Spring 2022

Muscular Dystrophy UK

Fighting muscle-wasting conditions

Campaign

The newsletter for our supporters

Inside:

- Reflections in Colour
- Town and Gown turns 40!
- Meet MDUK's first Equality, Diversity and Inclusion Ambassador

40 years and still running! Celebrating our Town and Gown's 40th anniversary

Fundraiser, Mike Cleaver, created the Oxford Town and Gown 10k in 1982, with inspiration from the first London Marathon. His son, Daniel, had Duchenne muscular dystrophy, and sadly died in 1990 at the age of 12. What started as a fun run to raise funds for MDUK has now become the professional, chip-timed and serious fun annual MDUK race it is today.

It's the only closed-road city centre 10k in Oxford, with a beautiful stretch along the River Cherwell, and a finish in the University Parks.

In May 2022, we're celebrating the Oxford Town and Gown 10k 40th anniversary. To commemorate the history of the event, we'll share stories of runners who raced in the very first event and are still running today! We're very excited about this landmark year and expect thousands of runners to join us and raise more than £200k to help beat muscle-wasting conditions.

In addition to the Oxford event, we also hold a Town and Gown 10k in Cambridge AND we have a programme of other running events, including half marathons and more 10k runs. Get involved, join #TeamOrange, and make every step count in the race to beat muscle-wasting conditions **www.musculardystrophyuk.org/get-involved/events/runs**

If you have an Oxford Town and Gown story to share, please let us know at events@musculardystrophyuk.org.

Welcome

Welcome to *Campaign* – the newsletter for supporters of MDUK.

Since our last newsletter some wonderful things have happened, from new research grants to virtual award ceremonies! Watch how we surprised the remarkable recipients of our President's Awards here www.musculardystrophyuk.org/Presidents-Awards-2021

On pages 6-7, there's an update about an MDUK-funded project, which explores gene therapy as a treatment for Duchenne and Becker muscular dystrophy. And we're delighted to introduce Tiffany Hesson, MDUK's first Equality, Diversity and Inclusion (EDI) Ambassador, on p10.

Importantly, new research we've funded now puts the number of people living with a muscle-wasting condition at 110,000. You can read the full story on our website www.musculardystrophyuk.org/new-research-study and we will also have a feature in our autumn edition of Campaign.

I hope you enjoy reading about MDUK's work, which we can only achieve thanks to your generosity.

Gabby Logan MBE
President, Muscular Dystrophy UK

PS: Check out the MDUK online shop **www.musculardytrophyuk.org/shop** and see if you can spot the new products that have just landed!

Our vision

A world with effective treatments and cures for all muscle-wasting conditions and no limits in life for individuals and families affected.

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On the cover: some of our brilliant Oxford Town and Gown runners.

Update from our Christmas Appeal family

Thank you so much to everyone who has donated to our Christmas appeal. Since Katherine Hutchinson shared her family's story, you've raised more than £32,000!

In 2016, when Joseph Hutchinson was four years old, his family were given the news that he had Duchenne muscular dystrophy. They were devastated but determined to find out all they could about the condition; their consultant advised them to contact MDUK. Since then, our helpline team have supported them with advice and information and, after struggles with their local council, helped them finally get the housing adaptations they needed.

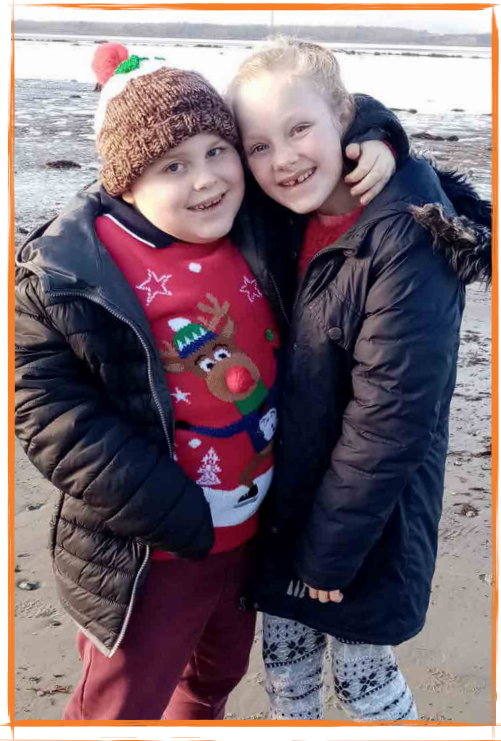
When COVID-19 hit in 2020, along with so many vital services, Joseph's physio and hospital appointments were cancelled. He also wasn't able to go swimming, which he loved to do. All of this affected his mobility and, in December 2020, Joseph started using a wheelchair full-time.

