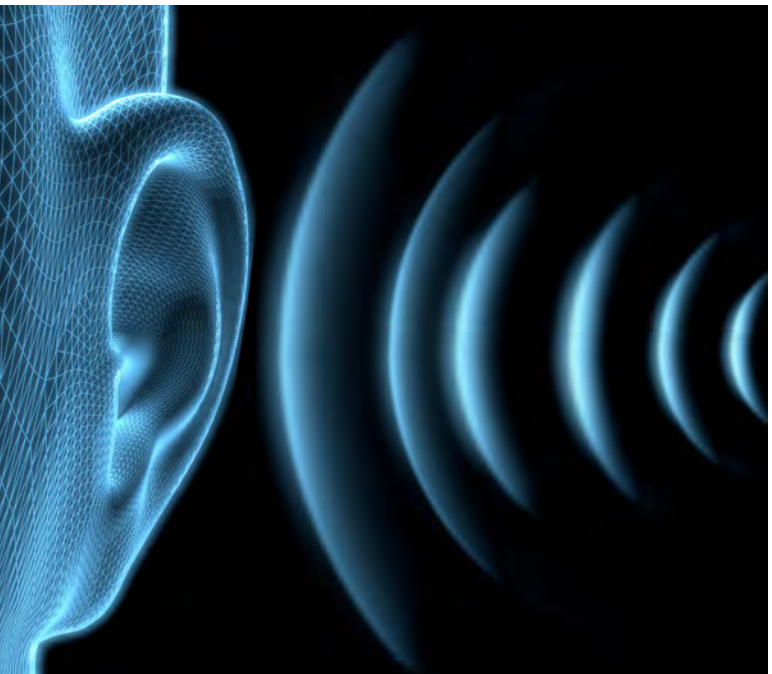
Hearing Loss in Children with Fabry Disease

A paper has been published online in the *Journal for Inherited Metabolic Diseases* describing the hearing loss in children with Fabry disease. The authors were prompted to study hearing loss in children with Fabry disease due to the only limited data available on the presence and degree. The methodology was to collect retrospectively audiograms of Dutch and Norwegian children with Fabry disease. First hearing sensitivity was determined by studying hearing thresholds at low, high and ultra-high frequencies in children with Fabry disease and comparing them to healthy children.

The results of 113 audiograms of 47 children with Fabry disease (20 boys with a median age at first audiogram of 12 years) were analysed. At baseline slight/mild or moderate to severe hearing loss was present in three children (6.4%, two boys). Follow-up measurements showed that three additional children developed hearing loss before the age of 18 years. Of these six children, five had sensorineural hearing loss, in the authors' opinions most likely caused by Fabry disease.

The conclusion is that a minority of children with Fabry disease show slight/mild or moderate to severe hearing loss, but their hearing thresholds are poorer than the reference values for normal hearing children.

Suntjens E, Dreschler WA, Hess-Erga J, Skrunes R, Wijburg FA, Linthorst GE, Tøndel C, Biegstraaten M. (2017) 'Hearing loss in children with Fabry disease'. *Journal for Inherited Metabolic Diseases*. 40(5):725-731.



Social functioning and behaviour in the Mucopolysaccharidosis IH (Hurler disease)

The aim of this study was to investigate the behavioural phenotype of MPSIH treated with haematopoietic stem cell transplantation, focusing on social functioning and sleep. Parental stress was also measured.

Twenty-two children with MPSIH with a mean age of 9 years 1 month, of whom ten were male (45%) participated in the study. Parents completed three questionnaires based on their child's social responsiveness, behaviour and sleep habits and one questionnaire concerning parental stress.

The results showed that 23% of children with MPSIH scored in the severe range of the social responsiveness measure suggesting significant difficulties in social functioning. Also 36% scored in the mild to moderate range suggesting milder but still marked difficulties in social interaction. The authors identified that although children with MPSIH did not show significantly higher rates of internalising, externalising or total behaviour problems than the normative sample, they received scores that were significantly higher on social, thought and attention problems and rule-breaking behaviour, and all the competence areas of the Child Behaviour checklist.

Parents of children with MPSIH did not score significantly higher on parental stress than parents in a normative sample. The authors conclusions were that parents of children with MPSIH rate their children as having problems with social functioning and various areas of competence more frequently than previously thought, with implications for clinical support.

Lehtonen A., Rust S., Jones S., Brown R., Hare D. (2017) Social Functioning and Behaviour in Mucopolysaccharidosis IH [Hurlers Syndrome]. In: *JIMD Reports*. Springer, Berlin, Heidelberg

FDA approves treatment for MPS VII

The US Food and Drug Administration has approved Mepsevii (vestronidase alfa-vjbk) to treat paediatric and adult patients with MPS VII, also known as Sly syndrome.

"This approval underscores the agency's commitment to making treatments available to patients with rare diseases," said Julie Beitz, M.D., director of the Office of Drug Evaluation III in the FDA's Center for Drug Evaluation and Research (CDER). "Prior to today's approval, patients with this rare, inherited condition had no approved treatment options."

MPS VII is a lysosomal storage disorder caused by deficiency of an enzyme called beta-glucuronidase, which causes an abnormal buildup of toxic materials in the body's cells. Mepsevii is an enzyme replacement therapy that works by replacing the missing enzyme.

Efficacy of Mepsevii was primarily assessed via the six-minute walk test in ten patients who could perform the test. After 24 weeks of treatment, the mean difference in distance walked relative to placebo was 18 meters. Additional follow-up for up to 120 weeks suggested continued improvement in three patients and stabilization in the others. Two patients in the Mepsevii development program experienced marked improvement in pulmonary function. Overall, the results observed would not have been anticipated in the absence of treatment.

MPS Consensus Meeting

Last December a Consensus Conference for Cognitive Endpoints in the MPS diseases co-organised by the National MPS Society and the MPS Society of the UK was held. As a result of the conference, the following papers are now published:

Cognitive and adaptive measurement endpoints for clinical trials in mucopolysaccharidoses types I, II, and III: A review of the literature
www.bit.ly/cognitiveendpointslitreview

Cognitive endpoints for therapy development for neuronopathic mucopolysaccharidoses: Results of a consensus procedure
www.bit.ly/cognitiveendpointstherapy

The overarching goal of the conference was to provide a forum for the consensus process. Expert clinicians from around the world participated providing a unique opportunity to present and hear subject-matter specific presentations and take part in workshops and discussion.

Two additional papers are being written from the workshops on the association of biomarkers/MRI/neurologic measures with cognition and on quality of life measures.

Gene editing in a human body has been attempted

BBC News reported on the first case of gene-editing occurring on cells inside a patient in November 2017. The experimental treatment was carried out on Brain Madeux, a 44 year old man from Arizona who has MPS II Hunter. Brian received an infusion that contains two molecular scissors (called zinc finger nucleases) which cut the DNA at a precise spot allowing a gap for new DNA to be inserted. The therapy is designed to become active once it gets inside the liver cells.

The trial will for now be measuring the safety of carrying out gene editing with more research required to see how effective the therapy might be. Sangamo Therapeutics who designed the therapy said:

"For the first time, a patient has received a therapy intended to precisely edit the DNA of cells directly inside the body."

Read the full story here: www.bbc.co.uk/news/health-42009929

Fabry members your help is needed

National Study on Involvement of the Heart in Fabry Disease

The team at University Hospital Birmingham have been conducting a national study supported by the MPS Society, which is focussing on involvement of the heart in Fabry disease. For this study we are collecting as much information as possible from all patients who have a cardiac device implanted – this would include a pacemaker, defibrillator or a Reveal device.

This study started in August and since then we have had great support from people coming forward providing extremely useful information. So far we have identified a total of 67 patients nationally who have cardiac devices implanted. Early data from University Hospital Birmingham has found that 56% of patients had abnormal heart rhythms picked up on routine device checks that needed treatment from either their device or a change in medication. Additionally, we found that all of these patients have a higher number of potential risk factors for developing these abnormal rhythms compared to those who didn't have any abnormalities found on device checks. These data and ongoing analysis will go a long way in furthering our understanding of risk factors that may predict involvement of the heart in Fabry disease and we are extremely grateful for your continued support.

For this study we are collecting as much information as possible from all patients who have a cardiac device implanted

If you have any of the cardiac devices listed above and you would be happy to help in this study, we would be extremely grateful if you could get in touch with Dr Ravi Vijapurapu the Clinical Research Fellow conducting this study on the following secure email address: fabry@uhb.nhs.uk.

The type of information requested from you is:

- What type of cardiac device do you have (pacemaker, defibrillator or Reveal).
- Which hospital do your pacemaker checks take place?
- If you have a defibrillator, have you ever had a shock from the device?
- Who is your consultant Cardiologist and where do they see you?

Thank you for your help in this very important study. If you have any questions or do not use email and would like the MPS Society to pass this information on please do contact us by post or telephone 0345 389 9901 and speak to Rebecca.

