A Snapshot of What We Got Up To This Year...!

Cure DM

Support ♥ Awareness ♥ Research

CureDM is a small yet mighty collective of families, carers, loved ones and special individuals. We would like to thank everyone who makes CureDM charity what it is.

We've seen our donations almost double in 2022 / 23 and our expenditure has increased to reflect this. This simply wouldn't have been possible without your support! We would like to sincerely give thanks to those whose efforts, contributions, and donations – big and small- make up the CureDM community.

Thank You!

All the Trustees on our panel have the unique perspective of being parents and carergivers to a child / young adult diagnosed with Congenital Myotonic Dystrophy or a similar neuromuscular condition...

Emma-Jayne Ashley
Founding Director & Trustee
Mum to Dregan (CDM)

Peter Ashley
Trustee & Chair
Dad to Dregan (CDM)

Stephen Uncles
Trustee
Dad to Matthew & husband to Paula (CDM & DM1)

Dr. Ali Kay
Trustee
Mum to Bertie (Ullrich CMD)

In 2022 we gave a warm welcome to two new additions to the CureDM Trustees...

Tamsyn Tate
Trustee
Mum to Daisy (CDM & DM1)

"I am really excited to join the CureDM team. My two-year-old daughter's diagnosis of CDM1 and my own subsequent diagnosis of DM1 means I can relate to those who have the condition whilst caring for a child with the severe form. With a career as a Marketing Manager, I'm hoping I can help raise the charity's profile and ultimately awareness for the disease. I am also keen to support others through their journey."

"I'm delighted to be joining the CureDM team. My one-year-old son was diagnosed with CDM shortly after birth along with my wife (DM1). The news came as a huge shock, as it does for many families. I look forward to contributing to the charity's fantastic work in community support, as well as raising awareness within the science and medical community to move towards effective treatment."

Liam Garwood
Dad to Louie, husband to Bayley (CDM & DM1)

(Learn more about the team by visiting our web site)

Ongoing Support

This year, like every year, we make efforts to let children and their families, carers and guardians know we are thinking of them and follow their journeys as much as possible. We try to offer emotional support and informative advice whenever we can. Many of the CureDM community face daily challenges such as the shock of diagnosis, illness, and often being in hospital. We hope, through our network, we can help support one another and let you know you are not alone.

"CureDM has been a lifeline of hope and reassurance to us. Getting the shock diagnosis, we didn't know where to turn for information and found so little positive information out there. The charity has really helped our understanding and made us feel more hopeful for the future."

Events of 2022

Families Day - Alton Towers - July

We were able to organise an incredible family day to give families affected by CDM the opportunity to enjoy the theme park with minimal cost to them. This also gave families, carers, and guardians across the UK the opportunity to meet face-to-face and offer a safe space to connect with others familiar with the issues of caring for children affected by CDM. We received lots of lovely feedback about how much fun the attendees had and what a positive experience it was.

If you attended Alton Towers this year you may have seen a friendly, somewhat cuddly character in the form of Miles the Mascot. We hope you enjoyed this new addition to our team. Watch out for him at our future events!

"What a fantastic experience for our daughter to meet with so many other children who share her condition. She's only met two other children who have this condition…

…I wish this charity had existed 11 years ago when we collected her from hospital…This charity is a wealth of knowledge on this condition and bringing us together is very special."

Disney On Ice - Sheffield, Birmingham, London - December

We were also able to offer a Winter family event this year. We secured spaces at three venues, offering 55 people the chance to enjoy a family trip to watch Disney on Ice live at Birmingham, Sheffield, or London with minimal personal cost to the families. It was lovely to be able to give the families another chance to meet up. For some it was the first time attending one of our events. We hope those who could make it made some unforgettable memories for the festive season! It was so successful that we are repeating the offer in 2023 - contact us if you are interested!

"[CureDM} has been a constant source of support and knowledge over the past year when the children have been ill, or I have had questions or concerns."

"We have really enjoyed and benefitted from the two meet ups (both Alton Towers in the Summer and Disney on Ice in December) where we got to meet many other families with children who have the same condition as my two...

...That for me is the best part as it has allowed me to build a network of support of other muns / dads who may be going through similar...This gives me hope and reassurance, knowing I am not alone!

Research

At last, 3 potential systemic treatments are in clinical trials (AMO Pharma, Avidity and Dyne) and more are on the way!

CureDM are working with the companies to make sure the trials measure what is important to us, that they are not too much of a burden, and are safe. We are also working with the UK regulatory bodies to speed up the approval process and make them available to everyone as soon as possible. Please get in touch if you'd like to know more or to be involved!

MDUK announced in 2022 that Dr.Chris Turner at UCL will be leading a natural history study of adults with Congenital DM1 (symptoms from birth). The study will give us information to further improve care and also provide data for clinical trials.

Members of CureDM advocated for this study and we are proud to be part-funding it.

We have been involved in producing a number of abstracts and posters that have been presented at conferences by CureDM, TREAT-NMD, and the UK DM Patient Registry (which we also part-fund) to explain to clinicians and researchers the real-world experiences of the people that actually live with Myotonic Dystrophy.

Thank you for the hundreds of responses to our ongoing survey that allowed this to happen!


