Autumn 2020

Muscular Dystrophy UK

Fighting muscle-wasting conditions

Campaign

The newsletter for our supporters

Inside:

VIRTUAL

CELEBRITY SPORTS QUIZ

With the UK in lockdown, we've found new ways for our dedicated supporters to continue helping to raise funds in support of MDUK's work.

This April, we launched our first ever virtual MDUK Celebrity Sports Quiz and supporters across the UK were able to join from the comfort of their sofas and test their sporting knowledge. The first quiz was such a success that it was followed by a second quiz in May.

The evenings were hosted by comedian Andy Zaltzman, and MDUK's own President Gabby Logan stepped into the role of quizmaster. Teams were also joined by a host of fabulous sporting celebs – including rugby player Charlie Hodgson, athletics star John Regis and cricketers Ryan Sidebottom, Phil Tufnell, Alec Stewart and Graeme Swann – who took part in a number of fun challenges.

Thanks to our wonderful supporters, the evenings raised an incredible £15,700!

MDUK President – and the evening's quizmaster – Gabby Logan

Former England cricketer Ryan Sidebottom, winner of the Tea Bag Challenge

Olympic athlete, John Regis took on the Straw Challenge

Welcome

I'm delighted to welcome you to the autumn edition of Campaign – the newsletter for supporters of MDUK.

Since our last edition earlier this year, many people will feel like the world has changed, but throughout the global pandemic, MDUK's mission has stayed the same – to ensure that every day counts for people living with muscle-wasting conditions.

Despite a loss of £2.8 million due to the pandemic, MDUK has adapted quickly to ensure it can continue to deliver some of its most vital services. On page 10 and 11, you'll read about how we've reshaped our services as well as created new ways to provide support during lockdown.

And on page 6, you can see a snapshot of life as a researcher during lockdown and how they've adjusted to ensure their vital work continues, even when they haven't been able to access the laboratory.

I hope you enjoy reading about the important work that has continued during this incredibly difficult time, all thanks to support such as yours.

Gabby Logan MBE
President, Muscular Dystrophy UK

PS: Our gorgeous new range of Christmas cards has arrived – you're sure to find the perfect cards to send your loved ones this Christmas. You can visit the MDUK online shop today: www.musculardystrophyuk.org/shop

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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FIND US ON

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DMN/2009

On the cover: Six-year-old Carmela takes part in the 2.6 Challenge for MDUK (see p5)

Six-year-old Carmela with mum, Lucy

Catching up with Carmela

When Carmela Chillery-Watson was three years old, she was diagnosed with LMNA congenital muscular dystrophy (LMNA CMD), a particularly rare muscle-wasting condition that will see Carmela's muscles deteriorate to the point of needing a power wheelchair full time, and for which there is presently no treatment or cure.

Carmela's mum, Lucy, says, "We've received a lot of support from MDUK since Carmela's diagnosis. When she was around three-and-a-half years old, MDUK helped us by providing supporting evidence for her Education, Health and Care plan. Their advice and guidance was invaluable, and Carmela was awarded the highest level of provision and now receives 1:1 teaching support at school."

"We've also received equipment grants from MDUK, as well as valuable advice about house adaptations and, through MDUK, have connected with other families who have a child with the same condition as Carmela."

Lucy says, "For me, Carmela's diagnosis and the way her condition develops means life can be an emotional rollercoaster. Being able to talk to another parent goes some way to lightening the load. It also gives me peace of mind knowing that MDUK is there to help at the drop of a hat and that they're only a phone call or email away. It takes away the pressure and anxiety of not knowing where to turn."

Lockdown has been particularly hard for Carmela, as her condition means that she has had to shield. During this time, she was unable to see her dad, Darren, as he is a driver for the NHS and, to protect Carmela, he had to sleep in a shed in the garden. Lockdown has also taken a physical toll on Carmela as she hasn't been able to do the exercises that help keep her muscles strong. This muscle strength and time together are things the family will never get back.

Lucy says, **"Carmela is such a positive little girl, and because she's never really known life any other way, she tends to focus on the things she can do, rather than the things she can't."**

And so this year, amid lockdown, Carmela decided that she wanted to do something to help support MDUK's work during the COVID-19 pandemic. In April, Carmela joined the 2.6 challenge, a UK-

wide fundraising event created to help charities raise vital funds during this difficult time.

Carmela has been doing laps of her home therapy assault course to raise money and has raised over £1,500. **But Carmela isn't stopping there!** She now has her sights set on her next challenge for MDUK which will have a Wonder Woman theme. As well as being Carmela's favourite character, Wonder Woman reminds Carmela of her mum because of the way Lucy has looked after her 24/7 during her time shielding.