Orchard Therapeutics Announces Extension of its Collaboration with Manchester University to Include Sanfilippo Syndrome Type B

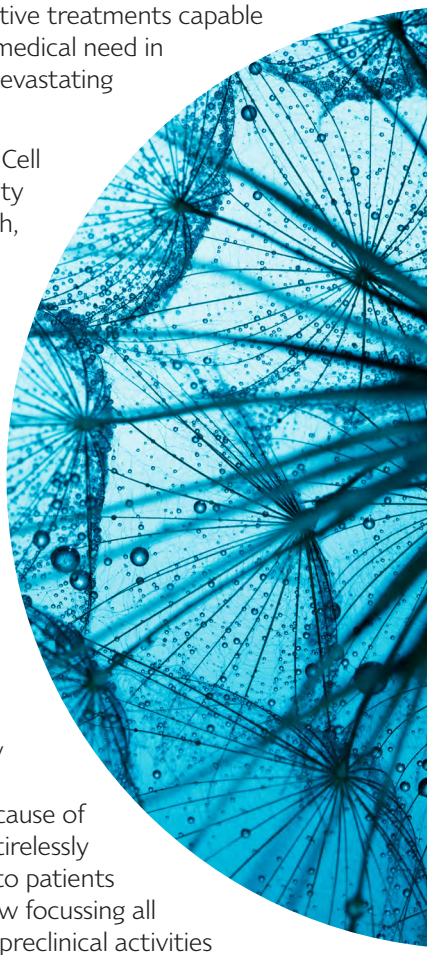
Orchard Therapeutics Limited, a clinical-stage biotechnology company dedicated to transforming the lives of patients with rare disorders through innovative gene therapies, has announced that it has acquired an exclusive license to develop lentivirus-based autologous ex-vivo gene therapy for Sanfilippo syndrome type B (or MPS-IIIB) f rom The University of Manchester, UK.

The technology, developed in Professor Brian Bigger's laboratory, and recently published in the journal *Brain*, involves the use of a high-titre lentiviral vector to drive the expression of a codon-optimized α-N-acetylglucosaminidase (NAGLU) gene under the control of the myeloid-specific CD11b promoter (LV.CD11b.NAGLU).

MPS-IIIB is a rare neurodegenerative inherited lysosomal storage disease caused by mutations in the NAGLU gene. The disease, which affects children as early as 2 years of age, results in severe and rapidly progressive brain disease and neurological symptoms. There is currently no effective treatment option for MPS-IIIB.

This programme in MPS-IIIB complements the existing collaboration program between Orchard, The University of Manchester and Manchester University NHS Foundation Trust in MPS-IIIA. Autologous ex-vivo lentiviral haematopoietic stem cell gene therapy is anticipated to correct neurological manifestations through the engraftment of subpopulations of haematopoietic stem cells in the central nervous system, thereby providing supranormal and widespread enzyme expression throughout the brain. In both MPS-IIIA and MPS-IIIB, preclinical studies have produced encouraging results showing a normalization of heparan sulphate levels in the brain and peripheral organs, as well as neurological disease correction.

Dr Jesus Garcia-Segovia, Orchard's VP Clinical Development, CNS and Metabolic Disorders stated: "The incorporation of MPS-IIIB into our development pipeline is a significant milestone in the consolidation of our neurometabolic franchise, which is currently focused on the development of autologous ex-vivo haematopoietic stem-cell gene therapy for children suffering from MPS-IIIA. We are very excited at the possibility of bringing effective treatments capable of addressing the high unmet medical need in children suffering from these devastating conditions".



Prof Brian Bigger, Professor of Cell and Gene Therapy in the Faculty of Biology, Medicine and Health, The University of Manchester commented: "It's incredibly exciting for us to work with our trusted partner Orchard Therapeutics to translate another autologous ex-vivo gene therapy that has demonstrated efficacy in a preclinical mouse model of MPS-IIIB into clinical development and scale-up".

Dr. Andrea Spezzi, Orchard's Chief Medical Officer added: "MPS-IIIA and MPS-IIIB are devastating diseases. Orchard and its collaborators are highly motivated to develop gene therapies to address the root cause of these disorders and will work tirelessly to make treatments available to patients as soon as possible. We are now focussing all our efforts on completing the preclinical activities required to enable the start of clinical studies in MPS-IIIA towards the end of 2018 and thereafter in MPS-IIIB".

Orchard's development pipeline of autologous ex-vivo gene therapies includes novel treatments for primary immune deficiencies, and inherited metabolic disorders including other undisclosed early and late-stage programmes.

MPS I and MPS II Expert Meetings

Join us on 28-29 April 2018 for two extraordinary expert meetings and a chance to share experiences and knowledge on MPS I and MPS II

Sally, a mum to Danny who has MPS II told us:

"I LOVED being at my first conference in 2017. I found many of the talks fascinating and really appreciated the chance to relax and enjoy meeting other families without having to worry about what Danny was up to. I know the MPS II meeting will deliver on all those things again, but with the added bonus of ALL the information being specific to Hunter Syndrome. Can't wait!"

focus on clinical trials

CLINICAL TRIAL TERMINOLOGY

Postmarketing

The phrase postmarketing requirements and commitments refers to studies and clinical trials that sponsors conduct after approval to gather additional information about a product's safety, efficacy, or optimal use. In the case of orphan drugs postmarketing approval is given by the European Medicines Agency (EMA)

European Medicines Agency (EMA)

About 30 million people living in the European Union (EU) suffer from a rare disease. The European Medicines Agency (EMA) plays a central role in facilitating the development and authorisation of medicines for rare diseases, which are termed '**orphan medicines**' in the medical world. The EMA provides the legal framework for sponsors (pharmaceutical companies) from clinical trial design through to marketing approval.

Inclusion criteria and exclusion criteria

Every clinical trial has specific requirements for who can or cannot participate. To participate in a clinical trial, participants must meet certain standards, called '**inclusion and exclusion criteria**'. These are used to protect the participants' safety during the clinical trial.

Clinical endpoints

In a clinical trial a **clinical endpoint** generally refers to occurrence of a disease, symptom, sign or laboratory abnormality that constitutes one of the target outcomes of the trial. Clinical trial **endpoints** can be classified as **primary** or **secondary**.

Primary endpoints measure outcomes that will answer the **primary** (or most important) question being asked by a clinical trial, such as whether a new treatment is better at preventing disease-related death than the standard therapy, which may be palliative.

Reimbursement

Clinical trial participants are usually asked to perform a variety of tasks and procedures that likely would not be requested during routine care. For example, they may need to undergo additional lab testing, x-ray procedures, answer questionnaires, or make extra trips to the clinic. When and how to offset subject inconvenience with some kind of compensation is decided by the sponsor during the protocol building, review and ethical and regulatory approval. It is a long standing practice intended to allow patients the ability to participate in clinical research without suffering financial hardship. Typically documentation is required such as receipts to get patients or their carers back to a cost neutral position.

How we can support you with clinical trials?

Although the advocacy team are not directly involved with clinical trials, the team can help you by sign-posting, providing information and addressing queries to help individuals and families to navigate the process.

We are here for advice at any stage of the trial and can support you with many aspects of the process.

PRIOR TO THE TRIAL

Thinking about a clinical trial?

The advocacy team can

- Give basic information regarding a trial such as the site, stage of trial and what might be involved.
- Give more information about eligibility criteria, for example is there an age limit or does a trial exclude from other trials?
- Help with definitions of the clinical trial – what is a natural history study? What is a placebo?
- Supply further details following an MPS magazine article or letter of interest and sign-post to the trial site.
- Discuss concerns and alternative options if available.
- Discuss the potential impacts on the wider family, finances, employment and education.
- Link members to other families who may also be on a trial.

DURING THE TRIAL

On a clinical trial – how can we help?

The advocacy team can

- Signpost to the correct team for queries.
- Assist the family and individual to have a good understanding of the trial and the expectations. This could be information about start and stop criteria.
- Encourage the family to ask questions.
- Explain that being on a clinical trial does not guarantee positive results or ensure the treatment will be available when the trial ends.
- Direct queries such as special funding requests.
- Assist the family or individual to raise any concerns with the trial.

END OF THE TRIAL

The trial has ended – what happens now?

The advocacy team can

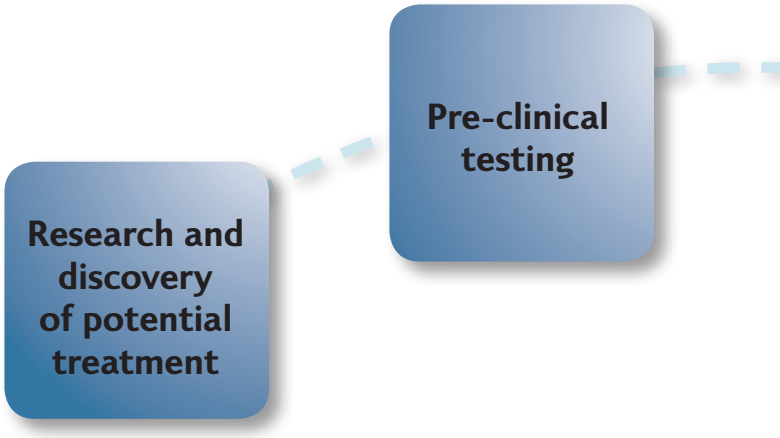
- Assist with queries regarding outcomes of the trial, the timescales expected for results to be completed and with compassionate use if this is available.
- Give information about the licensing process and how this works.
- Assist in the licensing process by representing the patient view.
- Can support families and individuals at meetings if required.
- Keep other members informed of progress of the treatment.

