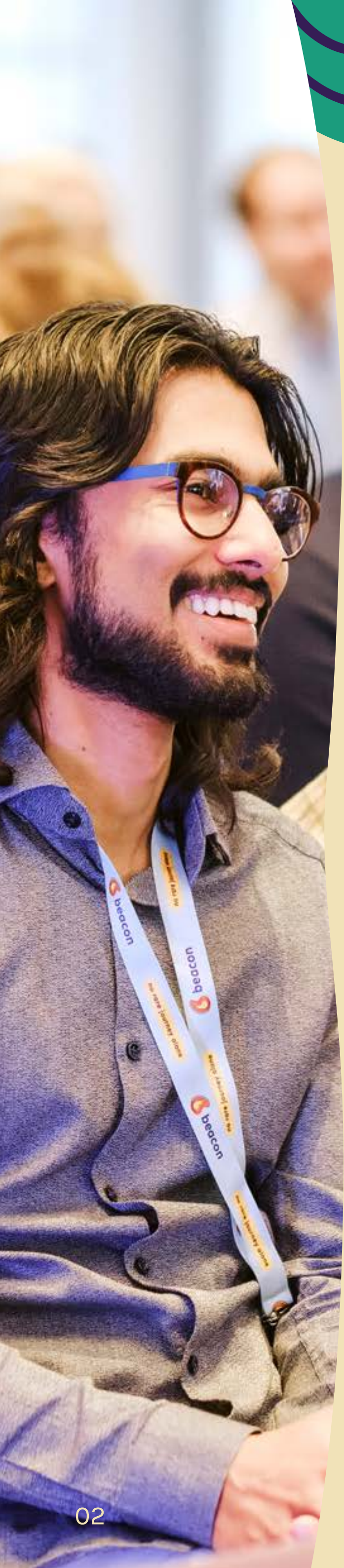
beacon
for rare diseases
no rare journey alone

Annual Review

2025



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Rick Thompson

There have been a lot of landmark achievements at Beacon over the course of 2025, each of which have helped to underscore our increasing reach and impact as a charity.

Working within the REMEDi4ALL consortium, we delivered our first ever training event for academics; helping researchers across Europe understand the end-to-end process of drug development and the central role of patient engagement.

The Manchester Rare Disease Showcase, our largest UK conference outside of London, saw the announcement of the extension of the UK rare disease framework by Ashley Dalton MP. Our diverse audience heard powerful patient stories, and in-depth discussions on the importance of diversity and inclusion in the rare disease agenda. It was a powerful event that really reminded me of the vibrancy and dedication of the rare disease community.

The success of the Showcase has really been mirrored across the year. We've seen huge growth in the users of our Resources Hub, over 280 delegates at our international drug repurposing conference, and some hugely engaging and successful patient group training initiatives. All this has allowed Beacon to support 319 patient groups in 2025—with each of these groups going on to transform the lives of those affected by rare conditions.

It is this direct impact on those isolated by rare conditions that is most important. I had the privilege of celebrating ten years with this charity in May 2025, and while it was humbling to receive many messages from across the community, I was most proud to reflect on the support we have delivered to the community in that time—and the transformative work our patient groups have achieved.

I can't wait to see what you all do in the next decade.

About Beacon

Our vision: A world in which no one faces their rare disease journey alone.

Beacon is a UK charity building a united rare disease community with patient groups at its heart.

Rare diseases affect between 3.5% and 5.9% of the global population — millions of people who struggle to receive a diagnosis, access timely and relevant information, obtain treatment, or even meet someone with the same condition.

Patient groups step in where healthcare or society leaves gaps; becoming a lifeline for these communities through emotional support, practical advice, and driving the development of new care pathways and treatments. Yet, many face significant challenges: **limited funding, reliance on volunteers, risk of burnout, and barriers to being recognised as equal partners in healthcare and research**.

Beacon exists to help these groups be the best they can be. Through training, guided programmes, community projects, and research initiatives, we upskill patient organisations, helping them grow, professionalise, and maximise their impact. Our projects bring together patient groups, researchers, healthcare professionals, and industry, creating opportunities to collaborate, share knowledge, and advance the field of rare diseases.

