Muscular Dystrophy UK

Fighting muscle-wasting conditions

Campaign

The newsletter for our supporters

Inside: A new era of research • Campaigning for easier access to benefits • Here for you: MDUK's peer support

Spring 2020

BGC Charity Day 2019

Celebrities and Muscular Dystrophy UK (MDUK) supporters came together and took to the trading floor as part of global stockbrokers, BGC Partners', 15th annual charity day.

Held at their offices in Canary Wharf, this special day of fundraising takes place each year in memory of the BGC staff who lost their lives during 9/11.

A host of famous faces including Beverly Knight, Kirsty Gallagher, Monty Panesar, Sam Allardyce and MDUK President, Gabby Logan MBE, gave their time to join the fight against muscle-wasting conditions and represent MDUK at this exciting event.

They tried their hand at trading, including closing deals with clients over the phone. As you'll see, it was a fun-filled day for celebrities and supporters alike, and we're grateful to BGC for supporting MDUK in this way.

Five-year-old Carmela was excited to meet comedian and BGC host, Jimmy Carr (l) and former football manager, Sam Allardyce (r).

MDUK's young supporters, like Luke Cramphorn and his mum, Lisa, took part in fun activities – and even met some exotic animals!

Beverly Knight (l), Gabby Logan MBE (c) and Kirsty Gallagher (r) were joined by BGC traders on the trading floor.

Welcome

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

On the opposite page you'll see some photos of the 2019 BGC Charity Day. It was a fantastic day enjoyed by everyone who attended and I was delighted to attend in my role as MDUK President.

You'll see an update from the McMenemy family who featured in our Christmas appeal – including seven-year-old Somhairle (pronounced Sorley). We had a wonderful response to the appeal and you can find out on p5 what the family has been up to since then.

You can also read the latest news about our MDUK Oxford Neuromuscular Centre, including an exciting new research project that could make more people with Duchenne muscular dystrophy eligible for gene therapy in the future.

I hope you enjoy finding out about the important work that your support is making possible. We're truly grateful for all you do for MDUK – you really are making a difference.

Gabby Logan MBE
President, Muscular Dystrophy UK

PLEASE NOTE: Most of the articles in this edition of *Campaign* were written before the coronavirus crisis took hold, and the images were taken at a time before social distancing was introduced.

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: Somhairle's school goes orange for a day (see p5)

Somhairle with mum Sarah and dad Gerry at his school for the recent Go Orange for a Day

Update from the McMenemy family

We'd like to say an enormous 'thank you' to everyone who gave to our Christmas appeal. The McMenemy family has been overwhelmed by the incredible response and, thanks to your kind gifts, you've raised over £32,000 since Sarah's letter.

In 2017, Sarah and her husband Gerry were told their little boy Somhairle had Duchenne muscular dystrophy.

In her letter, Sarah shared the challenges they faced as a family after this diagnosis: seeing their son in a wheelchair for the first time; feeling overwhelmed at the prospect of having to break the news to their friends and family; and how information and resources from MDUK helped them through this difficult time.

Since writing their letter, the McMenemys have had a busy start to 2020.

On Wednesday 5 February, the McMenemys went 'bright for the fight' and took part in our annual Go Orange for a Day campaign. Somhairle's entire school – including two teachers who dressed up as traffic cones – donned their brightest orange clothes.

The event was one of many across the country, which will help fund the groundbreaking research that brings hope to families like the McMenemys who live with muscle-wasting conditions.

Somhairle has also had a visit to the muscle clinic and an appointment at the Royal Children's Hospital in Glasgow. Somhairle had a bone density scan as well as a number of blood tests, and was extremely brave throughout all of these.

Your generosity means we can be there with information and advice to help families like Sarah's who are facing a muscle-wasting diagnosis. It means they can get the support they need, when they need it.

If you would like information or support on muscle-wasting conditions, please call our Information and Support Team on 0800 652 6352.

There's still time to donate to help children like Somhairle – visit: www.musculardystrophyuk.org/christmas

A new era in research

Prof Matthew Wood

We are committed to finding new treatments and, ultimately, cures for people with muscle-wasting conditions. Last year, thanks to the generous backing of our supporters, we established the MDUK Oxford Neuromuscular Centre.

The Centre is a partnership with the University of Oxford and aims to find potential new treatments and get them to patients faster. It has already attracted world-leading researchers from across the globe with Prof Laurent Servais, an internationally renowned neurologist, joining the Centre as Professor of Paediatric Neuromuscular Diseases.

Prof Matthew Wood, Professor of Neuroscience at the University of Oxford and director of the Centre, said, **"The MDUK Oxford Neuromuscular Centre will be one of the very first that takes drug development from basic research to clinical trial testing, and will also be increasing the capacity for clinical trials within the UK."**

Investigating new ways to make gene therapy more effective

One of the new research projects the MDUK Oxford Neuromuscular Centre

is undertaking is looking at how gene therapy is administered to patients.

Several gene therapies for muscle-wasting conditions are being tested in clinical trials, which use a harmless virus to deliver the desired gene into the body. Viruses can effectively carry information into our cells, making them ideal delivery vehicles for gene therapies.

However our bodies have naturally evolved to fight viruses. This means some patients will not be able to receive gene therapy (as their bodies will already be immune to the virus carrying the gene) while others may only get one dose before their bodies become immune. Instead of viruses, the research team led by Prof Wood is using exosomes – tiny packages found naturally inside the body – to carry a smaller version of the dystrophin gene (called micro-dystrophin), which is being used as a therapy for Duchenne muscular dystrophy. This could make more people with Duchenne eligible for gene therapy in the future and have similar implications for other muscle-wasting conditions.

Visit www.onmc.ox.ac.uk to find out more about the MDUK Oxford Neuromuscular Centre.

Prof Laurent Servais

Campaigning for easier access to benefits


Chloe Smith with her son, Jenson, and partner, Rikki Schroeder

Financial support is crucial when you're living with a muscle-wasting condition, but many people aren't receiving the benefits they need and can end up struggling financially, emotionally and practically. We are campaigning to make the system easier to navigate and for assessors to understand the complexity of muscle-wasting conditions.

Chloe Smith, whose three-year-old son, Jenson, has a muscle-wasting condition, knows how difficult the current welfare system can be. Following her son's diagnosis, Chloe reduced her working days and applied for Disability Living Allowance (DLA) to help care for him.

Chloe was denied the mobility component of the DLA because of Jenson's age at the time of application. She was also told she was not eligible for Carer's Allowance, which means she misses out on additional financial support despite caring for Jenson 35-plus hours a week.

"On top of that, we live in a first-floor flat and I have to carry Jenson up the stairs to get to it," says Chloe. "There's no parking and it's not big enough for all of Jenson's equipment. When we applied for local housing, we were placed as 'bronze banding', which is the lowest-priority category. We have now removed ourselves from the register because of the stress."

We increasingly hear stories of people like Chloe who struggle to fill in the necessary forms, are denied benefits that would help ease the financial burden, and may experience inaccurate assessments. It can have a serious impact on their quality of life, independence and mental health.

We published our report calling for change this year, along with a guide to help people with muscle-wasting conditions apply for Personal Independence Payment (PIP). Find out more at www.musculardystrophyuk.org/below-standard

Transforming support with artificial intelligence

We have teamed up with digital social enterprise Reason Digital and charities Stroke Association, MS Society and Parkinson's UK to transform the way we deliver health advice and information.

In this unique partnership, we're harnessing the power of artificial intelligence (AI) to deliver personalised advice and information via email.

We're calling it the Digital Health Assistant (DHA), and we believe it will transform the way millions of people in the UK, including those living with muscle-wasting conditions, receive health information and advice.

By gathering and interpreting data using machine-learning, the DHA will create and email content tailored to your specific needs, adapting to your circumstances over time, ensuring you get the most appropriate and effective ongoing support.

People with long-term conditions can struggle to get up-to-date, specific information, especially at the point of diagnosis and there are often not enough healthcare professionals available to pass this information on. We expect the DHA will dramatically improve access to quality practical and emotional care.

Representatives from the partnership met to launch the DHA

Thanks to support such as yours, we are able to invest in groundbreaking projects to improve individuals' and families' daily lives – we couldn't do it without you.

Visit www.musculardystrophyuk.org/DHA to read more about the project.

The latest news on Spinraza

Spinraza is currently the only treatment available on the NHS for spinal muscular atrophy (SMA), a severe neuromuscular condition that sees children irreversibly lose the ability to crawl, walk and swallow. In the most severe cases, babies with SMA are unlikely to see their second birthday, if untreated.

Spinraza is widely seen as a life-changing treatment; children who have received it have gone on to crawl and even walk.

The National Institute for Health and Care Excellence (NICE), the body that decides which treatments should be routinely funded by the NHS, originally rejected Spinraza. However, this decision was reversed following an 18-month campaign involving MDUK, SMA UK, clinicians and the wider SMA community. The announcement last year was good news for Mark Wilson, whose son, Aadi, has SMA Type 2.

"It means we can feel more excited about what the future might hold. We don't know how much impact it will have on our son, we just want the opportunity to try," says Mark.

Spinraza is now available through the NHS across the UK for people with SMA Types 1, 2 and 3. Children with SMA are starting to receive treatment but rollout is taking longer for adults, and not everyone who can benefit from the treatment is receiving it.

This means our campaign is not yet finished and we are working hard to make sure those who are eligible can get access to Spinraza – and to extend eligibility to all patients who stand to benefit from the treatment.

Your donations will help us continue our campaign to make Spinraza available to everyone with SMA. Find out more at www.musculardystrophyuk.org/fast-track

Panna, Aadi, baby Sahaana and Mark Wilson

Here for you: MDUK's peer support

Liz Williams was diagnosed with facioscapulohumeral muscular dystrophy (FSHD) in 2004. She now works closely with us to provide advice and support to others with muscle-wasting conditions.

Liz first heard about MDUK after visiting online forums to find out more about her condition. In 2012, she attended one of our first FSHD information days and can still recall the effect it had on her.

"I met so many people who I am still in contact with even now… I became more and more involved with MDUK and eventually, when the peer support scheme started, I jumped at the chance to volunteer."

Our peer support volunteers give their time to help others living with muscle-wasting conditions. This can include providing advice on issues such as home adaptations, planning a holiday or even starting a family.

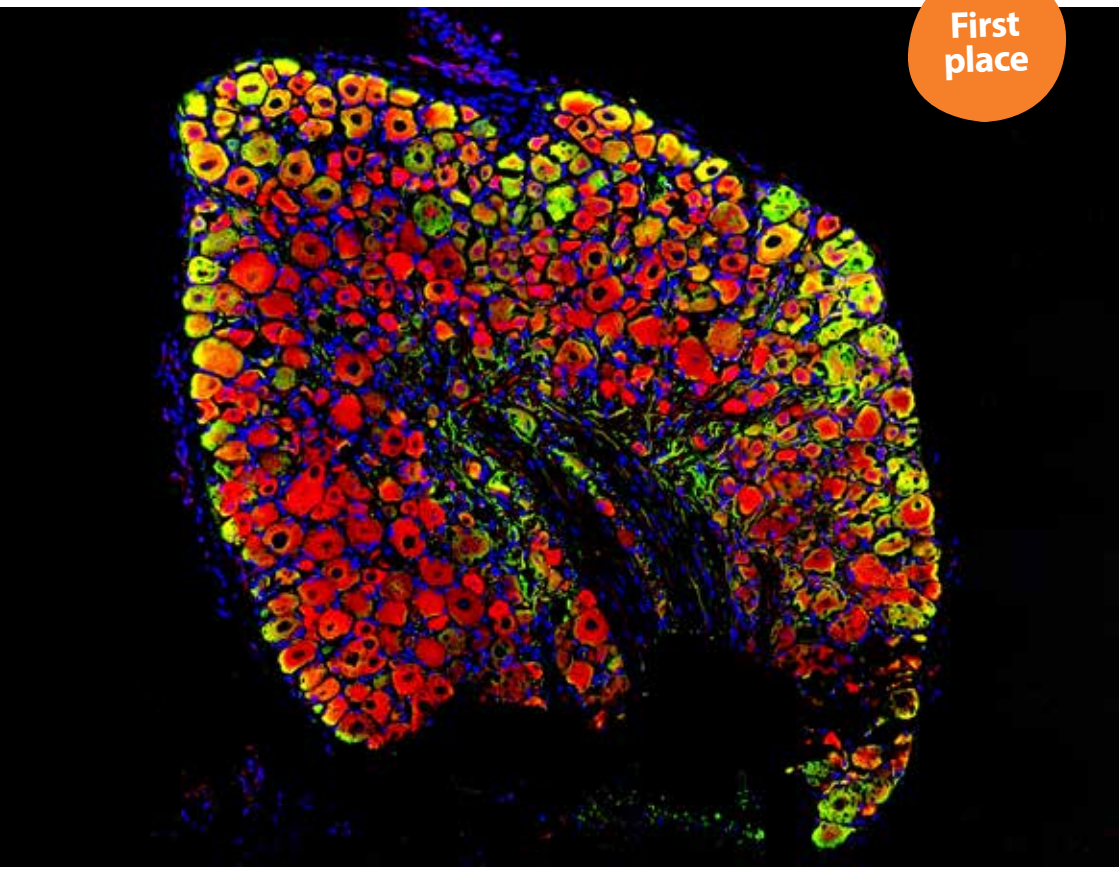
Liz says she gets just as much advice from those she helps as they do from her.

"I have been supported by and provided support to an amazing army of individuals. Not only have I maintained a level of independence I am happy with through the knowledge I have gained through others, I have a ping from happy endorphins every time I feel I have helped someone in some way."

Our peer support service relies on support from individuals. You can find out more about volunteering or make a donation by visiting www.musculardystrophyuk.org/get-involved

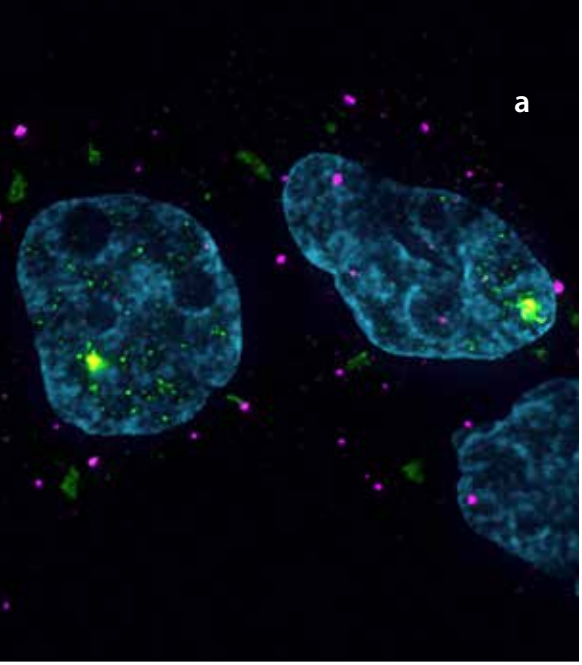
Research image competition

In September 2019, the MDUK research image competition was launched, giving neuromuscular researchers an opportunity to showcase their research in a different and engaging way for the public.



'Making sense of Charcot-Marie-Tooth disease' by Dr James Sleigh, University College London

Here we see Dorsal root ganglion (DRG) - collections of nerve cells that communicate the things we touch, smell, hear, taste and see, to the central nervous system. DRG are studied as part of research into some nerve-related conditions such as Charcot-Marie-Tooth disease. This image shows a section of a mouse DRG (red and green = nerve cells; blue = cell nuclei, where DNA is kept).

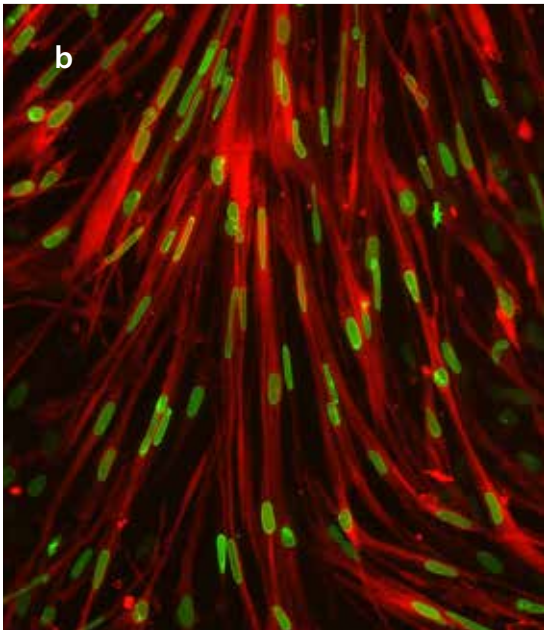


a) 'Myotonic dystrophy type 1: cells under stress' by MDUK funded researcher, Dr Judith Sleeman, University of St Andrews

Here we see cells from a model of myotonic dystrophy (MD), grown under conditions that stress the cell. In this image, the RNA (which is important in the processes used by cells to make proteins) is green and yellow, and the DNA (which carries genetic information that living things need to grow and function) is turquoise.

b) 'Modelling a skeletal muscle laminopathy using induced pluripotent stem cells' by MDUK funded researcher Daniel Moore, University College London

This image shows a model of the rare condition skeletal muscle laminopathy, using induced pluripotent stem cells (cells that can be turned into any other cell type in the lab). Models such as this one can be used to test therapies. (Red = muscle cell; green = nuclei.)



Visit www.musculardystrophyuk.org/research-news to find the latest research news.

Ways to support MDUK from home

We understand that the coronavirus pandemic is affecting everyone in many different ways. Just like you, we are facing uncertain times, and there is now an urgent need to raise more funds to meet the growing demand the COVID-19 crisis has had on our services.

For many of you, staying at home means that helping to fundraise will be more difficult at the moment, but on these pages, we have some fun and innovative ways you can still support MDUK's work – all from your own home.

Bake a Difference – host a virtual tea party!

Now, when you sign up to Bake a Difference, you can choose to host a virtual tea party from your home! Getting involved is easy and as well as being a fun (and delicious!) way to raise funds to support people living with muscle-wasting conditions, it will also give you and your friends a great reason to catch up via video-call and show off your baking skills.

To find out more about holding your own virtual tea party, visit the Bake a Difference zone online at www.musculardystrophyuk.org/bake-a-difference

Louise (pictured) is one of MDUK's Regional Development Managers who is here to support your fundraising activity. She's been busy baking at home, ready for her own Bake a Difference tea party.

Louise says, **"I love baking and my flourless chocolate cake is one of my favourites. I'm really looking forward to my tea party – there's nothing better than cake and a catch up!"**

And if you would prefer to host a tea party in person later in the year, register your interest by emailing **bakeadifference@musculardystrophyuk.org** or by phoning **0300 012 0172** and one of our Regional Development Managers will be in touch, when the time is right, to support you in your fundraising.

Join the fun with our Weekly Lottery

The MDUK Weekly Lottery is an exciting way to join the fight against muscle-wasting conditions as well as have the chance to win up to £10,000 – every Friday!

Weekly Lottery

You can join for just £1 per week and as well as our fantastic top prize, you'll also have the chance to win some great cash prizes from £5 to £1,000.

By joining the Weekly Lottery, you'll be supporting our vital work, every single week.

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

Raise funds for MDUK when you shop online

Whether you're doing your grocery shop, treating yourself to something special or even just paying your bills, signing up with one of our online shopping partners is a great way to raise free funds for MDUK.

We have four shopping partners to choose from, and when you sign up and shop, the retailer will make a free donation to your chosen charity.

With thousands of participating stores, it's an easy way to raise money for MDUK – at no cost to you.

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Visit www.musculardystrophyuk.org/shopping-partners to find out more.

LEAVE A GIFT IN YOUR WILL TO 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.

"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, Jack's granddad**

Muscular Dystrophy UK

Fighting muscle-wasting conditions

For more information about gifts in Wills, call Beth Glennie on **0300 012 0172** or email **legacy@musculardystrophyuk.org**

www.musculardystrophyuk.org/giftsinwills