Sometimes a trial can come to an end unexpectedly and families and individuals may require support at this time. The advocacy team can

- Sign-post for further advice and information.
- Assist families and individuals in raising their concerns.
- Offer emotional support.
- Support families and individuals at meetings.
- Guide the family or individual through the exit process.

The role of clinical trials in the development of a new treatment

The development of a new treatment takes many years. When there is sufficient understanding of the disease and the new potential treatment clinical trials will begin.



For the purposes of this article, we will be looking at interventional clinical trials, this means trials that include medical treatments, or drugs.

The basis of a clinical trial is to obtain information about new, experimental treatments (or existing treatments that need further study) by way of clinical research. The purpose of a clinical trial is usually to answer a question, or questions, about the treatment.

Preparing for clinical trials

Before clinical trials can start, a new treatment undergoes rigorous pre-clinical studies to determine that it is suitable to start testing in people.

In ultra-rare diseases, a natural history study is often undertaken. A group of untreated individuals with the disease are followed and tested over a number of months or years to further understanding of the condition. This can help with the design of the clinical trials and provide a control group against which the results of a new treatment can be compared.

Clinical trials are designed to efficiently measure the safety and efficacy, or effectiveness, of the treatment. A study protocol sets out information such as who can and cannot take part in the trial (the inclusion/exclusion criteria), scientific rationale for conducting the study, the objectives of the study and instructions for investigators conducting the clinical trial, for example dosing ranges and tests to be carried out. This ensures that data is collected in the same way across all clinical trial centres, which is important for analysing data and determining outcomes.

The relevant health authorities and ethics committees in each country where a clinical trial is taking place must give their approval before a clinical trial can take place.

Clinical trial phases

During the clinical development of a new treatment, different phases of clinical trials are undertaken. Completion of these phases can take several years.

Phase 1 and **Phase 2** trials are small and testing is carried out to check the safety of the drug. Safe and effective dosing ranges are established. Side effects and adverse events are monitored and a small amount of efficacy data is often collected.

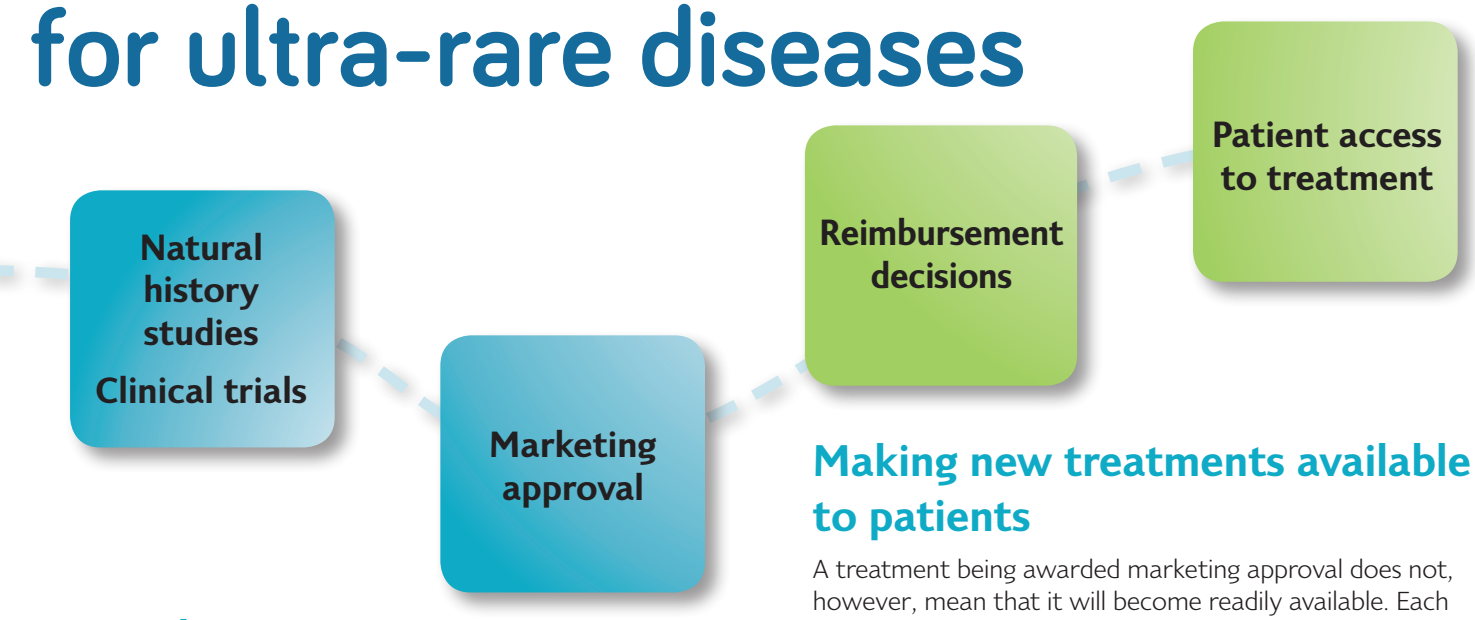
Phase 3 trials are conducted in a larger population, including more clinical trial sites and countries. This stage of clinical development will include pivotal trials which are those designed to provide evidence for a drug marketing approval. Further data is collected to continue to measure safety and obtain substantial data on the effectiveness of the treatment.

Phase 4 trials occur after the treatment has received marketing approval. They are used to understand the long-term effects of treatment in a greater number of individuals.

The end of a clinical trial

Due to the nature of research, a clinical development programme or individual clinical trial, might be stopped at any time, even a long running clinical trial. This may be, for example, because the data collected shows that the drug being tested is not meeting safety endpoints, efficacy endpoints or even for the business purposes of the company developing the treatment.

for ultra-rare diseases



Seeking marketing approval for a new treatment

At the end of a successful clinical development programme, marketing approval will be sought. This will involve submitting applications to regional or national bodies across the world such as the European Medicines Agency (EMA) for approval in the European Union and the Food and Drug Administration (FDA) in the USA. To gain approval the applicant supplies all the data they have gathered from their pre-clinical and clinical studies. This is reviewed and a decision is made to approve the treatment for use or not.

Accelerated approval in ultra-rare diseases

The very nature of ultra-rare diseases – where there is a very small patient base and a lack of available medical treatments – means that marketing approval for safe and effective therapies may be obtained quicker than usual due to the critical need for the treatments being tested. Accelerated approval can be applied for, which may mean marketing approval is given up to four times quicker than usual.

In Europe, this requires the EMA to award a treatment with a PRIME (Priority Medicines) status. PRIME status is only awarded to certain eligible products; it focuses on treatments that address an unmet medical need and that have the potential to bring a major therapeutic advantage to patients.

In the USA, this requires the FDA to award a treatment with a “breakthrough” status.

Making new treatments available to patients

A treatment being awarded marketing approval does not, however, mean that it will become readily available. Each country will decide whether they will reimburse the drug for use. This does become complicated as each country has a different healthcare system and, usually, an independent body or bodies to provide guidance around new treatments for use in the healthcare setting. For example, in the UK, the National Institute for Health and Care Excellence (NICE) makes recommendations on which new therapies should be available to patients and the individual health bodies for England, Scotland, Wales and Northern Ireland determine local availability.

The history of clinical trials

Clinical trials date back to biblical times, although it was during the 1700s when the first controlled clinical trial conducted by a physician was completed. However, an accurate methodology for the design of clinical trials was not seen until the 1920s. It was later still, in the 1940s, when a meticulous, systematic clinical trial was conducted within a framework that continues to be used today. It was from this time that development of the modern clinical trial format has been refined and stringent guidelines have been developed to protect the ethical interests of the patients participating in a clinical trial.

Although these advancements occurred relatively recently, clinical trials are important to our society. This is particularly true for the development of treatments for ultra-rare diseases. Indeed, in many cases, access to clinical trials is the only form of treatment available.

It is only through Sponsors continuing to fund clinical trials and the amazing people consenting to participate in clinical trials that the hope for those who are waiting for a treatment remains.

Trial and error

Daniella Vandepeer shares her experiences of family life on a clinical trial including her motivation for joining a trial, some of the difficulties the family faces and what she feels is important to share with others considering joining a clinical trial.

