SUPPORT | RESEARCH | AWARENESS

MPS Society
transforming lives through
support, research & awareness

Impact Report
2024

FROM THE GROUP CHIEF EXECUTIVE

4120 PEOPLE SUBSCRIBED TO OUR MONTHLY EMAIL NEWSLETTERS

665 INCOMING CALLS RECEIVED

2223 MEMBERS CONTACTED

781 OUTGOING CALLS MADE

Bob Stevens
Group Chief Executive

Since we formed in the 1980s, around the kitchen table of our founder, Christine Lavery, the MPS Society has focussed on making life better for people living with MPS.

The list of conditions we provide support for has grown over the years to include related lysosomal disorders like Fabry and Mucolipodosis, which would have otherwise had no patient support. The charity has evolved over these past 40 years but our focus on living the best life possible remains, and at the core of what we do now are three main objectives, to ensure our community have access to exceptional support and advice, world-class clinical care and effective treatments.

FABRY MATTERS CONFERENCE 2024

200 DELEGATES ATTENDED THE CONFERENCE

58 COMPLETED PATIENT SURVEYS

1240 MESSAGES EXCHANGED VIA THE APP

Exceptional support and advice

This year we expanded our support service to offer more online options. A growing population of our community joined our Support Hub Facebook Group, WhatsApp groups and weekly Teams calls to interact with their peers and provide support across the country to each other. We continue to focus on mental health and wellbeing and as well as referrals to RareMinds have pulled together information to help our community to feel empowered to support themselves.

World class clinical care

We collaborate with some of the best professionals in the field of rare disease to improve services for patients. This year we fully funded two grant applications made to the Clinical Scientific Advisory Committee (CSAC) and part funded the Dragon's Den style research pitch at the Fabry Matters Conference. We work closely with clinical teams and partnerships to improve all aspects of clinical care, including working on the MPS airway project and holding the first meeting to discuss the development of an emergency airway management plan for patients.

Effective treatments

The exciting and very successful Fabry Matters Conference 2024 was a major highlight of our year, collecting data and informing the patient community in a single productive weekend. This was a completely new approach to the way we run a conference and the insights collected will be used to empower the Fabry community and inform industry in developing treatments.

To oversee our activities we are lucky to have a group of trustees from different backgrounds, it includes those living with the conditions, family members of those with the conditions as well as scientists, clinicians and professionals. We are so grateful for the time they volunteer to the charity and look forward to developing our new strategy with time over the coming months.

Warmest wishes,
Bob

Supporting you

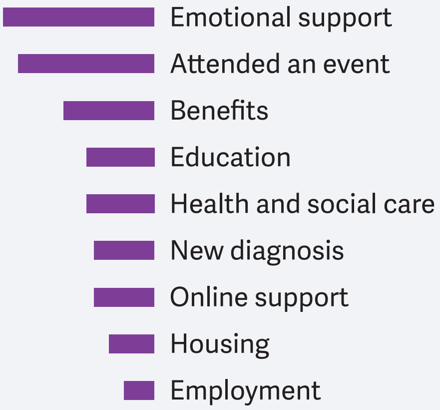
Our aim is to build a stronger, more connected community with YOU at the centre.

We want to make sure everything we do aligns with the needs of the people we support. We do this by listening to our members, seeking feedback about existing services and providing opportunities for members to meaningfully engage with us and influence the direction of the services we provide.

In 2024 we launched our Rare Voice Engagement Group to give our members a voice and help us to shape our services. Something you asked for was to let you know what we do. We have started to tailor our monthly newsletters so you get updates that are relevant to you and as part of this we also included a new section called Representing you to shed some light on the various meetings we attend and organisations we are involved with.

Thanks to some marvellous feedback and conversations at the Fabry Matters Conference we launched new initiatives for our Fabry community, including Fabry Focus and Wellbeing Wednesday. As well as working hard to make sure the Fabry community have an identity as part of the MPS Society.

The main areas of support we have assisted with:



> MPS Society has helped me feel less alone...having the opportunity to meet others similar to me and knowing there is a fantastic team of caring passionate people really makes a difference!

Our online support has grown and now includes more community groups:

MPS Social Hub

Fabry group

Time to chat for over 18s

Parents and carers

Bereaved support group

Regional group for Scottish Families

> Because we as a family have been supported by the same team, they have been able to provide knowledgeable support without us re-telling our story.

