TARGET MD

Spring 2020

Celebrating our amazing volunteers

One family's story of Spinraza

Transforming lives through research: news and updates

Muscular Dystrophy UK
Fighting muscle-wasting conditions

Muscular Dystrophy UK's lifestyle magazine
Helping you to live well with muscular dystrophy

Contents

The magazine for supporters of Muscular Dystrophy UK.

On the cover

Liz Williams, one of our peer support volunteers who talks about her experiences on p10. © Chris O'Donovan/MDUK

Muscular Dystrophy UK
61A Great Suffolk Street, London SE1 0BU

020 7803 4800

info@musculardystrophyuk.org

www.musculardystrophyuk.org

Editor: Laura Gibson Designer: Toby Maslin
targetmd@musculardystrophyuk.org
Advertising enquiries: Ross Hyland, Media Shed
rossh@media-shed.co.uk
020 3475 6814

Registered with FUNDRAISING REGULATOR

Registered Charity No. 205395 and Registered Scottish Charity No. SC039445

GUEST EDITOR

Welcome to the first edition of Target MD for 2020!

I am delighted to be guest editor for this issue. I joined MDUK just over a year ago and one thing was clear from the start – the passion and dedication of our supporters is a real force to be reckoned with.

Volunteers are the lifeblood of MDUK and our success is built on a strong foundation of support. In this issue you'll hear from a number of volunteers about what they do and why, from peer support to campaigning and sitting on our Lay Research Panel and Content Advisory Group.

Over the last 60 years, you have helped us become a real force for change and together we're making every day count for people with muscle-wasting conditions and their families. One father tells us of the moment he heard his son would receive Spinraza, the first treatment for spinal muscular atrophy (SMA), on the NHS. It is a potentially life-changing step that would not have been possible without families, doctors, researchers and charities coming together and giving their time to campaign for NHS funding.

One of my highlights last year was our National Conference in October. We welcomed more people than in previous years and I enjoyed the opportunity to speak to so many of you.

The President's Awards, where we celebrate the commitment and achievements of our supporters, are a big part of our National Conference. In this edition of Target MD we're celebrating our 2019 winners and calling for nominations for this year's awards. Keep reading to find out more, including how to make a nomination.

Thank you for helping us to be here for everyone affected by a muscle-wasting condition. We simply couldn't do it without you.

Rob Burley
Director of Campaigns, Care & Support, MDUK

I CAN HAVE A POSITIVE IMPACT

Peter Ashley © Chris O'Donovan/MDUK

Peter Ashley lives in North Lincolnshire with his wife, Emma-Jayne, and three children. His stepson Dregan, now 20, was diagnosed with congenital myotonic dystrophy five years ago.

As well as caring for Dregan full-time, Peter and Emma hold a number of voluntary roles for MDUK. They also raise money through their Family Fund and support other families locally and around the UK. They represent the myotonic dystrophy community in various European research and care organisations. Last year, Peter won the MDUK President's Award for Outstanding Achievement.

"Pete has dedicated the last 11 years to myself and the family he inherited, and his life to raising awareness of muscle-wasting conditions," says Peter's wife, Emma. "It has been his choice to dedicate practically all his time to help others. As long as Pete has his health, and a difference is being made to those who need it, I can't see him stopping anytime soon."

Peter's volunteering 'journey'

Peter's involvement with the charity began in 2014, after Dregan's diagnosis.

"We'd never heard of the condition," explains Peter. "We didn't know anything about it…The paediatric neurologist who diagnosed him said, 'Don't go home and Google it'...so we did.

> **When we got the diagnosis and realised how serious it was, it was a big hit.**

"It was upsetting that the implications for us were enormous…you realise you'll be looking after him for the rest of his life and it was a pretty grim looking future at the time. It was a big shock and it was a lot to take in, but we're the kind of people that like to know information, so we jumped into it and tried to find ways to improve Dregan's future. That's why we got in touch with MDUK."

Not really knowing where to turn after Dregan's diagnosis, Peter and Emma were spurred on after watching a local news report of a mum, Sarah Ruane, raising money and awareness for her son

who had congenital myotonic dystrophy. They got in touch with her and from there spoke to other families before finding out about us.

"Over the coming weeks and months, contact increased," says Peter. "Another mum who has a child with congenital myotonic dystrophy suggested that we could start up a Family Fund with MDUK. [We] had meetings in London and it just seemed like a no-brainer because we were thinking about the difficulties of raising funds and…the legal aspects of it. MDUK through the Family Fund do all that and leave the families to concentrate on raising the money. Since then, we've been involved in everything to do with the charity that came along."

With our help, Peter and Emma set up the Congenital Myotonic Dystrophy Fight Fund with three other families to raise money. With an initial ambition to raise £75,000, Peter, Emma and the other families smashed their target and have so far raised more than £120,000 for research into myotonic dystrophy.

Peter and Emma also began attending conferences to find out as much as they could about myotonic dystrophy and learn more about care and support.

"We thought…we need to know what's available out there – what's best practice," says Peter. "At the moment there are no treatments."

> **"It's quite a serious condition – it's life shortening – so what can we do to change that? That's the motivation for it all.**

Getting involved in research

It was from these conferences that Peter discovered more opportunities to support the work we do. He applied to become a member of our Lay Research Panel, which is involved in choosing which research projects should be funded by us every year.