Having had the opportunity to speak at the MPS conference this year, I wanted to put into words what being part of a clinical trial has really meant for us as a family. For those of you who listened to my talk, I was really quite honest, and incredibly optimistic because pretty much at the time, life was kind of plain sailing. Summer had just started, Caleb's health was in tip top shape and we were about to embark on a wonderful big family holiday with lots of hands to help us! So whilst everything I spoke about at the conference was fairly settled at the time, just one day later, we had some significant life changing news that would once again change things for both us going forward on our trial journey.

Sure there are loads of things I spoke about that are still very much our experience and reality, like the huge unforeseen costs this has had on our life financially, the unplanned surgeries, the missed career opportunities and the constant need to be organised and on top of diaries and calendars. You never can really fully prepare for how much time a clinical trial often takes up in your life, your mind, and your heart. We also have to take into account that life, as we know it, often throws us huge curveballs which there really is no other way to tackle but head on!

Our life changing news over the summer was another gift of life. During our trial journey, four years ago, as a family we embarked Pre Implantation Genetic Diagnosis IVF, also known as PGD. We were incredibly lucky and were blessed with another son, but this time without any further concern that he would be affected by the MPS II ratio of a one in two chance of being affected. We felt like we were a complete family. So in the summer when we very unexpectedly found out that we had conceived naturally, all of those wonderful feelings you usually have on news like this were actually replaced with fear and worry and the prospect of some very lengthy and invasive genetic testing. At the end of the summer once all of the testing had been completed, we received the good news that we were expecting a girl. Wonderful news on a genetic counselling front as no further testing would be needed throughout the pregnancy other than the standard 20 week anomaly scan.

A heavy load of information which joins the rest of the backlog of "medical" stuff you have to remember that comes with a clinical trial being a big part of your normal life. All of the above forms part of our extensive journey as a family living with a life-limiting condition, but taking a risk on something as scary as a clinical trial, and seven years later, seeing the both the benefits and impacts it all has on the course of how you imagined life would be. You constantly fit every piece together like a complicated puzzle, some days you ace it, and some days you are constantly turning your puzzle pieces around trying to fit them in to make it all work.

Our motivation for being part of a clinical trial from the very beginning was life extending. One of the very first conversations I had with our trial doctor went along the lines of preserving brain power and having less white matter form, at the time this was just more medical jargon to decipher,

> "You never can really fully prepare for how much time a clinical trial often takes up in your life, your mind, and your heart.

but something I did understand and I remember starkly was the possibility of adding 15–20 years on a life which was predicted to end prematurely. On top of all this, our doctor was very honest and answered all of our questions, he listened to our fears and supported us through a very tough time. It is a very stark reality when all you know (remember this was seven years ago) is the natural history of a disease which was very bleak, and diagnosing doctors telling you important milestones like walking, eating, talking, understanding were probably unlikely to last. Having digested this information from the diagnosing doctor prior to trial, we already had a mission to supersede the difficult reality which was out in front of us.

Caleb with his Grandpa

> “There are so many factors responsible for our Caleb’s positive progression namely because we took an unbelievable risk, but we have had great support, and understanding from school and other important figures in Caleb’s life

Starting on the trial was difficult and time consuming but we adapted well as a family to our new routine. And on top of it all we saw small milestones, which of course could be coincidence, but each day, each week, each month, and each year we have seen and experienced some really wonderful milestones and benefits, that we all believe had we not taken that risk we would be looking at a different journey.

To date we have seen no loss of skills which we worked tirelessly with Caleb to master, from toileting, eating with a knife and fork, swimming, understanding sign language, and enjoying life to its fullest. All these things when you are a mum of one child with a neuro-degenerative disease are so much more concentrated. It wasn’t until I had a second unaffected child that I actually fully understood the magnitude of the effort Mark and I had put into ensuring Caleb gained some of these important skills which have been so important in settling him into school from being incredibly independent to learning something every day.

There are so many factors responsible for our Caleb’s positive progression namely because we took an unbelievable risk, but we have had great support, and understanding from school and other important figures in Caleb’s life.

These years have not been without their difficulties, recovery from the first operation was probably the most daunting as it was all new and we were learning how to articulate it all. This starts to become a little easier

to digest, but you always retain a lot of information and understanding which unfortunately make every surgery more daunting. From a non-medical point of view all subsequent operations, general anaesthetics, and other procedures become much scarier. You get so far along on your journey with little or no issues, that when you are faced with a broken port, or another issue, you sway between the choice of either not receiving the drug you have relied so heavily on, or feeling incredibly guilty for making the decision to continue, cause some pain, and watch your child recover from yet another surgery.

You see each procedure will have a different recovery period, and there are so many factors to consider, particularly at varying ages, from your hyperactive five year old to a very oppositional ten year old. When your child is young, there are lots of things you can do to distract and get them to relax like endless TV watching and drawing and appreciated mummy cuddles. But when your child gets to a transitional age, and four years into a trial where you can see that the drug is almost certainly having an effect on your child's life for the better, like their memory, and their understanding, it becomes harder to placate them. Often, Caleb's lack of verbal communication is perceived by those around him, including medical staff to also affect his understanding. To some degree this is true, but Caleb has gained a new understanding of his life and he understands so much. As parents we have to explain over and over again to everyone Caleb's capabilities. You are constantly your child's advocate, and you know if he is given a bit of attention by a doctor or a nurse they would gain so much knowledge about what motivates him. He knows what the doctors are asking him and of him, and he understands that needles hurt and he remembers everything from an access tray routine to our car journey and the various routes we take to the hospital. Suddenly his condition has many other arms and legs attached to it in the form of other learning disabilities such as Autism and Attention Deficit Disorder. Something we feel that has come out of the trial is Hunter syndrome no longer comes first. This is amazing in our world.

In order for our little world with Caleb to turn, continuity is a must. Communication is essential in whatever form works for you as a family. We know all the things that make life that little bit easier for us as parents dedicating our lives effectively to our child. But most importantly we need to be one step ahead in knowing all the things that make life much easier for Caleb. From stopping off at his favourite service station to use the toilet to which ones have the best McDonalds. We try to stay at the same hotel each month so that Caleb knows every corridor and corner for exploring. The slightest change in this routine can sometimes result in a very uncooperative Caleb and will set the mood for the day, which when you are travelling in excess of 200 miles round trip can make for a very long journey. Most MPS families will agree that all forms of routine are a must, but unfortunately are not always possible. Being honest and communicating effectively helps the trial run smoothly, and we find explaining to Caleb and giving him the lead often makes the day easier for him and us. It is not always the case that everyone will think the same, feel the same or have the same outcomes you as a parent will. Medical professionals and other families will all have differing views of what being part of a trial entails. We have learned over the years that doctors are just like you and I - human. Like us, they are working parents, they are aiming for a better life for your child, they strive to be the best they possibly can, none of us are perfect, but we can all learn from each other by clear communication.

In spite of the monotonous travelling and the unforeseen circumstances that have arisen over the years we have been part of a clinical trial, we would absolutely do it all again. We have put our whole life into ensuring our son has the best chance of potentially having a wonderful quality and quantity of life. It may be hard at times watching your child cry in pain from a needle prick or being so cross they hit out because they can't tell you they are having a bad day. The reality of doing the absolute best for your child as well as contributing to medical journals paving the way for future treatments makes you feel like you are accomplishing something as a parent and making a huge difference in some way. Selfishly, Mark and I made a decision to see this out for our child as a family. We make all his decisions, we are Caleb's eyes, ears and voice and although it is hard, it is our life now and we have had to embrace that.

Along this journey we have dealt with so much from initial enrolment, to bringing new life into this world and going through bereavement. All of these things make you very resilient and sometimes a little cynical but maintaining a level head for your child overcomes a lot of life's adversities. For me, trusting my intuition as a mum and really believing that all the decisions I have to make on behalf of Caleb really are for the best. Something I have always adhered to is a clinical trial is just that, something that is being trialled. There is no right or wrong way of dealing with it, experiencing it, and going through it. Everyone's journey in life is completely different, as are children. No two are the same and you will ultimately see things in your child you won't see in others and vice versa. Having a pragmatic approach to this whole journey helps me through, and we treat most trips to hospital as our special family time.

“ Being honest and communicating effectively helps the trial run smoothly

Events

Glenarm Santa Experience

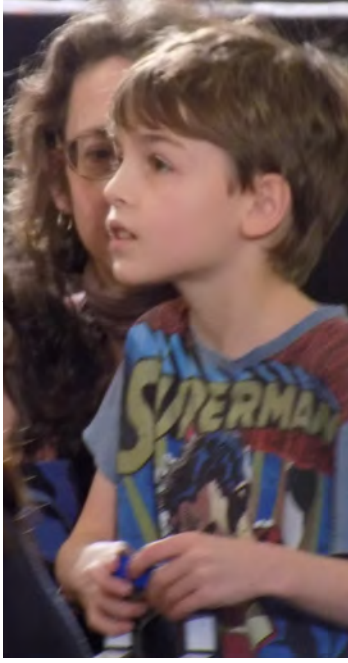
> To say it was a magical evening would be an understatement

It was a snowy Saturday in Glenarm and Santa had his feet up by the fire. He was all snuggled up for the evening with a mince pie (and some Guinness – he was in Ireland after all!) but the sneaky MPS Elf had brought some visitors! There was a knock at Santa's door and a crowd of rowdy boys and girls rushed to see Santa.