From our events, online resources, and international collaborations, Beacon equips, empowers, and connects those working to improve the rare disease experience. Through these efforts, we are breaking isolation, building confidence, and uniting the rare disease community into a stronger, more empowered, and collaborative force.


I ♡ Beacon because...
They are an empowering Support
network for the rare disease
Community. Giving us valuable
insight and connections to
training and opportunities small
Charities like ours Struggle with

Patient groups matter because...
We Champion our Community
with compassion, lived experience
and knowledge, to help bridge
the gap between diagnosis
and treatment.

I'm a rare disease advocate because...
I want to be part of the
Collective voice, to give
rare disease's the platform
they deserve.

Quick facts about rare diseases

In the UK and EU, a disease is considered rare if it **affects fewer than 1 in 2,000 people**

Rare diseases affect more people than cancer and AIDS combined

There are over 7,000 known rare diseases, yet only a **small fraction have licensed treatments or coordinated care**

The average rare disease patient receives **3 or more misdiagnoses**

Impact in action

Year at a glance

856 attendees at events

319 patient groups supported

27 live projects delivered

19 e-learning resources published

Masterclasses: **61**

Webinars: **97**

In 2025,
we welcomed
236 attendees
across patient group
training projects

Workshops: **36**

Mentoring: **42**

> Beacon's training is always spot on, delivering what small charities need at the time they need it. Their facilitators are always brilliant.
> Expert and approachable.
> Quality and usefulness assured!
> You know it's going to be good if it has Beacon's logo on it.

Timeline of the year

January

Launch: REMEDi4ALL Digital Academy

Webinar: Influencing research funding success

Resources Hub: 10 key platforms patient groups can use to enhance their capabilities

Webinar: Welcome to the REMEDi4ALL Digital Academy

February

Masterclass: Imposter syndrome: how to turn your inner critic into your biggest cheerleader

Webinar: Introduction to clinical trials

Webinar: Clinical trials for rare diseases

March

Webinar: Support and management of clinical trials

Masterclass: Launch of leadership masterclass

Masterclass: Conquer public speaking in London

Resources Hub: Imposter syndrome: how to turn your inner critic into your biggest cheerleader

Resources Hub: Introduction to prenatal screening

Resources Hub: Understanding the role of a genetic counsellor

Resources Hub: Patient group structures: finding the right fit

Resources Hub: Developing a peer support programme

Resources Hub: Creating inclusive and accessible events

Academic Bootcamp: from hypothesis to impact: navigating the repurposing pathway in Cambridge

Published: “Drug repurposing in Pancreatic Cancer” (Eve Hewitt) in Cancer Management and Research

April

Webinar: Partnering with academics

Ext: MayRo sits on the Jeans for Genes funding panel

Ext-Event: Rick gives a talk at the MRD and SITR pre-training webinar on the REMEDi4ALL drug repurposing initiative

May

RareChat: Strategic planning and sustainability

Conference: #iDR25: Medicines Reimagined in Amsterdam

Masterclass: Building capacity with AI

Celebration: Rick celebrates 10 years at Beacon

June

Masterclass: Networking with confidence in London

Showcase: The Cambridge Rare Disease Showcase

Patient Rep. Bootcamp: Understanding drug repurposing in Barcelona

July

Mentoring: Closing event for 2024–2025 cohort

Webinar: Funding call: Clinical trials to validate AI-driven drug repurposing

Celebration: Blayne celebrates 5 years at Beacon

August

Ext-Event: Megan gives a talk on how patient collaboration can drive rare disease research at a webinar for the LifeArc rare respiratory centre

September

Organisation: Welcomed 22 new members to our Patient Group Engagement Committee

Masterclass: Crafting content that captures your audience

Ext-Event: Megan and Rick attend RExPO in Barcelona to present on drug repurposing, patient engagement and training within REMEDi4ALL

Published: “Bake off star Andy Ryan reveals rare KTWS diagnosis” (Niamh Antrobus) in Media Planet’s Rare Disease supplement

October

Ext-Event: Hannah and Elizabeth attend WODC in Amsterdam where Hannah presents on industry-patient collaborations

Podcast: Launch of Rarely Heard Podcast Season 3

Workshop: Let’s get down to business in London

Hackathon: REMEDi4ALL repurposing hackathon for early career researchers in Barcelona

