

MPS magazine

Society for Mucopolysaccharide Diseases

Spring 2019 • www.mpsociety.org.uk

So many ways to raise

THREE FUNDRAISERS SHOW THREE DIFFERENT APPROACHES

GET INVOLVED!
MPS AWARENESS
WEEK 13-19 MAY

Look out for our new family and event photos in 2019 – including our cover model, Romy

EXCITING EVENTS

Photos and news from Lapland UK

INTERESTING INFO

Get ready for holiday with accessibility tips

TOPICAL TREATMENT

Three years on from the Vimizim MAA

MPS and related diseases

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

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

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

The MPS Society

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

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

Board of Trustees

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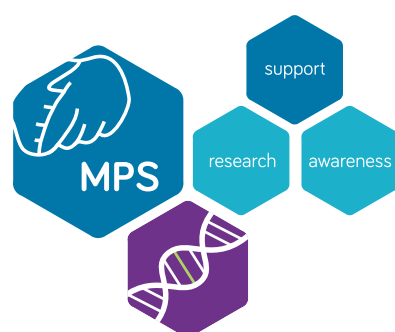
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In September 2018 we undertook a survey to help define the Vision of the MPS Society.

We sent survey invitations to all of our stakeholders – over 5000 in total.

You can now see a summary of the results of the survey on our website at:

mpssociety.org.uk/vision-survey-summary

Thank you to everyone who responded. You have helped to shape our future plans.



FUTURE OF VIMIZIM

Three years since the enzyme replacement therapy for MPS IVA was made available we investigate the results of the survey data



OVER THE WALL

Fun activity weekend for Fabry families



HAPPY HOLIDAYS

Handy travel tips no matter what your ability

WELCOME

This will be the last issue of the magazine in its current form before we move to a new-look, shorter, once-a-year, impactful newsletter! The magazine has been a great resource and one of the first things that Christine coordinated back in the early days of the MPS Society. However, life moves on and we have listened to your feedback and that you prefer to hear from us in other ways. Our plan is to provide more regular electronic communication so information is always up to date and focussed on what you want to hear about.

We will always take your opinion into account so if you want to get in touch with your thoughts about the magazine and its future then please email us at magazine@mpssociety.org.uk. And if you haven't already given us your email address please update your contact details so we can continue to stay in touch. Visit www.mpssociety.org.uk/keepintouch or phone 0345 389 9901.

From the Group Chief Executive



Bob Stevens
Group Chief Executive

Spring is here and it will soon be the MPS Conference 2019

I am really excited about our MPS conference being held in Coventry on the weekend of 28-30th June.

This year is different for many reasons, not least because it is in memory of our founder Christine Lavery MBE. It will also be more interactive and less formal. A chance for new members to mix with long-standing members. A chance to learn about the new science and more about the MPS conditions. It is also a great opportunity for us all to come together as one community. I know as an MPS dad how important that was to our family especially in the early days of our MPS journey.

Come along and discover that there is support, there is a place where we can all laugh and cry together, come and find out how you can be involved and be part of Team MPS but most of all come and discover that although you are rare you are not alone. You can book your place now at <https://cvent.me/e3AkD>

The Vision Project

Last autumn we launched the biggest stakeholder consultation in our history. The Vision Project, as it was named, gave all involved with the charity the opportunity to tell us what they wanted from Team MPS in the future. The results of the consultation, which informed our new goals that now reflect MPS and related diseases today, will be revealed along with our new three-year plan at the conference, so I urge you all to come along and have your say. At the end of the day it is your charity, we're here for you, and I am very proud to be the CEO of the MPS Society.

Ramblings of a CEO – The challenges facing funding of new and existing treatments

Some of you may be aware that I write a blog called Ramblings of a CEO and share it on the MPS Society website. In my last post I spoke about the challenges facing the rare disease community when talking about the reimbursement of new and existing treatments. NICE/NHS are becoming increasingly hostile towards funding these treatments and some treatments for rare diseases have already been refused on the grounds that they are just too expensive. Yes these are expensive treatments but that is primarily because of the number of patients involved. These treatments however are life changing in many cases and I speak from direct experience. Cost should not be a barrier to living the lives we all want and the MPS Society is working with our partners in the rare disease arena on a strategy to combat this real and present danger.

Finally, I want to remind you that we are a charity and are only able to support individuals and families with the help of generous donations from the public. We need your support now more than ever. Please consider making a regular gift of £5 a month to provide an easy to understand information pack for a newly diagnosed family. You can set up a regular donation in minutes here: www.mppsociety.org.uk/donate

Thank you and best wishes
Bob

“ Last autumn we launched the biggest stakeholder consultation in our history. The Vision Project, as it was named, gave all involved with the charity the opportunity to tell us what they wanted from Team MPS in the future.

News from the Board of Trustees

Board of Trustees

At the September Board, James Garthwaite was appointed Chair of the Trustees.

In December the Board approved a change in the Society's Articles of Association to reduce the representation of Society members on the Board from 75% down to a straight majority. This gives the Board more flexibility to recruit for specific expertise.

There have been several changes of trustees over the last six months. The Board will miss Paul Moody, Tim Summerton, Jessica Kafizas and Daniella Vandeeper, all of whom stepped-down between September and February.

In February we were delighted to welcome Professor Atul Mehta, Dr Gordon Harvey and Philip Pearson to the Board. Each brings valuable expertise: Atul was, until recently, a consultant haematologist at the Royal Free Hospital and is particularly familiar with Fabry; Gordon works in the pharmaceutical industry; Philip is a client servicing director in the IT sector and his son, Archie, has MPSVI.

Vision Project

In December, the Board of Trustees joined the staff team for a strategy day, building upon the findings of the 'Vision Project', the largest consultation with members in our history. It has helped the Society understand what our families, clinicians and other key stakeholders want from Team MPS in the future. It is guiding the development of the Charity's new plan, to be unveiled at the 2019 Conference in June.