Congenital and Childhood Myotonic Dystrophy Type 1 in the UK

Nikolenko Nikoletta[1], Ashley Emma-Jayne[2]

[1]National Hospital for Neurology and Neurosurgery, Queen Square, UCLH NHS Foundation Trust, UK

[2]Cure Myotonic Dystrophy UK Charity

Introduction

Aim

To identify the epidemiology and clinical characteristics of DM1 patients within the UK

Methods

Results

Conclusion

References

Acknowledgements and Contact

University College London Hospitals

UCL

Raising Awareness

Shining a light on Myotonic Dystrophy, especially the Congenital form, is crucial. Here are some of examples how we spread the word this year:

Celebrating International DM Awareness Day:

Social media came into its own, especially Facebook and Twitter, where many in the UK community and beyond showed support by changing their profile pictures, sharing information on the disease, upcoming trials, and posting personal stories. The national mourning of the Queen meant landmarks that were planned to light up green on the 15th of September were purple in respect for the monarchy. They have, however, all said they'll light up for us in 2023!

Growing Our Strong Online Presence:

As well as the CureDM web site, this year we added Instagram to our continuous, strong online presence on social media, helping us to share any news and breakthroughs. Our private, UK only, Facebook group continued to grow. It is only for those living with DM, focusing on peer support and day to day living.

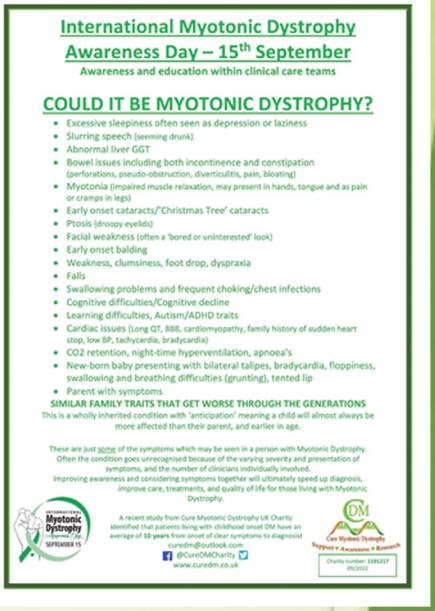
www.curedm.co.uk

Attending Conferences:

This year we got back to face-to-face conferences in the UK, Belgium, Italy and Canada and also presented at the prestigius IDMC. These allowed us to connect with the leading medical and scientific professionals, raising the profile of DM on a big stage.

Taking Part in Webinars:

In 2022/2023 we attended online conferences based in the UK, across Europe, Japan and the USA. As with in-person conferences, these webinars allow the exchange of medical advances in the field of neuromuscular disease and create collaborations that raise the profile and accelerate awareness of Myotonic Dystrophy.
Presenting the importance of patient preferences in webinars for ISPOR (Professional Society for Health Economics and Outcomes Research) and representing the community in online conferences is vital for keeping DM relevant in the eyes of the researchers.


International Myotonic Dystrophy Awareness Day – 15th September

Awareness and education within clinical care teams

COULD IT BE MYOTONIC DYSTROPHY?

- Excessive sleepiness often seen as depression or laziness
- Slurring speech (seeming drunk)
- Abnormal liver GGT
- Bowel issues including both incontinence and constipation (perforations, pseudo-obstruction, diverticulitis, pain, bloating)
- Myotonia (impaired muscle relaxation, may present in hands, tongue and as pain or cramps in legs)
- Early onset cataracts/'Christmas Tree' cataracts
- Ptosis (droopy eyelids)
- Facial weakness (often a 'bored or uninterested' look)
- Early onset balding
- Weakness, clumsiness, foot drop, dyspraxia
- Falls
- Swallowing problems and frequent choking/chest infections
- Cognitive difficulties/Cognitive decline
- Learning difficulties, Autism/ADHD traits
- Cardiac issues (Long QT, BBB, cardiomyopathy, family history of sudden heart stop, low BP, tachycardia, bradycardia)
- CO2 retention, night-time hyperventilation, apnoea's
- New-born baby presenting with bilateral talipes, bradycardia, floppiness, swallowing and breathing difficulties (grunting), tented lip
- Parent with symptoms

SIMILAR FAMILY TRAITS THAT GET WORSE THROUGH THE GENERATIONS

This is a wholly inherited condition with 'anticipation' meaning a child will almost always be more affected than their parent, and earlier in age.

These are just some of the symptoms which may be seen in a person with Myotonic Dystrophy. Often the condition goes unrecognised because of the varying severity and presentation of symptoms, and the number of clinicians individually involved.

Improving awareness and considering symptoms together will ultimately speed up diagnosis, improve care, treatments, and quality of life for those living with Myotonic Dystrophy.

A recent study from Cure Myotonic Dystrophy UK Charity identified that patients living with childhood onset DM have an average of 10 years from onset of clear symptoms to diagnosis

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www.curedm.co.uk

Cure Myotonic Dystrophy

Support • Awareness • Research

Charity number: 1191217

09/2022

Contacting Professionals:

We increased our efforts to make positive contact with medical professionals to bring recognition and further their understanding of the disease, to speed up diagnosis and implement the necessary care to their patients living with Myotonic Dystrophy earlier rather than later. Being members of Euro-DyMA (Federation of European DM Associations) and other DM specific associations enables us to bring meaningful discussion and collaboration to the very start of any project, making sure work is relevant for the community we represent.
This year we created a "Could it be Myotonic Dystrophy?" poster that has been distributed to clinicans and sent around the world. This can be downloaded from our website or please contact us for copies.

Raising Funds!

None of this would happen without the generous organisations that provide us with grants and, of course, the many amazing people that volunteer for us and selflessly raise the much needed money! You are fabulous!

For more information on CureDM and what we are doing behind the scenes, you can keep up to date on our social media pages, visit our website, or e-mail us at curedm@outlook.com

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