November

Workshop: Patient voice in clinical research

Showcase: The Manchester Rare Disease Showcase

Webinar: An introduction to rare diseases

Webinar: IP strategics for repurposing

Published: “Creating Connections” (Blayne Baker) in Rare Revolution Magazine

December

Webinar: Translational research for rare diseases

Organisation: Welcomed 5 new trustees

Scott Hutchings

Founder, CHAMP1 UK

Scott entered the rare disease world when his daughter Sienna was diagnosed with a CHAMP1 disorder, just before her third birthday in 2022. The diagnosis came as a letter in the post, with no further information, leaving Scott feeling like there was nowhere to turn. He began engaging with the US CHAMP1 Research Foundation, before going on to establish CHAMP1 UK.

"After I registered our charity, I remember thinking, What next? How can I get more involved in the rare disease world to help those with CHAMP1?"

Scott discovered Beacon while searching for support and connection.

> **Their website talked about the community and why patient groups matter. What really prompted me to reach out was their mission of ensuring that no one faces their rare journey alone. That really resonated with me, because that's exactly why I started CHAMP1.**

His engagement with Beacon quickly developed. His first conversation with Blayne helped make the rare disease landscape feel less daunting, and in 2024 he attended the Cambridge Rare Disease Showcase.

"It was a great chance to network and learn. Seeing people who were in my shoes five years ago presenting what they had achieved was amazing. I still speak to people today that I met at that event.

Since then, Scott has built his knowledge and confidence through Beacon's training and opportunities. He took part in the REMEDi4ALL drug repurposing bootcamp in Spain and, with a Beacon bursary, attended the 2025 International Drug Repurposing Conference in Amsterdam.

This was particularly valuable as drug repurposing is an area of interest for CHAMP1, enabling Scott to actively contribute to conversations with researchers and experts rather than simply observe.

> **Beacon are at pretty much every rare disease event I've ever been to, and it always feels like if Beacon is there, I'm in the right place.**

In 2025, Scott also took part in Beacon's Leadership Masterclass, where he developed his confidence as a leader within the rare disease community and tackled imposter syndrome.

> **I hate public speaking; the thought of standing up and talking about CHAMP1 used to fill me with dread. But through the coaching, the training, and pushing myself outside my comfort zone, Beacon helped me find my voice.**

This journey culminated in Scott presenting at the Cambridge Rare Disease Summit, where he shared his story and raised awareness of CHAMP1 with a room of researchers, clinicians and advocates.

"It's something I never thought I'd be able to do, let alone feel comfortable and confident doing it."

Scott's growing confidence also led him to apply to become a Beacon trustee.

"Initially, I thought, 'they're never going to want me'. But through the coaching, I started to recognise what I had to offer."

Having long recommended Beacon to others in the rare disease community, stepping into a trustee role felt like a natural next step.

> **Ever since I reached out to Beacon, it's felt like having someone in your corner.**

Patient group training

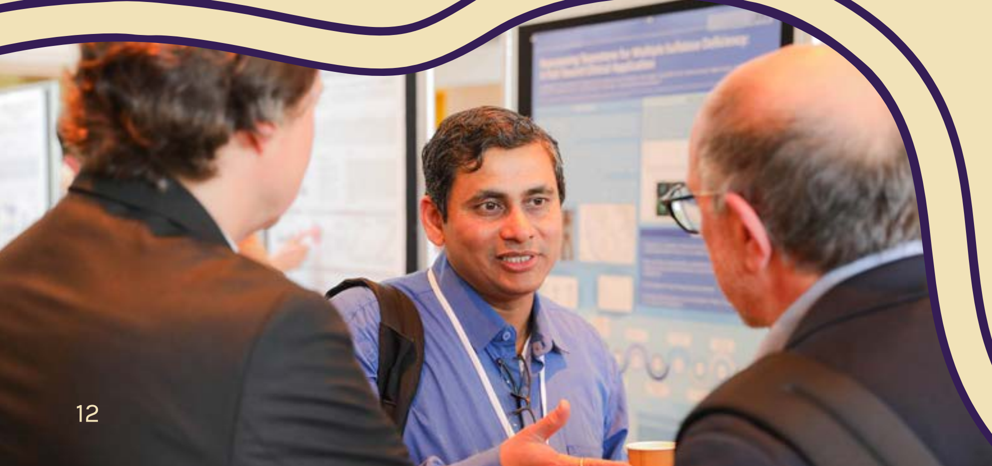
Business planning workshop

In October, we welcomed over 20 patient group leaders to London for a workshop focused on organisational sustainability. We highlighted the importance of business planning and demonstrated how leaders can use it as a tool to record their impact, use their resources effectively and communicate to funders and partners.

