Autumn 2022

Muscular Dystrophy UK

Fighting muscle-wasting conditions

Campaign

The newsletter for our supporters

Inside:

- Changing Places update
- Return of face-to-face Muscle Groups
- Impact report highlights

Will has the (Will) power!

We were thrilled to hear that Angela Turnbull, whose nephew Will, lives with Duchenne muscular dystrophy, is celebrating her successful, year-long campaign with Sunderland council to get a wheelchair accessible swing and roundabout installed in their local playground.

Angela's work will make a huge difference, not just in Will's life, but for many other local families. It may even help pave the way for more inclusive playgrounds across the country! Angela's campaign has attracted the attention of local and national press which has also helped raise awareness.

"It makes me so happy to see Will enjoying something like this in a wheelchair. It is an enormous achievement. There are plenty of other children with limited mobility who will also be able to make use of this incredible equipment and that makes me smile" said Angela.

Priya Manek, Head of Marketing and Communications said:

"It was such a joy to amplify Angela's efforts using our channels and help promote her campaign for a wheelchair friendly swing for her nephew in their local park. Just like Angela, so many of you have some incredible stories to tell and we for sure would love to hear them"

To share your story with us please email us at: pressoffice@musculardystrophyuk.org

To donate

To support our work with a donation please visit www.musculardystrophyuk.org/autumn22, call 0300 012 0172 or scan the QR code. Thank you!

Welcome

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

It's been an exciting summer and our Town and Gown run and Pedal Paddle Peak challenge were just two examples of our popular fundraising events. Overall, your support has enabled us to accomplish so much, and we've included some highlights from our 2021/22 Impact Report in this issue. You can read about how much you have helped us achieve, and are continuing to achieve, on pages 4, 5, 8, 9, 22 and 23.

On pages 18 and 19, we feature the variety of calls our helpline receives and the many ways we can support you if you need help. We were also delighted to be able to return to face-to-face Muscle Group meetings in 2022 and on page 12 you can read about how they can help fight loneliness.

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

Gabby Logan MBE
President, Muscular Dystrophy UK

PS: Check out the MDUK online shop www.musculardystrophyuk.org/shop and get in the Christmas spirit nice and early!

Our vision

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

Muscular Dystrophy UK
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www.musculardystrophyuk.org

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

DMN/2209

FIND US ON

Registered with FUNDRAISING REGULATOR

On the cover: a very excited Will Calvert enjoying an accessible swing in his local playground.

MDUK's Year in Numbers

Income generated:

£6.7m

We supported

2,078

people over the phone or email.

Supported the allocation of Government funding to Local Authorities that will see the installation of an additional

500+

Changing Places toilets in England over the next two years.

Funded 31

research projects, including nine new research grants.

Successfully concluded the

£1.2m appeal

for the MDUK Oxford Neuromuscular Centre.

We provided training to

OVER 1,600

NHS health and care professionals.

We worked on securing access to

SEVEN TREATMENTS

for four different conditions.

The gift of independence through treatment

Through our engagement in the appraisal processes for new drugs and treatments we have been able to help individuals living with muscle-wasting conditions access new opportunities to live with more freedom.

Finley is nine years old and lives with spinal muscular atrophy (SMA). His mum, Rosie, talks about the life-changing impact that receiving new treatments has had on Finley's strength, energy and freedom.

