MPS Matters 2025

Insights and IMPACT

MPS Society

transforming lives through support, research & awareness

Introduction

I wanted to approach the conference differently this time round. As we hadn't had one for so long it was a good opportunity to reflect on what our community wanted and needed from a meet up. I was able to think back to how nervous I felt attending for the first time as a dad of two boys with MPS II Hunter disease and to know that what I needed was to feel welcome. And boy did my team deliver. The feedback from those who attended was so positive. People felt welcome, people felt joy, people felt at home. Moreover, you could feel the camaraderie and buzz of a successful event.

Bob Stevens,
Group CEO

This report highlights the impact made by the event and the results of the various data collection carried out.

332 people attended the MPS Matters 2025 community weekend including:

- **143** community members
- **98** professionals
- **53** children
- **20** volunteers

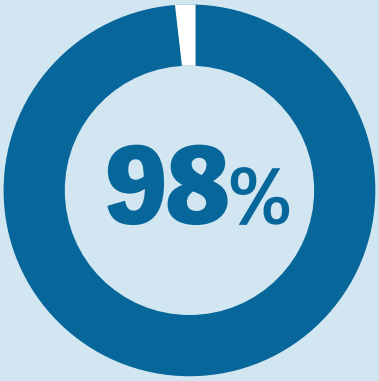
There were three tracks ...

- for parents/carers (18 sessions)
- for independent adults (13 sessions)
- for professionals (7 sessions)

We put on ...

- **28** presentations
- **9** workshops
- **1** expert meeting

And a research-focused dragon's den style feature and poster session



of respondents **thought the conference was well organised** (out of 41 responses)

Which covered topics like ...

Clinical updates | Taking ownership | Future choices | Identity and relationships

We used **Whova** to manage the agenda and share information. The app had:

- **189** users
- **158** private messages
- **636** event and session poll responses
- **152** community board messages

How people felt

"Everything was so thoughtfully put together, and the energy, creativity, and sense of community were truly inspiring. It was great to be involved, and I'm grateful for the opportunity to connect and learn from this wonderful group of people." *Speaker*

"Overwhelming is the top word, but everybody has made us feel so welcome. We haven't met other families like ours and this is so nice." *Parent*

"Meeting other families and having our own time. The childcare was amazing." *Parent*

"Meeting other families and being able to join sessions on topics which can help my son and help my understanding. Being able talk to the experts was also very helpful." *Parent*

"The conference was a lifeline, which we sorely missed. It made my daughter feel accepted by others, we also felt the lack of judgement from others, which means more than everything." *Parent*