"It seemed like an interesting thing to do," says Peter. "I filled in the form, got really positive feedback and started the fairly lengthy process of being included. I went down and attended one of the get-togethers as an observer and was proposed as a

member and accepted. It's been a great experience." Peter, who was recently elected Vice Chair of the panel, enjoys being involved although remembers the learning curve involved at the start.

"When I first signed up for it I assumed it was reading a couple of paragraphs for each application. By the the nature of the Lay Research Panel name, you would imagine you it would be written in such a way that anybody could understand it, but it's absolutely not that at all! By the time I joined, we'd had a couple of years of going to conferences and educating ourselves in the condition and we knew quite a lot, but I still felt out of my depth when we got our first ones to look at.

As time goes on and we look at more of the research proposals…you get to know about the conditions, and you get to know the language and how to read them, but a lot of them are very scientific. You have to do a lot of research just to simply understand what they're talking about in the proposal, which is hard work, but it's really interesting and rewarding."

To find out more about our volunteering opportunities visit www.musculardystrophyuk.org/volunteer

Emma and Peter Ashley © Chris O'Donovan/MDUK

Helping us shape our content

And then, in 2017, a new opportunity arose to join the MDUK Content Advisory Group and help shape the tone, language and information in our publications and on our website.

"Both myself and Emma signed up for that," Peter says. "It just seemed like a logical thing to do. It basically gives us advance notice of publications that are going out and the opportunity to have an input to make sure they're accurate and represent the community.

"We're an interesting cross section of people – there are health professionals, there are people with different conditions and with different skills. And we have some good discussions about what is being proposed in terms of press releases, leaflets, pamphlets, booklets, guides and the website."

"And it's a good thing to be part of, to hear feedback from a wide variety of people who all face different conditions. It's been really useful for us as well, just to have the opportunity to make sure that the information that goes out to the community is accurate because what might seem like a potentially very trivial inaccuracy or omission can actually have quite serious consequences for the people with this condition."

> **"It's just a great opportunity to actually have an input as a small user of a large charity that represents a lot of people in the UK.**

Recognising Peter's achievements

Last year, Peter was honoured with the Richard Attenborough Award for Outstanding Achievement – one of our President's Awards, which was presented at our National Conference.

Peter is humble about his accomplishments: "It makes me feel good that I can have that positive impact. It's all driven by trying to find out what's best for Dregan. At the end of the day, he's my son. He's my stepson, but he's my son. You know, you do those things for your family, don't you?"

Nominations for this year's President's Awards are open! Tell us who you think deserves an award this year and why at www.musculardystrophyuk.org/presidents-awards/nominate

HELPING TO MAKE EVERY DAY COUNT

Hilary Gray has been a fantastic fundraiser and committed supporter ever since her grandson was diagnosed with Duchenne muscular dystrophy seven years ago.

> **As a family our progress has been incredibly supported by Muscular Dystrophy UK . I just want to give something back.**

Hilary became one of our peer support volunteers in 2016 when the initiative began. Describing herself as an available and committed 'grandparent mentor,' she uses first-hand knowledge drawn from her grandson Callum's condition to help others. Hilary is currently supporting two families who are facing the myriad issues that typically surround a recent diagnosis.

"Callum was diagnosed after breaking his leg when he was three," recalls Hilary. "Two years later he had still not regained his correct gait. "Hilary's daughter (and Callum's mum), Emily, kept insisting that further tests be carried out.

Speaking about how much things have changed since then, Hilary says, "We really have come a very long way since the dark and dismal days of Callum's diagnosis, both on a personal basis as a family and on a wider scale, in terms of the growing hope there is out there for improved lives and eventual cures, as a result of scientific research."

Hilary also says that each year, the average age for people with this sort of condition to survive is really moving forward and the progress is well reflected in the more positive way stories of individuals is reported.

As well as giving her time as a peer support volunteer, Hilary has raised thousands to help us make every day count for people living with muscle-wasting conditions. She has organised and taken part in a huge range of activities, including arranging annual golf days at a Bracknell golf course (where Callum presents the prizes), planning regular race nights, holding pub quiz nights and getting involved in Go Orange for a Day every year.

Hilary has also taken to the skies to raise money – by jumping out of a plane, despite being terrified of heights!

"Hilary was very nervous before committing to the skydive last year," says Louise Moffat, our regional development manager for London. "It took a little persuasion by myself but in the end her enthusiasm to fundraise won and she took the leap. Her family and friends thoroughly appreciated her courage too and showed it with their donations."

> **She smashed the £399 fundraising target by over £1,000, raising a total of £1,600.**

To find out more about our volunteering opportunities visit www.musculardystrophyuk.org/volunteer

Hilary Gray

And it's not just Hilary who's giving her time for us – her grandson Callum was awarded a medal for Meritorious Conduct by the Scouts for his fundraising and raising awareness of muscular dystrophy. His activities include a car wash at his local fire station, several Move a Mile for Muscles events over the years and arranging a Go Orange fundraising day.

Thank you to Hilary, Callum and all our other volunteers, who are making every day count for people with muscle-wasting conditions.

Hilary's grandson, Callum

PEER SUPPORT CAN CHANGE LIVES

Liz Williams © Chris O'Donovan/MDUK

Liz Williams is an active and passionate advocate for people with Facioscapulohumeral muscular dystrophy (FSHD). Since her own diagnosis in 2004, Liz works closely with us to support others with a muscle-wasting condition. Liz chairs the FSH Support Group, which we support, and helps us develop information and resources to increase understanding of FSHD and wider issues affecting all people with muscle-wasting conditions. She is also one of our peer support volunteers. Liz tells us why she decided to get so involved.

