RESEARCH | AWARENESS | SUPPORT

Impact Report

2020/21

Adapt and overcome

Bob Stevens
Group Chief Executive

It's been a year of uncertainty. Covid has meant confusion over who should be shielding and the risks associated with vaccination. We had to rethink our events and face to face visits became a thing of the past. But we have worked with other patient organisations to develop webinars and guidance to inform our members as best we could. We responded to the need for mental health support with mindfulness courses and specialised support workshops and created more online content than ever before. Highlights of the year were the launch of the MPS Society's Clinical and Scientific Advisory Committee's research grants to fund projects in all areas of metabolic medicine, and of course how you, not just as members but also as supporters have helped to fundraise to provide more services and raised awareness of MPS, Fabry and other related diseases.

THIS YEAR WE HAVE SUPPORTED:

42 NEW MEMBERS

25 DISEASE TYPES

331 YOUNG ADULTS

> "You have picked me up off the floor so many times in the past."
>
> MEMBER FEEDBACK

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:

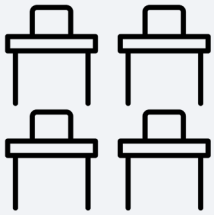
- exceptional support and advice
- world-class clinical care
- effective treatments

21

EVENTS HELD TO SUPPORT MENTAL HEALTH AND WELLBEING

OUR IMPACT IN 2020/21

1114
MEMBERS CONTACTED THROUGHOUT 2020-21



19
AWARENESS TALKS AND TRAINING DAYS WITH SCHOOLS AND SOCIAL SERVICES

70
BENEFIT APPLICATIONS SUPPORTED

269
MEETINGS OR CONSULTATIONS FOR OUR MEMBERS

10,518
POSTS VIEWED ON OUR BLOG

30,783
PEOPLE LOOKED AT CONTENT ON OUR FACEBOOK PAGE

2059
FOLLOWERS ON TWITTER

2502
PEOPLE SUBSCRIBE TO OUR MONTHLY EMAIL NEWSLETTER

ON AVERAGE
1575
PEOPLE VISITED OUR WEBSITE EACH MONTH

Highlights of the year

September 2020

We were fortunate enough to be featured on the Radio 4 Appeal, a weekly 3-minute programme that highlights the work of a charity and appeals for donations. Thanks to the lovely Kelly Mills for sharing her story about daughter Penny.

£18K
RAISED IN THE BIG GIVE CHRISTMAS CHALLENGE

January 2021

We launched a new research grant programme inviting applications for research grants in MPS, Fabry and related diseases reviewed by members of the MPS Society's Clinical and Scientific Advisory Committee (CSAC) and keeping up the strong tradition of funding cutting-edge research into rare diseases.

April 2021

We welcomed Mandy Carey to a brand new role at MPS Society as Head of Project Delivery.

"I am going to be involved in projects to help support patients and their families in dealing with mental health and wellbeing."

May 2021

Despite lockdown and Covid restrictions we managed another successful MPS Awareness Week this year. Our blue army did an amazing job raising awareness over social media and with friends and family creating a great buzz.

July 2020

We showcased the many skills of our community through the MPS has talent show. Although it had to be pre-recorded this time it didn't stop some amazing talent in singing, dancing, swimming, signing and much more!

October and November 2020

We held our first expert meetings focussing on ultra-rare diseases and tracheal surgery.

January 2021

Sophie Thomas handed over the reins after 15 years of leading our team of advocacy support officers to become Senior Head of Patient Services and Clinical Liaisons. In this role, she aims to maximise patient outcomes and wellbeing and to ensure patients remain the focus of decision making across all areas. In January she also took on a position as a patient and public voice partner in the NHS's Rare Disease Advisory Group (RDAG) working externally for the benefit of our members.

March 2021

We launched our mental health and well-being programme which included a series of specialised workshops and mindfulness courses aimed at adults, young adults and children. We also set up our well-being webpage offering video resources to help these groups to manage anxiety.