Finance

In December the Board also approved the budget for 2019, including an increase in staff numbers and a salary rise for all staff. At the February Board meeting, John Illankovan, presented the year-end accounts for 2018 which reassuringly showed a surplus for the year. The Board paid particular thanks to Bob Stevens for his role in achieving this positive outcome at the same time as navigating the Society through extensive changes.

The surplus in 2018 enabled the Board to allocate funds to invest in research. It also created a Clinical and Scientific Advisory Sub Committee to help

assess research grant applications. We will continue to need your support in order to carry on this great work in 2019.

Staff presentations

The Board had presentations from members of the MPS Commercial team (Jacqueline Adam, Alexandra Morrison, Benedicta Marshall-Andrew and Sarah DeCosta) in September setting out the impressive capabilities of the Society's wholly owned business. In February, Anna Thomas, who joined as Head of Fundraising and Communications in December, shared her perspective on the charity's communications and fundraising activities. Anna has exciting ideas for the future which will form part of the charity's new three year plan.



Please let us know if your contact details have changed. Update them at www.mpsociety.org.uk/keepintouch

WHAT'S ON?

Regional Clinics

Great Ormond Street Hospital

MPSI – 25 June 2019, 10 September 2019, 9 January 2020

MPSIII – 28 May 2019, 9 July 2019, 24 September 2019, 10 December 2019, 26 February 2020

MPSIV – 23 July 2019, 8 October 2019

Birmingham Children's Hospital

MPSI – 17 May 2019

HSCT & MPS I – 21 June 2019

MPSIII – 19 July 2019

MPSIII & LSDs & MSD, MLII – 18 October 2019, 20 December 2019 (am)

MPSIVA – 16 August 2019

MPSVI – 15 November 2019

Royal Manchester Children's Hospital, Willink Unit

POST HSCT 0-10 years – 12 April, 12 July, 11 October 2019

POST HSCT 10 years plus – 26 April, 19 July, 19 October 2019

Regional events

MPS National Conference in memory of Christine Lavery – 28-30 June 2019

Fabry Conference – 27-29 September 2019

Announcements

NEW MEMBERS

Kelly and Andrew Mills have been in contact with the Society. Their four year old daughter, Penny, has a diagnosis of MPS IIIA Sanfilippo Disease. The family live in the Staffordshire area.

Elis has recently been in contact with the MPS Society. He has a diagnosis of Fabry Disease. The family live in London.

Introducing Chrissy Fortune and Joanna Slade who have joined the MPS Commercial team

Hi, I'm Chrissy and I have recently joined MPS Commercial as a Clinical Project Administrator. My role involves organizing patient calls and carrying out questionnaires for those who are part of the Managed Access Agreement for Vimizim, which is a drug for those with MPS IVA, Morquio. When I'm not on the phone to patients I am getting involved with projects that Alex and Jackie need support with.

My previous position was within the construction industry (in the office, not on the tools!) and I am enjoying the shift into working within a new sector with such an important purpose. The whole team here are lovely and have taught me so much.

Outside of work I like spending time in my allotment or getting out for a good run, as I am preparing for an ultra-marathon this summer!



Apologies to Daniella Vandeeper and her family as we published a photo that wasn't actually Talia in the last edition of the magazine. Here is the lovely Talia.



I am Jo and am delighted to have joined MPS Commercial last autumn as their Clinical Trial Administrator. My main role is the reconciliation of the expense claim forms for our many patients attending clinical trials all over the world.

Many years ago I started my career in HR at Rolls Royce aero engines at their base in Bristol and my claim to fame whilst there was to be the first female member of HR staff to stand in a test bed during what is now the engine for the Euro Fighter being fired up for testing, every part of my body was vibrating but it was a truly exhilarating experience.

From Bristol my husband and I then moved up to Lytham St Annes on the Fylde Coast where we lived for 23 years whilst raising our two sons. 'Leafy' Lytham as it is often called is also known for holding the British Golf Open occasionally which was always an exciting event when the town became full of golf enthusiasts from all over the world and the locals kept their eyes peeled for celebs. Once our sons left home we made the decision to relocate down South to be closer to family and for my husband's work and now we have settled very happily in Tring.

For the last seventeen years I have been working part-time in admin for the NHS working in GP surgeries in Lytham and more latterly Berkhamsted where I had been working as a receptionist and also behind the scenes in letter coding from hospital consultants to GPs.

Outside of work I enjoy travelling, theatre, cooking, walking and spending time with my friends and family.



We would like to congratulate Paul and Lucy McKay on the safe arrival of baby Robin, Christine's granddaughter.

We welcomed Hannah Brock to the MPS Team in October 2018 for a week of work experience. Hannah undertook various tasks for each department and even took over Twitter for a day! if you're interested in any volunteer opportunities at MPS Society email us at volunteer@mpssociety.org.uk



Antony Selwood proudly shared photos of his daughter on her wedding day. He writes:

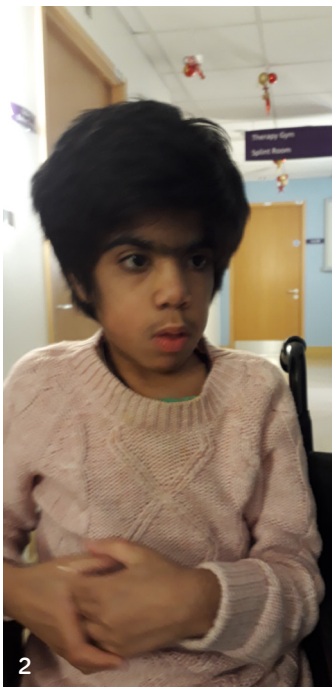
"I had a great August when my princess Emily got married. She married Dominic and he's a lovely lad."

Congratulations from all at the MPS Society.