Four speakers with extensive experience across small rare disease charities helped make this topic accessible and directly relevant to our community. They worked individually with leaders to help them create their own business plan on the day, ensuring they left with a tangible resource they could start using straight away.

The workshop provided rare disease leaders with valuable time to reflect on the long-term sustainability of their groups, something that is often difficult given their limited resources. Alongside the practical support, leaders had the opportunity to network throughout the day, helping to strengthen connections, learning and support across the community.

- **93%** of attendees said that attending the workshop has increased their sense of belonging to the rare disease community
- **87%** of attendees said they came away with practical skills and tools



> Beacon have done it again—set at the right pitch, with enough gentle support to make the process feel achievable

> Thank you all, without your help our charity would not exist

> A very useful event to grow a better understanding of strategy planning. Helpful and friendly people at Beacon

> Fantastic event and so valuable, thank you

Patient group training

Clinical trials workshop

In November, we hosted a **three-part online workshop** designed to **support rare disease patient group leaders** in **understanding clinical trials** while **strengthening their role in research**.

The training focused on building confidence, demystifying clinical research, and equipping patient groups with practical tools to meaningfully engage with trials across their lifecycle. Through **three interactive sessions**, **13 expert speakers**, and **real-world case studies**, the workshop highlighted how patient insight can help shape research that is both scientifically robust and relevant to the patient community.

26
attendees

- **78%** of participants reported an increase in knowledge following the workshop, with an average increase of **22%**
- **100%** of respondents said the workshop met their expectations, was relevant to their organisation, and worthwhile to attend

> “Beacon’s workshops are always on point for the needs of the patient group community. Their access to a range of experts within the field is immeasurably valuable and speakers are always of a high quality with a depth of practical experience. Beacon is an invaluable resource.”

Resources Hub

Developing peer support programmes

Peer support plays a vital role in helping patient groups build connections, reduce isolation, and improve wellbeing for people affected by rare diseases. However, many patient organisations lack the guidance, capacity, or confidence to establish peer support programmes in a meaningful and sustainable way for their communities.

To meet this need, we developed a comprehensive guide to support patient groups in designing, delivering, and evaluating peer support programmes. The guide provides practical guidance on programme models, safeguarding and confidentiality, peer-support recruitment and training, goal setting, funding, and impact measurement.

Importantly, this resource was developed through a collaborative community effort, with contributions from a Beacon volunteer and three rare disease patient groups who shared their first-hand experience of running peer support initiatives. Their insights are reflected throughout the guide as real-world case studies. Thanks to this collective effort, rare disease patient groups now have access to a clear framework and practical guidance to build meaningful, community-led programmes that strengthen connection, resilience, and wellbeing across the rare disease community.

- **100%** of feedback respondents agreed the guide increased their knowledge and understanding

> “This training was immensely helpful. Thank you so much!”

Resources Hub

GDPR guide

Many patient group leaders find the topic of data protection and General Data Protection Regulation (GDPR) daunting. Rare leaders know that their group must be GDPR compliant, but often don't know what that means or entails for their organisation.

Our top-tip guide, created with pro bono support from Costello Medical and Bristows LLP, was designed to help UK-based charities explore how data protection may affect their operations and how they can better manage GDPR. Our guide has helped groups understand their data protection responsibilities so they can use their community's data safely and securely.

When asked if the guide increased their knowledge and understanding of GDPR, users of the Resources Hub stated "**Strongly agree**," remarking:

> Really clear and well laid out and takes away the overwhelm of GDPR's reputation!

Community

Rarely Heard Season 3

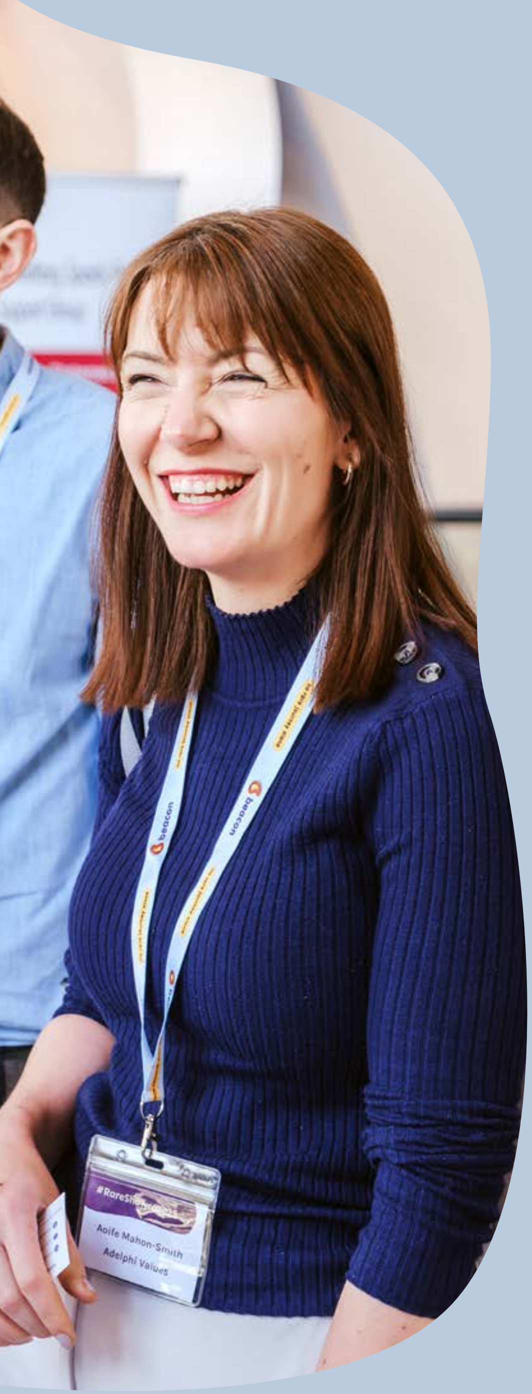
Season 3 of the Rarely Heard Podcast explored some of the most relevant themes facing the rare disease community today.

Featuring patient group leaders, clinicians, researchers and industry representatives, the season highlighted the role of specialist services in delivering care, the importance of reaching underrepresented communities, and the complexities in collaboration between patient groups and industry.

Across all episodes, Season 3 emphasised the value of lived experience and meaningful partnerships in shaping care, research and innovation in rare diseases.

170+ listens in **Rarely Heard** since we released Season 3.

> Each episode was fantastic with incredible speakers and beautifully facilitated.
>
> There is so much for my industry to learn especially from the latest episode 3!



Community

Manchester Rare Disease Showcase

In November, Beacon returned to Manchester to host another Rare Disease Showcase, becoming our second-largest UK event to date! The event brought together the full spectrum of the rare disease community – patient groups, researchers, clinicians, decision makers and life science professionals – creating a welcoming space for learning, collaboration and connection.

The programme featured more than 20 speakers exploring some of the most pressing opportunities and challenges in rare diseases today, from shaping policy and championing young voices in healthcare and research, to AI and digital innovation, tackling health inequalities, and the power of patient advocacy. Over 25 exhibitors from patient organisations and industry added an extra layer of energy, extending the impact of the day through shared insights, discussions and practical takeaways.

147
attendees

98% of attendees said they made new, meaningful connections

> **A brilliant and rewarding day**, full of community. It's refreshing to surround yourself with others looking to collaborate. I left feeling energised and hopeful.

> **The whole day was brilliant**—every talk filled with insights and emotion. Well done to all the speakers and especially Beacon for Rare Diseases for a terrific event.

> We had such an enjoyable time. You have brought together **such a strong community** and it is a great pleasure to be a part of it.

