



IMPACT REPORT

2021



A WORD FROM OUR CEO

Dr Lucy McKay



2021 was a momentous year for Medics4RareDiseases (M4RD), as an increasing number of stakeholders in the rare disease arena recognised how the charity is addressing the unmet needs of both medical professionals and people living with rare conditions.

In January 2021, the long-awaited UK Rare Diseases Framework was published by the Department of Health and Social Care. The Trustees and I compiled our thoughts in a blog, 'Priority 2: The key to unlocking the UK Rare Diseases Framework', sparking a lot of demand for M4RD's perspective. I am not trained in public affairs and found myself deep in the world of government policy. Luckily, I am well versed in the aims of M4RD, and I know what the rare disease community is asking for. This is what guides me in all the work I do.

A GROWING TEAM

To meet the increasing demands on myself and the charity, M4RD employed two new members of staff:



Jo McPherson
Operations and Finance Manager



Dr Emma Huskinson
Medical Communications Officer

Both Jo and Emma have been brilliant additions to the M4RD family, and having them on board means I can now take proper annual leave!

MAKING AN IMPACT

My role as CEO and figurehead of the organisation was, at times, consumed by policy and advocacy work. This is a responsibility I take very seriously because I see how the discussion had directly impact the community I serve. I am a member of the UK Rare Diseases Forum, which advises the UK Implementation Board, and I hope that through this channel, we can advance further towards our main goal of achieving mandatory undergraduate and postgraduate medical education on rare diseases, centred on the lived experience.

COMING UP

In the coming year, M4RD needs to grow as an organisation in order to take on an increasingly active role in advocacy and the delivery of rare disease medical education. This is reflected in M4RD's 2022 work programme.

PURPOSE, AIMS AND OBJECTIVES

M4RD aims to improve the lives of a certain portion of the public: Those living with rare diseases and their communities. It does this through raising awareness of the relevance of rare diseases in clinical medicine. The charity provides education about rare diseases and opportunities to develop a clinician's understanding of this large population group in the UK in order to better serve it.

A **RARE DISEASE** is defined in the UK as a condition that affects *fewer than 5 in 20,000 people*

Over **7000 RARE DISEASES** have been identified, which means that understanding and teaching about rare diseases in medical education is *extremely challenging*

However, this is a challenge that we cannot afford to avoid because approximately **3.5 MILLION PEOPLE IN THE UK HAVE A RARE DISEASE**



The challenges of having a rare disease do not stop after getting a diagnosis. People living with completely different diseases often report the same difficulties relating to healthcare, social care, family life, education, work and mental health.

There are many wonderful charities in the UK that support patients with rare diseases depending on their disease or specific need, and umbrella charities run successful public awareness campaigns.

M4RD specifically targets an audience that nobody else was concentrating on but that could have the greatest impact on both the diagnostic odyssey and the challenges that follow medical students and doctors in training.

M4RD was established with the objective of improving relief of sickness and preservation of health in those suffering from rare diseases throughout the world by:

- ✓ Advancing the education of medics, associated professionals and the public in rare diseases and genetic and genomic medicine
- ✓ Promoting research in all areas relating to rare diseases and genetic and genomic medicine and publishing useful results
- ✓ Endorsing improved care and treatment of those suffering from rare diseases

By creating a medical profession equipped to suspect, diagnose and manage rare diseases, we can help people living with rare diseases to have the best outcomes and reach their full potential. To do this, M4RD is driving an attitude change towards rare diseases amongst medical students and doctors in training. Without appropriate training, the idea that rare diseases are irrelevant to clinical practice will continue to contribute to the diagnostic odyssey. **We remedy this by presenting statistics to our audience and asking people with rare diseases to share their stories.** M4RD have developed a new approach to satisfactorily cover the subject of rare disease within undergraduate and postgraduate medical training. Finally, we're creating our own resources and promoting others' resources in order to support medical professionals when they need help on the subject of rare diseases.