On the 9th December I had the pleasure of taking some MPS families from across Northern Ireland to the Magical Glenarm Estate to meet Santa along with the MPS Elf. To say it was a magical evening would be an understatement.

Thank you to all those who battled the elements to be there. It truly is the highlight of my year!

Alison Wilson



Manchester pantomime

Following the success of last year's pantomime in Manchester the MPS Society chose to host the same event again this year.

This year's extravaganza was Aladdin and his Magic Lamp. Louise and Debbie from the advocacy team attended along with the MPS Elf to greet families to the event.

There was a hot buffet lunch to welcome everybody in from the cold and the families watched the performance from the comfort of their tables.

However, most children were so excited they chose to sit on the floor close to the performance. The actors happily involved the children in the story and it became very interactive – which the children loved.

The children helped with the treasure map, booing the baddie and helping Aladdin escape the cave. The children helped with Wishy Washy's laundry and managed to shrink some pants!

The MPS Elf also played a part in the pantomime and at one point was kidnapped by the baddie, but Louise and Debbie managed to rescue him and bring him back to MPS House.

Everybody appeared to have a fun time including the adults...oh no we didn't...oh yes we did...

> "Everybody appeared to have a fun time...oh no we didn't... oh yes we did...

> “It was a lovely experience thanks to all the MPS team for making this possible, much appreciated

Lapland UK

On Tuesday 12th December 2017 we had a very cold but extremely exciting day out at Lapland UK. Here is what the fun day looked like.

For some of us real snow and ice was the issue getting to Lapland UK. It was a bright sunny, but cold start. The families arrived with excited, but sleepy children, some were up before the birds!

The families were in either Husky or Reindeer group and the elves called us 'big folk' (adults) or 'little folk' (children) and the little folk were given a special passport which was stamped by the elves once they had completed some important tasks. Once the tasks were completed they could then collect their special jingle bell given by the elves. They also had Jingles (elf money) to spend in the Elf Village.

Elves collected us all and we took a walk to the enchanted forest, we were told the story about how the elves are trained to become Santa's helpers. We all had to learn a secret rhyme to enter the training workshops.

The first task for the trainee elves was to help finish making a polar bear and baby that Father Christmas will deliver to children on Christmas Eve.

Next stop was in Mother Christmas' kitchen where the trainee elves decorated their own gingerbread Christmas tree which had been lovingly baked by Mother Christmas and her elves. Lots of lovely sweets were there to decorate the Christmas trees and many were eaten before they got stuck on the tree. The children could then take them home to eat later, if they lasted that long!

Once the trainee elves had finished decorating they were asked to take a seat for story time with Mother Christmas. Mother Christmas showed us the wonderful gingerbread houses that she had made. Before we left Mother Christmas advised not to give the gingerbread trees to Father Christmas as he was already too "sideways tall".

Then out we went to the Elf Village, where we had fun on the ice rink and everyone could join in with. You could collect your special bell to confirm you were a trained elf for Father Christmas, write a letter with your wishes for Christmas and post it at the elf post office and spend your Jingles on lots of Christmas treats.

We took our MPS Society elf with us on the day and he was seen taking part in all the things that the children enjoyed. After meeting the huskies it was time for everyone to meet Father Christmas' reindeer. Many of the reindeer were lying down having a rest, conserving their energy for the delivery of the presents on Christmas Eve.

All the children had been very good and helped to get some of the toys ready for other boys and girls to have at Christmas and Father Christmas gave them all a cuddly husky to take home.

Our MPS Society elf didn't want to be left out and Father Christmas was happy for the staff and elf to have a picture with him.

So after a lovely day we all made our way home, a great time was had by all the big folk and little folk alike.

On behalf of all the staff at the MPS Society we wish you all a Happy New Year.

Rebecca Brandon
Advocacy Support Officer

Remember

Childhood Wood planting and weekend away for bereaved parents and partners

We are Dee and Monty from Birmingham and we attended the weekend away for bereaved parents on the weekend of the 14th of October. We went knowing only one other family, so we thought, but when we all met up on the Friday evening, there were a couple of other people we knew and others that we had to "study" as, like us, the years have passed and age changes the way we look now.

The weekend included a visit to Childhood Wood planned for the Saturday. We left the Hotel by coach all clutching our balloons. When we arrived at the Childhood Wood we all "marched"/walked slowly with care towards our special place in Sherwood Pines.

When we spotted Simon Lavery's bench we knew we had arrived, walking a little further on we came to the first of the two new boards, which had names on, pre-2010. Here Wilma Robins read the poem that means so much to us: "Remember" by Christina Rossetti.

Then, after a count down, the balloons were released. Many behaved themselves but like our children some were mischievous and got caught in trees. One dad climbed a tree, a few tried other ways to release the mischievous balloons and through their efforts most took flight.

The whole weekend was so very special to us as a couple, not least as it was the Birthday and Anniversary of our first baby, Katherine, whom we never took home.

Fabulous, fantastic, friendly, wonderful, wicked and much more. Thank you so much for the hard work that went into arranging the weekend for us and the other families, we cannot thank you enough. Old friendships have been renewed, new ones made, all with the same life experiences.

The venue couldn't have been better. You could either do different activities put on by the hotel or just relax or walk around the grounds. The evening entertainment was very good and most enjoyable.

Lastly may we say such a BIG THANK YOU to Christine, Bob Stevens and all who made the weekend happen. Meeting Bob was so good as he understands MPS personally, so will have the families in his heart...he's great.

Same again please!

Love Dee and Monty Russell xx

Clinical trial logistics

- We are experts in the ultra-rare diseases field, providing fully managed logistics to ensure patients can access clinical trials.
- Our service includes out of hours support, which can be accessed by non-English speaking families.
- We provide a patient-led service and are experienced in finding solutions to unique challenges.

Patient focussed research

- Our highly qualified and experienced team can create a bespoke solution to answer your research question.
- We will design and conduct a custom study to meet your needs including questionnaires, patient interviews and focus groups.
- Extend your research through our international network of patient organisations.

Managed access programme support

- Our dedicated Managed Access team are experienced in meeting the unique demands of the new and evolving Managed Access route for the treatment of rare diseases.
- We have well established contacts with expert clinicians and clinical centres, NHS England and NICE.

Medical communications

- We can help you publish the results of studies we have conducted on your behalf, or your own research.
- We can also develop disease awareness and patient directed materials.

MPS Commercial

How we help patients taking part in clinical trials

If you or your child are going to take part in a clinical trial, you may have to travel some distance to attend the site to receive your treatment and have the necessary follow-up visits and tests. In some cases, families will need to re-locate for the duration of the trial. The sponsor of the trial will help with arranging travel, accommodation if required and reimbursement of expenses. Often they will employ a clinical trial logistics company to help you with these aspects of taking part in the trial.

MPS Commercial provides logistical support to patients and their families. We ensure that travel and accommodation is suitable and aim to make these aspects of taking part in a trial as stress free as possible. Wherever possible, we visit the trial sites and assess the local travel and accommodation facilities for you.

In preparation for your first trial visit we will make sure that you have all the information you need and have an opportunity to ask questions. For the duration of the trial we will take care of your travel and accommodation bookings and the reimbursement of out of pocket expenses in line with the sponsors reimbursement policy. We also offer an approachable support service with access to a 24 hour, 7 days a week helpline.

If you are considering taking part in a trial and would like to know more about the support you might receive, please do get in touch with us at info@mpspact.com.

Team news

We are pleased to welcome a new member to our Clinical Trial Logistics Team.

My name is Sarah Buttfield, I started working at MPS Commercial in October 2017.

I have a background of working with children for ten years plus bringing up two of my own. I have also spent the last five years working in Reception for a local Health Centre.

I am very pleased to be working for MPS Commercial as a Clinical Trial Patient Coordinator as this involves working with families in a caring environment. The Advocacy team of the MPS Society have given me some training on the MPS conditions and the support they provide to our members of the Society. I am looking forward to finding out more and becoming more involved with the families.

I am learning something new every day and am very lucky to be working with such a friendly and dedicated team.

In my spare time I enjoy spending time with my family, walking our dog and going out for dinner. I have also started a Pilates class at the local gym that I attend.

Clinical trial logistics

Trip to the USA

Christine and Jo had a successful trip to Washington and Boston, USA, where they met with a number of pharma companies with research programmes in MPS.

Patient focussed research

Keep calm and complete a survey

In this issue we are taking a closer look at surveys and how they can benefit the MPS community.

The value of your survey answers

Patients with rare diseases are asked more than most to share information about their disease, but how is this information used?

Pharmaceutical companies, the regulators that approve new treatments and the bodies that decide whether the National Health Service can afford new therapies are increasingly seeking the patient's perspective of their disease to support their research and decision making.

