RESEARCH | AWARENESS | SUPPORT

MPS Society
transforming lives through
support, research & awareness

Impact Report 2023

FROM THE GROUP CHIEF EXECUTIVE

Bob Stevens
Group Chief Executive

ON AVERAGE
1352
PEOPLE VISITED OUR WEBSITE EACH MONTH

2677
PEOPLE SUBSCRIBED TO OUR MONTHLY EMAIL NEWSLETTER

We have spent 2023 readying ourselves for the future. We launched our strategic plan for the next three years and chose the priorities of improving the newborn screening process for rare diseases and increasing mental health support for our community.

Our hard work in advocating for access to new treatments for our community resulted in a number of highly-specialised treatments being recommended for use. This included a treatment for infantile lysosomal acid lipase deficiency (LAL D), an enzyme replacement therapy for Fabry disease and life-changing medication for the treatment of alpha mannosidosis.

Advocating and supporting our families will always be the cornerstone of the MPS Society and even in such a challenging financial climate our Advocacy Team have continued to support, advise and walk alongside our families, helping them to try and live the best lives they can.

Our face-to-face event schedule continued and alongside our usual fun family trips, we introduced a new wellbeing retreat for parents and carers and a special meet up for adults living with an MPS or related condition. These received great feedback, and we hope to do them again thanks to the brilliant sum raised during the Big Give Christmas Challenge.

This year, like many others, our influence and support has been felt globally. I went on a rare disease road trip, visiting colleagues and stakeholders in Canada and the US and advocating for our rare community. I was invited to speak at the Canadian MPS Society Conference and met and discussed therapies with pharma companies. We also continue to advocate for those in areas of the world who are not represented by their own patient organisation to gain access to novel treatments and therapies through compassionate usage programs.

It is always a privilege to spend my time fighting for our families both in the UK and around the globe and I came back inspired knowing that the MPS Society is changing the world of rare diseases one conversation at a time.

Warmest wishes,
Bob

Support Hub launched

Feedback from our membership showed a desire for more opportunities to link in with each other. We responded by setting up the Facebook Support Hub. This is an area where all members of the MPS Society UK can join and ask questions, share their thoughts and receive peer support. It's mediated by the Support and Advocacy Team so the discussion stays on topic and is helpful, but it is a place for people with MPS, Fabry and related conditions to share information and thoughts and feelings.

"Thank you for all the support you provide."

MPS SOCIETY MEMBER

THIS YEAR WE HAVE SUPPORTED:

61 NEW MEMBERS

27 DISEASE TYPES

350 IN-DEPTH CASES

SUPPORT IN 2023

Building a community

4183
MEMBERS CONTACTED THROUGHOUT 2023

We now support over 1800 families from diagnosis, through living with the condition, dealing with key life transitions and milestones, to bereavement and beyond. We have found more innovative ways to respond to the needs of our community, like setting up the Support Hub on Facebook which allows easier peer-to-peer communication. We also worked on new events in 2023, including a weekend for adults and young people with a condition.

In our membership survey you told us that more face-to-face events was important to you and that having a dedicated support officer was the most important service we offer. This is important to us too, and we will make sure we can continue to provide these services. We can't do this without your fundraising efforts, so thank you to everyone who has run a challenge, shook a bucket or dug deep this year.

30
AWARENESS TALKS AND TRAINING DAYS

186
CASES REFERRED TO RECEIVE MENTAL HEALTH SUPPORT

Angela's story

I became a member of the MPS Society on 14 October 1991. I rang them after we received the devastating news that our eldest son David had just been diagnosed with MPS III. Learning that your son has an illness that is going to rob him of everything from speech to his ability to walk, alongside extraordinary anti-social behaviour associated with MPS III and then, after all that, it would take his life probably before he reached adulthood, was devastating.

I feared that nothing could ever be normal again. However, the MPS Society introduced me to some wonderful people in very similar situations.

Nowadays, life has changed beyond all recognition. Our son's fight with MPS III is long over and all we have are memories. Nonetheless, the MPS Society is still there for us.

"Together, we learned to laugh again, became friends, shared information and gave each other hints and tips on how to cope with the different issues that MPS could bring."

Support events

It is our aim to provide MPS Society members and their families with appropriate and accessible face-to-face events which are fun but also offer the opportunity to meet, share experiences and create lifelong friendships. They also give our Advocacy & Support Team the chance to engage with members in a more casual setting.

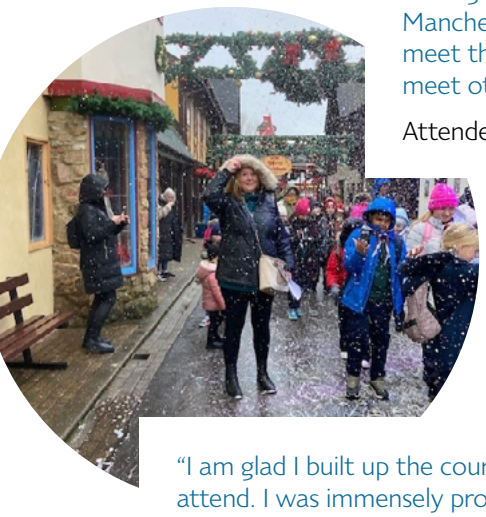
Throughout 2023, we organised numerous events for our families with locations across the UK, including a wellbeing retreat and independent adult weekend – both held for the first time, as well as fun days in Drayton Manor Park in the summer and Gulliver's Land at Christmas; plus, the annual Weekend of Hope and Remembrance. All received overwhelmingly positive feedback, stressing the importance of meeting other families and the significant impact peer-to-peer support has on them.