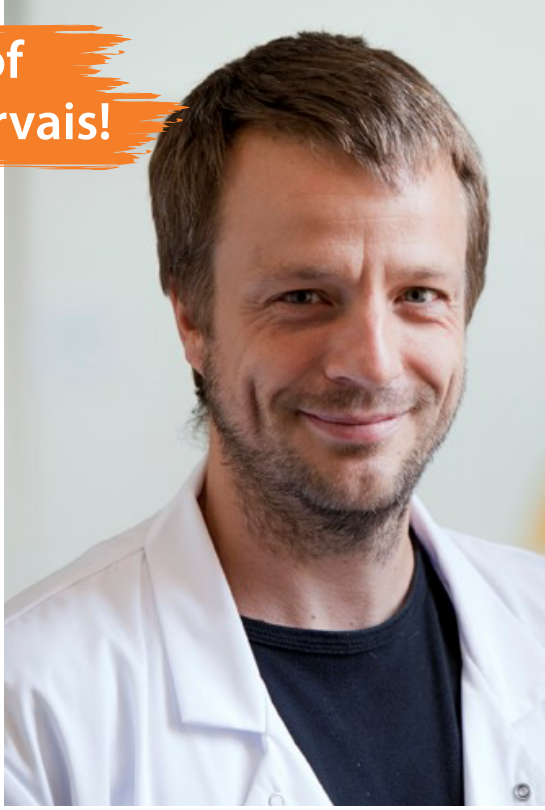
"It's incredible to think that there are a range of treatments available for SMA when there was nothing at all for years. Spinraza has given Finley so much more strength than he had before, and than what we had hopes of. However, Finley significantly struggled with the [infusion used to deliver Spinraza].

"Several months back, Finley changed treatments to Risdiplam; a treatment that MDUK, in partnership with SMA UK, fought for and secured access to, through a Managed Access Agreement. This is administered daily through Finley's feeding tube and is massively helping to maintain his strength and energy.

"Finley is delighted he doesn't have to experience any more pain with treatment, and it also means less time in hospital, which is always welcomed! Since starting treatments Finley has not had any emergency respiratory admissions. He is finally able to live the life of a nine-year-old schoolboy and we are able to live without so much sadness and worry.

"Recently, we've also got him a new powered wheelchair, that supersedes the chair he has had since he was three. He is really excited about having a new, faster, more efficient chair, and this would not have been possible without his strength and energy levels that have been maintained by the treatment he has been able to access. Our excitement is at fever pitch!"

Meet the other side of Professor Laurent Servais!



You may remember Professor Laurent Servais from our January fundraising appeal where he spoke passionately about his work at the MDUK Oxford Neuromuscular Centre and the future of neuromuscular research. Firstly, we wanted to say a huge thanks to everyone who donated to that appeal – you helped us raise over £28,000!

We wanted to find out a little more about what Professor Laurent Servais is like when he's not completing groundbreaking research, so we sat down for a behind the scenes Q&A!

Q What makes you get up in the morning?

A Actually, the two projects that I have been leading for the last year are what motivate me.

Firstly, we've been looking at the development of powerful outcome measures using wearable devices. This is a disruptive technology in comparison with traditional scales, and importantly, it has the power to make clinical trials much shorter with many less patients. I dream of conducting trials that are more objective which allow us to know much faster if a drug works or not. We are pretty close right now.

Secondly, I've been leading new-born screening of a severe and treatable disease. Setting up new-born screening for spinal muscular atrophy (SMA) and inspiring several programs around the world has convinced me about the potential of new-born screening to be game changing in our fight against rare diseases. I want to apply what we have done for SMA to 124 diseases, and probably more next year. If we can identify them early and treat them early, this will be transformative for patients.

Q What is the most exciting thing you've worked on?

A The odyssey of SMA

Q What do you do to relax after work?

A I relax by taking a walk, enjoying a Belgian beer or learning a foreign language with my daughter.

To read more about the appeal visit www.musculardystrophyuk.org/specialappeal

New-born screening

In March, the UK's first pilot study of new-born screening to detect spinal muscular atrophy (SMA) within days of birth was launched in the Thames Valley area. This study, designed by Professor Laurent Servais and his team, will be run at the MDUK Oxford Neuromuscular Centre and is crucial given that every five days a baby is born with SMA in the UK. Treatments are now available for SMA, and if delivered at birth, provide these babies with their best chance to live healthy and independent lives. However, without postnatal screening, by the time infants show symptoms and receive a diagnosis, most have lost 90% of nerve function. Professor Laurent Servais has said that "screening for SMA at birth will significantly increase a new-born's chance of survival and development" and there has been

a strong case for this across Europe and the US. We are proud to be working with SMA UK and a range of partners to seek the introduction of such a screening programme for pregnant women across the UK.

Looking back on what we've achieved

GOAL:

Fund high-quality research to find effective treatments and ultimately identify cures for all muscle wasting conditions.