But the great news is that, after many delays, the home modifications the family needed for Joseph are complete! He now has his own bathroom, with height-adjustable bath and wash basin, wash and dry toilet, and shower area. He has ceiling track hoists in both his bathroom and bedroom, along with a profiling bed, and plenty of accessible space.

Joseph has been able to start swimming again, he's able to move so much more in the water and do his "pool walking". The family have also re-joined an accessible cycling group that provides an accessible cycle for Joseph. Katherine, Joseph's mum, says, "It's wonderful to be able to get out and about again and put the worries of the housing adaptations process behind us.

"We also now have a wheelchair accessible vehicle, which has made a world of difference to our daily lives. We are so grateful to everyone at MDUK, who have consistently been by our side throughout everything, and I honestly don't know where we'd be without them."

Help us to be there for more families like Katherine's. There's still time to donate to the appeal here: www.musculardystrophyuk.org/Christmas. Thank you.



Gene therapy as treatment for Duchenne and Becker muscular dystrophy

Elena Marrosu (pictured right), has recently finished her MDUK-funded PhD, which may have implications for how to design gene therapy for the treatment of people with Duchenne and Becker muscular dystrophy. Elena's been working with Dr Federica Montanaro at the UCL Great Ormond Street Institute of Child Health in London.

Gene therapy aims to replace the dystrophin protein that people with Duchenne and Becker muscular dystrophy don't produce. The gene therapy treatment uses part of a virus to carry the dystrophin gene into muscle cells. However, the dystrophin gene is large, and the virus used to deliver the gene into muscles can only accommodate a limited amount of genetic material. For that reason, shorter versions of the dystrophin gene are used that can be effectively packaged into the virus. Using this shorter version of the dystrophin gene, known as microdystrophin, gene therapy works well in the skeletal muscles (those that connect to your bones and help you move).

However, researchers in Dr Montanaro's group had shown that microdystrophin was unable to interact with a certain protein in heart muscles, leading to the idea that microdystrophins don't function properly in the heart. This would be of particular concern, as heart failure is a major health issue in people with Duchenne and Becker muscular dystrophies.

Elena faced many of the difficulties experienced by many researchers on account of the COVID-19 pandemic and was unable to complete some key work without access to core facilities. But with some changes of focus, her project showed exciting outcomes, for example that loss of dystrophin affected how certain proteins were distributed inside the heart muscle cells of mice that are critical to maintain the function of cells. The incorrect distribution of these proteins, which was seen in the absence of dystrophin, was not corrected by microdystrophin. This implies that the standard gene therapy in Duchenne and Becker muscular dystrophy would lack some of the

functions associated with heart muscle. The researchers had the idea that the bits of the dystrophin protein missing in microdystrophin must be important for this function.

Elena's findings will inform future work to improve design of microdystrophins, to consider the specific effects in heart muscle.

"I was awarded an MDUK-funded studentship four years ago, and I felt incredibly lucky. The funding has enabled me not only to do my thesis but also to learn a lot. The charity has been there for all my research life. I'm very grateful."

For the latest news in research visit www.musculardystrophyuk.org/news

Outcomes of the 2021 grant round

We are pleased to announce that MDUK has awarded research grants to nine applicants in the 2021 new grants round, with a total award value of £639,185.

After the disappointment of having to cancel the 2020 new grants round, we were back on track in 2021, although with some restrictions we'd not had in previous years. The biggest of these were to limit the number of conditions we could cover, and limit the duration of the projects to 12 months.