This is especially important when the disease is very rare as there is generally much less information available from traditional sources such as articles published in medical journals and clinical trials will have involved much smaller numbers of patients than for a more common disease.

How sharing your experience can help:

- Gives researchers an understanding of the disease and what symptoms and effects they are trying to treat
- Identify ways to measure improvement by looking at the disease's natural course
- Determine what improvements are the most important to those with the disease
- Assess whether new types of drug delivery would be acceptable
- Design appropriate clinical trials
- Develop understanding of support needs
- Informs regulators and payers of the level of need for treatment

Every voice can contribute to the future care and treatment of those affected so please do keep on completing our surveys

How can we do it better?

We'd really like to know how we can improve the survey process. Let us know what would motivate you to take part, what puts you off and your ideas of how we could do it better. Share your views with us by e-mail **info@mpspact.com** or online at **www.surveymonkey.co.uk/r/Tellusyourthoughtsaboutoursurveys**

We need your help

We are still looking for people to take part in two of our current surveys

European MPS VII (Sly) survey

This disease is so rare that only a handful of cases have been identified across Europe.

We really need your help to understand this disease as there is very little information available for doctors, researchers and people who have been newly diagnosed.

Our survey is looking at three key areas:

Pre-diagnosis Who are individuals with MPS VII seeing in the early stages of their disease? Can we identify which doctors we need to give information about MPS VII to? This could help individuals who are currently undiagnosed, or who have been misdiagnosed.

Support needs What type of support do individuals and their families need? Could a European or national support programme be developed?

Effect on daily living How does the disease affect individuals? This can help all those affected understand the disease better. It can provide doctors with a better understanding of the overall care and support needed. It helps to identify what impact any new treatments are having. It can help with gaining access to therapy when health authorities are deciding whether to fund a new treatment or not.

MPS III (Sanfilippo) survey

The original European survey has recently been rolled out across the world to achieve our goal of 200 responses. The questionnaire is available in multiple languages and can be accessed on the MPS Society website, just go to the MPS Commercial page and look under Current research studies to take part.

If anyone is willing to share their experience of this condition please complete the survey at www.surveymonkey.co.uk/r/MPSVII2017 or email us at info@mpspact.com.

Research news

Understanding Fabry in families study

After receiving a phenomenal 723 responses to this survey from around the world, data analysis is underway. We hope to share the results from the UK with you in the Spring issue of the magazine.

International MPS III (Sanfilippo) survey

In support of this ongoing study Alex presented on the project to the German MPS Society Patient Conference held in Wurzbach in November. The German Society have supported the study by interviewing nearly 50 of their members affected by MPS III.

We are also pleased to report that our abstract entitled 'Patient organisations working in partnership to research the patient experience of rare-diseases – the MPS III survey,' has been accepted for poster presentation at the 14th WORLD symposium being held in San Diego from 5–9th February 2018.

Alex presenting at the German MPS Society Patient Conference

Photo by: Stefan Stark

Filming a patient video

Jackie travelled to beautiful Monmouthshire where she was warmly welcomed into the home of Megan and her parents to film a patient video about her pathway to diagnosis and what life is like living with alpha-mannosidosis. Keep an eye out on our website as we hope to premiere it soon!

Medical communications

MPS Commercial diary

5th–9th February 2018
14th Annual WORLD Symposium, San Diego, USA

We attended the WORLD Symposium and it was great to meet with so many pharmaceutical companies and showcase our research at the poster session:

Wednesday 7th Feb 2018, 4.30–6.30pm. Poster 250. Morrison A *et al*. Patient organisations working in partnership to research the patient experience of rare-diseases – the MPS III survey.

8th–9th June 2018
6th Fabry International Network (FIN) meeting, Vilnius, Lithuania

We will be presenting the results from our recent Understanding Fabry in families study at the annual FIN meeting.

2nd–4th August 2018
15th International MPS Symposium, San Diego, USA

We will be attending the International MPS Symposium and for the first time MPS Commercial will have a booth. Please feel free to pop by and say hi to the team if you are attending.

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

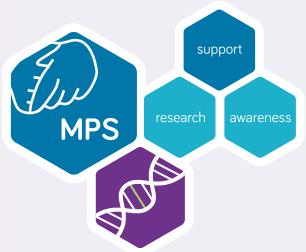
MPS Commercial trades as Patient Access to Clinical Trials (MPS PACT), and is a wholly owned, not for profit subsidiary of the Society for Mucopolysaccharide Diseases (the MPS Society), Registered Charity in England and Wales No. 1143472.

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.

You can now reach MPS Commercial on their own dedicated number: 0345 260 1087

Supporting the MPS Society

As the non-profit subsidiary of the MPS Society, we are proud of the contribution we are able to make to the work of the charity.



Managed access programme news

Results of the clinical measures from individuals with MPS IVA (Morquio A) enrolled on the English Managed Access Agreement for treatment with elosulfase alfa (Vimizim) were presented for the first time at the 14th WORLD symposium held in San Diego.

Managed access programme

Invited as speaker

Jackie was an invited speaker at a meeting entitled: Advocacy Powered Through Data Generation: Facilitating Medicine Approval and Access, in Amsterdam in November. Jackie's presentation, 'The Value of Patient-Focussed Data in Health Technology Assessment' showcased the quality of life data collection that MPS Commercial undertake as part of the elosulfase alfa (Vimizim) Managed Access Agreement.

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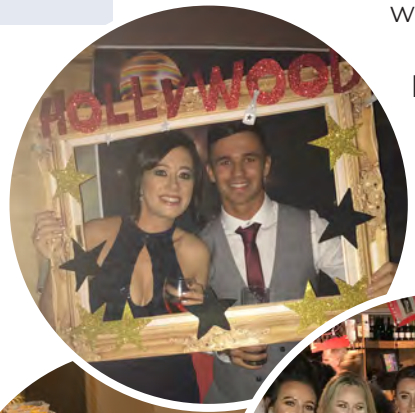
Fundraising

Iris Hitter organised a quiz night and charity open house, named as Jacqueline's Day, in memory of her daughter. The charity open house included a picture quiz, raffle, games and homemade cakes, teas and coffee. For the last two years Jacqueline's Day has raised a grand total of £373.00

Congratulations to Sally and her family who bid £50 to adopt our MPS Elf.

Hollywood Theme Party

We got in touch with Nicole after her Hollywood themed party on 29th September which was mentioned in the autumn 2017 issue. Nicole sent over some amazing photos from the night. They had created a night of great entertainment with dancing, a buffet and a raffle as well as information slides about the MPS Society to let everyone who attended know what the donations given on the evening were going towards. Nicole raised a total of £2011.00 which is incredible.



FIRBECK SAILING CLUB

Seas the day

Firbeck Sailing Club at Rother Valley Country Park held a big fun day for charity on the 20th August this year and supported MPS Society as their chosen charity and raised £800.00 this was all possible because of a connection they had with Wayne Bond who's child Harley has MPSIII Sanfilippo At the Winter warmer race day our Trustee Paul Moody went to receive the cheque and meet all the great fundraisers who took part in the event.

Happy Harvest

Bournemouth Jobcentre held a Harvest festival and raised £86.00 with a wonderful hamper in a raffle

Family fundraising

Inspired by his Grandparents', Anne and Ken Hooper and their fundraising for MPS, Tom Barrett ran the Bournemouth Half Marathon on Sunday 8th October, raising £761 for the charity. Anne and Ken first organised a charity walk for Bournemouth Hospital Gastrointestinal (GI) cancer in 1996 after Ken had been successfully treated for bowel cancer. The fundraising walk of approximately 4 miles started from their home in Throop, Bournemouth, taking in the scenic Mill and River Stour and culminated in a ploughman's lunch with Ringwood beer and a raffle in their garden. Many people were sponsored for the walk and it became an annual fun family and community event. After Ken and Anne's grandson, Jamie, was diagnosed with Sanfillippo in 2004 the proceeds of the walk alternated between MPS Society and Bournemouth Hospital GI cancer research. Over this period six walks have raised an incredible £19,602 for MPS. 2017 would have been MPS's turn to receive the proceeds but, having reached their 80th and 81st years respectively, Anne and Ken decided reluctantly that the organisation was becoming too much and that the walk, and perhaps more importantly for many the lunch, would no longer take place. As a family we have all been affected by this terrible disease and seen the amazing support and friendship the MPS Society have given Jamie and his parents and sisters. The end of this annual fun event was the motivation Tom needed to sign up for the Bournemouth Half and set up a fundraising page for MPS; he wrote that although stricken with this awful progressive and degenerative disease, his 18 year old cousin Jamie's "distinctive laugh and cheeky smile gladly remain."