"When I was first diagnosed with FSH muscular dystrophy back in 2004 I knew it was a reasonably rare condition in that I was unlikely to meet anyone else with the condition in my everyday life.

> **"I really wanted to find someone like me and the internet was my first port of call.**

"I was lucky to find a couple of forums created by others with the condition who wanted the same thing; to connect and share their experiences. Here I found my tribe.

"From this group I first heard about Muscular Dystrophy UK. MDUK in 2012 engaged with the FSH-MD online community to create one of their first Information Days. I remember over 100 people with the condition attended at that time.

"This was a huge success and there have been many information days since. I met so many people that day who I am still in contact with even now who represented the broad spectrum of how FSH can affect someone with the condition. I know that those who might be at the start of their journey can feel they don't want to see others who are perhaps further down the road of progression. But the majority of those reluctant to attend came away feeling it was a positive experience.

> **"I became more and more involved with MDUK and eventually, when the peer support scheme started, I jumped at the chance to volunteer.**

To find out more about our volunteering opportunities visit www.musculardystrophyuk.org/volunteer

"Whether it was planning my future, moving home, house adaptions, eventually using a wheelchair or even planning a holiday and where to go for those facing mobility challenges, I gained some knowledge from everyone I met.

"Since then, I have been supported by and provided support to an amazing army of individuals.

"The work varies so much from helping research, suggesting holidays, and even supporting people to go on and have a family. If that's not life changing I don't know what is.

"MDUK also provide Muscle Group meetings. I attend the Milton Keynes Muscle Group, which regularly welcomes 20 people with various forms of muscular dystrophy. It is such a positive group of people and we share our muscular dystrophy journey as well as find out about local services and how to access them. Without these amazing people I would have taken a lot longer to make the transition from walking to using a wheelchair full-time.

"They supported me when I needed to change my vehicle to a Wheelchair Accessible Van, with experience of what was possible and how to avoid pitfalls and delays. You regularly see Muscle Group attendees in the car park afterwards discussing their vehicles, lifts and hoists, wheelchairs, scooters etc.

"Being involved helps MDUK evolve to best support those with muscle-wasting conditions, whether it's campaigning, providing new vital information for the newly diagnosed, helping families with children, supporting people with work issues or benefit entitlement, or what research to focus on.

"Because I have learned so much over the last 15 years, I feel it is only right to pass on what I know.

"There isn't a text book for navigating FSH or any muscle-wasting condition for that matter. Everyone is an individual with different priorities and outlook. I try to assess what sort of support someone needs right now, arrange to give support in a way they

are comfortable with, sometimes reassure about what the future holds. I say they can ask anything at any time when they are ready, there is no set rulebook, and I suggest, signpost, not enforce my opinion and offer support without prejudice.

"Engaging with peer support [for MDUK], is not entirely selfless; 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. It's great for my mental health and has only ever been positive for me. If you have a couple of hours a week to spare, consider volunteering for peer support. I know how peer support can change lives – I've seen it first-hand."

To speak to one of our peer support volunteers, call our free Helpline on 0800 652 6352 in the first instance. If you have FSHD and would like to know more about the FSH Support Group, which Liz chairs, visit www.fsh-group.org

SPINRAZA: ONE FAMILY'S STORY

The Wilson family © Chris O'Donovan/MDUK

Last year, the first treatment for spinal muscular atrophy (SMA), Spinraza, was approved for use in England through a Manged Access Agreement. Through this agreement, people with SMA types 1, 2 and 3 can now access the treatment, although there are some restrictions attached. This followed the treatment becoming available in Scotland and roll-out is beginning in Wales and Northern Ireland.

While we know that not everyone who can benefit from the treatment is receiving it – and we are working hard to change this – the announcement last summer was good news for Mark Wilson. His son, Aadi, has SMA Type 2.

Mark remembers the moment he heard Spinraza was to be made available through the NHS.

"I had woken up early for work, turned my phone on, and saw an email breaking the news," he says. "My wife, Panna, who was pregnant at the time, was still fast asleep, but I had to tell her.

"We then immediately texted our family and friends with the news, and once I got to the school where I work my friends had plastered the door to my classroom with messages of congratulations."

"We were overjoyed.

For families like Aadi's, Spinraza represents hope. "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.

"We're lucky to have such a beautiful son, and he deserves the very best.

The family joined MDUK, SMA charities, clinicians and the wider SMA community in campaigning for Spinraza to be made available in what was a long, and often emotional, process. It took 18 months for NICE to approve the drug.

"The delays have been incredibly frustrating and completely unnecessary, and when we recount to our friends the story so far…they can't believe how slow the whole process is," says Mark.

Aadi has to travel to Sheffield for treatment as his local NHS trust in Leicester cannot deliver it.

"We will do everything to give him the greatest life we can.

Mark says, "Aadi keeps us positive, and like him we face every challenge with a determination and a smile."

To find out more about our Fast Track campaign, visit: www.musculardystrophyuk.org/fast-track

MDUK PRESIDENT'S AWARDS

Our annual President's Awards are an opportunity for us to celebrate the achievements of our amazing supporters, from researchers and campaigners to fundraisers and volunteers.