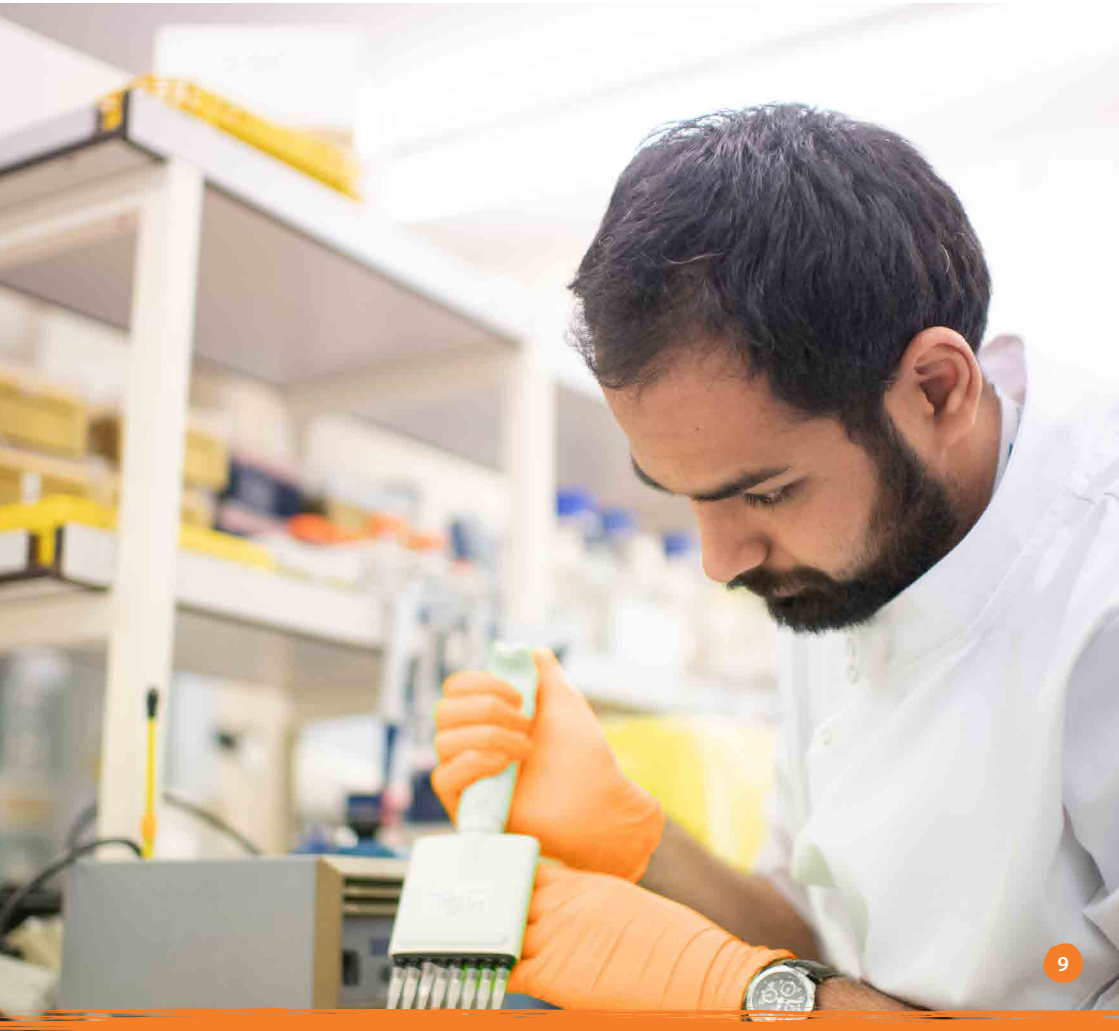
Despite these limitations, we received 20 applications, among which were some of a very high standard, making it hard to select the ones to fund.

Our final decision was to fund nine grants: one four-year PhD studentship, and eight projects. These cover a broad range of topics, from basic science through to clinical studies, with a focus on the following conditions: collagen 6-related congenital muscular dystrophy, congenital myotonic dystrophy, Duchenne muscular dystrophy, facioscapulohumeral muscular dystrophy (FSHD), spinal muscular atrophy (SMA), and type-1 myotonic dystrophy.

We're particularly pleased to announce that, along with our partners SMA UK, we've co-funded a four-year PhD studentship to study disease mechanisms in SMA, at the University of Edinburgh.

The 2022 grants round is currently underway, and we are hoping to make awards in the summer of this year for grants for up to three-year projects, and four-year PhD studentships, across a broad range of muscle-wasting conditions.