Thank you to our volunteers

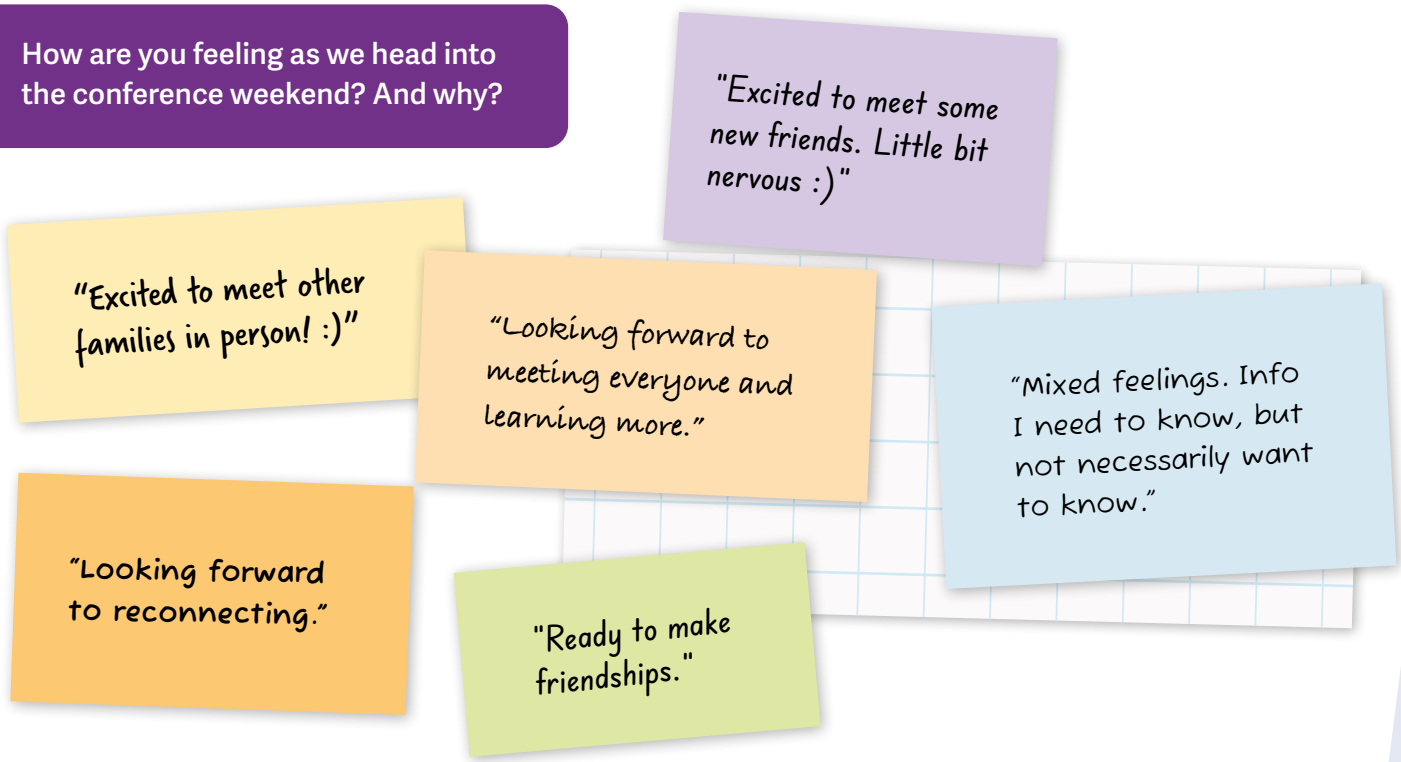
"It was an absolute privilege to attend as a volunteer, and as a bereaved MPS IIIA parent, it meant so much to be given the opportunity to care for two wonderful siblings living with the same condition as our daughter. It was an emotional but deeply meaningful experience, and I'm so grateful to have been part of it. *Volunteer*

Feedback for the weekend

We asked attendees to share how they were feeling when they arrived at the beginning of the weekend and again at the end of the weekend via mood boards at the entrance.

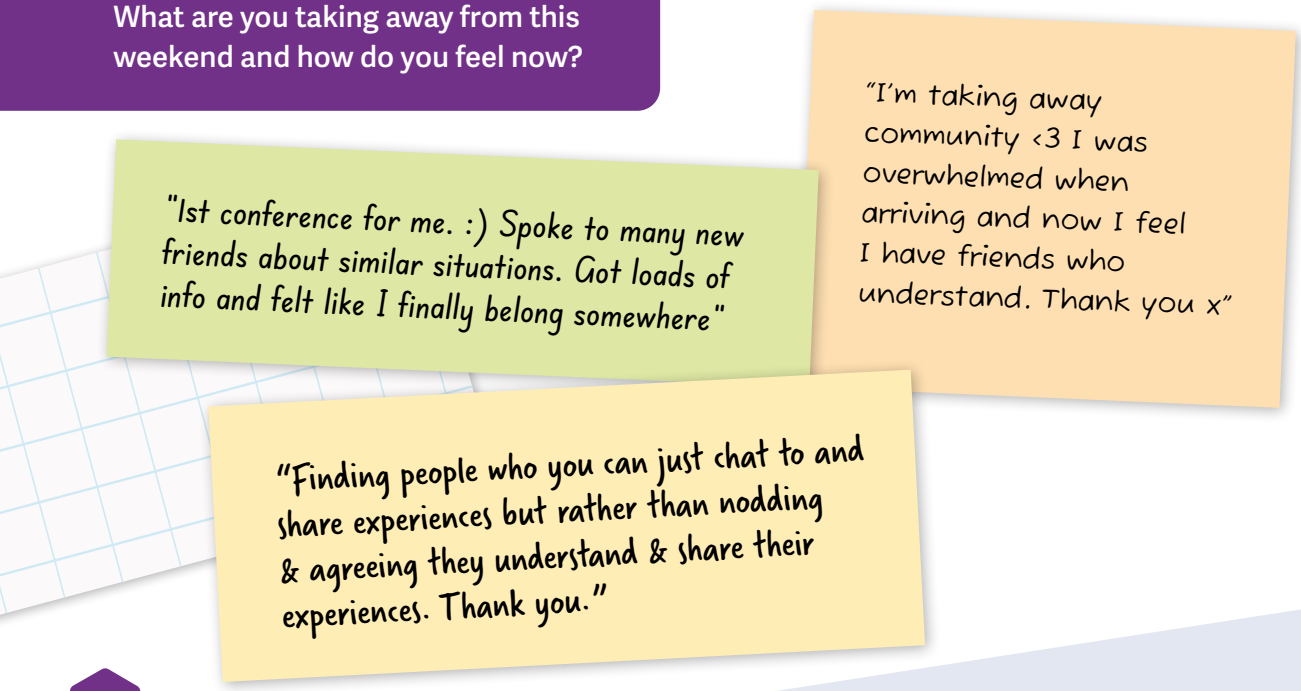
On arrival, the general sentiment was one of excitement and happiness, with some experiencing mixed feelings of nervousness or apprehension.