April 2021

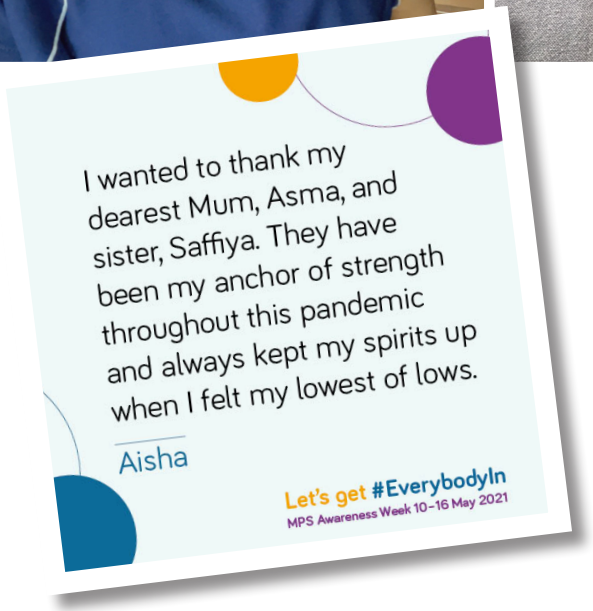
This Fabry Awareness month we featured a series of articles titled "Could it be Fabry?" from guest blogger Loretta MacInnes, a Fabry patient who wasn't diagnosed until she was 50 despite having symptoms since childhood. The aim was to raise awareness of the often unidentified symptoms of Fabry disease.

550
READ OUR FEATURED BLOGS

This awareness week we asked you to get involved by celebrating coming out of lockdown and looking to the future.

83 NEW FOLLOWERS ON SOCIAL MEDIA

10 PRESS ARTICLES PUBLISHED

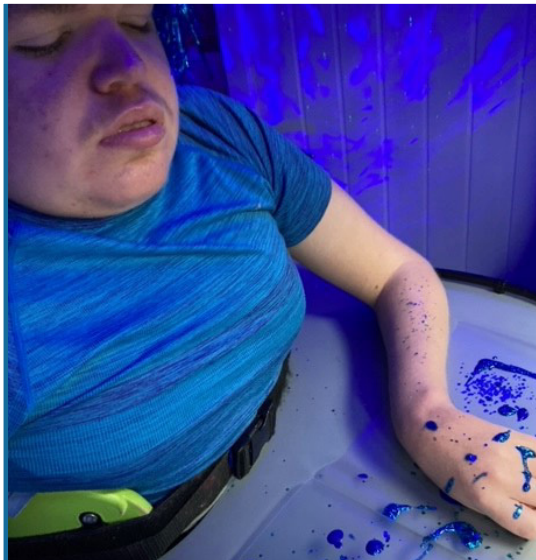


36K
PEOPLE REACHED THROUGHOUT THE WEEK

82%
NEW VISITORS TO OUR WEBSITE

YOU RAISED
£9000

Lexie, who is 13, organised a Wear it Blue day at her school of performing arts because her sister, Tillie Mae, has MPS III.



Ethan

From our community

It's thanks to some of the amazing support of our community that we provide the services for those who are living with rare diseases need the most. This is just a few of our wonderful community fundraisers.

Ethan Fanneran and family

Ethan is 12 years old and has MPS VI Maroteaux-Lamy Syndrome. This year he raised £3,000 for the MPS Society by walking over 100km in May leading up to our 'Wear it Blue' day as part of MPS Awareness Week. He chose 100km as this is the exact distance between where he lives in Canterbury and Great Ormond Street Hospital where he receives his weekly treatment for his condition.

Ethan's family has also raised nearly £15,000 needed to take him to Italy to take part in a groundbreaking trial which is searching for a cure. Ethan's mum, Katrina, says: "We have a very rare chance to cure our son's rare disease and we are one step closer to giving him the future we have only been able to dream about until now." She even started the 'Wear it Blue' day in honour of Ethan!