You can find details of all the research projects we fund here **www.musculardystrophyuk.org/research/our-research**



Meet Tiffany Hesson

MDUK's first Equality, Diversity and Inclusion (EDI) Amabassador

Tiffany Hesson has given up her job in banking to devote more time to her three-year-old son, Roman, who was diagnosed with a rare form of congenital muscular dystrophy (CMD), called LMNA-CMD, at the age of just a year. She also wants to focus on her new role as MDUK's first EDI ambassador.

"I'm thrilled to have this opportunity to help MDUK reach a more diverse community and get the message across that they're here for everyone living with a muscle-wasting condition; no-one is excluded. I want Roman to see that he's not the only black child with a rare muscle-wasting condition, and to be able to identify with the charity and other little children with conditions like his."

When Roman was diagnosed, Tiffany had never heard of Roman's condition, so searched on the internet for information.

"I found MDUK, which came up as the number one charity for people with muscle-wasting conditions. The more I learnt and understood about Roman's condition, the more I realised he would still be able to do things. Just differently.

"Roman is very accepting and adaptable and adjusts so well. But he's also really determined – managing to do things doctors told us he wouldn't be able to do. He breaks through every barrier. That's why I also want to help other mums and dads. I can identify with what they might be feeling, and I want to support them."

To hear more about becoming an EDI ambassador email:
EDI@musculardystrophyuk.org

NEW Facebook support group for parents

"When we first received the diagnosis of facioscapulohumeral muscular dystrophy (FSHD) for our twin boys, we were desperate to talk to other parents about their experiences. Getting peer support from others in a similar position to us has been invaluable and helps us feel we're not alone in facing this diagnosis," says Kerry Spink, mum of Oscar and Seb.

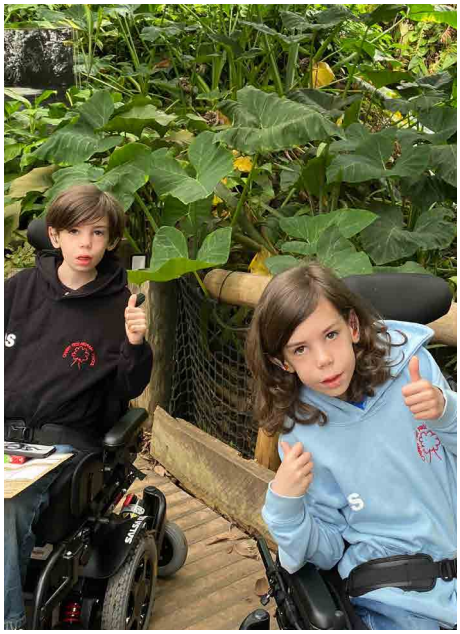
After listening to parents who told us what a support it was to meet others in similar situations, we came up with the idea of having a Facebook group just for parents of children living with muscle-wasting conditions. This group is now live and ready for people to join, no matter the age of your child or how recent the diagnosis.

We have four fantastic volunteer moderators, without whose support we wouldn't be able to create this community. The moderators all have children living with muscle-wasting conditions, and are there to make sure our group is a supportive, friendly, and a safe space for all parents.

You can join the group here www.facebook.com/groups/mdukparentsupportgroup

If you have any questions about our new Facebook parent support group, please get in touch with our Volunteer Engagement Manager, Calley Clay on c.clay@musculardystrophyuk.org.

We will also be continuing our virtual Muscle Group meetings this year, so visit www.musculardystrophyuk.org/get-support where you'll find dates and times for each region of the UK.



Making a difference to Karen's family

'Life-changing for Logan – challenging a DLA rejection'

Logan Lightly, a triplet, was diagnosed with Duchenne muscular dystrophy in February 2020. When his mum, Karen, applied for Disabled Living Allowance (DLA) for Logan, it was rejected. Karen said: "I was very thorough in completing my DLA form and I felt totally let down for Logan."

But Karen was not going to give up, so she got in touch with MDUK when she received her rejection letter.

Karen said: "I got amazing support from the advocacy team, who agreed my claim had been thorough. They asked me all the questions again and prepared a very strong letter stating the reasons why Logan was entitled to DLA. It made even less sense that he got turned down, as we had already successfully applied for a Blue Badge for Logan, with no issues."

With the support of our advocacy team, Logan's application went through mandatory reconsideration, which resulted in his getting low-rate mobility and middle-rate care.