"I was able to reach a much calmer, mindful state than when doing the online groups. This was such a lovely experience for me. As ever, a huge part of the benefit came from meeting with other parents and having long, in-depth conversations that just aren't possible online."

Attendee, Wellbeing retreat for the parents and carers

"I just wanted to say a huge thank you for organising the adult MPS weekend in Manchester. It was lovely to see old faces, meet those I'd only spoken to online, and meet others I'd never met before!"

Attendee, Independent Adult Weekend



Thank you to our generous funders for their support towards our Family Events over 2023 including: The Gosling Foundation, The Geoff and Fiona Squire Foundation and The National Lottery Community Fund.

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FACE-TO-FACE SUPPORT EVENTS HELD

"I am glad I built up the courage to attend. I was immensely proud of returning to the childhood wood which bought back so many good memories despite the sadness. It was a really special time for me, where I was able to catch up with very special friends and to also pay thanks to all of you for doing so well with the charity and the families. I know for sure that my mum would be and is very proud of you. It will be my commitment to return on a yearly basis. I cannot put into words how positive and effective the weekend was for my own mental health."

Attendee, Weekend of Hope and Remembrance

"This was our first MPS event and it was lovely to meet everyone! We will definitely be keeping in touch with the families we met and will look forward to future events".

Attendee, Drayton Manor Park

Hope for the future

Sophie Thomas, Senior Head of Patient Services and Clinical Liaisons

Fiona Stewart, Chair of the Clinical Scientific Advisory Committee

Highly-specialised treatments

As part of her role, our Senior Head of Patient Services and Clinical Liaisons works alongside other patient organisations and pharmaceutical companies to make sure highly-specialised treatments that are trialled successfully make it through the rigorous process NICE (The National Institute for Health and Care Excellence) and NHS England need to recommend these treatments for use with our conditions.

In the past year we have successfully campaigned for the following treatments.

Vestronidase alfa (Mepsevii®) for MPS VII was approved for routine commissioning for infants in November 2022. This is the first ERT approved outside of NICE (due to small patient numbers in the UK this was a separate process through NHS England) and is intended to be a bridging therapy for neonates and infants before Haematopoietic stem cell transplantation (HSCT).

Velmanase alfa (Lamzede®) to treat people with alpha mannosidosis who commence treatment under the age of 18 years. NICE approved the treatment in November 2023 after a five-year review process.

Sebelipase alfa (Kanuma®) was recommended – after nearly eight years in review – as an option for long-term enzyme replacement therapy for people with infantile lysosomal acid lipase deficiency (LAL D) if they are two years or younger when treatment starts in November 2023.

Pegunigalsidase alfa (Elfabrio®) for Fabry adults age 18 years and over was recommended as a treatment option in September 2023. In November 2023 the Scottish Medicines Consortium (SMC) released their decision not to recommend pegunigalsidase alfa (Elfabrio®) for the long-term treatment of adult patients with a confirmed diagnosis of Fabry. We remain committed to working with all parties to ensure that our members in Scotland have equitable access to therapies.

£355,956

AWARDED IN RESEARCH GRANTS SINCE CSAC LAUNCHED

Clinical Scientific Advisory Committee

Well, it has been a very exciting and busy time for CSAC. We were thrilled to have Professor Derralynn Hughes and Professor Simon Heales join Professor Bryan Winchester, Sophie Thomas and myself on CSAC, and more recently, Dr Simon Jones has also agreed to join our committee.

We have been assessing grant applications and making some awards. We were also delighted to see that some of the projects that had been delayed by Covid are now up and running.

It was great to hear that Professor Uma Ramaswami's work, which we helped fund, was accepted as a poster at WORLD 2024 and we were very proud to hear that one of our awardees won a prize at the British Inherited Metabolic Disease Group meeting for best presentation of papers presented.

This year also saw the first award of a summer studentship funded by the Christine Lavery Memorial Fund to mark 40 years of the MPS Society, to commemorate Christine's dedication to research, and to hopefully inspire interest from the up-and-coming generation.

On October 10th, we held our first virtual CSAC open day. We felt it was very important that our members and other interested parties were able to see who the members of CSAC are and to gain an idea of what our function is. I hope everyone was interested to see how much money had been awarded to support important work over the years, including the appointment of a young Dr Ed Wraith to his first consultant post!

CSAC is ready to work hard in 2024 as we open for applications again and will hopefully be able to help fund some more excellent projects.