Ethan's step-dad, Kevin, is taking on his own challenge and walking 100k along the South Coast in September for the MPS Society and the work that we do.

Priory Learning Trust

The Priory Learning Trust is an educational charity who support primary and secondary schools in the South West of England. They are made up of eight schools and have made us their Charity of the Year. One of the schools, The King Alfred School an Academy Sixth Form released a cover version of "Send Me On My Way" by Rusted Root to raise funds for the MPS Society.

It is the first-ever charity single to be released from the school and has been released on streaming platforms such as iTunes and Spotify. The single was made by 87 students and staff and encapsulates happiness and summertime. The popular song aims to raise a lot of money and also celebrate the bringing of people back together again now that the restrictions are beginning to lift.

Annabel Norris

A big congratulations to Annabel on completing the Salomon Serpent trail on 3 July in an amazing time of 2 and a half hours! She ran in honour of Sophia Scott and her family and has done them proud. She said: "It was super hard, but I loved it!"

Dan Hall

Dan took on a massive challenge in honour of his godson, Rory Hodge, who has MPS II to raise vital funds and awareness for the MPS Society.

In August he took on the Everesting challenge cycling up and down Waddington Fell in the Ribble until he reached the 29,031 feet elevation of Mount Everest!

He said: "This is really important to us so research and support can continue for families like Sarah and Sean's (Rory's parents). MPS Society is a wonderful charity providing support to families in a similar situation."

Lianne Mott

Lianne works for our sister company, RDRP and ran the Royal Parks Half Marathon for us last year. She had some running experience, but had never taken part in a race so long before. With the knowledge that she was supporting patients with MPS, Fabry and related diseases, she persevered and was delighted she was able to complete the challenge. Running is now a huge part of her life and has changed it for the better.

Marina Foster

After 18 years of raising funds and awareness for the MPS Society, Bristol-based charity shop Marina and Friends Fundraisers has finally closed its doors. From everyone here at the MPS Society and on behalf of all of our community, we would like to send a huge thank you to Marina for her hard work and dedication and to everyone who has worked with her and supported us over the years. Marina and Friends was started to honour Marina's grandchildren, Francesca and Josephine, and the popular community shop has raised an incredible £250,000 for the MPS Society over the years. It's definitely the end of an era but we'd like to continue to celebrate Marina's achievements.

National Lottery Community Fund award

£195K to build connections and reduce feelings of isolation

Our community's needs have been exacerbated by the COVID-19 pandemic and lockdown. From the threat to life to the limits to independence and social interaction, our community have never been in more need of our support.

To meet this need, we have adapted our plans for the next three years to support the MPS community to still build connections and receive the support they need through these times and we are delighted that The National Lottery Community Fund - Reaching Communities have awarded us £195k to help us achieve our plans.

With the funding, we will support the children and families affected by MPS and related diseases to build connections across the MPS community, reducing isolation.

"I feel as if I have grown in some confidence, because even a year ago, I would have started to panic about the prospect of speaking to people. I have always worried about not being understood." MPS Society member

Our response to the need for connection, is to provide opportunities to build relationships, encourage peer support and create a platform for those most affected to shape the services that matter to them.

We will build connections through this project over the next three years by working in four key ways:

1. Family events
2. Virtual wellbeing support
3. Young Person's Advisory Board
4. MPS community

Every member; the affected child, their parents, siblings and wider family and friends' network, will all benefit from access to our events and support services. The impact will be a more connected MPS-affected community, reducing isolation and providing support to families who would otherwise be isolated by their disease.

Masonic Charitable Foundation

Our fantastic grant funding from the Masonic Charitable Foundation of £62,725 over the past two years has almost come to an end. We are so delighted with everything we have been able to achieve with this funding, helping young people in the MPS & related diseases community through key life transitions.