Clinics

**ROYAL MANCHESTER
CHILDREN'S HOSPITAL
MPS I CLINIC
12 OCTOBER 2018**

(1)

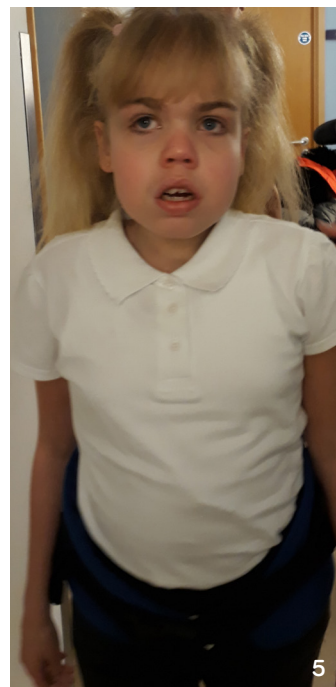
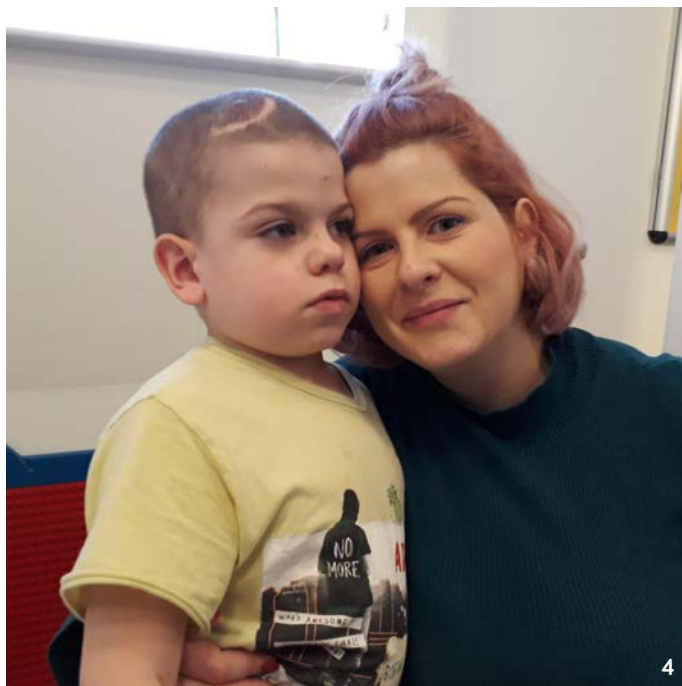


It was lovely to see new and existing members and the team at the Manchester clinic in October.

Louise Cleary, Advocacy Support Officer

**GREAT ORMOND STREET
HOSPITAL
MPS III CLINIC
11 DECEMBER 2018**

(2-5)



This small MPS III clinic was extremely busy, but it was lovely to see everyone

Louise Cleary, Advocacy Support Officer



**BIRMINGHAM
CHILDREN'S
HOSPITAL
FABRY
CLINIC
15 FEBRUARY
2019**
(6-11)



It was a pleasure again to be in the new Waterfall House for the Fabry clinic. It is so light and airy with lots of things to do for the children and young people. It was the traffic that was holding up the normal flow of the clinic so we all had time for a cuppa beforehand. It is always a surprise to see how much the children have grown when you haven't seen them for sometimes up to a year and more. I managed to catch up with everyone and find out how things are with school etc although most of the children were having an extra day off due to the clinic being on a Friday and then into half term the following week, lucky them, not sure about mums and dads... Thanks to all the staff for looking after me as always.
Rebecca Brandon, Advocacy Support Officer



Remembrance



Michael Steven Fitzgerald
“Showstoppa”

He was born on 28 June 1981 at UCL hospital and lived in London. He started at St Dominic’s nursery at four and then moved into the infants at five. He was diagnosed with Hunters at about three after Mark his brother had already been diagnosed at six. After years of attending GOSH for adenoids, grommets, ENT problems, carpal tunnel and growth problems, we moved out of London in 1990 and moved to Bedfordshire. This was difficult for our boys to leave their friends and family. It hit them hard at first but after starting his new school, St Martin de Porres and making other good friends, some life long, the move was a success. We continued with GOSH but had to move to Addenbrooks in Cambridge because they were too old. Michael went on to JFK in Hemel Hempstead and did his GCSEs. He again met some great friends. He moved on to Barnfield College to develop his skills in IT and then moved into work in this field. Michael had a habit of making friends easily and keeping them for life, friends who looked out for him, as he looked out for them. Michael struggled with his Hunters and only grew to 5ft tall, most of his friends would be much, much taller, but this didn’t matter to him. He could hold his own with one and all, with banter, fun and jokes. I said to Michael once when he was about 11 or 12 years old, not to worry when people stared at him, laugh and make a joke of it, advice that stood him well through his life. Both

our sons were smashing kids growing up and became terrific individuals. Despite their frustrating lives, they coped well through the adversity of Hunter syndrome. Michael was very persuasive when his brother got new skates for his eighth birthday – we went to the shop and guess who also came home with new skates (and knee pads, helmet and socks the works!). We couldn’t say no to him. They were good kids.

We were lucky to be able to go on holiday a couple of times a year, going to Disneyland in Florida a couple of times. There was a WCW wrestling show on and we managed to get tickets, the boys loved it. Michael got to see some of his heroes up close, great show and we even went to see one of the Star Wars films in Tampa Bay before it came to the UK. We drove all over Florida and down to the Keys, stayed in a condo for a week in Key Largo, had a great time driving across the state listening to our old cassette of The Mamas and The Papas, Dr Hook, Neil Diamond and more. We also had some memorable holidays at home in this country in holiday camps, very enjoyable.