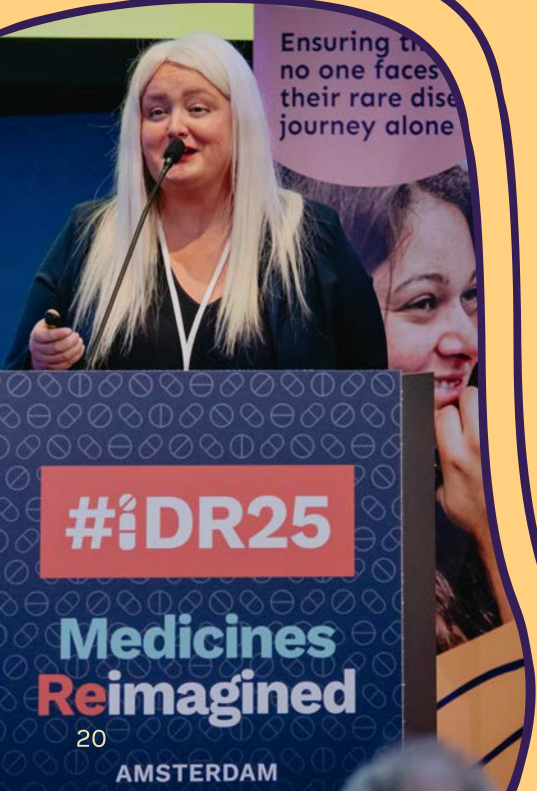
Research

iDR25

In May, Beacon headed to Amsterdam to co-host #iDR25: Medicines Reimagined in collaboration with REMEDi4ALL. Building on the success of our first international conference, #iDR24, this two-day conference welcomed 288 attendees from across the repurposing community, representing more than 30 countries and underlining the truly global nature of the event.

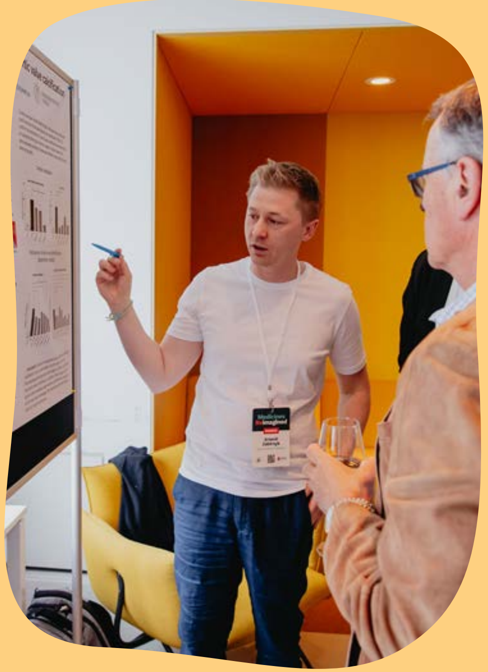
Once again, Beacon played a leading role in shaping and delivering the conference alongside colleagues at TEAMIT and Eatris. As well as co-organising the event, the team led the pathways on rare conditions and clinical trials, oversaw the lightning talk session, and delivered a patient-led plenary panel focused on patient engagement in repurposing—a highlight of the conference. For the first time, Beacon also ran a patient bursary scheme, supporting 17 recipients to attend, and coordinated the poster session, which showcased 18 posters.

Across the two days, 71 speakers from 12 countries shared their expertise, insights and experiences, creating a vibrant programme that reflected the diversity, ambition and collaborative spirit of the medicines repurposing community.



288
attendees

96% found attending to be beneficial



> It really was a tremendous two days. I leave with my patient advocacy batteries once again fully recharged (in fact they are overflowing) and ready to continue to fight the ultra-rare cause.

> This conference really gave me hope and ideas on how to move forward as a parent organisation to start research into treatment for our children.

> I thought the quality, breadth of topics and speakers was impressive and, from a patient perspective, very impactful.

Research

TrC4RD webinar series

As part of our involvement in the LifeArc Translational Centres for Rare Disease (TrC4RD) Hub we hosted a three-part webinar series: Introducing the rare disease ecosystem. Across the three sessions, we welcomed over 50 attendees involved in rare disease research projects. The main aim being to highlight the importance and impact of patient involvement in research.

Throughout the series, we introduced rare diseases and the key stakeholders within the landscape, we explored the power of the patient voice and the importance of collaboration to drive research forward, and we introduced patient and public involvement and engagement (PPIE)—what it means and how it can be built into research. We ensured that patient voice was embedded throughout the series, with talks from rare patient groups leaders, patient organisations, and lived experience patient partners.

57
attendees across the series

100% of attendees said the webinar **improved their understanding** of the rare disease landscape

The Lily Foundation

The Lily Foundation is a UK charity dedicated to supporting people affected by mitochondrial disease, a rare genetic condition that impairs the body's ability to produce energy properly. Founded in memory of Lily, the Foundation funds medical research, raises awareness, and provides support services to families living with these conditions. Its mission is to improve diagnosis, treatment options, and ultimately find a cure for mitochondrial diseases.

In 2025, The Lily Foundation first engaged with Beacon as a contributor to our Patient and Public Involvement and Engagement (PPIE) webinar series, sharing insights from their work with patients and families, offering practical examples of effective involvement, and bringing the lived experience perspective into discussions with researchers and professionals.

The Lily Foundation also went on to collaborate with Beacon through the LifeArc Translational Centres for Rare Diseases programme and attended the Rare Disease Showcase in Manchester, building connections across the rare disease community.

> **As a charity representing families affected by mitochondrial disease, we're passionate about ensuring patient voices are heard meaningfully in research and service development. We saw this as an opportunity to share our experience, advocate for best practice in PPIE, and learn from others working in this space.**
>
> **– Maria**

Through this partnership, The Lily Foundation has strengthened how it communicates the lived experience perspective and its role within wider PPIE conversations.

> **The collaboration with Beacon has helped us build confidence in articulating the value of meaningful PPIE and refine how we communicate lived experience to professional audiences. It has also enabled us to connect with others committed to improving patient involvement and ensured the voices of our community are represented in broader, sector-wide discussions.**
>
> **– Maria**

Alongside organisational collaboration, Beacon has also supported professional development within The Lily Foundation team. Katie Waller, Head of Patient Programmes, has taken part in Beacon's Public Speaking and Imposter Syndrome Masterclasses, as well as its mentoring programme.

> **All of Beacon's training has been fantastic and has helped me enormously to grow in confidence as I transitioned from a nursing role into patient advocacy. The opportunities are incredibly valuable, particularly in a space where there isn't a clear 'rulebook' and we are often learning as we go.**
>
> **Beacon not only provides high-quality training but also creates opportunities to connect with other patient advocates. You quickly realise you're not the only one facing the same challenges, and there's always someone who can offer support or advice.**
>
> **– Katie**

Through both organisational collaboration and individual development, The Lily Foundation's engagement with Beacon has strengthened its ability to champion patient voices, influence best practice in PPIE, and contribute to shaping the wider rare disease research landscape.

Thanks to our supporters

We are grateful for the support we receive from a range of funders. Beacon retains full editorial control over all of its work, and any funding received from pharmaceutical companies is used in line with the ABPI Code of Practice.

Funders

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Beacon has also received funding from the **UKRI Horizon Guarantee Fund** for our work in REMEDi4ALL, and **BioMarin Pharmaceutical** who supported The Manchester Rare Disease Showcase by way of a grant.

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Letter from our Chair

Sophie Costello

As Chair of Trustees, I am proud to reflect on a year of progress and impact for Beacon for Rare Diseases. Through 2025, Beacon strengthened its role at the heart of the rare disease community—equipping, connecting and championing patient groups across the UK.

What stands out is the level of work delivered by a team of this size. The timeline in this report shows that not a month goes by without trainings, events or initiatives.

Beacon's breadth and depth reflect its trusted, high-quality reputation, combining scientific rigour with a deep understanding of the rare disease landscape, and a personal approach that keeps patient groups seen, supported and empowered. I connected with many groups in 2025 and was moved by their gratitude toward Beacon. This year Beacon supported 319 patient groups, providing tools, knowledge and connections to help them grow and thrive. Case studies show Beacon unlocking the potential of rare disease organisations, enabling individuals and groups to build confidence, influence research, and drive meaningful change.

We are fortunate to have Rick Thompson leading the charity for a decade. His dedication, alongside the wider team, has built Beacon's culture, credibility and impact—translating into tangible benefits for the groups we serve. As a Board, we focus on long-term sustainability; in 2025 we welcomed five new trustees, reflecting Beacon's growing influence.