How are you feeling as we head into the conference weekend? And why?



When attendees left, they shared their feelings on the weekend. Their reflections were of **belonging**, **connection**, **joy** and **gratitude**. Many mentioned **friendship** and the **community spirit**, with standout experiences like the Build-A-Bear workshop and the Dads and lads session.

What are you taking away from this weekend and how do you feel now?



Research results

Results from online polls, surveys and post it note boards gave us some insight into what you were thinking and feeling about some of the most important matters to you.

Apart from a cure, what would you most like to see in the future for those affected by MPS?

"Better MDT working with health, education and social care. Early mental health support for families and on to adulthood."

"I would like more doctors or communities to be aware so it's easier for people to adapt when seeing someone with MPS."

"Mandatory training for healthcare professionals in rare diseases"

"Shine the lights to the world to see rare disease population can be empowered and making difference! Disabled but unstoppable <3"

What one thing would make your day-to-day life with MPS easier?

"For people to not speak to me like I am a child. To not have an infusion and to have friends would make it easier with having a condition."

"No bullies or mean people"

What does quality of life mean to you or your child?

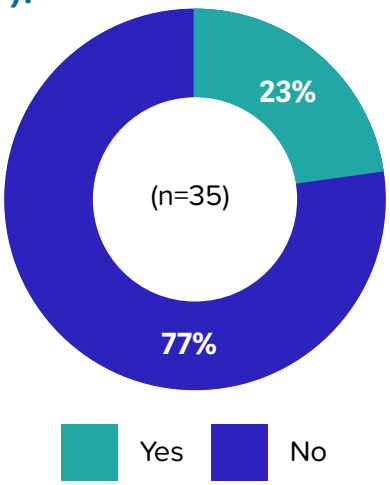
Top 5 asks of gene therapy

1. Pain-free and mobility improving treatments that are less invasive.
2. Access to gene therapies with cognitive and physical benefit, especially those that cross the blood-brain barrier.
3. A more compassionate and informative clinical trial process, including mental health support.
4. Clear, simplified information about trials and treatments, delivered early and in multiple formats.
5. Recognition of quality of life as a holistic goal that includes happiness, independence, and social connection.

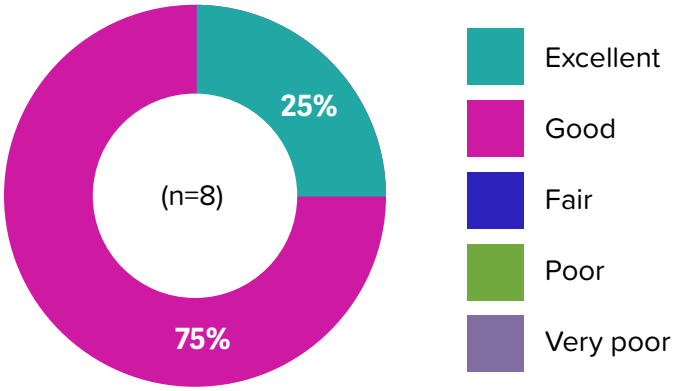
Rare Disease Research Partners Survey Results

PARTICIPATION IN RESEARCH

Have you ever participated in research with Rare Disease Research Partners (RDRP)?