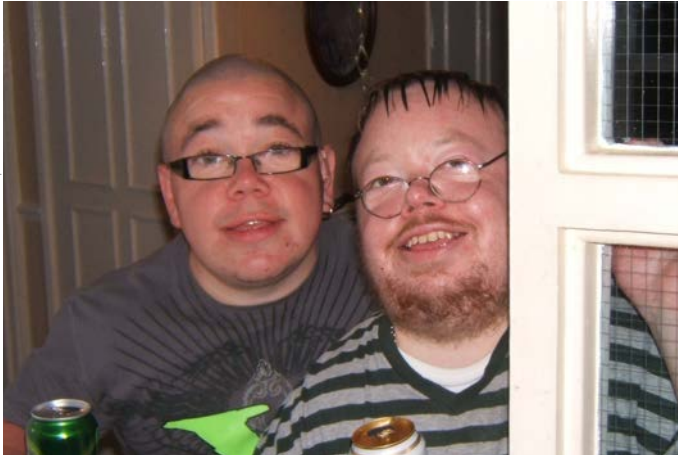
Michael loved his football, and growing up in the eighties, Liverpool was his team. He was a Spurs fan because of his Dad but soon sussed them out and changed to Liverpool, the team of the 80’s. I can still hear him shouting out “Suarez” or recently “Mo Salah”. He was a big fan of Jurgen Klopp, the manager. Football gave him a lot of good times and plenty of banter with his friends. He also loved his wrestling and UFC.

Michael loved his music anything from the 1960’s until today, loved burning his own CD back in the day. Michael worked most of his working life in IT, his last job working at Stoke Mandeville Hospital putting all the old patient records on to computer. He was happy working with computers, until the travelling got too much for him.

Michael loved driving and socialising with his friends, having a bevy or two and social media played a massive part in his life, “doing my correspondence” he would say.

Michael travelled to Thailand with friends in November 2018 only two months before he passed away and had booked another trip for February 2019. His friends went in honour of him.

Michael was a Jekyll and Hyde character because of his illness; he would take it out on his mum but always came back with a smile and cuddles for her. Mum was the real star of the family, her endless visits to the GP, various hospital appointments and for 37 years never missing, through all the ups and downs despite her own adversity. Mum was our champion.



We are proud to say we brought Michael up to be a proper person, funny “He was a dark horse” “Black Beauty”, friendly, loveable. We loved him to pieces. He is now buried with his brother Mark and they will “Never Walk Alone”.

Our son Michael. Mikey the Showstopper.

We would like to thank the MPS Society for all their help over the years, especially the late Christine Lavery and currently all the help received from Rebecca. We are grateful for the support of our family, and overwhelmed by the numbers of Michael's friends who travelled down from Bedfordshire to London for his funeral estimated at over 150, also his friends unable to come to the funeral, also those who travelled over to Addenbrooks hospital in Cambridge. We would also like to thank the medical team at Addenbrooks intensive care unit, and the metabolic unit, Dr Tan, Julie, and Liz, for all their help and support over the years.

Michael and Margaret Fitzgerald

In memory of Susan Clarke

Susan has been a keen supporter of the MPS Society since the birth of her granddaughter nearly 15 years ago. It was her final wish that rather than flowers at her funeral, friends and family contributed to our cause. They have raised £1000 in her memory. Susan was wife to Brian, Mum to Jane and David and devoted Nana to Melissa, Max, Erica and Spike.



Bereavements

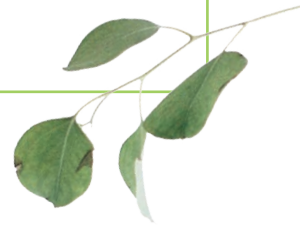
We also wish to extend our deepest sympathies to the family and friends of:

Muqadas Ilyas who had MPS II Hunter and passed away on 29 October 2018 at the age of 11.

Lachlan Picton who had MPS II Hunter and passed away on 9 November 2018 at the age of 8 months.

Michael Fitzgerald who had MPS II Hunter and passed away on 11 January 2019 at the age of 37.

Emma Whiteley who had MPS IIIA Sanfilippo and passed away on 25 February 2019 at the age of 25.





Your stories

This issue we're highlighting the stories of three of our fundraisers



The sibling

Harry Mills has been raising money at his school to help the MPS Society support people like his sister Penny. He organised a mufti day, made his own posters and advertised the event in his school newsletter. We caught up with Harry about his fundraising event and what life is like when your sibling has a rare disease.

What motivated you to fundraise?

Seeing other people do things to fundraise gave me the idea to raise money. I wanted to raise money that could help other children like my sister Penny.

How did you decide what you were going to do?

I spoke to my headteacher about how I could fundraise. She gave me a few ideas, we decided that a mufti day would be the best way and also a good way to tell others people about the MPS society.

Did it take a long time to plan?

My teachers helped me and a made a poster. It took a month to organise. We had our mufti day on the last day of term.

Did it go as you expected?

Yes, it was better. Lots of people donated more than the asked amount.

Have you got anything else planned for the future?

Yes, I'm hoping to organise a charity football match between my school and another one near us.

You clearly care a lot about your sister, what do you think life is like for her?

My sister is happy and excited all the time! She never stops eating, especially cheese. She doesn't know she is poorly which is good, I hope she can stay this happy all the time.



“Seeing other people do things to fundraise gave me the idea to raise money.”



The neighbours

Jason and Sarah Smith have definitely proved they love thy neighbour in all their fundraising efforts for Archie who lives next door and has MPS VI.

In 2018 Jason Smith took part in two long distance bike races to raise money for MPS Society. Jason is no stranger to an active fundraising challenge having completed ultra-marathons and the London to Paris cycle ride in the past. This time Jason's challenge was also inspired by a cycling hero of his:

“In 2018 I'm going to #BeMoreMike as in March 2017 we lost a cycling legend in Mike Hall. He inspired others to get out and ride. Mike's death has had a fundamental effect on many lives but it has sent a message across the world to go out and do that bike ride.”

Jason not only took on, organised and trained for his epic bike ride, he also promoted it widely to raise awareness and boost his fundraising, even appearing on the radio with Archie's dad, Phil.

“Archie is my reason to fundraise for the MPS. He's an active, football crazy, fun loving boy who has lived with MPS for over 10 years. Despite Archie's battles with MPS he's overcome such adversity to live his life by amazing us all in so many ways...The MPS has provided vital life-long weekly treatment for Archie and family support for over a decade. The MPS needs all the help it can to continue the life prolonging treatments that are so vital to the many MPS sufferers and families.”