10 RESOURCES AND PUBLICATIONS PRODUCED

68 NEW MEMBERS

277 JOINED THE MPS SUPPORT HUB

75.5% SATISFIED OR VERY SATISFIED WITH SUPPORT RECEIVED

25 EDUCATIONAL TALKS GIVEN

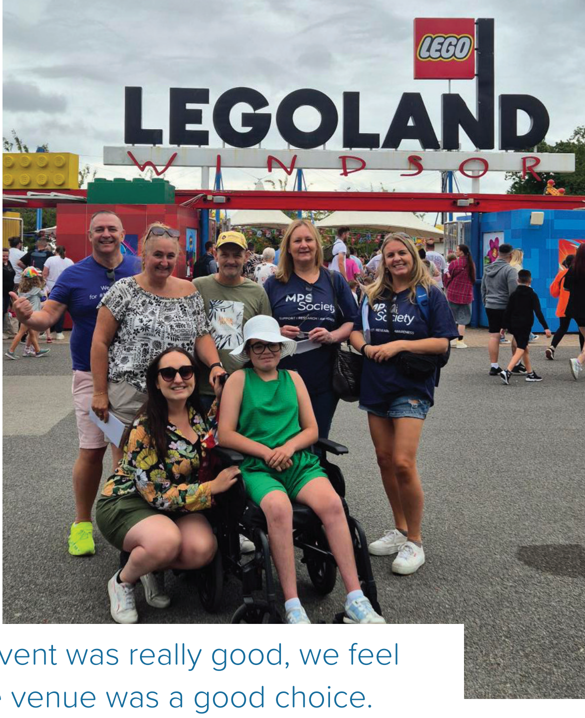
157 MEETINGS ATTENDED

26 CLINICS ATTENDED

Support events

Tailoring face-to-face events around the needs of our community and reaching all members across the UK has been a priority in 2024.

We offered opportunities for children, young people and families living with MPS, Fabry or a related condition to spend a day with people who understand, such as fun days out at Alton Towers, Legoland and Thomley. Regional events included an independent adult get-together in Manchester, Scottish meet & greet and Christmas experience in Northern Ireland. We also invited our bereaved community to come together to share memories and experiences. Face-to-face events offer a chance for our Support and Advocacy Team to meet with MPS Society members in a relaxed setting.



"This event was really good, we feel like the venue was a good choice. I loved meeting up with families that I communicate with via messenger in person and it was great just talking and catching up. The ride passes were a godsend because my little one struggles with queuing."

ATTENDEE, LEGOLAND

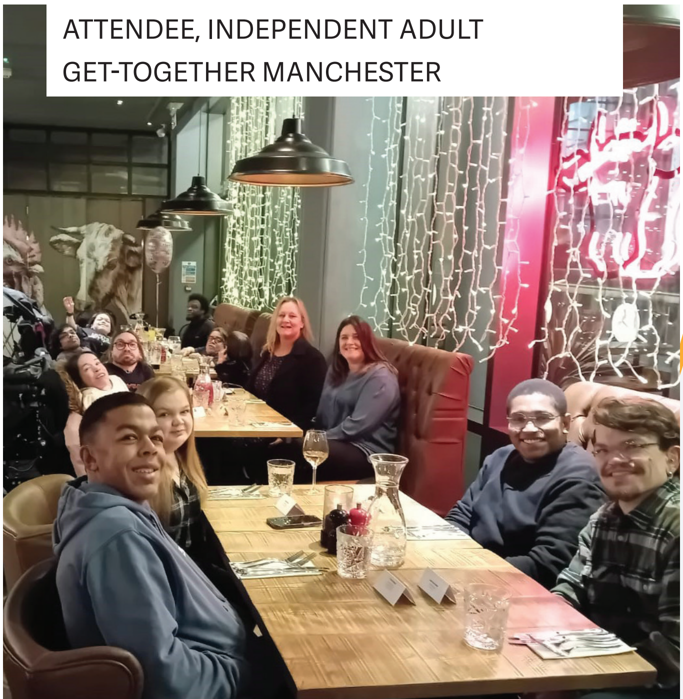
"We were feeling pretty nervous about going to our first MPS Society event. Our daughter is so early into this journey, that we're still not sure what having MPS means for her. We had the opportunity to chat to Bob which we really valued and we had a good catch up with Sally from Advocacy too. We met some really lovely families and look forward to seeing them again at future events."

ATTENDEE, THOMLEY FAMILY DAY

9 FACE-TO-FACE SUPPORT EVENTS HELD

"I really enjoyed getting together with people with my condition, chatting and having a laugh with them."

ATTENDEE, INDEPENDENT ADULT GET-TOGETHER MANCHESTER



Our families were unanimous in expressing the positive impact meeting other families and being able to share stories, tips and support has on them.

We sought your feedback after each event and listened to your suggestions, and will plan our 2025 event schedule accordingly, trying some new ideas whilst continuing to offer popular, regular events.