Last year, we asked you to tell us who you thought deserved a President's Award. It was so successful we're doing it again!

Nominations are now open for our 2020 President's Awards.

It's easy to put someone forward – all you have to do is fill in the nomination form online, giving as many details as possible about the person you are nominating. Winners will be announced in the autumn.

Meet last year's winners

Wayne Armsden won Fundraiser of the Year for his incredible efforts to raise money for his Family Fund, Archie's Army.

Dr Christopher Banerji took home the Early Career Scientist award for his research into FSHD.

David Duggins was honoured with the Community Achievement award for supporting us for over 50 years.

Kerry Thompson won Campaigner of the Year for persistent lobbying for more Changing Places toilets installed across England (read more on p19).

Peter Ashley received the coveted Richard Attenborough Award for Outstanding Achievement for his unwavering commitment to MDUK and other families (see p4).

Nominate someone special for a President's Award:
www.musculardystrophyuk.org/presidents-awards/nominate

SUPPORTING YOU TO GET THE BENEFITS YOU NEED

Financial support is crucial when you're living with a muscle-wasting condition, but many people aren't receiving the benefits they are entitled to and can struggle. MDUK is here for anyone who is finding this difficult and needs advice or support. We're also 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 an undiagnosed muscle-wasting condition, knows how difficult the current welfare system can be.

"Jenson is a joy to be around. He loves being outdoors and playing on the swings in his garden or visiting the beach and getting messy. He is our perfect baby boy, and I wouldn't change one thing about him. But every day is a challenge. "I'm not only Jenson's mum, I'm his carer, too."

"Jenson cannot talk other than to say 'Mum', and until very recently he was unable to walk. He mostly gets around by crawling or using his knees to shuffle along the floor. Jenson is…entirely dependent on myself and his dad, Rikki.

"I dropped days at work to care for him. Inevitably, this came with financial challenges, so I applied for Disability Living Allowance."

Chloe sought advice on applying for financial support, but still found the process difficult.

> **"The form is very detailed and specific, and I was really upset and stressed as it was just after Jenson's diagnosis.**

"At that time, you have enough to deal with – you don't need the additional pressure."

At the time of applying for financial support, Jenson was less than three years old. This meant Chloe could not apply for the mobility component of the Disability Living Allowance, despite being told her son would not walk until he's a bit older, or even ever.

Chloe does not qualify for Carer's Allowance either, which means she misses out on additional

Jenson Smith © Chloe Smith

financial support despite caring for Jenson 35-plus hours a week.

"On top of that, we currently live in a first-storey flat and 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. We have to keep his walker at his grandmother's house, six miles away.

"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.

> **"It's great that Muscular Dystrophy UK is campaigning on this, because change is needed.**

"Many people generally need financial support to assist the needs to their disabled children, perhaps from dropping hours in work or buying equipment they need. Everyone is different, and I feel like there needs to be less judgement and a more sympathetic attitude towards people who need support."

If you're having difficulties getting benefits, care and support, services or equipment, call our free helpline on 0800 652 6352 and find out how we can help.

We also have a step-by-step guide to help anyone applying for Personal Independence Payments (PIP). Email info@musculardystrophyuk.org to request a copy.

‘IF I WANT TO WEAR SEQUINS, I WILL’

Sulaiman Khan is an MDUK Trailblazer – part of our network of young, disabled campaigners - and an advocate for positive body image. He tells us why it’s time for better portrayals of disabled people on television and greater representation in advertising and marketing campaigns.

“Before I discovered colourful and sequined outfits, I only wore dull clothes. I used to think, ‘my body is too twisted’ or ‘I’m too lanky’ and was anxious about what I wore. But I don’t do that now. If I want to wear sequins, I will. If I want to wear shorts, I will.

“Disabled people need to see a lot more of themselves in the media, in adverts, on television. Around 20 percent of people are disabled, but only 0.06 percent of this segment is featured in advertising."

> **It’s essential to support each other, but we can’t do that if society doesn’t support us.**

A survey carried out by our Trailblazers found body image can have a big impact on emotional wellbeing. More than 4 in 5 people had been affected because of it. And almost half of the respondents were unhappy with how they look because they don’t see disabled people like themselves in advertising or marketing.

“Body image can have a big impact on mental health, and that needs to change,” says Sulaiman, who lives in London and has congenital muscular dystrophy.

> **Disabled people need to be seen for who they are, not painted as inspirational or brave.**

“It’s something I’ve had an issue with myself. Disabled people don’t fit into society’s paradigm of how you should look.”

Our Trailblazers are now calling for hairdressers and beauticians to consider the needs of disabled customers and for high street stores to be accessible.

“There needs to be changing rooms where disabled people can try clothes on, and accessible counters so you can actually buy the clothes,” says Sulaiman. “Those are the kinds of things that can harm body image. For many disabled people, buying clothes online is the only way to shop.”

He adds: “We have a long way to go, but there are some people who are making a difference. American model Jillian Mercado, who has muscular dystrophy, is great. And Jameela Jamil’s I WEIGH campaign is amazing because it values people for who they are.”

Sulaiman Khan

To find out more about Trailblazers, visit: www.musculardystrophyuk.org/trailblazers

INCREASING WORK OPPORTUNITIES FOR YOUNG PEOPLE

We know many young disabled people want to work, but find it hard to get a job due to a lack of opportunities and other barriers. It's why we launched our Moving Up work experience programme – to give young people with a disability the chance to show employers what they can do.