These are exciting but challenging times, and CSAC will aim to continue to provide both advice and support to the MPS Society and RDRP.

AWARENESS IN 2023

Spread the word

12
POSTS IN OUR NEW
SPOTLIGHT ON RARE CAMPAIGN

Fabry Awareness Month

"32, young, fun, living life and boom hit by what I can only describe as a tidal wave of fatigue and body pain that came from nowhere."

In 2023, Claire O'Meara was the face of our Fabry Awareness campaign and shared her story about finding out about her diagnosis and how she advocates for Fabry patients.

"I am right now in a good place, I feel back in control and finding my feet again on living, I have learnt what living with a rare disease means and that's doing things on my terms, advocating for myself and for others with Fabry disease, I especially bang the drum for females living with Fabry."

"The boy in the blue is just like you, And every other child and grown-up too."

MPS Awareness Week

We joined the International MPS Network to #chasethesigns of MPS and related conditions, highlighting key symptoms that, when spotted together, can help with early diagnosis. This campaign was shared over our social channels alongside our usual Wear it Blue appeal and a new MPS and mental health awareness project from Kevin Mullins called *The Boy in the Blue*. The project shared a poem, written by Kevin, which highlights the strength and resilience his step-son Ethan shows when managing his condition and people were encouraged to explore the themes from the poem using their preferred art form to bring it to life.

57K TOTAL IMPRESSIONS

550 WEBSITE VISITS

€12K RAISED BY YOU

HOW WE DO THIS

Thanks to you

In 2023 we recieved over

£23,000 from regular donations and payroll giving schemes

£9,000 from donations and tributes in memory of loved ones

£3,000 in one off donations

£133,228 from legacy donations

Thank you to our amazing community fundraisers who stun us every year with their imagination and effort.

Marathon marvels

Nine people took on the London Marathon for us and raised more than £31k. Thanks to Dave, Dale, Juan, David, Kevin, Stephen, Michael, Leanne and Caroline for their efforts.

Emma Venables carried out a gruelling 26 hour marathon of fitness and raised more than £9k on behalf of her friend's son, Freddie.

Fitties, Andrew and Keith, ran an astonishing 100km ultra and raised over £4k in the process.

Dan Lee trekked to Mount Everest base camp alongside a group of Co-op colleagues raising a fantastic £5k.

Groups of greatness

Cheshire Young Farmers chose us as their charity of the year and put on some amazing events throughout the year raising over £10k.

Enormous thank you to 5 Yard Rush and Murf who chose us as their charity of the year in 2023 and raised a fantastic £5,277 plus £726 in Gift Aid.

Two amazing charity fundraising balls were held this year. A huge thanks to the Scott family and the Brown family for spending so much time and effort on these gorgeous, glamorous and glitzy galas!

Conclusion of an epic challenge

The incredible Steven Gill concluded his epic challenge after two years. Steven and his friends and family set out to complete 59 events in honour of his dad who had Fabry and passed away in 2021. The #59events epic raised an impressive £18,003.

Big Give

During our Big Give Christmas Challenge you raised a fantastic £27,264.01 including Gift Aid and offline donations which will be used to help fund support events in 2024.

Facebook fundraising

Our social media challenges, Step into Spring and 60 Sit Ups in September raised a super £6k.

We want to say a huge thank you to Elizabeth Heath who took the bold decision to shave her head in 2014, then did it again in 2023.

"I wanted to try and get over £10,000 on the tenth anniversary of Jack's passing," says Elizabeth.

Together with the help of family, friends and colleagues she hit the £10k target in memory of her son Jack who sadly passed away in 2013.

Rare Disease Research Partners

Rare Disease Research Partners provide support research and access to treatment for people living with rare conditions. As a wholly-owned subsidiary of the MPS Society they invest any profit into the charity to help towards supporting our community.

In 2023, they attended important events and conferences, showcasing their research findings through poster presentations and publishing manuscripts and reports to further the knowledge and understanding of rare conditions. They helped over 250 people to participate in clinical trials and chaired focus groups on behalf of pharmaceutical clients.

They also won the coveted Chair's award in the BOBI awards 2023 for best approach to rare diseases.

£40,387

GIFTED TO THE MPS SOCIETY

Together we can transform lives

Thank you to those who shared their photos and stories for this impact report. Please keep telling us about your life with MPS, Fabry or a related lysosomal disease and your fundraising events. We try to share as many stories as possible on our website and we'd love to hear yours.

Help us to achieve our future plans and keep supporting everyone affected by MPS, Fabry and related lysosomal diseases through a regular gift, taking part in one of our many challenge events, getting your company involved or volunteering your time.

Contact us for more information:
fundraising@mpssociety.org.uk
mpssociety.org.uk/donate

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Who we are and what we do

The MPS Society is a charity committed to transforming lives through specialist knowledge, support and advocacy and research. **Our vision** is that people affected by our diseases live the lives they want. To make sure this happens we are working to ensure all our community have access to:

- exceptional support and advice
- world-class clinical care
- effective treatments