Sophie Thomas, Senior Head of Patient Services and Clinical Liaisons

Our head of patient services and clinical liaison has worked with colleagues to further the goals of the charity in delivering appropriate treatment and clinical care. Some highlights include:

- Responding to **NICE's** three-year strategy for involvement and engagement
- Meeting with metabolic teams to aid **cardiac specification plans** including patient information leaflets and training meetings
- Supported the implementation of a **new transition pathway** for LSD services
- Launched **PPPN (Patient, Professional, Partnership Network)** with a focus on clinical guideline development and LSD patient community survey
- Responded to **NICE's** consultation on methods and processes for including NICE technology appraisal recommendations in guidelines
- Shared information with our **LAL D community** about the discontinuation of the appraisal for people with late onset/non Wolmans form of the condition
- Received approval by **Scottish Medicines Consortium (SMC)** for pegunigalsidase alfa (ERT for Fabry) for use in adults

Following a successful launch in November 2023 of the **MPS airway project (MAP)**, we welcomed 24 attendees, representing six countries, to the first hybrid meeting in April 2024 in Wurzburg Germany to discuss complex airways with a focus on MPS II and MPS IVA. Proposed actions include development of international consensus guidelines, development of an emergency airway management plan for patients and discussion on the pathophysiology of the airways and trachea in MPS II and IVA.

- **Generation Study** – MPS were approached to review proposed family information sheets for the MPS conditions included in the study. Our amendments and suggestions were accepted.
- **All Wales Medicines Strategy Group** approved Naglazyme for use in Wales to treat MPS VI Maroteaux-Lamy.

Funding research

Fiona Stewart, Chair of the Clinical Scientific Advisory Committee

In 2024 our Clinical Scientific Advisory Committee (CSAC) paid out **£81,934** to grants and committed a further **£129,766** to fund future research. The committee were able to fully fund two grant applications. The first one focuses on MPS III Sanfilippo and the other revolves around the blood brain barrier. In addition, they offered partial funding in the form of a 'challenge grant' for a project looking at lysosomal damage repair.

We ran a virtual CSAC open day at the start of October which included presentations about what a biomarker is and how we measure it by Kevin Mills, an update on Brian Bigger's research, a look at a study to improve the quality of life of adults with Fabry disease using exercise by Sarah Gosling and Dan Bailey as well as an overview of what is happening in Rare Disease Research Partners (RDRP) by Alex Morrison. CSAC also gave funds towards two projects that took part in the Dragon's Den style competition at the Fabry Matters 2024 conference. These were:

- Establishing Novel Retinal Vascular and Neural Biomarkers in Fabry Patients Using Optical Coherence Tomography Angiography (OCT-A) for Assessing the Role of Treatment in Retinal Blood Flow and retinal vascular density
- Understanding atrial arrhythmia in Fabry Disease – the development of patient-specific in silico atrial models

£81,934

PAID OUT TO GRANTS BY CSAC IN 2024

AWARENESS IN 2024

Spread the word

ON AVERAGE
3716
PEOPLE VISITED OUR WEBSITE EACH MONTH

Fabry Awareness Month

April is Fabry Awareness Month and we have been sharing advice and expert knowledge about Fabry disease gathered at the Fabry Matters conference we held in early March. We raised awareness and highlighted what life is like living with Fabry by sharing professional presentations on topics such as nutrition, transition and mental health, as well as video clips of some of our members telling us what they want people to know about Fabry.

> “I wish people were more aware of it. Even my GP doesn’t know about Fabry.”

FABRY COMMUNITY MEMBER

We launched a new online Fabry education tool for professionals which offers them in-depth knowledge of Fabry disease and encouraged our members to share information with their GP, clinician or specialists.

MPS Society

Learn about Fabry disease

This new online Fabry education tool for professionals is free and easy to access. Learn through interactive online modules and knowledge checkpoints.

Current modules include:
- Fabry disease overview
- When to suspect Fabry disease
- Testing for Fabry disease
- Genotype-phenotype relationship
- Treating Fabry disease

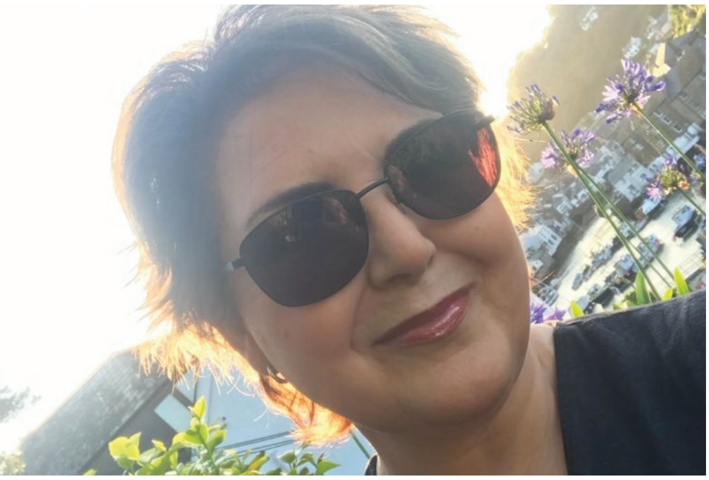
Scan me

www.mpssociety.org.uk/fabry-too

MPS Awareness Week

We shared member stories and resources to highlight the importance of mental health support for our community and why it is vital to provide specialist support for anyone living with or affected by MPS, Fabry or a related condition. To ensure continued provision of the mental health services for our community, we took part in the Big Give Kind2Mind appeal.

