RESEARCH | AWARENESS | SUPPORT

Impact Report

2022

FROM THE GROUP CHIEF EXECUTIVE

Bob Stevens
Group Chief Executive

THIS YEAR WE HAVE SUPPORTED:

60 NEW MEMBERS

27 DISEASE TYPES

300 IN-DEPTH CASES

2022 was a historic year, both for us as an organisation and indeed for the country as a whole. In September we joined with the rest of the country to mark the passing of Her Majesty The Queen Elizabeth II, mourning the loss of an inspirational person.

It's been a historic year in many ways for us this year. We welcomed 2022 by reaching a tremendous milestone as we celebrated our 40th year as a rare disease charity. The MPS Society started life back in 1982 when Christine Lavery MBE started a journey of hope, founding the Society of Mucopolysaccharide Diseases (MPS Society). Christine established the Society following her son's diagnosis of MPS II Hunter disease. Christine started to link up with other MPS families in the UK to offer mutual support and share much needed information. From these very small beginnings, around a kitchen table, the charity was born. Forty years later and we've evolved into a significant community, able to come together in times of need.

We've marked the remarkable achievement of our anniversary in a huge range of ways, with events focusing on particular issues each month as well as fundraisers of the month. As always we had our annual MPS Awareness Week, which was a huge success.

In November we were delighted to launch the Christine Lavery Memorial Fund, to honour her memory and dedication to research and the community. The fund will go towards supporting a university student to undertake a summer laboratory scheme on a research project that will study an MPS or related disease. The student will receive a salary based on a relevant living wage, meaning this will be accessible to students from all backgrounds.

We share the LSD Collaborative survey, which was designed by the 11 charities in the Collaborative who work together to support patients with lysosomal disorders. Thank you to everyone who filled in the survey. Your feedback as always is really important and helps change the landscape of rare diseases.

People are at the heart of what we do and that extends to our amazing team. Every year we carry out a staff survey. This year we are delighted to report that 100% of our staff feel encouraged to improve their physical and mental well-being. This is particularly important to us as mental well-being has been a key focus of our work this year, with the introduction of more counselling and advocacy services for our community.

Our staff were also instrumental in making headway with new medical treatments. The National Institute for Health and Care Excellence (NICE) provided positive feedback regarding MPS Society's submission for Highly Specialised Technology Re-Evaluation for Vimizim, following the recent news that it has been recommended by NICE (National Institute for Health and Care Excellence) for treatment in England and Wales.

We were involved in a number of new ways of raising awareness. We collaborated with Design Science to create new animations explaining life with an MPS disease. These were created through a series of workshops with people living with rare diseases and are aimed at newly diagnosed families and professionals who may not have heard of these diseases.

It has been a fantastic year and we want to thank each and every one of you for your help and support. We value our community and supporters and know that what we do would not be possible without you.

We look forward to another year of success.

Warmest wishes,
Bob

Support we offer

Through our Advocacy and Support service in 2022, we have provided a wide range of support, both practical and emotional. Our team has had direct contact with over 1,100 of our members, including over 60 new members who joined this year.

We have provided support in a number of ways:

Connection – 46 cases

Emotional support – 109 cases

Independence – 32 cases

Transition – 12 cases

We have delivered over 300 in-depth cases of support, for example with Education, Health and Care (EHC) plans, social care issues and continuing healthcare meetings.

Lilia

6-year-old Lilia was diagnosed with MPS III B last June. The advocacy team helped her family with the process of claiming disability benefits, supporting specialist information which was instrumental to the claim. They also provided support in putting together Lilia's EHC plan and a support package for the whole family. They helped with her transition from mainstream to a specialist school. As well as this they offered counselling services and emotional support.

Lilia's mother said:

"We are extremely grateful for all the help and support we have received. They have definitely made the months since Lilia's diagnosis more bearable."

Oliver

The team has supported Oliver and his family for many years following his diagnosis at the age of 7, Oliver is now 24 years old. His Support and Advocacy Officer has provided practical guidance through health, social care and education systems as well as emotional support. Oliver is now living in his own home, with a care team around him. His Support and Advocacy Officer has been a consistent member of the multi-disciplinary team, representing Oliver at meetings, supporting communication and working with his family and local professionals throughout his transition.