My sincere thanks to our team, trustees, partners and funders, and above all to the patient groups who inspire everything we do. It is a privilege to support them.

Sophie

Connect with us

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The Resources Hub

Newsletter

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Credits and acknowledgements

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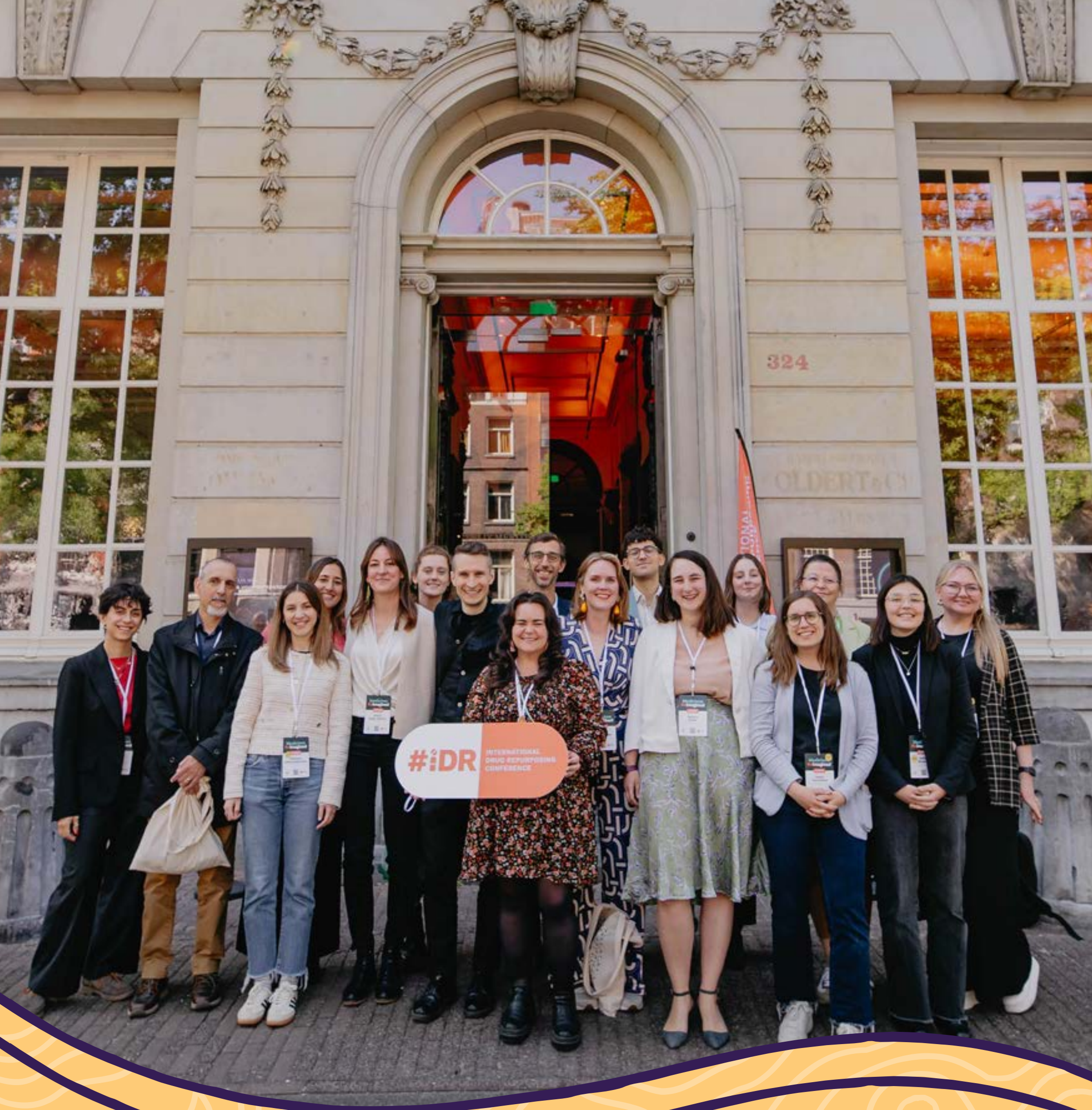

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