Sarah took a more social approach to her fundraising and organised “Archie's Charity Night”. The event included magic, music and a visit from Barricade the Transformer.

The community

The Young Farmer's Club in Diss, Norfolk were amazing in supporting the Society as their charity of the year in 2018. This group of young people have come together to support a member of their community and it sounds like they had a great time doing it!

MPS Society Conference
28–30 June 2019

Book now at:
cvent.me/e3AkD



The Diss Young Farmer's Club (YFC) chose the MPS Society as their charity of the year as we support their ex-chairman's family. This community group really pulled the stops out over the year to raise an incredible £9083.20.

Their fundraising efforts included a jumble sale, Christmas carol singing and, at the club's 75th anniversary ball in June, an auction and game of Heads or Tails. The event at the Norfolk Showground attracted 660 past and present club members, and helped raise the profile of the work of the MPS Society.

Diss YFC press officer Edward Saunders said: "The rare diseases are progressive and life limiting, and with an ex-chairman's family currently in receipt of support from the organisation, it was an obvious choice for the club to endorse the charity."

They presented this impressive sized cheque to Bob Stevens who has visited the Diss YFC meetings and attended the annual ball. Bob said "for a community group to do so much for one of their own is heart warming. The Society are incredibly grateful."

“ It was an obvious choice for the club to endorse the charity



Interested in supporting the MPS Society as your Charity of the Year? Get in touch with Anna at fundraising@mpssociety.org.uk to see how we can work together.

Events

Over the wall

A family weekend organised for members with Fabry disease and their families thanks to sponsorship from Shire.

Harding family

We arrived at the activity centre at teatime and were quickly introduced to Kath, our Over The Wall volunteer,

who was just lovely. We had some food and then had the opportunity to go to our room and unpack. The rooms were lovely and the beds were really comfortable.

All the parents went off for a meeting, whilst the youngsters were able to socialise in the common area. Alfie and George especially liked the air hockey, table tennis and Jenga.

We had an early start on the Saturday, the cooked breakfast was popular all round and set us up for all the activities to follow. There were opportunities to do art, drama, music and a treasure hunt which was brilliant fun. In the afternoon with the sun shining we all ventured outside. We were all brave and gave the zip wire a go, which turned out to be not as scary as it looked although it was very high. Football and archery was really popular with Alfie and George loved the abseiling and rock climbing.

In the evening there was a disco and some of the volunteers took on roles to entertain, such as a fortune teller and magician. This was a huge hit with the adults and kids. Board games were also very popular, Alfie loved playing Cluedo with his new friends and volunteers.

Sunday we packed up ready to head home at lunchtime. But not before we all came together to see photos of all the wonderful things we had done over the weekend.

We all had an amazing time thanks to Over The Wall. It was great seeing the kids making new friends in a relaxed environment. The volunteers and staff were all brilliant, we had so much fun. We were very thankful that we had the opportunity to go and feel privileged to have been invited. If other families get the opportunity to go our advice would be, definitely go!



Matthews family

I have to say the biggest thank you to the MPS Society for organising a weekend away with others who have Fabry disease as part of their family.

We went away in the early autumn, many of us slightly nervous at what the adventure camp would entail but encouraged that we were promised we wouldn't be sleeping in tents!

My husband Simon couldn't be with us, as he was poorly that weekend with a chest infection and we arrived late on the Friday evening, already quite exhausted and feeling slightly lost that we weren't together as a family as hoped.

On arrival, we were given water bottles with our names on, two volunteers to take care of us, play and chat with us throughout our stay and the warmest welcome from the team.

We bonded as a family, as a group of people who experience Fabry in a variety of ways and were so touched by the experience. We zip wired, we painted t-shirts, completed a treasure hunt, played charades, danced at a disco, ate lots of great food and felt encouraged by what we were able to do and laughed lots.

On the last day, we made memory boxes of our time together and my two children, Charlotte and James, still hold this and the whole experience dear in their hearts.

I came home and cried. I cried because it was so wonderful and I was so overwhelmed by the generosity of both the MPS Society and the volunteers at Over the Wall. I felt I didn't deserve it...but the truth is it's hard to have Fabry disease within your family. It's an invisible illness and nobody truly understands how it feels to live with it and the challenges it brings unless you have it... but this weekend provided us with the space to talk and share with others who have the condition and we left feeling not quite so alone.



“ We had the most amazing day! I was overwhelmed with the beauty and love of so many special people that we share this crazy cruel life with.





Lapland UK

This year Christmas has been extra magical for our family. We received some very special invitations from Father Christmas himself, asking if Seren and Jacob would like to come and visit him at his secret toy factory deep in the forest to help the elves with their work. Of course their little faces lit up with excitement. We all went to bed excited that night ready for an early start the following morning.

We arrived at Lapland UK with two very excited children, and I think we were just as excited as they were! The children were greeted by a very friendly elf on arrival, whilst we checked in and were given our 'team husky' stickers. The children were each given an elf passport which they needed to enter Lapland. We then went through a magical walk way into the elven forest where we were met by two charming elven folk who told us all about the magical forest. Soon we were being led through the door into snow covered Lapland.

Our first stop was the toy factory where we were introduced to the chief toy maker and the mischievous elf conker. The children all made a soft penguin toy, ready to go on Father Christmas's sleigh to be delivered on Christmas Eve. Conker stamped their elf passports for helping make the toys. After that, we went to visit Mother Christmas and her elves in her kitchen. The children loved decorating their own delicious gingerbread biscuits, and enjoyed sitting down to a story told by Mother Christmas. Once again the passports were stamped. We then went to explore the elf village. It was everything you'd expect an elf village to be and more! We enjoyed giving ice skating a go, the children were very good and skated rings around me! We stopped off at the elf post office to get our final elf passport stamp of the day, and we had a look around the bauble shop which was full of beautiful hand painted Christmas baubles. The children were very excited to visit the elf emporium where we purchased two penguins just like the ones the children had made at the toy factory. We were all in sweet heaven when we visited Pixie Mixie's sweet shop! We saw some beautiful husky dogs and stopped to say hello to them, the children loved stroking them and finding out about them from their handlers. There were lots of interesting characters wandering around the village, we had a chat with a very tall elf!