Suzanne Barrett

Sporty fundraisers

Left to right: Sabea Bradley, Ben Ingram, Pete Osborne and Nick Harrison

On 15th October 2017 four members of staff from Manor Court Hotel in Bridlington ran the Bridlington Half Marathon to raise money for the MPS Society. Pete Osborne, whose mum, sister and brother all have Fabry Disease, contacted the society when he was planning the event and set up a JustGiving page for sponsorship as well as an information table in the hotel giving guests the opportunity to learn about what the MPS Society does and to donate money. Alongside Pete were Ben Ingram, Sabea Bradley and Nick Harrison, his colleagues and friends at the hotel. Together they trained to be fit enough to run the 13.1 mile course. It was a blustery October day by the sea in East Yorkshire when the four joined about 500 other runners setting off down the promenade at the beginning of their run. They all completed the course and between them raised over £900 for the MPS Society.

I took on the challenge of a 100 mile bike ride on the 24th September starting at the National Indoor Arena in Birmingham and ending at the same point. I am doing this as a very close friend of mine has a child who has been diagnosed with MPS II (Hunters syndrome). I had a brilliant day at the velo Birmingham raising money for the MPS Society. My official finish time was 5:34:51. By the time I had finished the total raised was almost double the target figure so I couldn't be happier.

Adam Foster

London

Amanda Scott and Andy West (Darren Scott's best friend)

"I am Darren's best friend and I have known him since aged 12. Since the diagnosis there have been some dark moments, very dark moments. So it has been tough but I feel I've helped in my own way, day to day. His reason for getting up in the morning is to try and find a cure, live for today and make sure Sophia lives life to the full. As for Amanda, she has been the rock. I have not come across someone as resilient as Amanda in my life. But she needs an outlet, something else to focus on as well as fighting this terrible disease. The London Marathon I think would be perfect for her. Where do I come into this? Well, I am a regular runner (8.5 miles, five days a week) and have promised to train and run the London Marathon with Amanda. We live very close to each other, so training together will not be a problem and I will be getting Darren out pounding the streets on occasion as well! I think it would be great for Amanda knowing that she has someone by her side during training with the same end game as her – to run the marathon and raise awareness and funds for MPS diseases. I love running but I know loads of people find it a lonely past time. I don't want Amanda to feel alone, I want her to feel excited, energised, enjoy training and be part of a journey which can bring a smile to her face and Darren's." *Andy*

Jessica Edgecombe

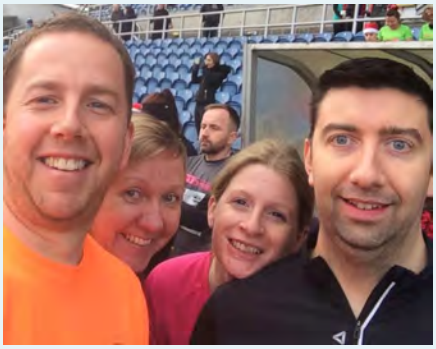
One of our long standing supporters Adam Gosling volunteered Jessica to run this year's London Marathon "Jess is a fab chef, she comes and supports my sailing team from time to time and looks after us superbly" *Adam Gosling*

Marathon Runners

Every year we have such a great response from fundraisers wanting to take on the London Marathon, so thank you to everyone who applied or showed an interest and we hope to secure more places in the future

Rebecca Caplan

"I have been inspired by a number of friends who have recently completed some serious challenges and always watched the London Marathon and felt it would, one day, be fantastic to give it a go. However, at 52 next year, I don't think my body will wait much longer! Tom and Louis Garthwaite are very good friends and their parents, James and Claire, have been close friends for over 30 years. We also know Olly Gosling very well – my husband, Peter, is his Godfather. We are therefore very aware of both Hunters and Hurlers and the serious challenges these give Tom, Louis and Olly. So, I know 3 young people with MPS and their families extremely well. I would love to run the marathon for them – to raise funds for the charity and to spread awareness of these conditions. I am not a runner, certainly nothing over 5K so this will be a huge challenge but one that I would relish."



Katy Brown and Lindsay Johnson

"None of us have ever run a marathon before but all four of us – Katy, Simon, Ian and Lindsay – are going for it this year. We've all been entering races together for four years, slowly working up from 10ks to half marathons, but never any more than that – until now! Lindsay and myself have MPS Society places, Simon was lucky enough to get a place in the ballot. Ian, meanwhile, managed to get a place through another charity but joins us in MPS spirit! The training is going well – as is the fundraising – with £800 totted up already. We're aiming to get to £2,000 and we're working hard to add up the miles and the money we raise." *Katy*

Great Runs

North

The Great North went really well, I completed the run in 2 hours 28 minutes taking 30 minutes off my time from last year. More importantly I raised over £370 with an extra £80 for Gift Aid. My colleague lost her son Michael on his 13th birthday to MPS and she was the reason I contacted the organisation. I ran with the mini bear that the MPS sent me. We called him Michael bear, Michael after my colleague's son. I am the one wearing the MPS tshirt in the pink hat, it was an absolute pleasure to run and raise money for the MPS Society. My sister in law is called Samantha Unsworth, we've been friends for over 30 years and I ended up marrying her brother 15 years ago. So it was a good day doing a run with her. Samantha ran for a local charity raising money for research into brain haemorrhages. I believe the final amount raised was £490.00

Kathrine Winter

Deborah and Mark Burniston took on the Great North and Great Manchester Half this year.

Great South Run – Portsmouth – 10 miles – 22nd October 2017

After weeks of hard training the day had finally arrived. With strong winds forecast we waited at the start with trepidation.

Stuart had never ran a race before and through regular training and diet he had shed over 2 stone and was ready to go. Chris was more used to a fitness regime, being a Personal Trainer, but nonetheless it was still a daunting challenge for him. We both didn't want to let our family and supporters down. The first 8 miles went according to plan, running around the Historic Dockyard, passing HMS Victory and going through the streets of Portsmouth. There were so many runners it was like the M25 on a bad day! The final 2 miles was a different challenge altogether. Already tired we faced the windswept seafront of Southsea. With the wind nearly blowing us off course, the last leg was gruelling, but we made it. We both achieved Personal Bests, Chris 1hr 20mins and Stuart 1hr 38mins. We are delighted to have raised over £615 in support of the MPS Society which we believe is a very worthwhile cause. Can't wait until next year!!!

Chris Hill and Stuart Plenderleith

It was a great time I loved it had a fantastic atmosphere, I finished in 2 hours and 15 minutes. I've managed to raise £375.00 with gift aid. I did this because my brother (age 9) and his younger friend called Jack both have Sanfilippo syndrome.
Katie Playle

Thanks for the opportunity to run the Great North Run it was fantastic. I managed the course in just over 2 hours and had a wonderful time. I managed to raise £252.00 on my JustGiving page and £ 148.00 in cash totalling £400.00.
Graham Martin

East

Running for Dylan

Dylan, my 2 year old son, has been diagnosed with MPS III (also known as Sanfilippo syndrome). Zab, Mark, Shaun, Gavin, Jack, Melissa, Louise and myself ran the Great East Run.

Through BT donate we raised £351.50 plus £64.78 in Gift Aid as well as an extra £335.00 sent by cheque with the possibility of more match funding to come

Darryl Brook

South

The Great South Run was good although hard work. My goal was to do it in less than 1 hour 30 minutes. Although the wind was gusty, particularly along the last 2 miles so I actually did it in 1.29, so pleased with that. I think I managed to raise £ 874.00, so pleased with that. Next year I think 1.20 will be my target.
Lee Shepherd

A walk in the park

On the 31st October 2017, all the staff from MPS House went on a walk to celebrate the 35th year of the MPS Society. The walk lasted 35 minutes and was a 3 mile round trip from Westwood Park, Little Chalfont to the Bedford Arms, Chenies.

We started the day dressing up in our "Wicked" fancy dress attire taking into account that we would also need to wear considerably sensible walking shoes, as the route was set out across the countryside and likely to be muddy. We all gathered at Westwood Park car park at around 10:00am dressed up in tutus, funny hats and accessories with our wellies on, ready to walk.

We had a great surprise when Cookie Brown and her daughter decided to join us dressed as witches and were lucky with the weather. We started our walk along the edge of woodland to meet Christine and Bob at the other side of the park, we carried on through the wood and along an escarpment-like footpath along the top of a field with wonderful views across Chess Valley. Walking past manor house and gardens making our way along the village green to the Bedford arms for some much needed refreshments provided as a good gesture from the Bedford Arms team.

We took some time to rest and enjoy the moment before heading back towards MPS House on the same route. We arrived back just before midday and had raised just under £300 on completing our walk, enjoying every minute knowing that this would go a long way in helping with research and support for the future. It was a great time to bond and raise money and took little effort to organise.

If you would like to get out in the fresh air to support MPS Society and organise a similar event – whether large or small – visit our webpage where you'll find posters, sponsorship forms and registration forms as well as a step-by-step guide to a perfect *Walk, Toddle or Roll*.

www.mpssociety.org.uk/walkabouts

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

Paddy's Whiskers held an end of season big busk on the beach and chose to support the MPS Society for local boy, Corey, raising £226.18.