Jack Blackburn, who has mitochondrial disease, proved himself an asset to the team during a month-long stint of work experience with our Trailblazers team last summer.

"It was great having Jack working in the Trailblazers team," says Trailblazer Manager, Lauren West. "Jack helped with our Wellbeing Camps and other project administration. He was highly efficient and motivated and proved an asset to our team and MDUK more broadly."

Jack, 19, is now studying chemistry and physics at A-level and hopes to go into medicine.

"What I really want to do is become a doctor specialising in helping people with muscular dystrophy."

Emma Vogelmann, who manages our Moving Up programme, says, "We launched Moving Up in 2015 in response to our investigation, which found a lack of employment and work experience opportunities open to young disabled people.

> **We have helped dozens of disabled people to find work.**

"We offer flexibility in length of placement, hours and the possibility of remote working. We provide equipment to make the workplace accessible and to give people what they need to do the best job possible. And we offer work experience in a completely supportive environment to test what adjustments are needed and build confidence to ask for those in other workplaces."

As well as the benefits for those seeking work

Jack Blackburn

experience, Emma says the Moving Up programme is also well received by businesses, including creative companies like 20Ten and Wall to Wall Media.

"Companies benefit from partnering with an experienced charity that can offer practical guidance on embracing disability in the workplace, both in terms of staff and customers."

The Moving Up programme is funded by us thanks to a grant from the City Bridge Trust. All work experience placements begin in our London office with the potential opportunity to take advantage of our links with external organisations for further placements in the South East.

Emma Vogelmann, MDUK Work Experience Development Officer

To find out more about our Moving Up programme and register your interest, please contact Emma Vogelmann, MDUK's Work Experience Development Officer, on e.vogelmann@musculardystrophyuk.org or 020 7803 2872.

POWERING AHEAD

Ever since the Wheelchair Football Association (WFA) was formed in 2005 to govern the sport, Powerchair football has been on a steady rise in popularity and numbers. Not surprising for the unique football format that provides the only active team participation sport for people who use electric powered wheelchairs.

Ellie Renton with brother, William

As nine-year-old Ellie Renton from Middlesbrough says, "Powerchair is wonderful because it is something that I CAN do as opposed to all the things that I CAN'T. Also, there is the brilliant chance to make new friends, who are similar to me, with disabilities."

Practice sessions are the highlights in Ellie's week and getting her first Powerchair in 2015, made a huge difference to Ellie and her family's life.

What is Powerchair football?

Powerchair football is a four-a-side version of football, which is played indoors on a basketball court or similar surface.

It is an all-inclusive, unique sport that provides opportunities for disabled people of all ages to access the beautiful game of football. At nine years old, Ellie is one of the youngest registered players while the oldest is 71. More than half of players registered with the WFA have a muscle-wasting condition.

The WFA are recognised by the Football Association (FA) as the sport's lead organisation and the WFA's laws are sanctioned by them.

Where can you play?

There are around 108 affiliated Powerchair Football teams within 48 clubs across England, with around 1000 players training on a weekly basis. The WFA oversees eight competitions; including the Muscular Dystrophy UK National League Premiership and Championship, six regional leagues and the WFA Cup. There are also a number of club-organised tournaments, festivals and less formal friendly events that are run outside of the formal WFA competition structure, giving new clubs and inexperienced players opportunities to take part in the sport.

If you would like to give Powerchair football a try, find out more, you can find out more, including where your nearest club is, on our website: www.musculardystrophyuk.org/powerchair

CHANGING PLACES SUCCESS

Being able to get out and about is something many people take for granted. But we know this can be difficult when you're living with a progressive muscle-wasting condition.

Inadequate public toilets are just one part of this problem: around 250,000 people in the UK need fully accessible Changing Places toilets. These are larger spaces with a hoist, changing table and room for two carers as well as a wheelchair.

Without them, Kerry Thompson, our 2019 Campaigner of the Year, says going on days out can be 'a military operation'.

"It's like having a small child," she says.

> **I should be able to leave my house without a second thought, but the reality is I can't do that.**

"Instead I have to plan ahead, have to remember to take wipes and spare trousers."

Kerry, who lives in Milton Keynes and has FHL1 myofibrillar myopathy, relies on Changing Places toilets. "People everywhere need Changing Places to go to the theatre, to the cinema, to visit the countryside.

"It's one thing having them in a shopping centre or supermarket – which, don't get me wrong, is great and needed – but what about the museums, the beautiful gardens, the pubs and restaurants? It would be nice to go to those places, too."

As co-chairs of the Changing Places Consortium, we are campaigning for these facilities to be installed in all big public places, including city centres, shopping centres, entertainment and leisure venues and hospitals. There are currently

Kerry Thompson with singer/songwriter, Tony Hadley

about 1,350 of these fully accessible facilities across the UK – 200 more than this time last year. That's an encouraging step in the right direction, but there is still work to be done.

"There are parts of the country that don't have any toilets at all," says Kerry.

Venues that have registered facilities in the last year include Windsor Castle – 'a dream' for Kerry – and London Zoo. The Government has also consulted on introducing legislation to make Changing Places toilets mandatory in new, large public buildings, while, thanks to Kerry, Tesco committed to installing facilities at stores with the greatest need.

"That all started when Muscular Dystrophy UK asked me if I would go to a meeting with Tesco to explain what Changing Places are and why they are important," she explains. "It was quite daunting because it was the first time I had done anything like that, but I'm so glad I did. Now, they have just registered their 41st facility."