See how Carmela is getting on by visiting:
www.walkwithcarmela.org

The research that will help Carmela and children like her

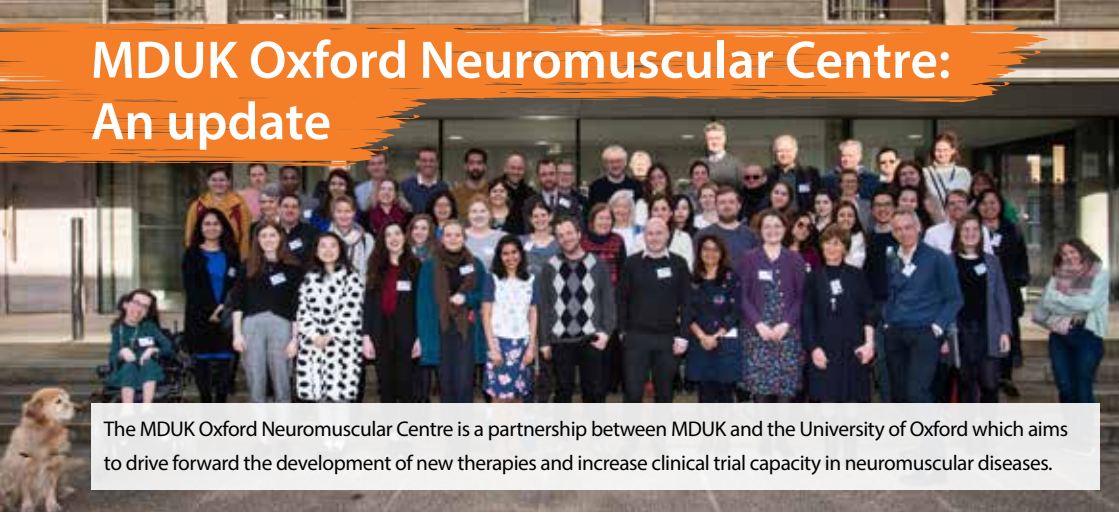
Changes (mutations) in the LMNA gene cause a group of muscle-wasting conditions called laminopathies. People with laminopathies can be affected very differently, even if they have the same condition or even the exact same mutation. At the Myology Institute in Paris, Dr Gisèle Bonne and her team are undertaking a research project to find out why this is the case.

The project which is co-funded by MDUK and Cure CMD is a collaborative partnership established to support world-class research into LMNA. This research could not only inform how to clinically care for people with LMNA but could also help identify new therapies to treat these conditions.

Dr Bonne's team have been working with international colleagues to find samples (including muscle biopsies) kindly donated by patients with LMNA from across the world. So far, the team have used these samples to identify some differences in the LMNA genes and have also begun to see how different changes in these genes can affect the muscle's function at a cellular level. This research will hopefully allow Dr Bonne and her team to better understand why some people with this condition show very different symptoms to others.

Follow the latest research news by visiting
www.musculardystrophyuk.org/research-news

MDUK Oxford Neuromuscular Centre: An update



The MDUK Oxford Neuromuscular Centre is a partnership between MDUK and the University of Oxford which aims to drive forward the development of new therapies and increase clinical trial capacity in neuromuscular diseases.

This February, the Centre held its first annual scientific meeting for ONMC research group members which was opened by Professor Matthew Wood (Centre Director) and Dr Kate Adcock (Director of Research and Innovation at MDUK) who reflected on the exciting developments in the field and the importance of the research carried out at the Centre.

The scientific talks were kicked-off with a keynote lecture by Professor Carsten Bönnemann (National Institute of Health, USA). There was a series of excellent scientific talks from researchers highlighting the breadth of neuromuscular conditions that are studied by teams within the Centre. These included Dr Richard Webster who spoke about his research to understand the mechanisms that enable signals to travel from nerves to the muscles and his work in congenital myasthenic syndromes (a group of inherited conditions which prevent the signal coming from nerves reaching the muscle). In addition, Dr Victoria Nesbit discussed her research into the natural history of mitochondrial disorders and how she uses her findings to develop the best clinical practice for these disorders.

Attendees also participated in an engaging panel discussion chaired by Professor Laurent Servais with expert input from neuromuscular researchers from around the world. The discussion focused on new developments in gene therapies for neuromuscular diseases and top tips to help guide the Centre's work in these exciting times.