We would like to extend a big thank you to our key supporters who donated emergency funds due to the pandemic, including The Gosling Foundation, Global's Make Some Noise, The Big Lottery and The Julia and Hans Rausing Trust, along with grants from a number of pharmaceutical companies. We were also very grateful to a number of charitable trusts and foundations for their grants and donations towards various aspects of our support services, including: The Masonic Charitable Foundation, the Eveson Charitable Trust, the Summerfield Charitable Trust, the Wixamtree Trust, the Pilkington Charities Fund, the Borrows Charitable Trust and the Adint Charitable Trust.

New look events

In the last two years we were unable to carry out a number of activities, particularly face to face, such as our annual conference, due to Covid lockdowns and the additional risk posed by getting our community together in person. We have however played a central role in supporting the community by providing a greatly expanded online provision including workshops, webinars, mental health support and an expanded young people provision.

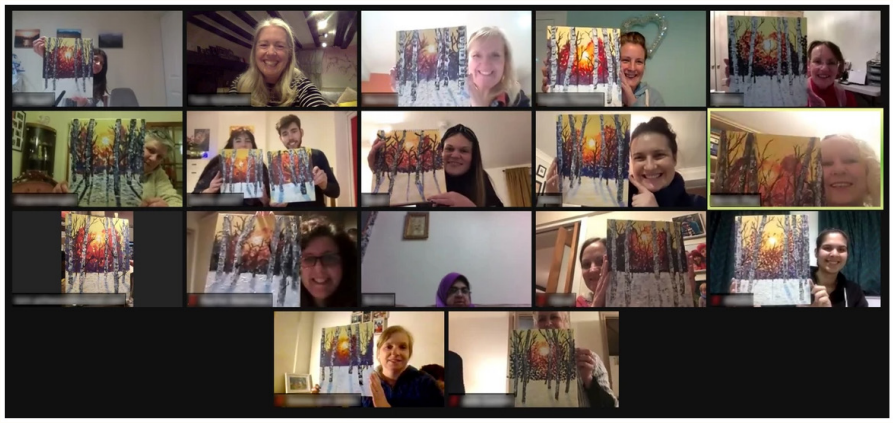
New to this year were the sibling workshops run by sibling support charity, Sibs. We also offered sensory workshops for parents and children to attend. There were specialist webinars on Fabry treatment updates and how the vaccine might affect people living with Inherited Metabolic Diseases. We also made time for some fun with painting workshops, kids' parties, scavenger hunts, Christmas panto, make your own decoration and much more!

We also continued our support of young adults by hosting four formal sessions and some online chats with our Rare Voices group and held an online event for our bereaved families to get together.

All of this is available for free to our members thanks to the funding and donations from everyone who supports the charity.

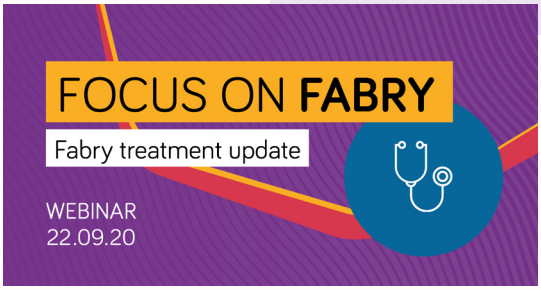
40
EVENTS HOSTED

MORE THAN
600
RSVPS



> "It was one of the most rewarding courses I have ever undertaken...[it] was especially good to meet with other members."
>
> MEMBER FEEDBACK

The Fabry Treatment update webinar was the most well attended event we held.



Several resources were produced as a result of the learning from our webinars.

Rare Disease Research Partners

Rare Disease Research Partners

SUPPORT RESEARCH AND ACCESS TO TREATMENT FOR PEOPLE LIVING WITH RARE CONDITIONS

Our wholly-owned subsidiary, MPS Commercial, underwent a successful rebrand in January 2020, changing its name to Rare Disease Research Partners (RDRP), to better reflect the work they do.