> **It feels amazing to have achieved this.**

"Now, I can go forward and understand as a campaigner the best way to encourage venues to build Changing Places toilets."

To find out more about Changing Places, visit www.musculardystrophyuk.org/changingplaces

A NEW ERA OF RESEARCH

Professor Matthew Wood © Suki Mok/MDUK

Professor Laurent Servais

We are committed to finding new treatments and ultimately cures for people with muscle-wasting and associated conditions. Last year we established our new MDUK Oxford Neuromuscular Centre, which is a partnership with the University of Oxford, to find potential new treatments and get them to patients faster.

The centre has already attracted international talent to Oxford to work alongside other world-leading researchers in the historic city.

"The MDUK Oxford Neuromuscular Centre is an extremely exciting new project," says Professor Matthew Wood, Professor of Neuroscience at the University of Oxford and Director of the new centre.

> **Patients with neuromuscular diseases are desperately in need of new treatments.**

"I think for the first time ever, we are at a phase in the history of these diseases where it is now possible to develop treatments, mainly genetic treatments, for devastating neuromuscular diseases.

"The MDUK Oxford Neuromuscular Centre will therefore be one of the very first that takes drug development through from basic research to clinical trial testing and increasing the capacity for clinical trials within the UK.

Someone who is equally as positive about the future is Professor Laurent Servais, a globally respected neurologist who joined the team last September as Professor of Paediatric Neuromuscular Diseases.

"I'm pretty sure that here in the MDUK Oxford Neuromuscular Centre, we have the potential to make things change," he says.

Professor Servais has previously overseen numerous clinical trials to test treatments for Duchenne muscular dystrophy and spinal muscular atrophy (SMA). He also led the newborn screening programme for SMA in southern Belgium.

"I would like to see some disease disappearing. I would like to be able to tell my grandson or my granddaughter, 'when I was a young myologist I saw kids with this disease but now it doesn't exist anymore'. I think we have the possibility to do it."

> **That makes me very excited: not to treat, not to cure, but to contribute to make disease disappear."**

Find out more about the Centre and its team at www.onmc.ox.ac.uk

RESEARCH UPDATES

Genome editing shows promise for congenital muscular dystrophy

A new study has shown that editing the LAMA1 gene can prevent and reverse symptoms in a mouse model of congenital muscular dystrophy type 1A (MDC1A). LAMA1 is known as a 'modifier' because it can alter the severity of MDC1A. The researchers activated LAMA1 using genome editing tools. This approach isn't specific to the mutation causing the condition, so could potentially benefit a wide range of patients in future.

One of the concerns with genome editing is the potential for unwanted edits being made by the 'molecular scissors'. The researchers in this study did not observe any unwanted edits in the mice using their technique, which is encouraging. However there's still a lot to do before this could be tested in people with MDC1A.

New genetic study of myotonic dystrophy type 1

Researchers at Glasgow University have identified genetic factors that influence the severity of myotonic dystrophy type 1 (DM1). DM1 is caused by a mutation in which a triplet of letters in the DNA – called CTG – appears more times than it should. Generally, the higher the number of CTG repeats, the earlier that symptoms will appear and the more severe they will be.

The researchers assessed the number of inherited CTG repeats in DNA samples from 255 patients who took part in the international OPTIMISTIC trial. They found that this number determined the age at which symptoms first appeared and predicted participants' muscle function.

These results confirm the importance of carefully counting the number of CTG repeats as part of clinical trials in DM1. They also help to explain variability between DM1 patients and will inform the design of clinical trials.

Discovering more about the FSHD gene

Scientists from Fred Hutchinson Cancer Research Center, USA, have identified a new role for DUX4, the gene that is mutated in facioscapulohumeral muscular dystrophy (FSHD). The researchers studied DUX4 in different types of cancers. They found that some cancer cells use DUX4 like an invisibility cloak, to avoid detection by the body's immune system. This also protects the cancer cells from immunotherapies.

This discovery suggests that drugs that block DUX4 could be used to boost effectiveness of anti-cancer therapies. This is good news for FSHD, as it could potentially mean more money and resources being directed towards the development of DUX4 targeting therapies.

Clinical trial updates

Promising results for DMD steroid alternative
Results from a six-month study testing vamolorone in boys with Duchenne muscular dystrophy (DMD) have been published. Vamorolone is an anti-inflammatory drug thought to have fewer side effects than steroid treatments currently prescribed for DMD. The study showed that the treatment was safe and improved muscle function in a dose-dependent manner. Vamorolone is currently being tested in a global phase 2b trial called VISION-DMD, which includes UK trial sites.

Delays in OPMD gene therapy development
Benitec Biopharma has reported delays in the development of its gene therapy to treat oculopharyngeal muscular dystrophy (OPMD). The company has decided that further work needs to be done to improve the delivery of the gene therapy. Unfortunately, this means that the timelines for the first clinical trial has been postponed until further notice.

Promising results from SMA SUNFISH trial
Pharmaceutical company Roche has announced positive news from Part 2 of the SUNFISH study. The trial evaluated the drug risdiplam in people aged two to 25 years with Type 2 or 3 spinal muscular atrophy (SMA). It showed the drug to be safe and effective in increasing muscle function.