Oliver's parents said:

"We see you as part of Oliver's extended family support. You have been involved with us since Oliver's diagnosis in 2006 and we feel so lucky to have you as part of his support team. We would never have achieved all we have without you and the MPS Society."

Our commitment to mental well-being

Living with a rare condition can be a challenging and isolating experience. We understand this and work to help with any feelings of loneliness that members of our community feel. We are currently working on our Building Connections Project, which is ongoing and stretching into 2023. Some of the results of the work will be released soon but so far we have managed to create content which gives practical, helpful advice such as our blog that gives "Top Tips to Combat Loneliness" created using the ideas from our community on how they overcome loneliness.

In fact this project ties into one of our key concerns: the mental health and well-being of our community overall. In 2022 we held events that focused on mental health such as Children's Mental Health week in February in partnership with Place2Be. We also celebrated World Mental Health Day on 11 October.

We continued to offer our telephone and online counselling service (open to any patient or family member over 16) as well as our online mindfulness courses thanks in large part to generous donations from charitable trusts.

"The course was quite life-changing"

PREVIOUS PARTICIPANT, PARENT

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

PREVIOUS PARTICIPANT WITH FABRY DISEASE

With the help of a grant from the National Lottery Community Fund – Awards for All we launched the Rareminds therapy course for 16–18-year-olds, run by a qualified psychotherapist. Studies have shown that teenagers and young people living with MPS and related conditions are more likely to suffer from anxiety, low self-esteem and depression. So we were able to help these young members better learn new coping strategies, make connections and improve their mental well-being.

We also began to offer the ability to book Advocacy appointments online to make life easier for individuals and for clinicians referring patients.

Face-to-face events

We returned to face-to-face events this year with trips to Legoland, Drayton Manor and Gulliver's Land. It was so good to see our families connect once again and this remains an important part of what we do.

The Wood of Remembrance and Hope

The Wood of Remembrance and Hope (originally known as the Childhood Wood) was created in Sherwood Forest in 1993, when the MPS Society was granted a licence to plant a wood of oak saplings cloned from the forest's Great Oak. The project is a way for our members to commemorate the lives of loved ones they have lost to MPS, Fabry and related diseases. We organise an annual weekend for our bereaved families, when bereaved families can visit the wood with the support of MPS staff and other families and plant a sapling as a positive way of celebrating the life of a loved one.

2022 saw the refurbishment of the wood, including new fencing, surfacing, entrance signs and memory boards. This was made possible through a grant from The Geoff and Fiona Squire Foundation along with very kind donations from MPS Society members. As a result of this generous funding, the wood has been transformed into a more welcoming and family-friendly place where our members can go at any time to spend a day close to the loved one they have lost.

A big thank you to all our donors over the years who have made this special place possible.

Disease booklets

We know that being diagnosed with a rare disease is life-changing and patients can struggle to come to terms with it. To help them understand more we created disease booklets for them to learn more about the diseases and how we can help them to live well. They are designed to help those affected, both patients and their families, and to understand the causes and effects. They draw on the experiences of parents, carers, families and medical professionals as well as medical literature.

Clinical liaison

This March marked 20 years since Sophie Thomas started working for the MPS Society. She joined as an Advocacy Support Assistant and has since gone on to become Senior Head of Patient Services and Clinical Liaisons.

Sophie plays a critical role in our work and this year has been no exception. One aspect of her role that makes a significant difference is communication. Sophie works closely with our clinical centres, attending annual clinical meetings and one-to-ones with the LSD Collaborative. She updates them on clinical perspectives as well as delivering patient feedback relating to their experience in attending LSD centres and metabolic appointments. She also works closely with the NHS. She's involved with peer reviewing and sitting on the rare disease advisory group, which is a group that puts forward recommendations to NHS England in respect to specialist services. This involves reviewing all sorts of protocols and policy changes.