Mrs M Conquest, grandmother to Joseph Hiller, is a member of a small church house group of St James the Less Hadleigh Essex who raised £40.

Kate Evans had to back out of Surrey Half in March but was able to take part in the Lion Charity half on 18th June and raised £900.

Richard Kitchen donated £20 after the Advocacy Service Team helped with his PIP claim.

Anne and Mike Palmer gave £15 that had been collected between the family made from loose change.

Cookie (Eleanor) Brown raised £200 by sorting out her shed and selling flower arrangements sundries to fellow flower arranging friends.

Richard Dunn sent in a donation of £20 on behalf of Ella Dobson who donated after reading his book "A Chance to Live".

Unison in Surrey donated £50 which was chosen from hard working activists in their region after they had distributed prizes.

Arlene Murray has raised a further £81.10 through collection pots.

Dress down Friday at Currencies Direct raised £112.21.

Ross Stuart donated £74.22 which was originally raised for his son, Jack, for a visit to Disney World in Florida. Unfortunately, Jack sadly passed away in December 2014 so when Ross closed the bank account he chose to send the remaining balance to help others with MPS disease.

A long overdue thank you to Maureen Ryles who raised £579.50 plus £54.63 Gift Aid in the Edinburgh Marathon in 2016.

Catherine Groves and Ian Davis raised money for MPS Society in lieu of their wedding gifts in September sending in a total of £390.00 plus £97.50 in Gift Aid.

Allen Fencing Limited held a gold day this July and raised £2,840.00 which was sent in via Davis Eaton.

David and Alison Patton raised £380 at Oscar's Christening.

Paula Sheridan raised £439.48 in two challenges: Virgin Money London 10k and the Richmond Half Marathon.

Marina and Friends charity shop in Bristol has raised a further £4,080.63 which has now brought in a total of £181,376.22 for MPS.

Starkey & Brown donated £100 because one of their customers has a daughter with MPS IIIA.

Wayne Pollock Exclusive Luxury holiday company donated £200.00 in lieu of Christmas cards.

Falcon Food services donate monthly to charity and one of the employees put forward MPS Society as beneficiary because of excellent support to him and his family. We received £150.

Stephen McCawille completed the Brooks Snowdonia Marathon and raised £105. He has completed six challenges overall and collectively raised £1,263.15.

Myra Williams donated three tickets for a performance of The Lion King in London which we advertised on Facebook. These were snatched up by Karen Sainsbury for £50.

Matt Lamb sent a fundraising donation of £175 raised by Safe Haven Day Nursery where his son attends. The team at Safe Haven wore blue and held a raffle to raise the money.

Shaista Yaqoob raised £200 selling homemade curry at her work place.

Portland Medical Practice held a Christmas jumper day and raised £65.

The Rustic Crust and The Brown Cow raised £174.16 when the Rustic Crust celebrated its 3rd birthday and donated 10% of every pizza order to the MPS Society.

Lucy and Rebecca Frank raised £248.05 selling cakes at school for her cousin who has Sanfilippo syndrome

The pupils at Rigby College raised £497.57.

Throughout 2017 Emma Siddall and Wayne Bond have been triumphant with fundraising and awareness. We have received a further £780 this quarter from various fundraising events.

Heather Taylor collected £50 in donations in lieu of Christmas cards from attendees at a keep fit class in Darlington and chose MPS Society because of a connection to Claire Tolley.

Samantha Forbes raised £220 through a Christmas decoration and raffle fundraiser with friends and family.

Donations

Subkelz Costa, Richard Kitchen, Mr R. Collett, Carolyn Metaxa, Michael Gough, F.G Robinson, Jackie Cooper, Wilma Robins, David Gordon, Barbara Watt, Glenn Mckee, Mrs. A Palmer, Graham Bell, Mrs S Vivier, S.G Clarke, Janet Gremo, Mrs C. Shorthouse, Mr Trevor Brown, Mrs M Lyon, Mr & Mrs Lavelle, Beryl Bird, Mr & Mrs P.M.C Rock, John Byrom, Norman Saville, Justin Berry, Mr & Mrs Naish, Mrs Baker, Miss L Ricketts, Mrs A.J.Gunary, Mrs Powell, Mrs F Gee, Christine Hume, Mrs Macintyre, Rob Kenton, Les King, Patricia Skidmore, Pam Hope, Shirley Bown, Moira Darke, Mrs E Mason, Christine Fitzpatrick, Maureen Loveday, Carol Copsey, Antony Selwood, Shirley Stewart, Iris Hitter, Anne Franklin, Peter Hawkins, Lewis Brown-Sawyer, Anne Ridley, Holly Nowell, Wendy McGinn, Damian Carr, Marlene Murty, Pauline Headland, Kay Todd, Mrs J B Hudson, Carol Copsey, Mr & Mrs Mayhew, Ruth Hall, Mrs G Plummer, Mrs & Mrs Danny Todd, Mr P Swayne, Ms S Mooney, Wendy McGinn, Laura Sutherland, Ian Mahoney, Kathryn Wallis, Brian Tilbury, Andrew Culley, Alan Hall, Jenny Quant, Robert Kenton, Mrs D Jowett, Valerie Morley, Linda Rowland, Jude Butler, Dorothea Gallagher, Mrs M Mcgrattan, Donna Quinn, Amy Hague, Peter Archard, Jacqueline Alcantara, Kathleen Hiller, Kenneth Silman, Matthew Lamb, Richard Kitchen, Sara Juul, Ann Calveley, Emilio Davila, Teresa Alvarez, Sally Mitcham, Mark Hughes, Jean Mossman, Ross Silcock, Katie Kafizas, Lindsey Jephcott, David Warren, Miss E Jenkins, Pat Rowan, Keith Hotston, Nigel Nicholls, Mr & Mrs Matthews, Robert Jones, Ann Calveley, Sally Reis, Shabana Kausar

Regular contributions by Standing Order or Give As You Earn

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

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

Ellen Nicholson, Dinah Adair, Damien Adair, Donal Drayne, Langlea House, Vivienne Culley, Mr & Mrs Matthews, Ellen Nicholson

In memory

Paul Franklin, Mrs Joyce Eleanor Mary Arnold, Jamie Piromalli, Dean Lewis, Gracie Bella Sims, Bob Silcock, Denis Rowan, Shujah Altaf

Thank you to those who donated via the Weather Lottery:

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

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

We are so grateful for all who have donated in memory of Christine Lavery. We will work hard to continue her mission at the MPS Society and make sure she leaves a lasting legacy.

Jenny Wilson; Janet Richardson; Linda Pack; Taryn Treger; Nedeem Al-Astrabadi; Linda Golding; Wendy Preston; Chris Regester; Robert Proctor; Mark, Jeanne and Ryan Dant; Kazue Lidderdale; Nicolas Koebel; Stephen Ma On Tat; Ann-Marie Watson; Jennifer Greenberg; Terence Ballard; David Stephens; Jacqueline Harris; Angharad Watson; Mary Davison; Barbara Pollard; Ian and Ann Hedgecock; Save babies through Screening Foundation; Rosemary and Harry Nurse; Manda, Greig, Emma and Millie Stuart; Moira and Keith Dorke; Mrs A.B Clifford; Jenny and Andy Hardy; Janet Richardson; Jessica Reid; Elizabeth Svennevik; Roy and Sue Jones; David Russell; Alan Bowen; Kathryn Maters; Madalena Avila; Alessandra Callegari; John and Polly Cleall; Keith and Jackie Blackmore

Have you heard of Ultra Challenges?
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7 great adventure challenges!
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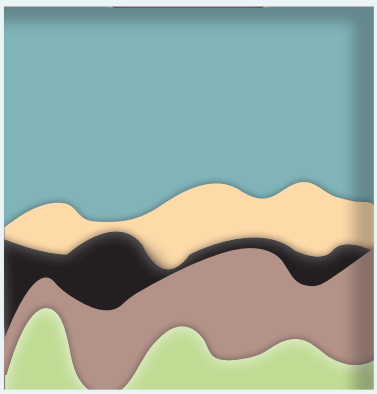
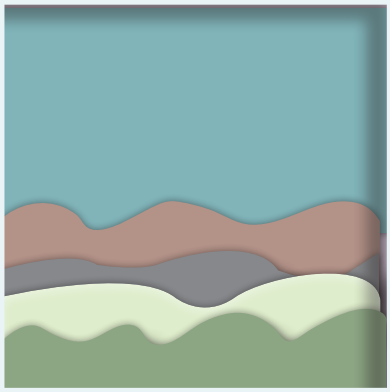
www.ultrachallenge.com

2018 Challenges

The challenges to be a part of this year we have limited places left in our Great North, Great South and Prudential Ride however if you have secured your own place on your own challenge this year please do let us know, we appreciate all of you that choose to take on various challenges to support the MPS Society



Prudential Ride
28th July 2018



Great North Run
9th September 2018

Great South Run
20th October 2018

Interested in taking part get in touch at
fundraising@mpssociety.org.uk

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