Risdiplam is an oral drug that works in a similar way to Spinraza, increasing SMN protein levels by targeting the SMN2 gene. Roche has said its findings will be shared with the US Food and Drug Administration (FDA) and the European Medicines Agency (EMA).

Early positive results from DMD trial
Capricor Therapeutics has released positive preliminary results from its HOPE-2 Phase II clinical trial testing CAP-1002 in steroid-treated boys and young men with Duchenne muscular dystrophy (DMD). CAP-1002 is a stem cell therapy that is thought to stimulate muscle regeneration and reduce muscle scarring. After six months, participants treated with CAP-1002 had better shoulder, arm and hand mobility than those in the placebo group. Early positive signs in tests that measure lung and heart function were also reported.

CNM drug receives Orphan Drug designation
Dynacure has announced that the EMA has granted Orphan Drug designation to DYN101 for the treatment of patients with centronuclear myopathy (CNM). This designation gives Dynacure certain financial benefits that will help to lower the cost of developing the drug. DYN101 is a molecular patch designed to switch off DNM2, a gene that is overactive in CNM. The first clinical trial testing DYN101 in patients is expected to begin soon.

FDA rejects DMD exon skipping drug
The US Food and Drug Administration (FDA) has rejected Sarepta Therapeutics' application for accelerated approval of golodirsen. Golodirsen is an exon skipping drug that targets exon 53 of the dystrophin gene, which could potentially treat eight percent of patients with Duchenne muscular dystrophy (DMD).

The rejection from the FDA does not affect the ongoing ESSENCE trial, which is assessing the safety and efficacy of golodirsen. ESSENCE will generate clinical evidence to support future applications for approval of the treatment.

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The MDUK Lay Research Panel © MDUK

HELPING VOLUNTEERS MAKE EVERY DAY COUNT

We want to be there for everyone living with a muscle-wasting condition, from the moment of diagnosis and beyond. But we know we can't do this without the help of our dedicated supporters and volunteers.

Last year we started looking at what we can do to inspire, attract and support volunteers to help us achieve our ambitions. Joel Rackham, our Volunteer Engagement Manager, explains how we're involving people with muscle-wasting conditions to help shape the way we do this:

"In November 2018 I was thrilled to begin working on our volunteering project at MDUK. For the last 60 years, we have relied on volunteers to support the work we do, and many of our successes would have been impossible without them.

> **We want to make sure volunteers remain at the centre of what we do.**

"Our aim is to support more people affected by a muscle-wasting condition by getting our volunteers more involved with what we do. To help us do this we formed a Volunteer Steering Group at the beginning of 2019. It has seven members, including one of our Trustees, Sheila Hawkins, who also has professional experience of the sector.

"The group is already helping to make a difference by advising on ideas such as the introduction of new events and programmes to support people living with a muscle-wasting condition. This includes trialling youth groups through paediatric clinics that are led and organised by volunteers, and developing our peer support network to support more people.

"We have also introduced new systems to support our volunteers through training, and developed a new Volunteer Handbook.

> **Our volunteers are important to us and we want to make sure they are fully supported and have the opportunity to get more involved.**

"Over the next year, we'll be launching a survey to make sure we hear from all our volunteers about how we can work together to make every day count."

To find out more about our volunteering opportunities visit www.musculardystrophyuk.org/volunteer

COMING TOGETHER FOR NATIONAL CONFERENCE

Last October, we welcomed lots of you to our National Conference. Working with researchers, healthcare professionals, campaigners and others, the event was described by attendees as inspirational, informative and supportive.

We looked back at the progress that has been made since MDUK was founded 60 years ago. Back then, research into muscle-wasting and associated conditions was largely neglected, the life expectancy for people living with Duchenne muscular dystrophy was around 14 years and attitudes towards people with disabilities were mainly negative.

Now, pioneering research has led to over 150 clinical trials globally so far. Better care and support has seen life expectancy increase and equality legislation, combined with improved access, means more people with muscle-wasting conditions have greater opportunities, visibility and aspirations than ever before. We know there is still a long way to go though.

We wanted your National Conference to give you the opportunity to discuss the topics and issues we know are important to you. Breakout sessions included advice on financial and practical support, looking after your mental health and supporting a child with a muscle-wasting condition into adulthood. There were also research Q&A sessions, which proved extremely popular.

And of course we celebrated the winners of our 2019 President's Awards, who were presented with their trophies by MDUK Vice President, Michael Attenborough CBE – (see page 14).

"The Living with a muscle-wasting condition session told us of the stories that were inspiring and promising of a better future. It was super optimistic giving hope to many."

Thank you to everyone who came along and made the day special.

ROYAL RECOGNITION

We are delighted that a number of our supporters have been recognised by the Queen for their commitment to raising money and awareness to help beat muscle-wasting conditions.

Our President, **Gabby Logan** was awarded an MBE in the New Year's Honours List for 'services to sports broadcast and promoting women in sport'. She was joined on the honours list by MDUK Vice President **Charles Manby**, who was also given an MBE. Meanwhile, **Sue Barnley**, who runs the Help4Harry Family Fund, was awarded a BEM – British Empire Medal – for 'voluntary and charitable services'.

They follow in the footsteps of **Claire O'Hanlon**, from County Tyrone in Northern Ireland, who received an MBE last year. Claire chairs our Northern Ireland Council and has raised substantial money for MDUK and a number of Duchenne charities since her son Luke was diagnosed with the condition, aged 13 months, in 2012.