Finally it was time to see the man himself. We were led down a very enchanting snow covered trail, with lots of charming little wooden elf houses to admire along the way. We could even hear some elves whistling away in their houses. At the end of the trail we were greeted by Father Christmas's reindeers! The children were beyond excited at this point, and after looking at the sleigh, we led down to a very magical looking log cabin in the forest. We went inside and were greeted by jolly Father Christmas himself. He had a lovely chat with the children about all the things they have achieved this year, and he was very happy to tell them they were on the good list! They were told they were now honorary elves and were given bells to put on their hats. Father Christmas gave them an early Christmas present, a beautiful husky soft toy each. We posed for a family photo which we will cherish. We had a wonderful day and made so many happy memories. It was lovely to share it with other MPS families. A diagnosis like Hurler Syndrome can often be a lonely path to walk as it's so rare. Meeting up with other families and their children is very special and helps you to feel less alone. Thank you to all of the wonderful staff at the MPS society for organising such a magical day out!

Seren and family



ANOTHER SURVEY?

What happens to the information you give in a survey? Why your input is so important.

So you have been contacted again and asked to take part in a survey. Unless you are part of the camp that enjoys a good questionnaire (pointing the finger at our MPS Commercial Research and Communications team), it might not be such an attractive prospect to take up your precious time ticking yet more boxes.

What is the purpose of it? The fact is that the information you provide us on your experiences is very important. Once you've finished your survey and submitted it our Research and Communications team get started. You'll see below just how much can be done with your help.

As an example let's look at the MPS III survey we conducted in 2017. MPS Commercial would like to extend thanks again to the families from 13 countries around the world who participated and shared their stories on diagnosis and how MPS III affects them.

Here is the latest update on how your information is being used and the difference it can make.

The international MPS III survey

Your information is helping us to:

Support the development of new treatments



All the results were written up and shared with the pharmaceutical companies who sponsored the study. They are using the information you supplied to:

- Understand more about the disease from your perspective
- Understand why diagnosis can take several years and support initiatives for earlier diagnosis
- Help them to describe the 'burden of illness.' This key piece of information is used when:

- Seeking approval for a new treatment from the European Medicines Agency
- Submitting evidence to NICE for the treatment to be made available on the NHS

Burden of illness is a measure of the impact of disease on the individual and the economic costs of care and treatment

Increase awareness in the medical and research community



Your information made it into the poster session at the WORLD symposium in the USA. The event is the largest of its kind and takes place every February. It is a key date in

the diary for researchers, pharmaceutical companies and patient organisations working on MPS and other lysosomal storage disorders.

As a result of you taking the time to reply to our survey, we were able to create two posters using the information gathered and present them to a wide audience of people. These are people who care about the future care of those with MPS III. One poster focussed on the journey from first symptoms to obtaining a diagnosis. The second described the burden of illness. Both generated a lot of interest and discussion. Plans are underway to publish the studies in full in a scientific journal.

Support the work of the MPS Society

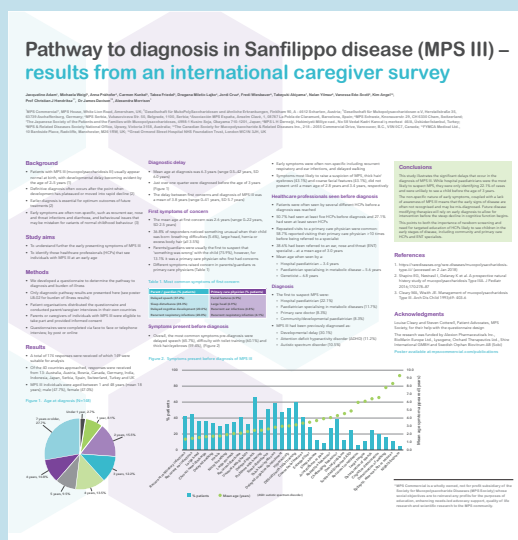


Your information can be used in a wealth of positive ways. We are also looking at ways to use the information in-house to support the disease awareness campaigns of the MPS Society.

Presenting posters at WORLD

Our posters were presented alongside hundreds of others as part of the poster session, where everyone attending the conference can read and take photographs of them.

There was also a special session where we spoke about the study with doctors and researchers and answered any questions.



Research & treatment

The Vimizim Managed Access Agreement three years on

It's now been three years since Vimizim (elosulfase alfa) was made available in England through the Managed Access Agreement (MAA).

Vimizim is the first available enzyme replacement therapy for MPS IVA. It has been available in England since December 2015 under a Managed Access Agreement.

This ground breaking agreement was the result of a campaign for access to treatment by the MPS Society and a collaboration between us, NICE, NHS England, BioMarin and the medical community. It provides a workable solution, making treatment available while more information is collected on Vimizim long-term benefits.

At the three year point, more than sixty adults and children with MPS IVA in England are receiving Vimizim treatment and those living in Wales and Northern Ireland are receiving treatment under a similar arrangement. Access in Scotland is determined on a case by case basis.

Those on treatment in England have to attend their clinic twice a year for tests. They also have to complete questionnaires with our MPS Commercial team that track the effect of treatment on quality of life. All this information is then combined to assess how well the treatment is working.

Those on the MAA will continue to stay on treatment until December 2020 if their tests show that Vimizim is benefitting them. Whether or not treatment will be available after December 2020 will depend on the decision made by NICE. We are currently engaging with all those involved in the development and running of the MAA to support this final review.