"I felt this decision acknowledged the condition Logan has," Karen said. "Receiving DLA now means that Logan can access things that I couldn't afford previously, such as horse riding and extra swimming lessons, which make a world of difference to him.

"It was so important to us to get this support. I had proof Logan required more care, as I have my two other triplets to compare against, but the DWP initially overlooked this. MDUK helped me at a time when I really didn't know where to turn, and I'll always be incredibly grateful for that."

If you need help getting the care, support, services or benefits you're entitled to, get in touch with us on our Freephone helpline on 0800 652 6352 or at info@musculardystrophyuk.org.

Reflections in Colour

The idea for Reflections in Colour was developed by Andrew Robertson, who has limb-girdle muscular dystrophy, and Chris Howard, renowned cinematographer, after they shared a fundraising 'Kent Castles' walk.

When Andrew saw the photographs that Chris had taken, he contacted people from all over the UK with a muscle-wasting condition to invite them to choose one of the pictures and share their thoughts, or reflections, on them. These often-poignant comments on how the pictures relate to various life journeys work beautifully alongside the thought-provoking images.

Between them, Andrew and Chris put the images and reflections together for a stunning online exhibition. The exhibition was so popular, with many people purchasing prints, that Chris put together two books, and donated a percentage of the profits from sales to MDUK. Chris said, 'Reflections in Colour has become a brilliant way to give people a greater understanding of what it is like to live with a muscle-wasting condition.' You can buy the book from our online shop at shop.musculardystrophyuk.org/products/reflections-in-colour.

To read more about how Reflections in Colour came about, visit: www.musculardystrophyuk.org/get-involved/do-your-own-fundraising/reflections-in-colour

Get involved

Congratulations to our Christmas raffle winners!

Dave, from North London, won our top prize of £3,000. "My grandson Dylan has UCMD [Ullrich congenital muscular dystrophy] and has never been able to walk. He is due for an operation on his scoliosis this year. I think the prize money will mainly go on him and his brilliant mum, Genevieve, who is the main carer."

Play the Spring raffle now! **www.musculardystrophyuk.org/raffle**

Ripples – the easy way to fundraise

Ripples – previously known as Roundups – is a way of raising money for us, pennies at a time! It's like a digital version of a collection box, so you can 'round-up' every time you make a payment with your bank card. Round your payments up to 10p, 50p or £1 – you choose, you are in control. When your donations reach £5, a payment will be made to your chosen charity – hopefully MDUK!

Visit www.joinripples.org/charities/disability/muscular-dystrophy-uk to find out more

ripples™

Friends of MDUK competition winner

Patricia Lock has had an exciting few months! Towards the end of last year, as a Friends of MDUK member, she won our prize draw – two nights' accommodation for two people, in a UK/European destination, courtesy of Handiscover accessible breaks! Then, in January 2022, Patricia received MDUK's Volunteer of the Year President's Award! Congratulations Patricia, and we hope you enjoy your well-deserved break.

If you're interested in becoming a Friend of MDUK, please visit www.musculardystrophyuk.org/friends where you can see all the benefits of becoming a member.

Gifts in Wills

Gifts in Wills are incredibly important to MDUK. In fact, they fund around 30 percent of everything we do. Every single gift, no matter how large or small, helps our work to beat muscle-wasting conditions – we rely almost entirely on voluntary donations for our research to find treatments and cures, access to specialist support through the NHS, and advocacy and support services to remove limits in life for individuals and families living with muscle-wasting conditions.

For further information about leaving a gift in your Will, including how to make or update your Will for free, visit www.musculardystrophyuk.org/giftsinwills or call Grace Moran on 020 7803 4845.

Muscular Dystrophy UK

Fighting muscle-wasting conditions

Weekly **Lottery**

Play our Weekly Lottery and you could win £10,000!

Here's an exciting way for you to join the fight against muscle-wasting conditions and help people like Luke – play the MDUK Weekly Lottery!

Playing is easy – from just £1 a week you can have the chance to win some great cash prizes from £5 to £1,000 – and a whopping first prize of £10,000!

Enter today at **www.musculardystrophyuk.org/lottery** or by phoning our Weekly Lottery hotline on **01628 821 983** (Mon-Fri, 9am-5pm)

Scan here to join our lottery today!

Luke Cramphorn who lives with Duchenne muscular dystrophy

£10,000 JACKPOT

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