Thank you to everyone who has supported the Centre, you can read more at: www.onmc.ox.ac.uk

The life of a researcher in lockdown

When the UK moved into lockdown, scientists around the country suddenly found themselves without access to a laboratory and their normal research environment. MDUK-funded researcher Professor Antonella Spinazzola tells us how she and her team have progressed their research, during this time, even from home.

What we do

My research focuses on parts of cells called mitochondria which convert food into a form of energy that our body can use. My colleagues and I study how mitochondria are made and function, what happens when things go wrong, and how we can fix them. We have a special interest in the DNA inside the mitochondria, as defects in this can cause a number of conditions that lead to weakness, pain and fatigue in muscles.

By gaining an understaning of the mechanisms that cause these conditions, we hope to develop therapies that can halt or reverse their progression.

A challenging start to lockdown

The first few weeks in lockdown was an unsettling and frustrating time. My team had spent months preparing to test a number of chemicals on a disease model to see whether they could help treat a particular type of mitochondrial disorder.

We'd also planned to conduct a study to test the safety and efficacy of a new compound in subjects with mitochondrial myopathy - a project supported by MDUK.

However, when lockdown started, we needed to stop all experimental work within seven days.

The new normal

I initially found it difficult to establish a new routine while working from home. It took me a few weeks to settle into this new way of working, but I felt more determined than ever to progress the research projects that could make a real difference.

Much of my research cannot be performed without specialized equipment used in a laboratory, such as microscopes, incubators, and disease models, from cells derived from patients to transgenic animals.

Yet somehow I ended up just as busy as usual in lockdown. I have been fortunate, as in the past years my group has been gathering enough data that could be analysed and processed and assembled into scientific papers. It is important to process our data and publish papers so that other researchers can see our findings and, hopefully, build on them in their own research.

We were also able to use remote connections to switch from physical meetings to virtual ones, and used these to revise projects, discuss papers, and make plans to prioritise activities on our return to the lab.

Looking to the future

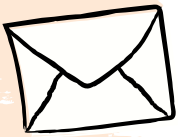
Finally, I'm pleased to say that at the end of July, we were able to return to the lab – albeit with many restrictions in place. Despite these restricitions, I was eager to return to the research that I love, because, as Stephen Hawking said 'science is not only a disciple of reason but, also, one of romance and passion'.

To read more about Professor Spinazzola's research project, visit:
www.musculardystrophyuk.org/mito-study

Here to help during lockdown

Throughout the coronavirus (COVID-19) pandemic, MDUK has continually adapted to ensure people living with muscle-wasting conditions still have access to our most vital support services when they need them most.

Between 1 March and 31 July this year, we received **953 requests for support via our helpline, on email, and through neuromuscular clinics.** Of these, almost over one third related to COVID-19, including topics like shielding, alert cards and care plans.



How we've helped

Like many organisations in the UK, MDUK has felt the impact of the pandemic. As the charity's resources changed, we had to respond quickly to the increasing and urgent needs of the people we support.

We assessed where the need was greatest, and how we could best deliver our key services – such as our helpline and advocacy services, despite having had to furlough part of the team. Existing volunteers were given specialised training around Personal Independence Payment (PIP) applications and became instrumental in helping to meet the demand for this service and supporting people through the (sometimes daunting) application process. This enabled MDUK staff members to respond quickly to other requests for support and continue to provide more specialised services, such as MDUK's peer support programme, practical advice and guidance in response to queries, and support for people who needed to appeal PIP or DLA decisions.

New support during lockdown

The risk of the virus to people living with muscle wasting conditions and extended shielding periods meant that there was an increase in anxiety, stress and isolation so we worked with a group of professional life coaches who volunteered for the charity to provide free one-to-one coaching sessions.

Within the first week of lockdown, MDUK also launched a series of Facebook live Q&A sessions. The MDUK Helpline team, which specialises in accessing benefits and other support, was on hand to answer questions. Additionally, a group of neuromuscular specialist clinicians had been established who helped the team ensure they could provide accurate clinical information about coronavirus and muscle-wasting conditions.

Making a real difference to Tobias

Thea, mum to 7-year-old Tobias, applied for Disability Living Allowance (DLA) in January 2020. Though the application was accepted, payments were awarded at the lowest rate, which his family felt was lower than he was entitled to.

MDUK's advocacy team provided support through the appeal process and in June, Tobias and his family received the good news that their appeal had been successful and Tobias would receive payments at a higher rate.