In Scotland, Paisley man, **Connor Colhourn**, had the privilege of meeting the Queen at the Palace of Holyroodhouse last summer. He was invited to a garden party in recognition of his positive attitude to living with Duchenne muscular dystrophy and charity fundraising.

Connor was diagnosed with Duchenne when he was two years old. Now in his twenties, he has helped to raise thousands of pounds for MDUK. Connor also participated in the Queen's Baton Relay for the 2014 Commonwealth Games.

Claire O'Hanlon

Connor Colhourn (right)

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THANK YOU FOR TAKING PART

By taking part in events across the UK, you're helping ensure we can be here for everyone affected today, tomorrow and beyond. Together, we're determined to make every day count for people living with muscle-wasting and associated conditions.

London Marathon

A big thank you to the 92 runners who took on the Virgin Money London Marathon for us last year. Thanks to your incredible effort (and stamina) we raised in excess of £234,000! This year, we have more than 100 people taking part and we can't wait to see how they get on.

Microscope Ball

Former rugby star, Will Greenwood was the compere for our 36th Microscope Ball, which was held last October. The event was well attended with 740 people there on the night, raising a total of £380,000.

BGC Charity Day

We enjoyed the company of Beverly Knight, Gabby Logan, Kirsty Gallagher, Monty Panesar and Sam Allardyce at last year's BGC fundraising event.

Held, as always, on 9/11, all of our supporters did a great job of representing us at the event. One of big highlights was our fantastic young supporter, Carmela, shouting, "Show me the money," at the traders!

Great Chef's Dinner

Around 400 guests came along to a special Great Chef's Dinner in London organised by the Q Trust in aid of MDUK. Hosted by Prue Leith, the exclusive event involved other top chefs such as Tom Parker Bowles, Richard Corrigan and Skye Gyngell. The dinner proved to be a great success, raising a staggering £140,000!

Oxford and Cambridge Town and Gown

Thank you to Newton Europe who sponsored our Oxford and Cambridge Town and Gown running events last year. More than 7,000 runners got involved in total, raising an incredible £279,313. If you fancy giving one of these 10K runs a go this year, get in touch! The Oxford Town and Gown run is on 3 May while our Cambridge 10K is on 18 October.

If you would like to support MDUK, or to organise your own event, please contact events@musculardystrophyuk.org


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WHAT YOU CAN GET ON BOARD WITH

Whether you're looking for a local group for help and support, an information event or want to get involved in one of our fundraisers, we have just what you need!

Skydives

Throughout March, we're looking for brave souls to take a leap of faith for MDUK – by jumping out of a plane! If an outdoor jump isn't your style, why not try an indoor skydive instead? All we ask is that you raise a minimum £399 for outdoor skydiving or £100 for indoor ones.

Translational research conference

Scientists from across the globe are expected to come together in London for the 2020 UK Neuromuscular Translational Research Conference on 22 and 23 April. The conference, which will showcase the latest research developments, will be followed by a patient information day on 24 April. More details coming soon.

Scottish Conference

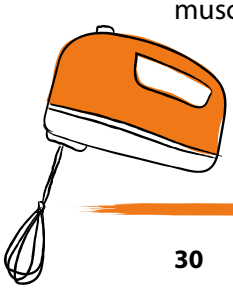
Our Scottish Conference will be held on Saturday 6 June. Join us in Glasgow to find out about some of the latest research developments, hear from inspiring speakers and spend the day catching up with old and new friends.

Pedal, Paddle, Peak

Back for a fifth year, we're once again challenging teams of four to cycle, canoe and trek their way around the Lake District. Entries are now open for our Pedal, Paddle, Peak event, which takes place in July. It's a great way to get your family, friends and even work colleagues involved.

Bake a Difference

Bake a Difference is changing – instead of asking you to help us raise some dough for one week in October, you can now do it all year round! Every cake you bake at any time of the year will help us make every day count for people living with muscle-wasting conditions.



Find our full list of events at www.musculardystrophyuk.org/events

DAVID WILLIAMS CAN NOW ENJOY THE OPEN ROAD THANKS TO LEWIS REED

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David Williams from Chester has difficulty controlling his limbs and is unable to walk but is now enjoying the freedom that comes with your own vehicle as he is now the proud owner of a converted VW Shuttle SE wheelchair accessible vehicle from Lewis Reed (WAV) Ltd.

This is David's first drive from wheelchair vehicle and features bespoke controls fitted that include a hand controlled steering tiller and a toe operated keypad to operate the lights, indicators, etc. The arm to which the tiller is attached is positioned in front of him in the driving position once the steering wheel has been removed. The unique design of the system also allows other people to drive the vehicle, all they have to do is unplug the arm which holds the tiller system and plug the steering wheel back in.

David has now had his vehicle since 2015 and is delighted with it as he explained:

> "The vehicle has been a dream come true... more than that in fact. I never thought I would be able to drive. I live with my elderly mum who no longer likes to drive long distances so being able to drive myself means we can get out and about independently and without the vehicle from Lewis Reed we would both be pretty much housebound. I am also a journalist who specialises in motorsport so I can now drive to race meetings myself. Beyond all else I love the sense of freedom that being able to drive brings".

David was assessed by Motability who then put together a "package" consisting of the Lewis Reed conversion with bespoke adaptions to suit him.

To find out more about the full range of vehicle conversations available from Lewis Reed, call us free* on **0800 247 1001**.

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