




IMPACT REPORT

2022



A WORD FROM OUR **CEO**

Dr Lucy McKay



Medics4RareDiseases (M4RD) has continued to address the unmet needs of both medical professionals and people living with rare conditions in 2022. The charity is increasingly recognised as pivotal for designing and delivering medical education on rare diseases. By working with the delivery partners of the UK Rare Diseases Framework, we hope to facilitate a more coordinated, national response to Priority 2 going forward.

A WORD FROM OUR CEO - Dr Lucy McKay

MAKING AN IMPACT

2022 is the first year that I headed into **Rare Disease Day** (28th February) with a team. Jo and Emma had joined the previous year, meaning that the charity could make a much greater impact on this important day. Emma's experience in communications allowed us to have a coordinated message for the day and to be featured in a much wider variety of media, including on the back page of The Times' Rare Disease Day supplement.

RISING TO THE CHALLENGE OF A NEW ROLE

Melissa, M4RD's Training and Education Officer, joined the team in mid-February and hit the ground running! This new role has been game-changing in terms of delivering **Rare Disease 101** training sessions to medics throughout the country. She has also spent the year preparing to launch an online survey for medical students about their understanding of rare disease and their learning requirements called **RISE UK with Cardiff University**.



ON-DEMAND TRAINING

This year, the team delivered **13 training sessions** to a number of clinical groups, including paediatricians, GPs and medical students. Currently, these are organised 'on demand', demonstrating that medics want this education and that they recommend it to their colleagues. This is paving the way towards installing Rare Disease 101 into regular training and education for doctors and future doctors.

A POPULAR YEAR

M4RD Learn, our e-learning platform, has nearly **900** users. Melissa has teamed up with **Kym Winter** from Rareminds to create a new Mental Health course that will launch on M4RD Learn in 2023. Digital learning is going to continue to grow as we have now welcomed **Eleanor** to the team as M4RD's Digital Projects Officer. With Eleanor's expertise on the team, The Rare Disease Podcast 4 Medics is on track to hit 4000 downloads by the end of the year!



A GROWING TEAM

The charity has spent 2 years rapidly building this team to meet the demands being placed on it to design and deliver patient-centered, single-discipline education on rare disease. We've been demonstrating that our approach benefits patients and clinicians alike, and now we need to keep rolling it out through:

- M4RD's own channels
- Medical schools
- Postgraduate training
- Putting pressure in the right places within policy and public bodies

I am determined to keep using my dual experiences as advocate and medic to ask the much-needed questions; to keep examining why so many people living with rare conditions today still face similar challenges to the friends I grew up with; to keep the focus on tangible benefits to the rare disease community.

OUR BOARD OF TRUSTEES

The M4RD Board of Trustees continue to contribute generously to the success of the charity



Two trustees are GPs and have been involved in M4RD (in its previous forms) since 2011. **Dr Hannah Grant** is an international lacrosse player, but for M4RD she provides insight into day-to-day life and learning needs of being a GP in the NHS. M4RD Treasurer, **Dr Debra Fine**, was an accountant prior to training to become a doctor and this range of experience is invaluable to the role.



Lindsay Birrell is the Vice Chair of M4RD; her prior experience working for Metabolic Support UK as CEO has been invaluable to Lucy and the rest of the Trustee Board.



Dan Jeffries is M4RD's patient representative, but has many skills within IT. His day job of creating online interactive educational software means he provides bespoke and greatly discounted services in the creation and development of M4RD's learning management system, M4RD Learn.



M4RD Chair, **Chris France**, is the founder of a successful e-commerce company and has been pivotal in getting M4RD's structure established, mentoring Lucy and steering the charity in the direction of successful strategies.

PURPOSE, AIMS AND OBJECTIVES

Medics4RareDiseases (M4RD) aims to improve the lives of 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 receiving a diagnosis. People living with completely different diseases often report the same difficulties in terms of healthcare, social care, family life, education, work and mental health. There are many wonderful charities in the UK that support rare disease patients depending on their disease

or specific need. Umbrella charities run successful public awareness campaigns. However, M4RD specifically targets an audience that nobody else was concentrating on but could make the greatest impact on the diagnostic odyssey and the challenges that follow medical students and doctors in training.

M4RD was established with the objectives of relieving sickness and preserving the health of those suffering from rare diseases throughout the world by:

✓ Advancing the education of medics, associated professionals and the public in rare diseases, genetic and genomic medicine

✓ Promoting research in all areas relating to rare diseases, genetic and genomic medicine and publishing the 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 have the best outcomes and live full lives. 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 the statistics to our audience and asking people living 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 to support medical professionals when they need help regarding rare disease.



WHAT WE DID IN 2022

LESSONS LEARNED FROM THE COVID-19 PANDEMIC

During early 2020, a group of advocates came together to take Action for Rare Disease Empowerment (ARDEnt). The groups' aim was to shed light on the **unseen impact** of the pandemic on people living with rare conditions.