Treatment work

Sophie is very involved in work focusing on the approval of new treatments and patient access. This includes working with National Institute for Health and Care Excellence (NICE), the Scottish Medicines Consortium (SMC) and Welsh Health Specialised Services Committee (WHSSC). These regulatory bodies decide if and how treatment should be reimbursed by evaluating whether the treatment is:

- cost-effective
- clinically effective
- how treatment impacts and improves patient and carers quality of life and activities of daily living

The input of the MPS Society is vital, to not only convey the patient experience of the condition and treatment but to bring in the clinical experience and views also. We do this by undertaking surveys, questionnaires, focus groups and interviews with patients and clinicians to really understand how the condition impacts patients.

"A main part of our role is not only giving the collective patient view but to identify patients who may with our support act as patient experts representing the community. It is important for the committee to hear directly from patients about how the disease impacts them, what they view are the advantages and disadvantages of receiving treatment and the effects treatment has on their quality of life," says Sophie.

Last year we were involved in the approval of two treatments by NICE and two with SMC. There are three currently underway with NICE, with committee meetings scheduled in 2023

Major achievements this year

In 2022 we were thrilled that NICE recommend Atidarsagene autotemcel (Libmeldy®) as an option for the treatment of late infantile and early juvenile metachromatic leukodystrophy (MLD). Sophie (on behalf of the MPS Society) along with two other MLD patient organisations (MLD Support Association UK and ArchAngel MLD Trust) were crucial in delivering information to the committee that informed the decision. NICE commended the patient organisations for their submissions providing detailed feedback from a survey on the effect of Libmeldy® on quality of life.

MLD is a rare, serious, and life-limiting condition that significantly affects the lives of people with the condition, their families and carers. This gene therapy treatment if given pre-symptomatically has the potential to prevent disease progression, with children living normal active lives. One patient expert stated "With an MLD diagnosis for two of our children, one untreated and the other seven years post-transplant, we have witnessed first-hand the pain, torture and devastation this monstrous condition unleashes on innocent children. We have also seen the results of this ground-breaking treatment and the second chance at life it brings. We are thrilled those other children will have access to this therapy."

In April 2022 the SMC also approved Atidarsagene autotemcel (Libmeldy®) under its ultra-orphan criteria

Another wonderful piece of news arrived in June when we were delighted that NICE approved Vimizim for treatment in England and Wales. The data that MPS Society and Rare Disease Research Partners (RDRP) collected and submitted was a huge factor in the committee saying yes.

NICE stated in a letter "...the patient submissions were very thorough and of the highest standard the committee has seen so far and the committee were not sure they could have asked for more. There was a view that the patient participation outshone the submission and participation from the company's for this evaluation."

Rare Disease Research Partners

RDRP put patients at the centre of their business to deliver unique research insights, outstanding support for people taking part in clinical trials and access to new treatments. They help patients with reimbursement, and provide expert rare disease communications. In 2022 the RDRP Clinical Trial Support Team supported rare disease patients and their families with 1889 clinical trial study visits worldwide and the Research and Medical Communications Team published 10 papers and posters.

Find out more: www.rd-rp.com

40 years of the MPS Society

2022 marked an important milestone for us as we celebrated our 40th birthday. That's 40 years of raising awareness and making an impact for all those affected by MPS, Fabry and related lysosomal diseases!

We began the celebrations with our 40th Anniversary Challenge. Members of our community came up with fun fundraising challenges based around the number 40. From doing 40 sit-ups to baking 40 blue cookies, we welcomed all ideas.

We selected a focus each month on all the things that make us special so we could look forward to a year of celebrations.

We selected a fundraiser of the month and shared their story: www.mpssociety.org.uk/our-resources/tags/fundraiser-of-the-month

January
We launched our 40th Anniversary Challenge which raised money throughout the year.

February
Rare Voices, our group of young people organised a quiz.

March
We focussed on the history of research at MPS Society and published a booklet on some important research milestones.

April
In Fabry Awareness Month our Fabry stories were read 731 times.

May
We launched our 40th anniversary video at a House of Commons event.

June
We focussed on our reason for being here, our members.Your stories were read by 929 people in June.

July
We returned to face-to-face events at Legoland, Windsor.

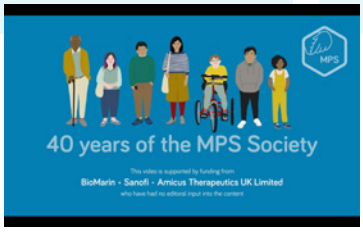
August
Our core, the Support and Advocacy Team were the focus this month.