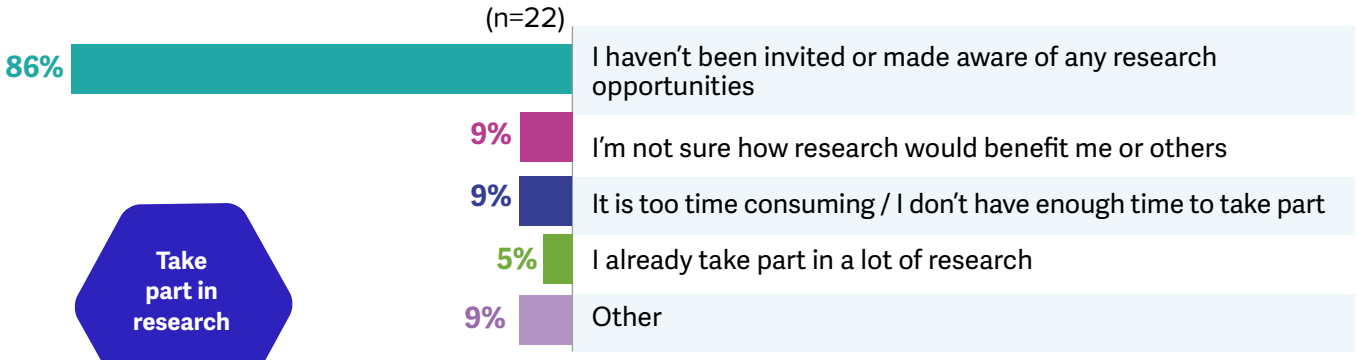


How would you rate your overall experience of participating in research with RDRP?



All 8 participants that have taken part in research with RDRP have had a **good** or **excellent** experience with us.

Why have you not participated in research with Rare Disease Research Partners (RDRP)?



The majority of participants responded that they **had not been invited or made aware of research opportunities**.

Take part in research


Rare Disease Research Partners

We know that you may have been asked many times to take part in research activities such as surveys, interviews and focus groups. These can feel time consuming, confusing or repetitive. You may even ask yourself: Is this really making a difference? The answer is, YES.

Why take part in research?

Help shape the future of Rare Conditions

There are more than 7000 rare conditions with more being identified all the time - your experience, whether as a patient or a caregiver, is incredibly valuable. 95% of rare conditions have no approved treatment. It can take 4-8 YEARS to receive a diagnosis.[2]

1 IN 17 people will be affected by a rare condition during their lifetime.[1]

1.Genetic Alliance: Facts and Figures. [Accessed 5 June 2025]. Available from https://geneticalliance.org.uk/campaigns-and-research/facts-and-figures/
2.The Lancet Global Health. The Landscape for Rare diseases in 2024. The Lancet Global Health. 2024;12(3): E341. Available from https://www.thelancet.com/journals/langlo/article/PIIS2214-109X(24)00056-1/fulltext

It can be especially hard to find participants for these types of research in ultra-rare conditions. If you are interested in taking part in research and are happy to be contacted by Rare Disease Research Partners with opportunities to do so, please scan the QR code to sign up.

SCAN ME!

One of the main barriers in rare condition research is understanding how the condition progresses over time and its impact on the daily life of the person with the condition and their families. This gap in understanding makes it more difficult to prove the benefits of treatments, affecting their chance of being approved for use and made available on the NHS.

By taking part in research and sharing your experience, even for just a few minutes in a short survey, you are helping build the knowledge which is essential for:

- **Faster diagnosis** – by telling us about early symptoms and patterns
- **Improved symptom management** – by sharing what matters most in the patient's everyday life
- **Developing better treatments** – by highlighting unmet needs
- **Support for families and caregivers** – by helping us understand what caregiving involves

www.rd-rp.com
services@rd-rp.com
0345 260 1087

Help shape the future of rare disease care.
Your story can drive real change!

Please note that Rare Disease Research Partners does not conduct clinical trials – if you are interested in taking part in a trial speak to your disease specialist.

Research opportunities are published in the MPS Society monthly newsletter or via direct emails or posted within the WhatsApp support group.

Find out more at **www.mpssociety.org.uk/research-database-reg**

If you are interested in taking part in future opportunities, please contact **services@rd-rp.com**.

Please note that all research is voluntary and you are not obliged to take part in all or any research opportunities sent to you.

PATIENT, CAREGIVER AND HEALTHCARE PROFESSIONALS (HCP) PERSPECTIVES

We asked patients, caregivers and HCPs what they thought was the most challenging aspect of MPS or a related lysosomal condition.