WHAT WE DID IN 2021

THE UNUSUAL SUSPECTS: RARE DISEASE IN EVERYDAY MEDICINE

M4RD's annual signature event in partnership with the Royal Society of Medicine (RSM) has always been held live in London on a weekday afternoon. However, due to the ongoing pandemic, the 2021 symposium was held during the evening as an interactive online meeting. As a result, it was able to reach a live global audience of over 200 participants.

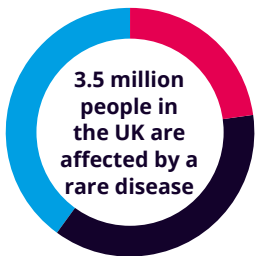
"Best event in Rare Disease Week so far – informative, varied and great pace!"

96% of clinical delegates said that this event will prompt them to consider a rare disease in their clinical practice

"This has taught me the prevalence of rare diseases collectively and has inspired me to ask more questions regarding the patients I see."

Delegates were asked:

"Were you aware that 3.5 million people in the UK (1 in 17) are affected by a rare disease in their lifetime?"



23% Were aware

37% Knew that rare diseases are common as a whole but did not realise how common

40% Did not know that so many people in the UK are affected by a rare disease

January

February

RARE DISEASE 101

Our learning module launched successfully at the beginning of 2021 and has been completed by 54 users.

This basic online and interactive training in rare disease for medical professionals is free to access, and the first module consists of 10 short lessons, including:

- *The Diagnostic Odyssey*
- *Challenges of Having a Rare Disease*
- *Patient Groups*
- *Diagnostic Tools*

The module caught the attention of medical professionals, educators and public health officials in Australia, where M4RD are working with two universities to create an Australia-specific version of Rare Disease 101.



RARE 101

March

April

APPOINTMENT OF JO McPHERSON

Operations and Finance Manager



WHOSE VOICE IS IT ANYWAY?

RareQoL and M4RD hosted an engagement session on the UK Rare Diseases Framework and the England Action Plan – on behalf of NHS England and NHS Improvement – called *“Whose Voice Is it Anyway?”*

“There seems to be an imbalance in power, with medical professionals having most of the power and patients having less.”

- Dr Sondra Butterworth

“Striving for health equity doesn't mean providing the same services for everybody; it's about having appropriate services for our diverse community.”

- Kerry-Leeson Beevers

“Youth participation in rare and chronic condition healthcare settings is fundamental to service design and delivery...”

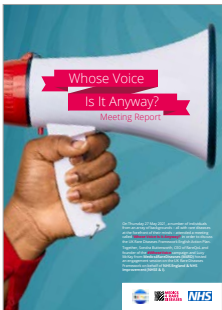
- Laurence Woolard

“The Bangladeshi and Pakistani communities that I work with in London are often labelled hard to reach, but that's based on assumptions rather than knowledge.”

- Shwetha Ramachandrappa

“We need an open and plain channel of communication that goes both ways between patients and healthcare professionals. It needs to be very well understood and not just a tick-box exercise.”

- Dr Pushpa Hossain



[CLICK HERE](#) to read the full report

May

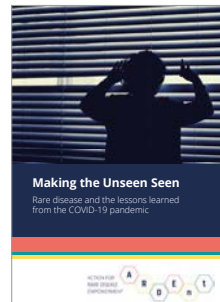
MAKING THE UNSEEN SEEN

Working collaboratively with Rare Revolution and Cambridge Rare Disease Network (CRDN), M4RD helped form **Action for Rare Diseases Empowerment (ARDEnt)**.

ARDEnt brought together over 30 stakeholders from across different sectors to look into the risks and benefits posed by the rare disease pandemic.

The report *‘Making the Unseen Seen’* was published in May, followed by ARDEnt’s inaugural webinar, which attracted over 140 participants.

Our efforts continue as we work to achieve the recommendations made from our findings.



[CLICK HERE](#) to read the full report

June

July

August

APPOINTMENT OF DR EMMA HUSKINSON

Medical Communications Officer



September

THE RARE DISEASE PODCAST 4 MEDICS

In 2021, M4RD marked two international awareness days with a special podcast feature.