They have continued to grow and expand their services to the wider rare disease community while still offering trusted clinical trial logistics management and providing more evidence and research-based services and specialist medical communications.

Alongside their varied contract work and support for the MPS Society and other rare disease patient organisations, they became corporate members of the British Healthcare Business Intelligence Association and run the research grant programme for the MPS society. This year they gifted the MPS Society their profit of £319,000.

Clinical Trial Support

The last year has been a challenging year for the Clinical Trial Support Team. I don't think it will come as a surprise to anyone that the pandemic has caused difficulties for those travelling to their study visits, so we have been working with our clients to try and alleviate the stress and anxiety for families. This has included:

- temporarily relocating families to their clinical trial sites
- checking COVID restrictions per country for those patients still able to travel and assisting families with completing necessary travel paperwork
- checking that all our vendors have processes and precautions in place to ensure minimal risk of exposure to COVID for passengers
- arranging COVID tests where necessary
- arranging quarantine accommodation where necessary.
- keeping aware of any potential last-minute disruptions to flights due to the pandemic
- looking into alternative travel options for patients who don't want to travel by plane
- providing contingency plans to our clients to show how we could continue to support patients during COVID.

In fact, we authored a poster for the 2021 World Conference in February showing the additional support provided to patients and their families during the pandemic.

For us, it has been really encouraging to see the Sponsors of studies do what they can to make participation easier for patients during the pandemic. As already mentioned, patients and their families have been temporarily relocated, or the Sponsor has opened sites more local to the patient.

During this period, we have supported nearly 2200 study visits and 20 relocated families. While there was initially a drop-off in patient travel, we are now seeing this increase again as borders open and travel restrictions ease.

Research and Medical Communications

The Research and Medical Communications Team have been incredibly busy with an increased demand for our services. During COVID restrictions we have been very fortunate to be able to recruit new members to the team and offer two university students one year of research-based work experience placements.

Our pharmaceutical company client base has expanded, and we have supported them in a variety of patient-focussed and new treatment-related projects. Alongside this we have worked closely with the MPS Society to publish new research into the gastro-intestinal issues associated with Sanfilippo, and generate evidence for patient organisation submissions to NICE for the evaluation of gene therapy in metachromatic leukodystrophy and the re-evaluation of enzyme replacement therapy for Morquio A.

We would like to take this opportunity to thank the MPS Society members, their families, other patient organisations and their members, doctors, nurses and other healthcare professionals who have taken part in surveys, focus groups, advisory boards and even videoed themselves to support our projects.

Over the past year, we have been able to share MPS Society and RDRP research with the international healthcare community by virtually attending both the WORLD symposium and the International MPS Symposium, presenting five posters in total and presenting on Morquio A at WORLD.

And we have been pleased to provide professional writing services for a growing library of patient-friendly materials including handouts for the MPS Society's webinar series on new treatments and clinical trials, another two issues of Fabry Findings for the Fabry International Network, guidance on COVID-19 and a booklet for patients being treated for Batten's Disease.

Support

Our priority, as ever, is to the families we support, our team, volunteers and supporters. Our commitment to ensuring that every person affected by an MPS or related disease has access to a supportive community has been more important than ever due to the COVID-19 pandemic. The majority of our members have been classed as more 'at risk' of COVID-19 under NHS guidance this past year. This has had major implications on the families we support and how we have reacted to their needs.

Threat to life

Coronavirus poses a severe threat to life for our community and, understandably, the families we support are particularly anxious about protecting their loved ones from contracting the virus. For most, this means strict self-isolation.

We introduced mindfulnesses courses for Fabry adult patients and parents and carers of children with MPS or related diseases. We held mental health and wellbeing workshops and then adapted these into online resources which can be accessed by all our members.

Limited Independence

Independence is a key issue for our members who will struggle with having to rely on others for support with everyday tasks outside the home, such as shopping, commuting, work, etc., that they are used to doing themselves.

Our Covid resource pages offered signposting to places where families could go for extra support. We also aimed to share up to date and accurate information as frequently as possible about changes to shielding and vaccinations so our families stayed informed.

Social isolation

Social isolation and distancing means that many of our members feel lonely and isolated. Disruption to routine can be particularly challenging for our younger members and those with neurological degeneration and/or learning disabilities. This can lead to increased challenging behaviours and outbursts for these members who are frustrated and trying to understand what is going on.

Our sensory workshops helped to provide relief and entertainment to children who were struggling through lockdown. We also offered workshops and video resources for young adults and children who were experiencing anxiety.

Challenges for parents and carers

The majority of our community rely on parents, family and partners for their personal care. Social distancing increases care responsibilities as day groups, schools and workplaces close. Carers will now have to juggle 24/7 care responsibilities with their jobs; caring and home-schooling their other children; housework and any other responsibilities with little to no respite.

Though we couldn't be there physically to support our members, we hopefully provided some respite in the many online events we held that offered a little time off for our over-worked families.

MENTAL HEALTH AWARENESS TRAINING WAS ROLLED OUT TO THE WHOLE ORGANISATION

> The help and support we have received has been invaluable. The outcomes may have been very different without you.

MEMBER FEEDBACK

OUR SUPPORT AND ADVOCACY TEAM UNDERTOOK SPECIAL EDUCATIONAL NEEDS LAW TRAINING

Disruption for siblings

Siblings of children with an MPS or related disease are also affected. The demanding physical and emotional needs of affected members means that siblings may have to help with care, home-school alone and entertain themselves in the home. This can be particularly isolating and disruptive for younger siblings.

We introduced specialist workshops for siblings of all ages, through to adults, who were coping with the added strain on life that a child with extra needs places on a family.

Cancelled treatments

There is no cure for MPS and related diseases, but many members rely on regular enzyme replacement and other therapies and treatments to alleviate or reduce symptoms. With the demand on the NHS, treatments are being cancelled and our members are encouraged to not attend any 'non-essential' appointments to limit their chances of contracting the virus. This means that our members condition may deteriorate and symptoms worsen.

As well as funding research into new treatment areas that will benefit our members we are looking at new ways to improve the support we offer our members when they attend specialist clinics.

8 ADVOCACY FACTSHEETS UPDATED

Medical research

The pandemic has had an impact on current and planned medical research programmes as participants will be unable to attend appointments and healthcare professionals will be drafted into Coronavirus emergency response. This will be disruptive with some research programmes being paused and others needing to be started again after the outbreak is under control.

RDRP have continued to support our members who are on clinical trials and to make sure the logistics are as simple and easy as possible.

NHS implications

Where possible, those with MPS and related diseases will receive any clinical care or treatment in their own home to minimise the risk of coming into contact with contagions. With the current demand on the NHS, this means that many of the healthcare professionals treating our community will be unfamiliar with this rare group of diseases. In addition, carers and parents are going to be increasingly relied on to administer some treatments, medications and injections.

We continue to work closely with NHS England and NICE to make sure the fastest and fairest system for allowing our members to receive treatment is available. We watch with interest the results of the Rare Diseases Framework plans that are currently being written. We supported two treatments going through the NICE Highly Specialised Technologies evaluation, including in-depth surveys to gather information on patient carer involvement and the burden.

Together we can transform lives

Thank you to those who shared their photos and stories for this newsletter. Please keep telling us about your life with MPS, Fabry or related diseases and your fundraisng 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 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-now

MPS Society
MPS House, Repton Place,
White Lion Road, Amersham,
Buckinghamshire HP7 9LP
0345 389 9901

@MPSSocietyUK
fb.com/mpssociety
www.mpssociety.org.uk

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