We funded **eight innovative projects**.

We **funded a four-year PhD studentship** as part of our SMA PhD Partnership with Spinal Muscular Atrophy UK.

We launched a **full-capacity 2022 grant round** without any restrictions imposed due to Covid-19.

GOAL:

Ensure everyone has access to specialist NHS care from a multidisciplinary team

We secured NHS funding for **two new** neuromuscular specialist posts, totaling a NHS investment of **up to £7.5m per year** in specialist services.

We supported clinical networks and **provided training to over 1,600 healthcare** professionals through online training modules, to better equip them to meet the needs of people affected by muscle-wasting conditions.

Taken from our 2021/22 Impact Report

GOAL:

Provide services and promote opportunities to enable each affected individual to live as independent a life as they wish

We responded to **more than 2,078 requests** for information or support over the phone and by email, and **9,354 people used our online forum** to share experiences and support.

We saw **a 3.7% increase** on the previous year in the number of visits to the care and support section of our website.

GOAL:

Generate income to continue supporting the community

Total income for the year **£6.7m**, a **20% increase** compared to 2020/21

More supporters took on fundraising challenges, and corporations chose us as their charity of the year.

The MDUK Oxford Neuromuscular Centre **appeal brought in over £1.2m**.

Thanks to the generosity of our funders, our Trust team had one of their most successful fundraising years ever, **raising over £1m**.

Being there for Darren

We were there for Darren when he needed us. Darren Woolrich, 50, was diagnosed with Charcot-Marie-Tooth disease (CMT) officially in 2021. After only being awarded the standard rate for the daily living and mobility component of Personal Independence Payment (PIP), Darren reached out to MDUK for support.

Darren felt the PIP decision didn't accurately portray his circumstances and that he should be awarded a higher rate. However, he couldn't face going through this process again as he was struggling to come to terms with his disability and the effects it had on his life.

Whilst receiving support from MDUK, Darren came a long way in accepting his condition and saw massive improvements in his mental wellbeing. Prior to this, Darren struggled to speak openly about his condition. He said 'Speaking with you and going through everything has really helped me come to terms with my situation and accept it more'.

Paige, MDUK's Helpline, Information and Support Officer, spent time getting to know his situation and gain as much information as possible to be able to write him a supporting letter to try and get his PIP award increased. With this support, Darren's application went through mandatory consideration, and he was awarded the enhanced rate for mobility which increased his income by a significant amount.

Darren was incredibly grateful for our help, and the outcome of the appeal.

"Thank you so much for my letter, Paige, seeing everything about my condition and my story written in front of me, from my view, was really helpful and eye-opening for me. I cannot thank you enough."

Our Information, Advocacy and Care Team are here to support you. Whether you have just received a diagnosis, need some practical information or would just like to speak to someone who understands what you're going through, we can help.

You can contact the team by calling our free helpline on 0800 652 6352 or email info@musculardystrophyuk.org.

Policy Update

Over the last few months, the policy team has been hard at work. A key focus has been in the changes to funding of services with the upcoming NHS reform. This has involved working closely with our Neuromuscular networks to build a rapport with local commissioners (i.e. those responsible within NHS England for funding decisions) to address local gaps in services.

We have also relaunched our Mental Health Matters campaign to upskill key healthcare professionals involved in neuromuscular care to better support people with a muscle-wasting condition.

Over the summer, we undertook a survey into the impact of the increasing cost of living and how this is impacting those with muscle-wasting conditions. Our findings will be published at the end of September with key recommendations to ensure better support is available when needed.

If you want to hear more about our current projects, please contact the policy team at campaigns@musculardystrophyuk.org

Michaela Regan,
Head of Policy and Campaigns

Fighting loneliness with the return of face-to-face Muscle Groups

Paula and Andy Bailey at the Leeds muscle group.

Having a muscle-wasting condition often means adjusting to a new and unexpected reality, and many people struggle with feeling lonely as they process this. But meeting other people in similar situations and talking about the things that matter to you can really help.