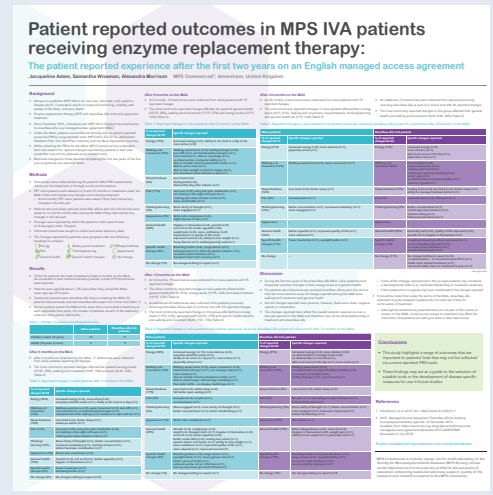
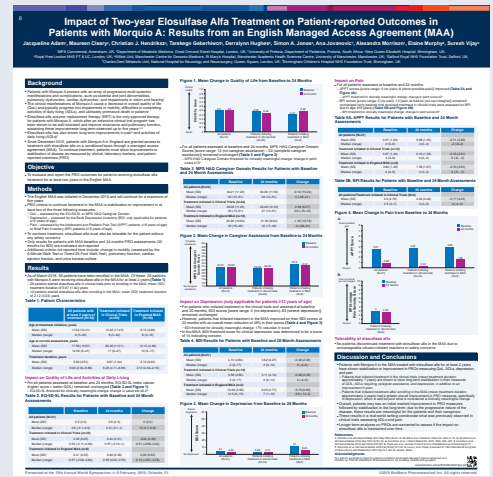


What do the results look like so far?

Results presented at the WORLD symposium in February showed that most measures of quality of life have remained stable during the MAA, even in those who have been on treatment for many years (those who took part in clinical trials have now been on treatment for an average of seven years). They also showed that children were experiencing less pain.

MPS Commercial have also been collecting testimonies to capture the benefits that those on treatment report. These results were also presented at WORLD. The most common changes you tell us about are:

- An increase in energy levels and stamina that mean you can be more active
- Less pain
- Feeling less tired or sleeping better

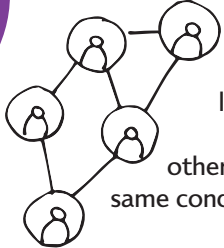


Thank you to all MAA Vimizim patients

All patients currently part of the MAA will be used to hearing from the MPS Commercial team on a frequent basis in order to carry out questionnaires relating to treatment. We'd like to thank you all for your continued co-operation over the last three years. If you would like to see the full posters presented at WORLD you can view them on the MPS Commercial website www.mpscommercial.com/publications

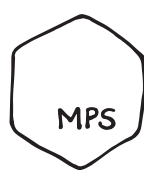


We have been asking a series of questions over social media to get an insight from you, the experts, as to the best approach to the unique situations you find yourselves in. If you have a question you want to ask the experts let us know at magazine@mpssociety.org.uk



We would have liked to have been able to contact other families with the same condition for support.

What did you find most useful on diagnosis?



out that there was a rare disease society who put me in touch with other MPS families and assisted with access to treatment.



One thing Debbie told me on the phone that meant so much to me: 'Remember that he's still the same boy that he was yesterday.' Very wise words and very comforting.

A full explanation including life expectancy which I asked about. I appreciated that honesty. A clear plan to see other specialists and an early follow up appointment to enable me to ask everything I didn't think to at the time!



Honesty was important. I didn't want things to be sugar-coated, but definitely to be delivered with compassion and understanding.



We are always here from that first diagnosis and throughout your journey. Call the advocacy support team on 0345 389 9901 for any support you need.

Information & resources

Accessible holidays

After our last *Ask the experts* article in the autumn 2018 issue of the magazine, **Sophie Dowsett** researched and found a selection of websites to help you have a happy holiday no matter what your ability

Are you the adventurous type?

Seable holiday offers accessible active holidays for the visually impaired and wheelchair users.

www.seable.co.uk

For the festival lovers

Festival Spirit promises to make dreams a reality offering festival experiences for life-limited and disabled people.

www.festivalspirit.org

City break your thing?

Disability Horizons, an online disability lifestyle publication, reviews the top five most accessible cities for wheelchair users.

www.disabilityhorizons.com/top-five-accessible-cities-for-wheelchair-users

Or are you a beach bum?

Again from Disability Horizons, a round up of the best accessible beaches in the UK and where to hire beach wheelchairs.

www.disabilityhorizons.com/best-accessible-beaches-and-beach-wheelchairs-in-the-uk

Need a helping hand?

3H helping hands for holidays provide subsidised group holidays for the physically disabled at well researched venues – giving their carers a valuable period of respite at the same time. They also run and programme offering grants to assist disabled people and their families on low income to organise a UK holiday for themselves.

www.3hfund.org.uk

Don't forget to...

Get the app...

Plan your holiday with the TripTripHurray app - a travel platform for people with specific needs that lets you quickly and easily search for accommodation, public transport, places of interest, shops, restaurants and services. It's effectively a personalised trip adviser whether you're travelling with a baby, dog, in a wheelchair or have special dietary needs.

Get sharing socially...

Use Its Accessible app to help you find and share accessible hot spots, including bars, restaurants, hotels and car parks. Led by member reviews it offers a range of places that are accessible to people with mobility difficulties.

Learn the lingo...

Lonely Planet have put together a free Accessible Travel Phrasebook which gathers together some disability-specific words and phrases translated into as many different languages as they possibly could.

www.shop.lonelyplanet.com/products/accessible-travel-phrasebook-ebook-1



Fundraising

Thank you to everyone who has collected money, sent in a donation or carried out fundraising for the MPS Society. We really appreciate what you do. We can't feature everyone in the magazine so we have set up a thank you page on our website to celebrate the wonderful fundraisers and donors we have. Visit mpssociety.org.uk/thankyou to read about all the wonderful people who have helped to support the MPS Society and individuals and families with MPS, Fabry and related diseases in the UK.