Patients
Dealing with their symptoms and mobility issues.

Caregivers
Care responsibilities, symptom management, feeling helpless and a concern for the future.

HCPs
For **patients** their symptoms, the care burden on themselves and their families.
For **caregivers** the progression of disease.

Caregivers reported that their **biggest concern** when caring for someone with MPS or a related lysosomal storage condition was a concern for **the future.**

HCPs find the **most difficult conversations** to have with their patients are the conversations **around disease progression**.

To help support these conversations, HCPs would like more support with

- Better visual materials
- Input from palliative care colleagues
- Psychological support for patients

If HCPs could help their patients better understand one key aspect of their MPS or related lysosomal storage condition, it would be disease progression & the patient journey.

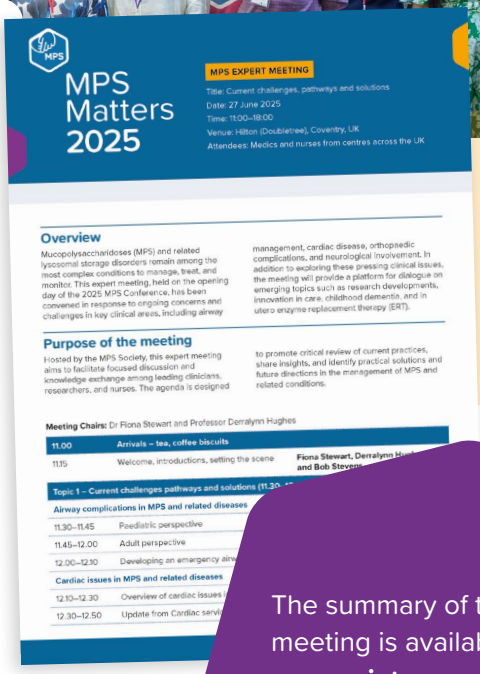
Participants with MPS or a related lysosomal disease find it **moderately, very or extremely important** to receive a plain language summary of the research results.

Expert Meeting

The Expert Meeting has received positive feedback. Speakers described it as an excellent and memorable day, valued not only for the rich discussions but also for the chance to reconnect with colleagues, the society and patients.

The summary report was equally well received, praised as a clear and useful record of the meeting's outcomes with some already putting it to work in their professional settings.

"Thank you for hosting and inviting me to the esteemed MPS matters conference. I thoroughly enjoyed reconnecting with the Society, colleagues, and, most importantly, the patients. It was an unforgettable experience and I learnt so much."


MPS Matters 2025

MPS EXPERT MEETING

Title: Current challenges, pathways and solutions
Date: 27 June 2025
Time: 11:00–18:00
Venue: Hilton (Doubletree), Coventry, UK
Attendees: Medics and nurses from centres across the UK

Overview

Mucopolysaccharidoses (MPS) and related lysosomal storage disorders remain among the most complex conditions to manage, treat, and monitor. This expert meeting, held on the opening day of the 2025 MPS Conference, has been convened in response to ongoing concerns and challenges in key clinical areas, including airway management, cardiac disease, orthopaedic complications, and neurological involvement. In addition to exploring these pressing clinical issues, the meeting will provide a platform for dialogue on emerging topics such as research developments, innovation in care, childhood dementia, and in utero enzyme replacement therapy (ERT).

Purpose of the meeting

Hosted by the MPS Society, this expert meeting aims to facilitate focused discussion and knowledge exchange among leading clinicians, researchers, and nurses. The agenda is designed to promote critical review of current practices, share insights, and identify practical solutions and future directions in the management of MPS and related conditions.

Meeting Chairs: Dr Fiona Stewart and Professor Derralynn Hughes

11.00	Arrivals – tea, coffee biscuits	
11.15	Welcome, introductions, setting the scene	Fiona Stewart, Derralynn Hughes and Bob Steven

Topic 1 – Current challenges pathways and solutions (11.30–

Airway complications in MPS and related diseases

11.30–11.45	Paediatric perspective
11.45–12.00	Adult perspective
12.00–12.30	Developing an emergency airw

Cardiac issues in MPS and related diseases

12.10–12.30	Overview of cardiac issues
12.30–12.50	Update from Cardiac servi

The summary of the expert meeting is available at **mpssociety.org.uk/expert-meeting-summary**

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The Gosling Foundation · Est. 1985 ·

ALEXION AstraZeneca Rare Disease

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