During the COVID-19 pandemic, we continued our Muscle Groups virtually, but we are delighted to say that this summer we welcomed the return of in-person meetings again! It is a fantastic opportunity for people living with muscle-wasting conditions and their families to come together and socialise over a cup of tea like we used to.

We know there is value in virtual events, so to keep them inclusive, most will still have an option to access them virtually as well. As the summer schedule of in-person meetings has now finished we will continue virtually as the weather turns colder, returning to in-person meetings in spring 2023.

"We encourage anyone who may be considering joining a Muscle Group meeting to do so. There's so much support and help available".

Jane and Mark Field
West Midlands Muscle Group

Register your interest in the Muscle Groups, and keep up-to-date, by visiting www.musculardystrophyuk.org/joinagroup

It's in our DNA!

In February 2022, we launched a fantastic year-long partnership with Tikiboo clothing, an award winning active wear company. This partnership was born out of a great shared purpose; Tikiboo clothing is bright, bold and wants to be seen. This statement coincides with our annual 'Go Bright' day and that's where the conversation started.

Together, we designed the #MusclesMatter collection and Tikiboo are donating 20% of all sales of this range to MDUK. In just the first quarter of the partnership a fantastic £12,000 was raised! The collection consists of neon leggings for both adults and children, bra tops, hoodies for both adults and children, t-shirts and more. One of their striking designs features a DNA theme and more designs will be launched in the new year.

If you run a business or think your employer would like to talk about a charity partnership, please contact Martin Hywood, Corporate New Business Officer at m.hywood@musculardystrophyuk.org or simply call Martin on 07899 917 868. He'd love to hear from you!

Changing the world – one toilet at a time!

We are delighted to be partnering with the Department of Levelling Up in supporting the government's £30 million programme to install a further 500+ Changing Places Toilets over the next two years in England. MDUK will be working with 200 local authorities so we can help them use this funding to increase the number of Changing Places toilets in England to over 1,800.

The programme was launched in spring 2022 and saw £23.5 million allocated to different councils in England. The next phase will be launched later this year when the remaining £6.5 million can be applied for by local authorities, particularly in areas where there is currently little or no Changing Places toilet provision.

As co-chairs of the Changing Places consortium, MDUK have already worked with local users to ensure that the regions most in need applied for funding and we will continue to be heavily involved in the programme by providing specialist expertise to support local councils that are awarded funding.

This is where you can help! We have developed a new toolkit to support campaigners with the tools, resources, information and guidance on how you can best make a difference and influence local authorities to apply for future funding and have even more Changing Places Toilets installed. All the information is on the website and available ready to support our campaigners www.changing-places.org/get-involved/local-campaign

We'd also like to introduce the new Changing Places Toilet monthly newsletter – Engaged. Check it out on our website www.changing-places.org/news and remember we're here to support you with everything Changing Places related!

Making all the difference

Changing Places toilets can make a huge difference to people living with neuromuscular conditions, like 8-year-old Fraser, who has Duchenne muscular dystrophy.

Fraser Simmonds lives with Duchenne muscular dystrophy and has never been able to walk. He uses a powered wheelchair and needs assistance to use the toilet. His mum, Shelley, explains why Changing Places toilets are so important to families like hers.

"Regular disabled toilets are dark, dingy and smelly. They're rarely found clean and they often are filled with dirty nappies which are left to overflow and fester. Fraser needs help removing his clothes to use the toilet and I have to kneel on the floor to support him with this. They are often far too small and I dread to think what I've knelt in over the years.

We like to explore different places and have lots of adventures. Changing Places toilets allow us to do that without the stress of wondering what the loo will be like. It makes such a difference to open the door of a Changing Places toilet and find a bright and clean space waiting for us. Changing Places toilets make disabled people feel like they matter; going to the toilet is a basic human right and everyone deserves the same dignity."

MDUK are proud to co-chair the Changing Places Consortium to help meet our vision that everyone affected by muscle-wasting conditions has access to clean, secure toilets and changing facilities wherever they are.

Find your nearest Changing Places toilet here www.changing-places.org/find

Epidemiology study

New research – funded by MDUK – reveals 110,000 people in the UK now live with muscle-wasting conditions

Tens of thousands more people in the UK are living with rare, muscle-wasting conditions than previously thought. Research we've funded has found 110,000 people are living with associated conditions, compared with the earlier figure of 70,000.