We still love hearing from you and if you want to send us your story and a photo we will always try to feature you. Send any stories and information about your fundraising to fundraising@mpssociety.org.uk



Jurassic Coast Challenge

"I am very pleased to let everyone know that I am alive and well since completing my very long Jurassic Coast Challenge for charity on 21–22 July 2018. I walked 100 km from Poole Harbour to Bridport to raise money for the Society for Mucopolysaccharide Diseases.

I ranked 45th out of 318 women finishing the challenge in 23 hours 56 minutes (my personal cut off time was 25 hours) and ranked overall 133rd out of 619 participants.

Needless to say, with sore toes, the challenge is not for the faint hearted, but certainly one worth remembering!

I just also wanted to say a huge thank you to those that have supported me. Your contributions are very generous, and I am very thankful."

Love and Light, Mystique

I completed the race in 1.29 hours my fastest time yet. It was also the hardest one as well. I completed it with my cousin Terrie Ann Brown and friend Andy Heazleton who all were running for the MPS Society. The weather was beautiful and unusually warm. (I'm not going to say it was too warm!!!). A great day and such a good feeling that money was raised for the MPS Society.

Lee Shepherd, Great South Run

Great South Run £1,177.49 raised with 5 runners

The Great South Run takes place in Portsmouth the south coast's best 10 mile course through the streets of Portsmouth, passing landmarks such as HMS Victory and the Spinnaker Tower. The course is almost flat, with minimal slopes around the course, which makes fast times achievable.

Get your GSR place for 2019 on 20th Oct: bit.ly/MPSGreatSouthRun

The fundraiser's fundraiser

Jocelyn Keates has been settling into the role of Fundraising Administrator at MPS Society and, as part of the job, is supporting those fundraisers who are "doing their own thing". She has been so impressed with everyone's efforts and wanted to say a particular thank you to Steve Rendell who has been super busy fundraising and raising awareness for the charity.

"I wanted to give an extra special shout out to Steve Rendell who took part in The London Landmarks Half marathon on Sunday 24th of March all in aid of Harvey Brown who has Morquio and the MPS Society. Steve has been relentless in his efforts to raise funds for us including; bucket collections at his beloved Millwall Football ground (where Harvey is a regular mascot), donated football items that he raffled, friends who did collections at local pubs and those who donated on his Just Giving Page. Steve has also been spreading the word with his story in local newspapers and interviews. He has surpassed his expectations and target for this fundraiser and hit £6k which is ever increasing! Thank you Steve from all of us!"

See Steve and Harvey on the day of the run here: bit.ly/SteveHarveyClip

Millie Stuart organised a birthday fundraiser on Facebook donating to the MPS Society in memory of her brother Jack.

"As everyone knows this Society means the world to me and my family and of course my very special brother"

Thank you to mum, Amanda, who let us know what Millie had organised.

To start our own birthday fundraiser visit www.facebook.com/mpssociety



Photos courtesy of Haddenham.net

Haddenham Mummies

Massive thanks to the Haddenham Mummies who raised £2,385 in their local community during their traditional mummies' plays put on at pubs and village events in the run up to Christmas. They chose eight good causes and the MPS Society was fortunate to receive a cheque for £480 from this jolly crew.



Great North Run £4,122.06 raised with 10 runners

Starting at the centre of Newcastle, our runners crossed the iconic Tyne Bridge before finishing in the coastal town of South Shields.

"We all did really well Mark and I finished in 2.30 hours and the others in 2.36 hours, which was OK considering the injuries we had. We held a charity race night on the 14th December which would have been Jake's 18th birthday. We are all deliberating whether we run again next year but I'm sure we will be"

Mark and Debbie Burniston (who ran with Michael Riley and Michael Hair who also supported MPS)

"On the day I met Juliette as we were walking to the start of the marathon, I have now linked up with her via a Facebook run group which is great, I'm going to see if I can persuade her to do the great south with me! I saw two

ladies during the run, one who had an older style vest and was running for Jake but I didn't catch her name and Kate who was running for her brother who had sanfillipo, amazing amongst the thousands to meet a lot of the other runners running for MPS."

Terrie Shepherd

"I've run the Great North Run for the second time this year on behalf of the MPS Society. The training was tough but after entering other events like the York 10k, Darlington 10k and Lindley 10k, just by wearing the vest, lots of people were asking about the charity and how people are affected by MPS diseases. The GNR had a fantastic atmosphere, with the red arrow planes passing above us at the start and finish. I saw fellow MPS runners in passing and

the crowds were so supportive. The steel pan bands and other music really kept me motivated. What a great day! I completed the race minutes faster this year, 2.14 hours, and I'd be happy to do it again for the MPS Society next year."

Katie Playe

"We had an amazing day. We managed to finish in 2.43 hours. We were of course very proud of ourselves, however especially proud to be running and raising money for MPS. Samantha has a barbers salon and has been raising money through a sponsor form. She will add the money to our just giving page. We would like to thank you for allowing us to run and raise money for this amazing charity."

Kathrine and Samantha x



Get your GNR place for 2019 on
8th Sep: bit.ly/MPSGreatNorthRun



Get involved

MPS Awareness Week 13–19 May 2019

Awareness information stand

Hold an information stand on any day of Awareness Week in your local community, school or work place.

Wear it Blue

On 15 May Wear it Blue in school, your work place or local community. Simply wear a blue t-shirt or why not paint your face? What you do is totally up to you! Combine it with a blue bake sale to raise even more funds!

Local bucket collections

We need volunteers to raise funds in your local area. This could be bucket collecting outside a local shopping centre or supermarket. We can help you with permissions.

Shout about it!

Share our social media posts with your network. Register today for your social media toolkit with advice on how to do this and look out for our posts @mpssociety and @mpssocietyuk

Thank you in advance for every pound you raise and every post you share!



We want everyone to know about the signs and symptoms of MPS and related diseases. Early diagnosis could mean better treatment or may even save a life. Help us make a difference this Awareness Week and register for your free pack here: www.mpssociety.org.uk/mps-awareness-week

For more information
get in touch
fundraising@mpssociety.org.uk
0345 389 9901



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