Lucy joined the ARDEnt webinar series '**Lessons learned from the COVID-19 pandemic**' held in association with the Medicine and Me Section at The Royal Society of Medicine.

The series covered the themes outlined in the Making The Unseen Seen report:

- Diagnostic delay
- Health and social care
- Clinical trials and drug development
- UK Rare Diseases Framework and action plans



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

January

WALES PAEDIATRIC TRAINING DAY

M4RD organised its first training event in Wales.

27 attendees joined the hybrid event, which featured:

- **Rare Disease 101**, delivered by Lucy
- **Patient stories**, provided by Same But Different
- **A clinician's perspective**, delivered by Dr Graham Shortland
- **Genetic & Genomics**, explained by a genetic counsellor based in Wales
- **An introduction to advocacy**, delivered by Genetic Alliance UK



February

THE UNUSUAL SUSPECTS

M4RD's annual event was held in association with the Medical Genetics Section of the Royal Society of Medicine.

Last year, the online presence of the symposium meant that a **higher proportion of international delegates** could take part and this year was no different, with over **330 registrations**.

94% of attendees said that this event would impact their clinical practice

"I will try to keep a rare disease on my differential diagnosis list from now on, which I had been somewhat brushing off in the past, unless it was more obvious than the more common diseases; I will pay more attention to learning about the rare diseases collectively."



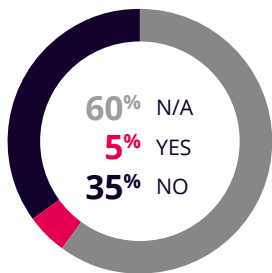
[CLICK HERE](#) to read the full report

STUDENT VOICE PRIZE 2021

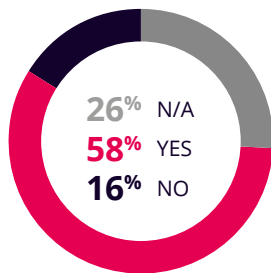
M4RD and Beacon co-hosted the 'Beyond the Student Voice Prize: Continuing your involvement in rare disease' webinar.

28 attendees joined us

"I received broad rare disease education in my training."



"I have experienced elements of the diagnostic odyssey in my life."



The Student Voice Prize

CLINICAL TRIALS AND EARLY ACCESS PROGRAMMES MINI-MODULE

With support from Bionical Emas, we launched our Clinical Trials and Early Access Programmes mini-module.

Our Clinical Trials lesson covers:

- What a clinical trial is used for
- How clinical trials are designed and executed
- How they may differ in the context of rare disease

The Early Access Programmes lesson is an introduction to this poorly understood area of medicine. It is important to understand on a basic level when it is appropriate for patients to have access to investigational medicines.



RARE DISEASE 101 AUSTRALIA

In collaboration with Western Australia's Rare Care Centre and the Australian Government Department of Health and Aged Care, **Rare Disease 101 Australia** was successfully launched.



March

April

May

BUILDING CONNECTIONS

Kelly and Jason, both **medical students from Hong Kong**, joined us as part of their international placement programme for one day.

They spent time with us at emotive's office and joined us for a podcast episode.

"The placement was enjoyable and fruitful. I believe future participants will enjoy it too."



UNDERSTANDING RARE DISEASE

M4RD co-hosted **'Understanding rare disease: communicating with patients'** – an interactive virtual event initiated and funded by Takeda for the **#IAmNumber17** campaign.

There were a range of attendees from different years of universities from across the country, and the event explored the value of effective and sensitive communication skills when it comes to handling complex conditions, such as rare disease.

Attendees also had the chance to speak directly with some of the 'I am Number 17' **campaign changemakers** and hear first-hand about how their diagnostic odysseys came to an end, through perseverance and working together with the help of their health care professionals/consultants.

#IAmNumber17

RARE DISEASE 101 TRAINING

M4RD held a Rare Disease 101 **training session for paramedic trainees** at Oxford Brookes University.

40 students joined us

Lucy delivered the basic Rare Disease 101 element, which was tweaked for this specific audience concentrating on where they can make a difference:

- Communication during handover
- Listening to patients and using their information they provide
- Being open to seeing unusual presentations

A survivor of a heart attack from SCAD shared her story, including tips for paramedics.

Pippa, from Addison's Disease Self Help Group, explained what patient alert cards are for and how to use them.

June

MEET OUR NEW AMBASSADOR, GRACE KNIGHT

Grace Knight, a final-year medical student from Keele University, spent 3 weeks with us on elective placement and enjoyed the experience so much that she signed up to become an ambassador!

"It was honestly excellent. I would highly recommend and really valued my time as part of the team. I had trained for 6 years, across five trusts and two universities and no one had even mentioned rare disease before I came across M4RD."



July

TEACHING SESSIONS

Dr Lauren Ferretti, M4RD Clinical Ambassador, gave teaching sessions at St George's Paediatrics Department.

"I'm still buzzing slightly from this morning's teaching - thank you all so much! I feel like it was a real success - the feedback so far has been really positive."