These findings come at an important time when decisions are being made about the future of health service commissioning and delivery. The new figure puts the number of people living with muscle-wasting conditions on a par with more well-known conditions, such as multiple sclerosis and Parkinson's disease, and we'll use these findings to fight for better healthcare for our community.

The methods used for the new study are different from those used before, so you can't directly compare the two figures. But the new results do suggest an increase in life expectancy among those living with muscle-wasting conditions.

Dr Iain Carey led the study at St George's University of London. He and his team used millions of healthcare records collected from GPs across the UK between 2000 and 2019. The findings have now been published in the journal PLOS ONE.

Dr Carey said:
"We're grateful to MDUK for funding our research, which has revealed a far greater number of people living with neuromuscular conditions than previously estimated. Indeed, a rise in prevalence among older age groups suggests that some of these conditions are now much more common within an ageing population. It is therefore important that multidisciplinary health and social teams are made aware of this new estimate as soon as possible."

Our CEO, Catherine Woodhead, believes decision-makers should engage with these findings as a matter of priority:
"No wonder neuromuscular specialist services are so stretched. They already work tirelessly to meet the complex needs of people with muscle-wasting conditions, and this research shows clearly that they need further resources. This growing population needs support, and they are often overlooked. They cannot be left behind."

The news also comes after the publication of the charity's Shining a Light reports, which highlight how the impact of COVID-19 has exacerbated historic issues around the paucity of neuromuscular service provision. These reports offer recommendations on how care can be improved, and MDUK is engaging with health services across the four nations, as well as the Department of Health and Social Care, to influence how neuromuscular services are provided.

If you have a muscle-wasting condition and need support, contact our helpline on 0800 652 6352 or find out more on our website www.musculardystrophyuk.org/get-support

We'd love to know more about you and why you support MDUK. Complete our supporter survey here: www.musculardystrophyuk.org/survey2022

A day in the life of our helpline

Paige and Romla - Helpline, Information and Support Officers at MDUK – describe some of the calls our helpline receives.

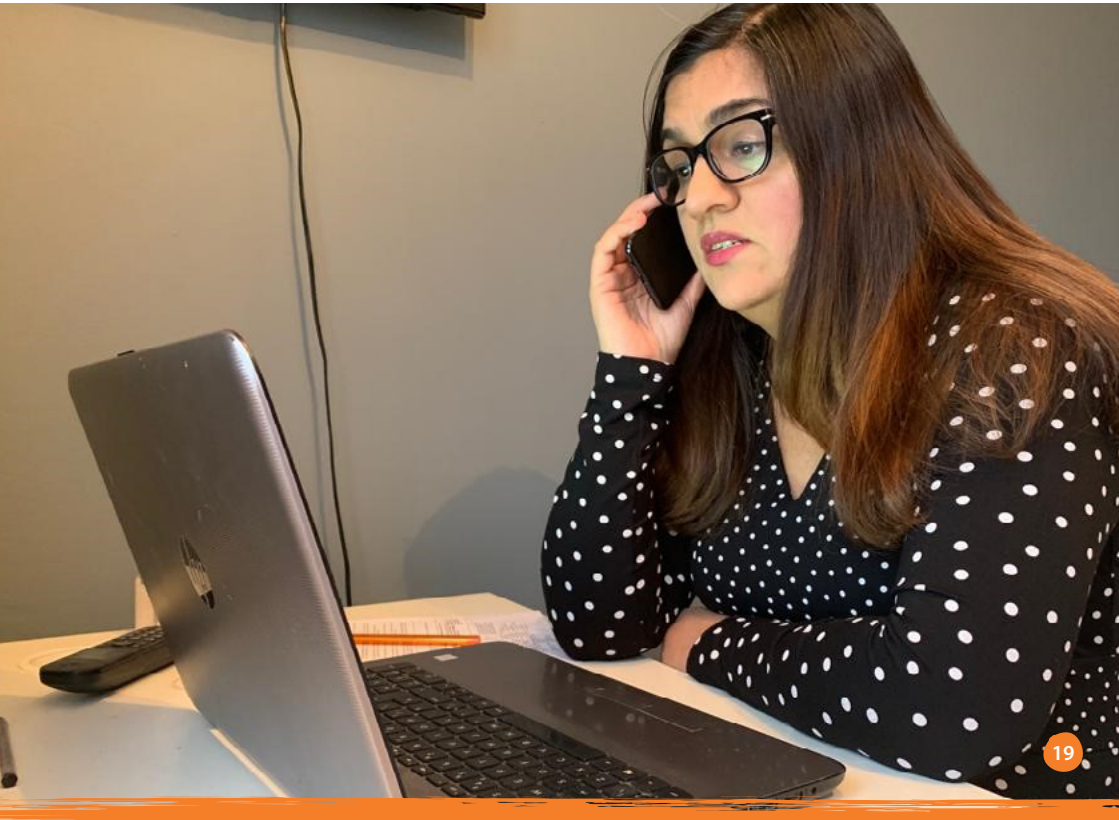
Paige – Today I spoke to a person who had recently been diagnosed with Inclusion Body Myositis (IBM). Since their diagnosis, they had been struggling with their mental wellbeing and had been feeling very alone as other people were not aware of what IBM was and they had also never met anyone else with the condition. They wanted to know if we could help. I recommended our Peer Support network, explaining that we could put them in touch with a trained volunteer who also has IBM, so they could speak with someone who may understand how they are feeling and to also get advice and information too. I also told them about the MDUK local Muscle Groups where people with muscle-wasting conditions can come together regularly. When I mentioned the ways we can support them, they were so happy to hear they could finally speak to someone who understands what they're going through.

Romla – I spoke to someone today who was calling about their husband. His condition had progressed, and he was struggling with tasks around the house, such as safely getting into the shower and out of the bath. They explained that they've seen helpful aids but as they are on a low income, are concerned about how to fund them. I told them that they would likely benefit from being referred to an Occupational Therapist (OT) and offered to find the contact details for their local authority. I explained that an OT can complete an assessment at home and some aids and equipment can be provided by the local authority. I explained that there may be certain specialist equipment that cannot be funded by the local authority but let them know that they could get back in touch with us so we could explore what other grants are available to them. They were relieved to hear that they can get some help without having to fund it all.

Paige - The last call I had was about a Personal Independence Payment application; an individual living with Becker muscular dystrophy was unhappy with what

he had been awarded. He was told by his neuromuscular care advisor that we can support with challenging a decision outcome and wanted to know how. I explained that I would be able to write a supporting letter for the mandatory reconsideration to explain how his condition affects his daily living and mobility needs and support him to argue his case about why the initial decision was incorrect and why he would benefit from a higher award. We discussed the process of sending out a consent form, as we would be dealing with his personal information, arranged a phone call to discuss the current award and supporting letter and established when the supporting letter would need to be sent to the Department for Work and Pensions. After our conversation, he was hopeful that, with my support, he could get the outcome he would like and thanked me for my help.

If you need help or support, you can contact our team by calling our free helpline on 0800 652 6352 or email info@musculardystrophyuk.org.



Get Involved

Shop and raise money for us – at no extra cost to you

Shop at smile.amazon.co.uk, select us as your chosen charity and Amazon will give us a donation! You pay exactly the same as you would on the main Amazon site so there is no extra cost to you. Or if you really love your online shopping, why not sign up to Give As You Live www.giveasyoulive.com and do your online shopping through their site?

They have over 5,500 stores on their site, and we will receive a donation for each purchase you make. The site is very easy to use, and you can even add a quick shortcut to your browser. Both are fantastic ways to raise money for us for doing shopping you were going to do anyway!

MDUK Christmas Shop!

Have you started your Christmas shopping yet? Whether it's browsing the catalogue, or looking online, take a peek inside our shop to see our selection of Christmas cards, home and bath scents and gifts. Check out our new exclusive Christmas selection of baseball caps and zippered hoodies. Or perhaps our super soft cuddle puppy husky is more appealing! Profits made through our shop are another great way of supporting individuals and families living with muscle-wasting conditions.

Check it out now:
shop.musculardystrophyuk.org

Raffle

We are pleased to announce our Spring Raffle First Place Winner is... **Anne from Bedford!**

Anne has been a generous supporter of MDUK since her grandson, Joe, was diagnosed with Duchenne muscular dystrophy when he was only 8 months old. Joe is a lively 5-year-old who loves going on family bike rides, being read to and playing puzzles. Thanks to his early diagnosis, his mobility has been assessed and supported, allowing him to enjoy life to the fullest. The raffle helps us support families like Joe's to access the care they need and make every day easier.

The runner-up prizes went to supporters in Leeds and Barnet.

Thank you everyone for playing and our next raffle will be available to enter from October. Good luck!

Celebration giving – birthday fundraisers

Do you have enough 'stuff'? Instead of receiving birthday or Christmas presents you won't use or – shhh, don't like! – why not ask for donations to your favourite charity? It is easy to set up an online Facebook Fundraiser or, if you prefer, simply ask for donations that you can send on to us here at Muscular Dystrophy UK. You can ask for donations in lieu of presents, fundraise for a charity close to your heart or even embark on a challenge to increase those donations!

For tons of fundraising ideas, visit our website: www.musculardystrophyuk.org/fundraisingideas

Looking forward

Increasing access to treatments is a core part of our work to fight muscle-wasting conditions. This year, we helped to secure or improve access to seven treatments that help people with Duchenne muscular dystrophy, SMA, Pompe disease and myotonia in adults with non-dystrophic myotonic disorders.

Let's take a closer look...

Translarna/Ataluren

A treatment for people with Duchenne muscular dystrophy with a nonsense genetic mutation currently available in England through a Managed Access Agreement (MAA) and in Scotland through the ultra-orphan Pathway.

MDUK is a member of the Managed Access Oversight Group (MAOG) and this year we helped to secure an extension to the MAA and began preparation for the full NICE appraisal in 2022/23.

Spinraza/nusinersen

A treatment for people with SMA currently available in England through a MAA) and in Scotland through the ultra-orphan Pathway.

As a member of the MAOG, we worked with SMA UK to help ensure that more people became eligible to access the treatment.

Evrysdi/risdiplam

An oral treatment for people with SMA. This year, working with SMA UK, we successfully helped to secure access to the treatment in England through a MAA. We helped to secure approval of the treatment in Scotland by the Scottish Medicines Consortium.

Zolgensma/onasemnogene abeparvovec

A gene therapy for SMA. It addresses the genetic cause of SMA by delivering a functional copy of the SMN1 gene into nerve cells. This gene is critical for the function of the nerves that control muscles.

This year we worked with SMA UK to help successfully secure NICE approval of the treatment for babies under 12 months who fit the criteria. It is also available in Scotland.

Avalglucosidase alfa

A potential treatment for Pompe disease. This year we actively engaged in the early stages of the NICE assessment process of the treatment.

Cipaglucosidase alfa (with miglustat)

Another potential treatment for Pompe disease. This year we actively engaged in the early stages of the NICE assessment process of the treatment.

Mexiletine/NaMasucula

A treatment for myotonia in adults with non-dystrophic myotonic disorders. This year we helped to secure NICE approval for the treatment in England. MDUK had previously played a role in ensuring the treatment was approved for use in Scotland by the Scottish Medicines Consortium.

WILL YOU LEAVE A GIFT

to help us create a future free from the limitations of muscular dystrophy?

Gifts in Wills fund over 30% of everything we do at MDUK.

"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 grandad

For a confidential discussion about leaving a gift in your Will or to find out more about our Free Will Service, call Grace Moran on 0207 803 4845 or email legacy@musculardystrophyuk.org. Alternatively, scan the QR code.

www.musculardystrophyuk.org/giftsinwills

We have partnered with the National Free Wills Network to offer our supporters the opportunity to have a simple Will written or amended for free. There is no obligation to leave a Gift to MDUK however we hope you might consider it.

Muscular Dystrophy UK
Fighting muscle-wasting conditions

NATIONAL FREE WILLS NETWORK

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