'Doctors are patients too' was released on International Myotonic Dystrophy Awareness Day. M4RD CEO Lucy interviewed Dr Genevieve Allum to talk about her diagnostic journey and the ripple effect of being diagnosed with a genetic disease.

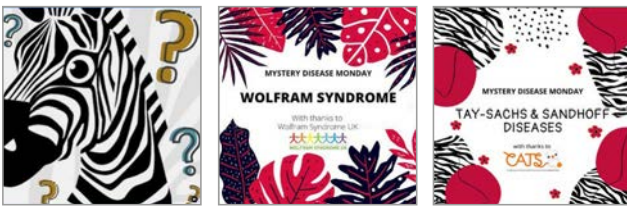
For World Heart Day, Lucy interviewed Dr David Adlam and patient advocates from BeatSCAD for the *'Not your usual heart attack'* podcast. The interview gave a brief overview of spontaneous coronary artery dissection (SCAD), and the panel discussed the challenges faced in diagnosing the condition and the biases faced by patients.

We went on to launch *The Rare Disease Podcast 4 Medics*, which is available through Spotify and Apple Podcast.



MYSTERY MONDAY

During 2021, 23 rare conditions were featured



October

STUDENT VOICE PRIZE 2021

The Student Voice Prize 2021 was launched with Beacon. The theme was rare disease and health inequalities and intersectionality.

There were 50 entries and 29 patient group pairings.

The winner will speak at the annual symposia for both M4RD and Beacon and will have their winning essay published in the *Orphanet Journal of Rare Diseases*.



November

M4RD AMBASSADOR

Applications to become an M4RD Ambassador were opened to recruit the 2022/23 cohort

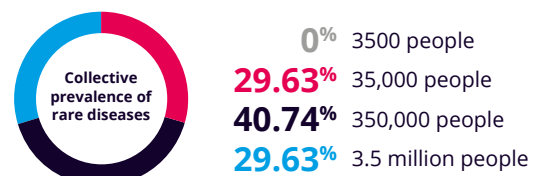


December

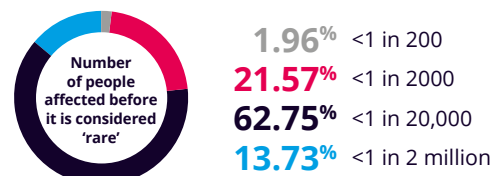
RARE DISEASES IN PRIMARY CARE

M4RD and the Royal College of General Practitioners (RCGP) hosted an Alexion-sponsored webinar, *Rare Diseases in Primary Care: Assessment and Management*

Attendees underestimated the collective prevalence of rare diseases



and underestimated how many people a disease has to affect before it is considered 'rare'



Ongoing in 2022



PLANNING FOR 2022

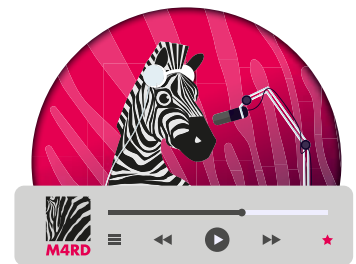
In 2022, M4RD aims to:



Host its annual meeting with the RSM, 'The Unusual Suspects: Rare disease in everyday medicine' on 9 February 2022

Continue to engage with the Department of Health and Social Care and its delivery partners

Organise a series of webinars with the RSM's Medicine and Me section on 'Lessons learned from the COVID-19 pandemic' in collaboration with the ARDent group



Launch a Rare Disease 101 mini module focusing on clinical trials and early access programmes, as well as some 'deep-dive' modules on mental health and care coordination

Recruit two project assistants

Launch the second and third series of the Rare Disease Podcast 4 Medics



Launch the Student Voice Prize 2022 with Beacon

Induct a new cohort of patient, clinical and medical student ambassadors

ANNUAL REPORT AND ACCOUNTS

The Trustees, with support from the staff team, decide the charity's fundraising goal for each year based on funds needed to achieve the planned activities for the year.

In 2021 the fundraising goal was £80,000. £101,330 was raised through sponsorship and donations. Total expenditure for the year was £74,700.

Find out more from our full [annual reports](#)



With thanks to our 2021 sponsors and donors:

