

Spring 2021

Muscular Dystrophy UK

Fighting muscle-wasting conditions



Campaign

The newsletter for our supporters

Inside:

- Out and about with MDUK fundraisers
- Big news for Changing Places
- Moving Up in 2021

Out and about for MDUK

Here we share some snapshots of our dedicated supporters and their efforts to raise funds and awareness for MDUK during lockdown.

Jordan Mossom (pictured front cover and top right) who has Duchenne muscular dystrophy, raised awareness for the condition by creating a portrait of himself wearing his brightest clothes each day throughout February as part of MDUK's Go Bright. He said taking part helped to lift the depression and lack of motivation he had been experiencing during lockdown.

Amy Williams (centre right) took on a triathlon challenge, walking, cycling and rowing 60km in memory of her best friend, Jack, who had Duchenne muscular dystrophy. Amy, who has cerebral palsy and epilepsy, adapted the challenges for her condition by using a walking frame, a tricycle and a rowing machine.

Ben Ridsdale (bottom right) is embarking on a three-day bus adventure to raise money for MDUK in memory of his two best friends, Matt and Rory, who both had Duchenne muscular dystrophy. Ben, who is visually impaired, will travel from Crewe to London using only his free bus pass, taking around 15 buses to complete his journey.



Welcome

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

Since our last edition, we have continued our work to make sure individuals and families with muscle-wasting conditions have the vital information and support they need during these uncertain times.

On p8, you can find out about a promising treatment for SMA Type 1, known as Zolgensma, and MDUK's work to make sure it's accessible to those who need it.

And on p9, you'll read some exciting news from our Changing Places campaign and how MDUK will be supporting the government to ensure these accessible toilet facilities are available for those who need them.

On p12 and 13, you'll find out how MDUK's new Work Experience Development Officer, Jack McLellan, will be supporting young disabled people to gain valuable work experience in the year to come.

I hope you enjoy reading about the incredible impact your generous support is having on the lives of people living with muscle-wasting conditions.



Gabby Logan MBE
President, Muscular Dystrophy UK



PS: Some exciting new products have arrived at the MDUK online shop, including our stylish string shoppers. Check out our range of gorgeous gifts and homewares at www.muscular dystrophyuk.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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On the cover: Jordan Mossom, a photographer who has been raising awareness for MDUK.



The Gibbs family: Tobin (7) and big sister Ophelia (11) with mum, Thea and dad, Simon

Update from the Gibbs family

A huge 'thank you' to everyone who kindly donated to our Christmas appeal. Since Thea Gibbs wrote to you in November about her son, Tobi, we have received an incredible £35,000 in response to the appeal – and the donations are still coming in!

At the start of 2020, seven-year-old Tobi received a neuromuscular diagnosis, and the family was told that it looked like a congenital myopathy. While this was scary news, it enabled a plan to be put in place for further investigations. But then the pandemic came along, shutting everything down, and the plan went out the window. During this difficult time, MDUK's Helpline and advocacy services were there when the Gibbs family needed it most.

Now, a year on, Tobi is enjoying being back at school after lockdown, although he has struggled with fatigue, sore muscles and emotions. He is also finally about to receive a scan so that doctors can take a closer look at his heart, followed by a general anaesthetic to take muscle biopsies. The family hopes this will lead to a full diagnosis for Tobi, and provide further information to support his Individual Pupil Resourcing Agreement (IPRA) to make sure Tobi has the support he needs while attending school.

We're also delighted to share the news that Margot, a Goldendoodle puppy, has just joined the Gibbs household (pictured below). The family hopes that in time, she can be trained as a therapy dog for Tobi, to enrich his life even further.

Thanks to kindness such as yours, MDUK can be there for families like Tobi's who are facing a diagnosis with a muscle-wasting condition, making sure they get the information and support they need when they need it most.



There's still time to donate to the appeal, visit:
www.musculardystrophyuk.org/Christmas

Improving care for adults with Duchenne

In 2020, MDUK was delighted to share updates on two major projects from the Adult North Star Network that will improve care and support for adults living with Duchenne muscular dystrophy.

These include the expansion of the North Star natural history database and the first-ever national *Standards of Care for adults with Duchenne muscular dystrophy*.

MDUK has funded the North Star Network for children, led by Professor Francesco Muntoni and Dr Adnan Manzur, since 2004. The network, which includes 23 neuromuscular centres across the UK, has established a range of regular assessments of children with Duchenne muscular dystrophy to help better understand the progression of the condition.

The centres upload clinical data to a central database, creating what is known as a natural history study. The network, and the data it collects, help to improve the care that is offered to children, to inform the design of clinical trials and to monitor the potential effects of treatments.



For example, data collected in the North Star database showed the benefits of long-term steroid treatments in boys with Duchenne muscular dystrophy. This information led to clinicians across the country using steroids to treat children with the condition. As a result, children benefiting from the use of steroids are now walking for around three-and-a-half years longer than children in the previous decade.

2020 saw the exciting extension to the North Star natural history database – the **Adult North Star database** – which has now gone live. This means that, for the first time ever, neuromuscular centres are recording the impact of Duchenne muscular dystrophy on adults. This marks an important moment and opens up the possibility of adults getting potential new treatments and clinical trials in the future.

Another important update from the Adult North Star Network in 2020 was the creation of the first-ever national *Standards of Care*

for Adults with Duchenne muscular dystrophy. While best practice care considerations for the care of children living with the condition have been in place since 2010, there had previously been none for adults.

In 2016, MDUK funded a PhD studentship with Professor Ros Quinlivan (pictured left), Consultant in Neuromuscular Disorders at University College London Hospitals, to drive forward the work of the network of specialist health professionals who provide care for adults with Duchenne muscular dystrophy. Professor Quinlivan leads this network, now known as the Adult North Star Network.

In December 2020, Professor Quinlivan presented the first-ever standards of care for adults with Duchenne to the World Duchenne Academy. These will provide best practice guidance for clinicians on areas such as respiratory and cardiac care, helping them provide the

best clinical care to adults with Duchenne muscular dystrophy. While these standards of care are intended for use by clinicians, MDUK has started working on a version tailored for people with the condition. This information will help people with Duchenne muscular dystrophy, as well as their families, know what standards of care to expect from healthcare services. This, along with therapy standards of care, will be available this year.

Vivek Gohil (pictured), knows all too well how important it is to have national standards of care.

“I noticed a change in the care I received when I moved from my children’s neuromuscular team into adult services, with less time and access to a multi-disciplinary team who are experts in Duchenne. This all happened at a time when I felt very alone and my condition was really deteriorating and I actually needed more expert support. I hope this new guidance means we can level up care for adults with Duchenne across the country.”

Get the latest updates on the North Star Network and other breaking news by visiting:
www.muscular dystrophyuk.org/news



A promising treatment for SMA Type 1

We are delighted to bring you the news that a one-time gene therapy used to treat infants with SMA Type 1, called Zolgensma, has been approved by the Scottish Medicines Consortium (SMC) for restricted use within NHS Scotland.

Babies born with SMA Type 1 are unable to sit or roll unaided. Studies have shown that, after a single infusion treatment of Zolgensma, these babies were able to reach milestones such as sitting, crawling and even walking.

This treatment addresses the genetic cause of SMA by delivering a functional copy of the SMN1 gene into nerve cells. This gene is needed to make a protein that is critical for the function of the nerves that control muscles.

Thanks to support such as yours, MDUK has been working with SMA UK, TreatSMA and clinicians to advocate for access to this innovative treatment.

In Scotland, the SMC has said Zolgensma can be given by NHS Scotland for infants where both copies of the SMN1 gene are faulty and who have a clinical diagnosis of SMA type 1, and for some affected infants before they develop symptoms of SMA. NICE, the body that advises what treatments should be available

on the NHS in England, are also reviewing Zolgensma to treat eligible infants with SMA Type 1. We expect them to publish their final guidance this summer.

During this review period, with advice from leading experts in the treatment of SMA, NHS England will be making Zolgensma available to some eligible infants who fall outside of NICE's criteria for receiving the treatment.

Dr Kate Adcock, Director of Research and Innovation at MDUK, said:

“This news means more affected children will be able to access the world around them, attend nursery and school, and join in with their family. This treatment will bring hope to families facing this diagnosis”.

Until more details are released about how families will get access to the treatments, we suggest families speak to their consultants.

Read our latest research news by visiting: www.musculardystrophyuk.org/news

A big step for Changing Places



The government has announced plans for distributing its £30m fund to increase the number of Changing Places toilets in existing buildings across England. Thanks to kindness such as yours, MDUK will support the government to make sure these facilities are available to more of the quarter of a million people in the UK who need them.

Local authorities will soon be invited to 'opt in' to receive a proportion of the funding, based on need, to install facilities in their communities. MDUK has worked with the Ministry of Housing, Communities & Local Government (MHCLG) to help identify how best to direct the funding, which was first announced in the March 2020 budget, and how we can support local authorities once the funding has been distributed.

This investment means thousands of disabled people across England, including those with muscle-wasting

conditions, will have greater access to public places, including locations such as leisure and sports facilities, cinemas, and arts venues.

MDUK, in partnership with MHCLG and the Research Institute for Disabled Consumers, will also be undertaking the biggest consultation to date with Changing Places users in England to find out what their needs and priorities are.

Kerry Thompson, one of MDUK's Changing Places campaigners, says: **"The biggest challenge I face when going anywhere outside my home is locating a Changing Places facility. They really do make a world of difference when planning days out so this announcement will open up a whole new world for the hundreds of thousands of people who rely on them."**



To find out more about how you can help support the Changing Places campaign, visit: www.muscular dystrophyuk.org/changing-places-toilets

Providing support during lockdown and beyond

During the pandemic, MDUK has remained as committed as ever to supporting everyone in the UK who lives with a muscle-wasting condition, along with their families. After what has been a challenging year, the development and roll-out of COVID-19 vaccines across the UK brings hope.

Since the start of the pandemic in March 2020, MDUK's helpline team has received over **400 requests for support relating directly to COVID-19**, as well as nearly **300 requests for our alert cards and care plans**. The COVID-19 guidance on our website, which we are constantly updating, has received **over 41,000 unique page views**.

By working in collaboration with other charities, we have successfully ensured that the Government has heard and acted upon the concerns of people affected by muscle-wasting conditions around access to vaccines. Like many charities, we were concerned that our community wasn't on the priority list, as early plans for the roll-out of vaccines were announced. After we raised this with the Joint Committee for Vaccination and Immunisation (JCVI), people classed as clinically



extremely vulnerable were moved into priority group four.

In February, we joined other members of the Shielding and High Risk Coalition in a meeting with the Minister for Covid Vaccine Deployment, Nadim Zahawi MP. In the same month, we were pleased to note that people living with muscle-wasting conditions, who weren't classified as clinically extremely vulnerable, would be included in priority group six for vaccination.

Thanks to support such as yours, MDUK's dedicated Advocacy, Information and Support team has continued to be on hand with up-to-date and accurate information. And with your support, we've been able to inform and influence important decisions about the roll-out of COVID-19 vaccines.

Making a difference to Janice

Janice Robson first contacted MDUK's Advocacy, Support and Information team in June 2019, after her application to renew her Blue Badge was unsuccessful. MDUK's advocacy team supported Janice to appeal this decision, using their knowledge and expertise to help present her case.

"MDUK was just incredible and so friendly too," said Janice.

"They wrote a letter for my council about my Blue Badge, explaining why it was suitable for me and why I should be given one. Thanks to their team I got my badge back – they really made such a difference."

Janice's experience with MDUK meant that in January 2021, when she had concerns around receiving a COVID-19 vaccine, she knew exactly where to turn.

"I've been shielding since March last year and have barely left the house, so I knew I wanted the vaccine if it was offered to me. But when I was invited to have it, I had a lot of questions and felt quite worried.

"Would the vaccine be safe for me to receive considering I take medications to manage my condition? Would it make my condition worse? There was just so much information about the vaccine from all over the place, it made it hard to know what to make of it all. I felt so worried that

I couldn't sleep one night, so I decided to call MDUK the next day – and I'm so glad I did.

"When I called the MDUK helpline, they listened to my concerns and went out of their way to help me. Their team provided me with information from neuromuscular specialists about the vaccine and my condition, and by the time it came to having my vaccine in January, I felt confident it was the right thing for me."



Moving Up in 2021

The COVID-19 pandemic has brought about many changes to the way people work, with greater numbers of people working from home or working flexibly. The UK's unemployment rate has also grown significantly during this time, with fewer job vacancies available than in previous years.* Jack McLellan, MDUK's Work Experience Development Officer, tells us how he'll be moving the work experience project onwards and upwards in 2021.

I know all too well the challenges young disabled people face when trying to find work. In 2016, I joined MDUK's work experience programme, Moving Up, as a participant after spending years as a graduate struggling to find work. After many unsuccessful applications, my confidence had taken a real knock.

With generous funding from the City Bridge Trust, Moving Up gives young disabled people an opportunity to gain valuable work experience and skills, as well as ongoing support, mentoring and careers. My placement with MDUK's advocacy team enabled me to learn so much, not just about advocacy services, but also about how to balance work with my disability. Most importantly, it boosted my confidence and, not long after my placement finished, I successfully applied for my first full-time role. I joined the HR department at MDUK, where I gained valuable skills that I have carried through my career. Five years on, I'm now working in

MDUK's Moving Up employability programme. My own experiences have made me determined to help other young disabled people gain the skills and confidence they need to stand out in an increasingly crowded job market.

During my first month in the role, I met with people from other disability charities as well as MPs to discuss the disability employment gap, the impact of the COVID-19 pandemic and, ultimately, how MDUK can work with these organisations to address these issues.

I also attended a workshop run by the London Recovery Board – the committee leading London's recovery from the coronavirus crisis. I joined over 200 participants from London charities to explore how we can support communities impacted by the virus and I look forward to using what I learnt from the workshop to help people living with muscle-wasting conditions.

I'm delighted to say that in January 2021, we took on our first Moving Up participant since the pandemic began. They had to work remotely, which came with its challenges but, working with teams across MDUK, we've created a programme to make sure participants feel supported and can get the most out of the experience. I'll also be offering more long-term help to participants, including guidance on key skills they need when preparing to enter the workplace.

Working life won't be the same again, but I'm excited to play my part in making sure disabled people are part of what the workplace looks like in the future.

If you are a young disabled person looking for work, or know someone who is, you can find out more about MDUK's Moving Up programme by visiting www.muscular dystrophyuk.org/workexperience



Get involved

Did you know that you can help MDUK raise funds for FREE just by shopping online?

Whether you're treating yourself, buying gifts or doing your weekly shop online, when you shop through Give as you Live online (GAYL), you can raise free funds for MDUK and it won't cost you a penny extra! We talk to GAYL-user, Catriona Parker from our fundraising team, about how easy it is to use.

How does it work?

GAYL has partnered with over 4,000 retailers so that when you shop via GAYL online and make a purchase, the retailer donates a percentage of the purchase price to a charity of your choice as a thank you.

What sorts of amounts can you raise?

Different retailers give different percentages ranging from 1-10%. For example, I bought a pair of shoes on sale for £22, and MDUK received a donation of 66p. Another time I spent £259 on holiday accommodation (pre-COVID) and MDUK received a fantastic £6.46. So far, supporters



have raised over £2,800 for MDUK simply by shopping online. That's money given to MDUK, at absolutely no cost to the individual or the charity, for shopping they were going to do anyway!

Do you have any tips for using GAYL?

It's very frustrating to make an online purchase then realise afterwards you've forgotten to use Give as you Live! So I have installed their donation reminder for Google Chrome and now, every time I go onto a participating site, a handy reminder pops up. There is also a GAYL app if you shop from your phone or tablet.

How can people sign up?

You can sign up online by visiting www.giveasyoulive.com/charity/muscular dystrophy and, if you have any questions about Give as you Live and how it works, please email us at reply@muscular dystrophyuk.org and we'll be very happy to help.

Cambridge Running Festival

TTP Cambridge Half Marathon has joined forces with MDUK Town and Gown 10k to launch The Cambridge Festival of Running, taking place in October 2021.

The landmark Cambridge Festival of Running will feature:

- **MDUK Town and Gown 10k and Junior 3k**
Saturday 16 October
- **TTP Cambridge Half Marathon**
Sunday 17 October.

Both the 10k and Half Marathon routes take runners through the city of Cambridge and past countless historic landmarks, with TTP Cambridge Half Marathon's route passing through two iconic University colleges.

To find out more about the races and how to take part, visit www.townandgown10k.com/cambridge



The Great Muscle Raffle is now OPEN!

And when you enter, you could win our incredible top prize of £3,000 – imagine winning a prize like that!

Each chance to win is just £1, and every entry helps make sure MDUK can continue to be there for families, providing information and support when they need it most.

Enter today at www.muscular dystrophyuk.org/raffle

Congratulations to our Christmas Raffle winners!

First prize – £3,000 | Mrs Hooper, BOSTON

Second prize – £250 | Mr Symonds, VERWOOD

Third prize – £50 | Ms Mengezeleli, STANMORE

Thank you to everyone who took part.



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IN YOUR WILL
TO CREATE A
future free
from the limitations
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WITH YOUR HELP
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"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
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For more information about gifts in Wills,
call Beth Glennie on **0300 012 0172** or email
legacy@muscular dystrophyuk.org

www.muscular dystrophyuk.org/giftsinwills



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