September
We planted saplings at the newly renovated Wood of Remembrance and Hope.

October
We said thank you to the long standing friends who have collaborated with us and helped to define the work we do.

November
We could not let the year pass without honouring Christine Lavery in her birthday month.

December
We smashed our ambitious target raising over £28K in our Big Give Christmas Challenge.



Watch it here: www.mpssociety.org.uk/40th-anniversary

Find out more here: www.mpssociety.org.uk/christine-lavery

Awareness

Fabry Awareness Month

In April we celebrated Fabry Awareness Month. We wanted to highlight what life was like living with Fabry disease and raise awareness of this disease. We heard from a number of guest bloggers who spoke about living with Fabry, as well as discussing treatment options and sharing tips on managing symptoms. We also highlighted the extremely important link between heat intolerance and Fabry disease, something that is really important to understand when managing symptoms.

We also gave thanks to our fundraiser of the month, Steven Gill, who has been fundraising in aid of Fabry disease.

Steven Gill lost his dad to Fabry when he was 56 years old. Steven set himself an extraordinary mission to raise awareness, by doing 59 challenges to raise a whopping £20,000.

Steven's father Andrew was diagnosed with Fabry disease aged 48, a week before Steven's wedding. Although he had long experienced symptoms of the disease, their sporadic nature and lack of understanding meant it was hard to diagnose.

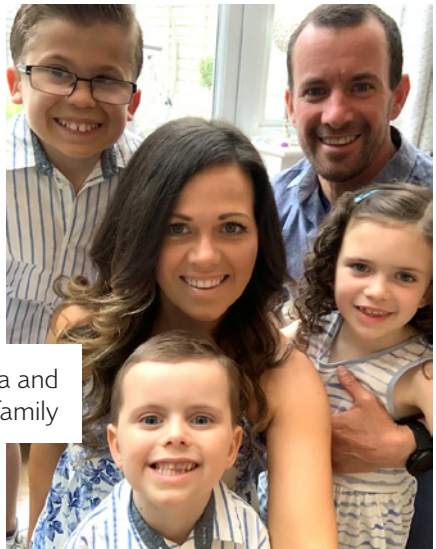
"It is always easier to join up the dots once the equation has been solved, but I suspect Dad, deep down, knew something was wrong," says Steven.

Steven and his dad

52K PEOPLE REACHED

66 NEW FOLLOWERS

£8K RAISED BY YOU



Katrina and family

MPS Awareness Week

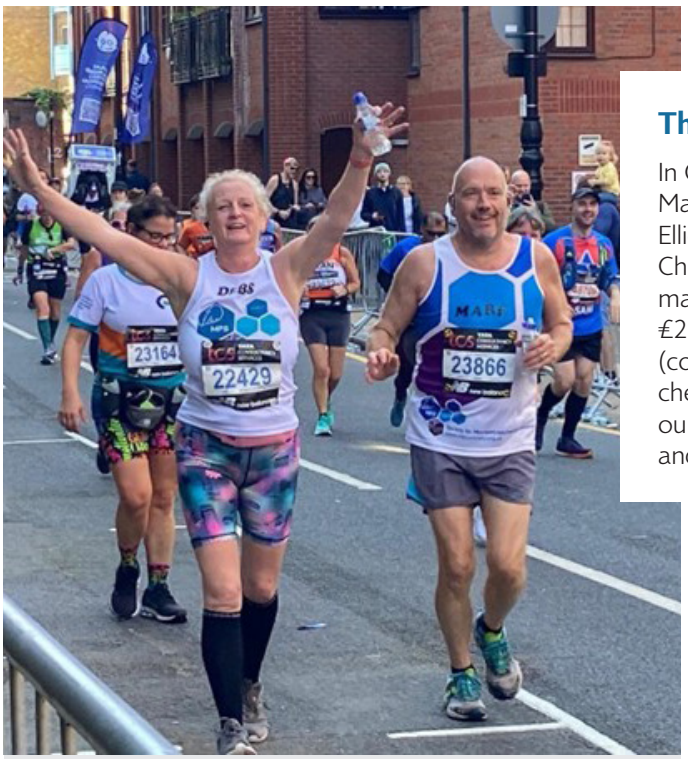
MPS Awareness week is always an important week in our calendar, and this year's was no exception. It took place between 9-15 May and we began the week with a trip to the House of Commons, to discuss the impact of our work and mark our anniversary. It also marked another milestone, as we celebrated ten years of Wear it Blue – an event started by Katrina Fanneran-Mullins, a member of our community and parent to Ethan who was diagnosed with MPS in 2011. This event has seen thousands of people around the world posting photos on Facebook and Twitter of themselves and their families wearing blue, in honour of all those with MPS.