"We already felt in a state of crisis because of lockdown and finding out about the decision on Tobias' DLA really heightened this. MDUK guided us throughout the appeal process and were always there with specialist knowledge and to reassure us." says Thea, Tobias' mum.

To help make sure we can continue to deliver vital services to people living with muscle-wasting conditions, you can donate by visiting: www.musculardystrophyuk.org/autumn20

Muscles Matter 2020: online sessions

This autumn, we have moved our National Conference online and will be holding a series of virtual seminars from now until October.

We will be sharing a diverse programme covering advances in condition-specific research as well as support and advice on living well and how we are driving change for specialist care and support. All sessions are free to attend. Book your place today at: **www.musculardystrophyuk.org/MusclesMatter2020**

Condition-specific seminars:

Duchenne muscular dystrophy
Wed 9 September, 4 – 5.35pm

Mitochondrial disease
Mon 14 September, 1 – 2.35pm

Limb girdle muscular dystrophy
Wed 30 September, 10 – 11.35am

Becker muscular dystrophy
Sat 10 Oct, 10 – 11.35am

Charcot-Marie-Tooth
Thurs 22 October, 4 – 5.35pm

Practical advice seminars:

Assistive technology
Wed 9 September, 6 – 7.10pm

Managing education
Mon 14 September, 4.30 – 5.40pm

Navigating the world of employment
Wed 30 September, 12 – 1.10pm

Accessing financial support
Sat 10 Oct, 12 – 1.10pm

Accessing services in the world of COVID-19
Thurs 22 October, 6 – 7.10pm

Changing Places campaign gets a boost

A major change to guidance for building rules in England will require Changing Places toilet facilities to be designed and built into new public buildings – including shopping centres, supermarkets and arts venues – from next year.

As well as this, the Department for Transport, in partnership with Muscular Dystrophy UK, has also announced £1.27m to install 37 more changing places at service stations across England. These new facilities will give people with complex needs and their carers the confidence and freedom to make more journeys by road as coronavirus restrictions ease.

Rob Burley, Director of Campaigns, Care and Support at Muscular Dystrophy UK, said:

"This is huge news for the quarter of a million people in the UK who need Changing Places toilets. Having access to these much-needed facilities increases independence and improves quality of life. The changes to legislation will make it easier for disabled people and their families to enjoy activities that many take for granted."

As co-chairs of the Changing Places Consortium, we're delighted with this progress but will continue our work to make sure these facilities are available to those who need them in all buildings and venues.

To find out more about Changing Places toilets and how you can get involved in our campaign, visit: www.changing-places.org

Get Involved

go bright for the fight!

Go Bright for the Fight! February 2021

Make muscles matter this February and Go Bright for MDUK!

Wherever you are, join people all over the UK and wear your brightest clothes to help raise vital funds to support people living with muscle-wasting conditions. You can also take part in some of our fun Go Bright challenges on the day!

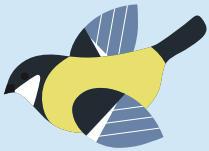
Find out more by visiting www.musculardystrophyuk/go-bright or email go.bright@musculardystrophyuk.org to register your interest.

MDUK shop

Our new range of Christmas cards and gifts has arrived at the MDUK online shop. With a wide selection to choose from, and stocking fillers starting at just £1, you're sure to find something to delight your loved ones on Christmas morning.

Shop online at www.musculardystrophyuk.org/shop

Congratulations to our Great Muscle Raffle winners!



1st prize £3,000 Mrs Harrison, St Helens

2nd prize £250 Mrs Tregunno, East Grinstead

3rd prize £50 Mrs Vann, Rainham

Thank you to everyone who took part and do keep an eye out for the MDUK Christmas Raffle which will launch in October. It's a fun and easy way to support our work and with a top prize of £3,000, you don't want to miss out!

From early October you can play in the Christmas Raffle by visiting: www.musculardystrophyuk.org/raffle

Gifts in Wills

When you choose to leave a gift in your Will, you'll be helping create a future free from the limitations of muscular dystrophy.

With your help, the next generation of research scientists will finish what we've started.

For more information about gifts in Wills, please phone 0300 012 0172 (Mon-Fri, 9am–5pm) or email legacy@musculardystrophyuk.org www.musculardystrophyuk.org/giftsinwills

"We were devastated when we found out our beautiful grandson, Jack, had Duchenne. All we wanted to do was take this horrible condition away. We can't do that, we know. But your gift in your Will could make that happen one day."

Steve Gauder, pictured with Jack

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)

Luke de Bruin who lives with Duchenne muscular dystrophy