On our social channels and website we encouraged supporters to Wear it Blue and were delighted to share a gallery of the many dedicated participants. We also joined the International MPS Network in the #itsabouttime campaign, raising awareness that for people living with MPS every second matters.



> “Functioning in a state of urgent response was my normal. I learned it is not possible to pour from an empty jug.”

MOTHER OF A DAUGHTER WITH MPS

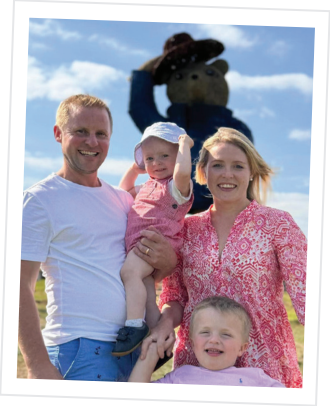
71K TOTAL IMPRESSIONS

1647 WEBSITE VISITS

£28K RAISED BY YOU

Thanks to you

A heartfelt thank you to our outstanding community fundraisers, who inspire us every year.



Paddington wears it blue

A giant thank you to Snugburys Ice Cream for choosing to support the MPS Society in honour of the Goodwin family who have a son with one of our conditions. They raised over £9,000. If you haven't come across Snugburys before, visit their famous ice cream shop in Nantwich, Cheshire where you can also meet a 45-foot-tall Paddington Bear.

Around the rock

Thank you to Tom, Scott and Sam for taking on the *Around the rock* challenge in memory of Ollie. They raised over £11,000 for the MPS Society and Acorns Children's Hospice. "I'm really thrilled we managed to raise so much for you," said Tom.

Running for #TeamMPS

We've had some amazing runners this year. Jon, Jess, Hana, Damian, Craig, Kevin, James and Joe were our London Marathon team and raised over £52,000 – the most ever!

A huge MPS Society thank you to Michael who completed the London Landmarks Half Marathon raising £2,370, to our Royal Parks Half Marathon runners Sam, Nicolette and Pete who raised over £5,600 and to Paul, who took on the Great North Run and raised over £450.

In 2024 we received over

£22,000 from regular donations and payroll giving schemes

£10,000 from donations and tributes in memory of loved ones

£7,000 in one off donations

£87,000 from challenge events

MPS Awareness Week

You shared our posts more than ever before and absolutely smashed our fundraising target raising over £28,000. We had a great variety of events such as the Kind2Mind appeal, so many Wear it Blue events, cake sales, coffee mornings, a golf day and donations from some very kind individuals and organisations.

You made this our best ever Big Give Christmas Challenge by raising over £32,000 for our community events which are vital for our members:

> "These outings allow us to have special days together, creating memories away from the stresses of daily life."

Thanks to everyone who shared our stories and posts. We couldn't have achieved any of this without you, our pledgers, donors and fundraisers.

Rare Disease Research Partners

In 2024, the RDRP Clinical Trial Support team assisted 117 patients in attending over 992 study visits, supporting clinical trials for MPS II, MPS IIIA, MPS IIIB, MPS IIID, Fabry and MLD conditions. Expanding their expertise, the team also began collaborating with pharmaceutical companies and patient organisations to support patient recruitment for research surveys, helping to gain deeper insights into these conditions.

Meanwhile the communications team provided support for the important work on airways management in MPS and carried out all research activities at the Fabry Matters Conference. Their research helped provide a greater understanding of rare diseases through presentations made at international medical conferences on Fabry, Alpha-mannosidosis and MPS II and eventually led to the development of support materials assisting Fabry patients in navigating their care and treatment.

£150,569

GIFTED TO THE MPS SOCIETY

Together we can transform lives

Thank you...

...to the trusts and grant-making organisations who have supported us in 2024 including The National Lottery Community Fund

...to our corporate funders who have contributed to the work we have done this year

...to all the regular donations we receive including those from anonymous donors, we don't know who you are but we are so grateful for your support

...to everyone who has left a legacy in their will to help support families with MPS and related conditions in the future.

Your support makes such a difference. Find out more about how we use your donations to transform lives at www.mpssociety.org.uk/our-impact

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

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