GP ONLINE

GPs are obviously a key demographic in terms of raising awareness for rare diseases, and GP Online is a popular resource for them.

The 'Viewpoint' article was a full profile piece about Lucy, with excellent brand exposure for M4RD. With the editor giving us free reign to create the article, we were able to effectively incorporate the charity's key messages, along with a link to M4RD Learn.

Thanks to Alexion, our 2022 partner, for the introduction; it was a brilliant opportunity to communicate directly with such an important target audience.



Championing
and informing
general practice
professionals

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

August

MEET OUR NEW AMBASSADOR, AMY STEELE

Amy Steele, a first-year medical student from the University of Manchester, joined us for a one-day placement and went on to become an ambassador!

"From this placement, I hoped to gain a better understanding of the aims of M4RD and what they do to help patients with rare diseases. I also hoped to improve my own understanding of the realities of living with a rare disease, and hence why it is so important for medical professionals to be educated about rare diseases in order to be able to best support rare disease patients."



INTERVIEW WITH GENOMICS ENGLAND

Lucy was interviewed by Genomics England CEO, Chris Wigley, for an episode of 'The G Word'.



[CLICK HERE](#) to listen

AMBASSADOR INDUCTION

At the beginning of September, we hosted an induction evening for our 13 new Ambassadors - made up of patients, medical students and clinicians. As well as sharing information about the many events and opportunities available to help inform our projects and teaching opportunities, we talked about the importance of communicating the lived experience of having a rare disease and how this is fundamental in our message to medical students and doctors in training.

Dan Jeffries joined us to talk about his own experiences of being a patient advocate, and we were also treated to a presentation from **Zainab Alani, Fatima Alani** and **Dr Agata Oliwa** who spoke of how they started their own rare disease society at the University of Glasgow.

Dr Thomas Dunne, who joined our programme as a medical student many years ago, talked about how he has fulfilled his role as an ambassador over the years and the projects that he has been involved in.

In total, we now have
10 clinicians, 5 medical students
and **4 patient ambassadors!**



September

ZIMBABWE STUDENTS ON PLACEMENT

M4RD took on **3 medical students** from Zimbabwe for a 3-month online placement.

These students are members of the national rare disease charity in Harare, known as **Child and Youth Care Zimbabwe**.

"Thank you so much for the opportunity. It was an insightful and amazing experience! I personally really learned a lot from the placement. I am ready to apply what I learned to our own rare disease projects here in Zimbabwe. It was an honour, and I really do hope to continue working with you!"

THE LIBRARY

M4RD started to develop 'The Library' – an HCP-restricted area to share educational non-promotional materials from its industry partners. The aim is to share excellent resources in a compliant fashion in one place.



STUDENT VOICE PRIZE

The 2022 competition saw entries from students across 44 different universities in 11 different countries.

67 medical students entered with **65 PAG pairings**

"I highly enjoyed writing and learning about the lived experiences of patients with rare diseases. As a future doctor, I now have more empathy and a better understanding about how patients with rare diseases navigate the healthcare system and their experiences in doing so."

"Learning about a rare disease is incredibly motivating. It makes you, as a future health professional, determined to do better and be better for the sake of others."

RARE DISEASE PODCAST 4 MEDICS

Each week, we continue to release interviews with people from across the rare disease and medical world, looking at different experiences and perspectives while providing pragmatic tips and advice for healthcare professionals.

Season 2 reached **2122 downloads** and Season 3 reached **4000!**



Available via Spotify and Apple

"Thank you so much for all your hard work on the podcast for vascular EDS. It meant a lot to our community to be given such a great opportunity to raise awareness and have a voice. You are doing great work. It has given me the confidence to advocate for others in my situation."

October

ARI PATEL AND LUCY SPOKE TO THE COMMUNITY PAEDIATRICIANS

Lucy was joined by **Arti Patel** from Unique in giving a teaching session on reforming rare disease at Sunshine House Clinic (part of the Evelina Guy's and St Thomas' NHS Foundation Trust). This talk was for the paediatricians at the Evelina Sunshine House Clinic.



November

MENTAL HEALTH AND RARE DISEASE COURSE

The authoring of a Mental Health and Rare Disease e-learning course was successfully completed by M4RD and Rareminds, ready to be launched in 2023.

The module consists of **eight interactive lessons**, each focusing on a different topic related to the challenges that living with a rare disease poses to a person's mental health.



December

Ongoing throughout the year

MYSTERY MONDAY ON INSTAGRAM STORIES

During 2022, **34 rare conditions** were featured, with over:

650
votes

3750
story views

"Huge thanks again for helping increase awareness of Usher syndrome! You all are doing amazing work!"

"Thank you to the PAGs who provide information for this feature."



WITH THANKS TO OUR 2022 SPONSORS AND DONORS:



And with thanks to Novartis for sponsoring the M4RD Mystery Monday feature, and to the James Tudor Foundation and the Grace Trust for donating towards the further development of M4RD Learn.

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