MPS Awareness Week 2022 was a great success. We managed to spread awareness and reach so many people across the UK and globally. We reached 52,000 people over the course of the week with a combined 942,000 social media reach and an incredible 1469% increase in Instagram profile views.

Fundraising

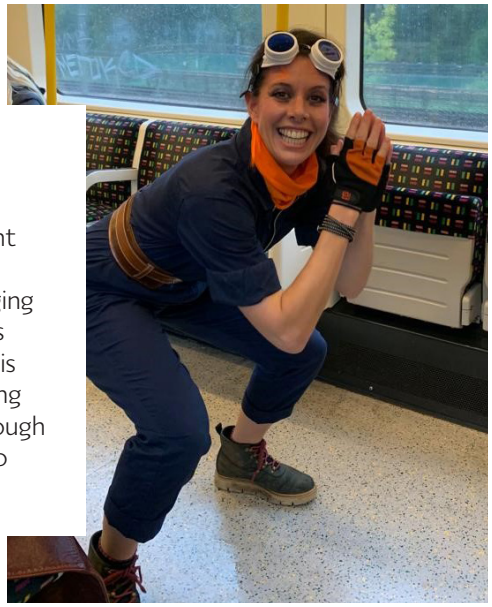
Our members are at the very heart of what we do. Helping our community with the day-to-day challenges they face is absolutely critical to our mission. We want to give them hope and support. It's a large part of why we exist.

In turn, we couldn't do what we do without their help so we want to say a huge thank you to everyone who has helped fundraise with us this year! Here are some of the many wonderful fundraisers and challenges that our community have carried out this year.



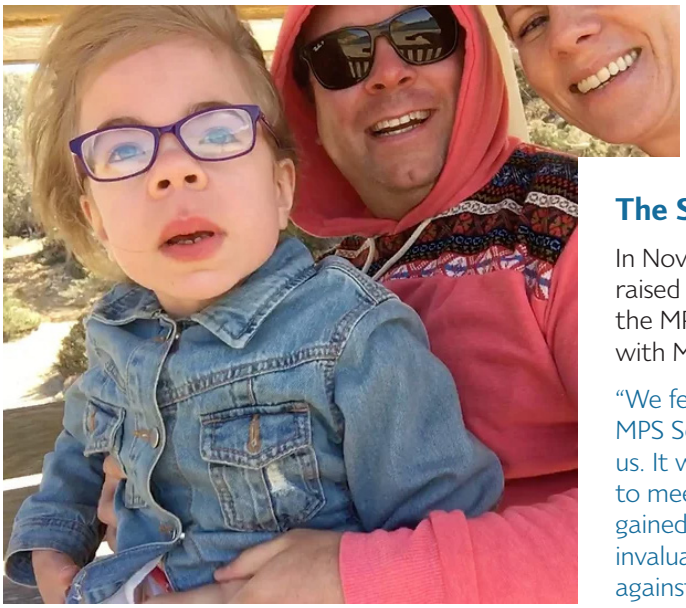
The London Marathon

In October, 13 amazing runners ran in the TCS London Marathon. Our 2022 team consisted of Luke, Shelley, Elliot, James, Ant, Mark, Debbie, Tim, Gemma, Adam, Chris, Duncan and Michael. Together they have raised a massive £18,598. With Gift Aid, their total is well over £20,000! The sun shone that day and our cheer squad (complete with kazoos and bright blue pompoms) cheered them all on as they sped past. Well done to all our runners and a huge thank you for your team spirit and dedication.



Facebook squats

In September our "2,000 Squats in September" Facebook challenge event got underway. Our amazing team of squatters made us so proud, challenging themselves to squat at least 66 times per day for the whole month, which is no mean feat. They raised a staggering £4,418 and we can't thank them enough for their hard work and dedication to this tricky challenge.



The Scott family

In November we celebrated the Scott family who have raised an incredible £50,000 for various charities, including the MPS Society, after their daughter Sophia was diagnosed with MPS IIIA Sanfilippo, seven years ago.

"We felt lost and isolated," says Sophia's dad Darren. "The MPS Society was there to provide guidance and support for us. It was always good to attend the MPS Society conference to meet with others in the same position and the knowledge gained from the medical and scientific updates were invaluable in helping provide knowledge and hope in our fight against this currently untreatable condition."

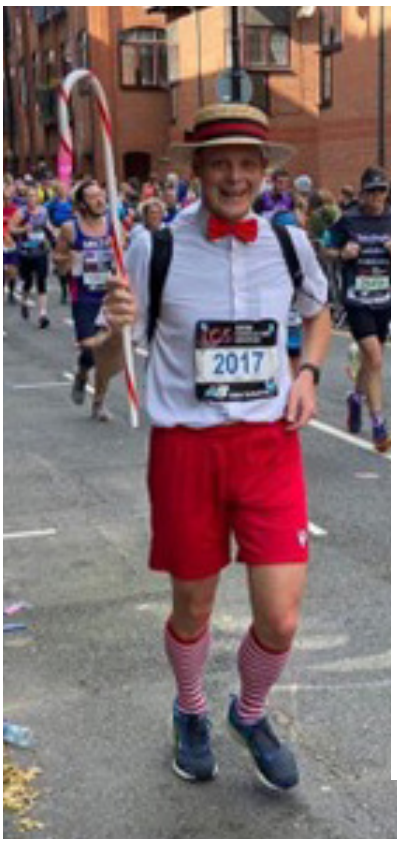
The Hampden Arms

Louise Lucas is the owner of the pub The Hampden Arms in Great Hampden, Bucks. Louise has been fundraising and raising awareness for MPS for many years now, after a close friend's child was diagnosed. The pub's fundraising quizzes have become a local must-attend event!

She says: "Our Boxing Day quiz has become a legend in its own right - with the pub full to bursting - some years customers have had to go home to get their own chair as we have been so busy!"

Towersey Morris men

In February, we celebrated the remarkable achievements of Andy Hardy and the Towersey Morris men, of which Andy is a member. The group started to raise money for research into MPS when Andy's son Matthew, who had MPS II Hunter, was a boy. Matthew died in 1994 aged 13. After his death, Andy joined the group and became a member of the Haddenham Mummers, too. The Towersey Morris men and Haddenham Mummers have raised thousands of pounds for the MPS Society over the past 35 years.



Duncan Clarke

When Duncan's, niece, Lilia, was diagnosed with MPS III last June, it took her friends and family a long time to come to terms with her newly-diagnosed disease.

Not one for sitting around, Duncan set himself a "crazy challenge" of completing 12 marathons in 12 months to raise funds for the MPS Society.

Duncan says: "The MPS Society has provided a massive amount of support to my brother, Jonathan's family, providing workshops, counselling, and sibling support. They have even given presentations at Lilia's school to raise awareness."

Grants and trusts

We are so grateful for all the grants we've been awarded this year and the support they have meant for our community. Thank you.

National Lottery Community Fund award

We are now in the second year of this fantastic three-year grant from the National Lottery Community Fund. The grant from the National Lottery is helping us to deliver our project "Rare but not alone – community, connection and peer-support". We are helping our community to build connections through this project from April 2021 to March 2024. Our project aims to create a more connected MPS community by providing support to families, opportunities to build relationships and encouraging peer support.

We are achieving this through activities under four headings:

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

We are very grateful to the National Lottery Community Fund for this substantial multi-year grant which is helping us to make such a difference to the lives of the MPS and related diseases community.

The Masonic Charitable Foundation grant

Our much-appreciated two-year grant from the Masonic Charitable Foundation came to an end in February. This grant focused on helping our young members make key life transitions. We were very pleased with everything we were able to achieve with this funding, helping young people in the MPS and related diseases community. A huge thank you to the Masonic Charitable Foundation on behalf of everyone we have been able to support!

Gosling Foundation

In June we received a substantial donation from the Gosling Foundation to go towards essential website and communications upgrades. We are planning to upgrade and redevelop the MPS Society website, making sure it is easy to navigate, understands the user's journey and offers suggestions for what they may be looking for, so it will be quick and easy to find relevant information. We were delighted to receive this grant from the Gosling Foundation towards this vital project which will benefit everyone who uses our website.

ON AVERAGE

3487

PEOPLE VISIT OUR WEBSITE EACH MONTH

Research

£214,455
AWARDED IN RESEARCH GRANTS

The MPS Society's Clinical and Scientific Advisory Committee have reviewed an overwhelming number of research grant applications over the past year, the highest volume that we have ever seen.

It is an amazing achievement for the MPS Society to have provided so many grants to support some brilliant studies that will no doubt have huge benefits to our patient community and it is a very exciting time for research into MPS and related lysosomal conditions, with a very active community of scientists and clinicians always looking to understand and discover more and more.

In 2020 two projects were awarded funding but were delayed due to Covid and it seems that things are fortunately starting to move forward now:

- Evaluation of a Digital Microfluidics Platform for Rapid Assessment of Lysosomal Enzyme Activity in Dried Blood Spots.
- Does Hydrotherapy alleviate pain and improve functional mobility in patients with Mucopolysaccharidosis?

Lysosomal Disorders: Covid-19 impact – Home infusions and patient wellbeing: patient survey

During the pandemic, over 90% of out-patient appointments were converted to virtual clinics. Over 50% of our patients had to interrupt ERT at home, as there were concerns with home care nurses attending the patients' homes during the lockdown period. This project proposed a short survey in the first instance to obtain a single time point information of patient wellbeing during the pandemic. The team plan that the survey will enable a more objective measure of quantifying the unmet psychological needs of LSD patients.

How can we Prognosticate airway and Respiratory functiOn decline in MPS IVA? From 'bIg data' in rare diseases to preciSion medicinE (PROMISE)

This project aims to expand an existing international registry, using data on individual patients' disease progression and treatment response to help doctors predict future negative outcomes before they happen, thereby allowing treatment decisions to be more informed. The team will develop a mathematical model using existing and new prospectively collected data to calculate the risk of worsening airway and lung problems in people with MPS IVA. They aim to build upon the work that we have pioneered in the area including 3D tracheal reconstructive modelling, virtual endoscopy and augmented reality, with the ambition to extend this technology to create an airway database of all children with MPS-IVA.

Physical activity and sedentary behaviour in the enhancement of mental health and quality of life in Fabry disease

People with Fabry disease often suffer from multiple medical conditions, physical disabilities, mental health problems and lower quality of life. Being physically active and sitting less are linked to better mental health, but there is not any research on this in individuals with Fabry disease. This research involves a study to look at whether physical activity levels and sitting time are linked to mental health (depression and anxiety) and quality of life in adults with Fabry disease.

How effective is a virtual psycho-education group for adults with Fabry disease in improving self-reported wellbeing?

Higher rates of mental health issues are reported in the Fabry disease (FD) population than in the general population. These are associated with greater adaptive functioning issues, self-reported cognitive complaints and pain. There is some evidence to suggest that psychological interventions can help. Lower intensity psychological interventions such as CBT groups are effective in treating anxiety and depression in patients with chronic health conditions. Group interventions have advantages over individual therapy in providing opportunities for normalisation and patient knowledge sharing, and increased efficiencies for services. This study involves the development and piloting of a virtual, group, psycho-educational package for the management of psychological symptoms of Fabry disease, and quantitative and qualitative analysis of its efficacy.

We are also reviewing a further four applications currently so feel so inspired by the great work that is going on in the research community.

We launched the Christine Lavery Memorial Fund on 21 November, Christine's birthday, to honour her memory and dedication to research and improving the lives of our community. The fund will go towards supporting a university student to undertake a summer vacation scheme on a research project that will study an MPS or related disease.

Find out more:
www.mpssociety.org.uk/for-professionals

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