Muscular Dystrophy UK

Fighting muscle-wasting conditions

Autumn 2021


# Campaign

**The newsletter for our supporters**

**Inside:**

- Remembering our Royal Patron
- A lifeline for boys with Duchenne
- Providing support after lockdown

# A story of Jon, #MyMateMartin and BBC Radio 4


**MDUK recently featured in a BBC Radio 4 Appeal, which aired on Sunday 25 April 2021. We've had a fantastic response to the Appeal, which has brought in £22,000 to date.**

The Appeal focused on the extraordinary friendship between Jon Richardson, stand-up comic and star of *8 out of 10 Cats*, and Martin Hywood, MDUK supporter and a fellow Leeds United fan. Martin and Jon share a love of football, lively banter and help each other through the tough times.

Martin, who has limb girdle muscular dystrophy, was classed as clinically extremely vulnerable when lockdown began. This meant he spent more than a year shielding at home during the pandemic, and during this time lost the ability to walk.

Martin said: "It's great to have a mate like Jon… he's a fantastic friend who endorses all the good things I'm trying to achieve, especially when I'm trying to raise awareness and funds for MDUK."

**If you haven't heard their entertaining and heartfelt appeal, you can still listen at: www.musculardystrophyuk.org/bbc-radio-4-appeal**

# Welcome

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

On p4-5, we remember HRH Prince Philip and look back at his time as Royal Patron of MDUK and the commitment he showed to the charity.

We also share on p6-7 an exciting update on Translarna, a drug used to treat symptoms of Duchenne muscular dystrophy, and find out the positive impact this is having on people with the condition.

And on p11-12, you can 'meet' our team of Information, Advocacy and Support Officers who provide invaluable support to families across the UK.

I hope you'll enjoy reading about the work that your kind support makes possible, and how it is making a very real difference to people's lives – today and for the future.

**Gabby Logan MBE**
President, Muscular Dystrophy UK

**PS:** Visit **shop.musculardystrophyuk.org** to discover this year's gorgeous range of Christmas gifts, cards and stocking fillers.

**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: HRH Prince Philip at an event in 2016 celebrating 50 years of Royal patronage.

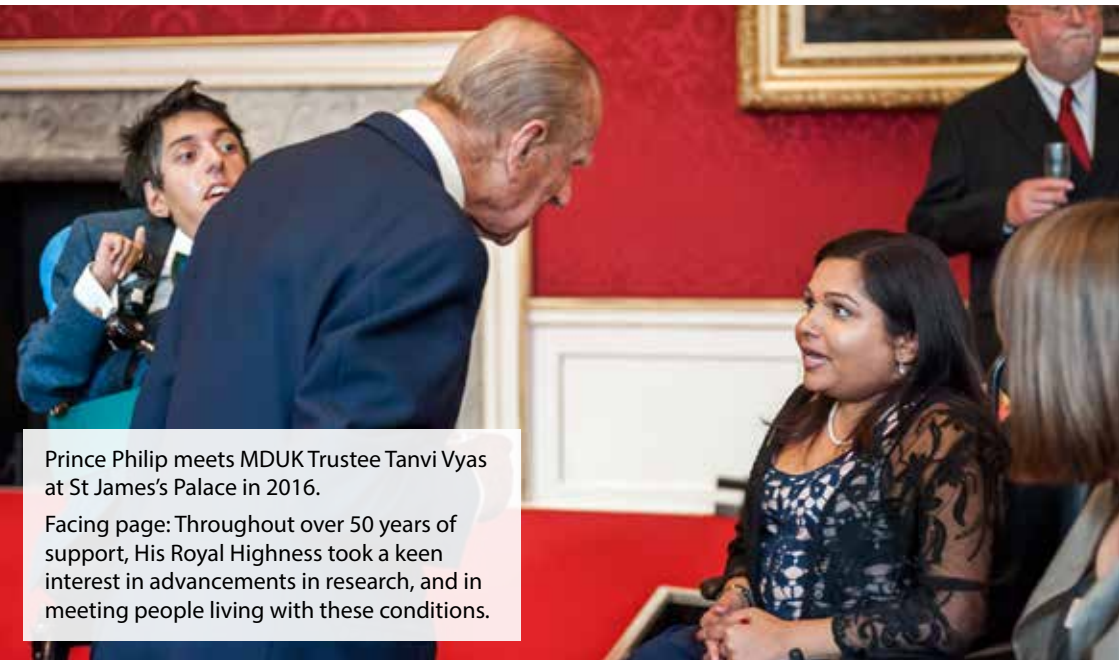
# Remembering HRH Prince Philip

**We were deeply saddened to learn of the death of His Royal Highness, Prince Philip, our Royal Patron on 9 April 2021. For over 50 years, Prince Philip showed his unwavering support for MDUK and his commitment to the fight against muscle-wasting conditions.**

Tanvi Vyas, MDUK Trustee, met the Duke on two separate occasions: **"I found Prince Philip to be really charismatic, charming and well-humoured. He had an incredible memory, and recalled meeting me on a previous occasion, which was really surprising considering the number of people he must meet. It's been wonderful that Prince Philip supported the charity and its aims for so many years."**

HRH Prince Philip became a Patron of MDUK in 1966 – a time when very little was known or understood about muscular dystrophy. Prince Philip's backing, along with that of our community of supporters, has been instrumental in helping us advance the search for treatments and cures, as well as clinical support and care for people living with muscle-wasting conditions.

**You can read more about this progress in *50 Years Together*, an online version of our 2016 publication honouring Prince Philip's outstanding half-century of service. www.musculardystrophyuk.org/50-years-together**



Prince Philip meets MDUK Trustee Tanvi Vyas at St James's Palace in 2016.

Facing page: Throughout over 50 years of support, His Royal Highness took a keen interest in advancements in research, and in meeting people living with these conditions.

# A lifeline for boys with Duchenne

**In May this year, we learnt that access to a drug used to help treat symptoms of Duchenne muscular dystrophy has been extended for use on the NHS in England until January 2023.**

The drug, Translarna, can help treat a version of Duchenne muscular dystrophy caused by nonsense mutations. These are changes in the DNA, which affect the length and functioning of the dystrophin protein, accounting for around 10 to 15 percent of diagnoses.

Eligible boys have been receiving the drug under a Managed Access Agreement (MAA), due to expire in July 2021. An MAA is an agreement between NHS England, NICE (the body that advises which treatments should be available on the NHS in England) and the drug manufacturer, allowing patients to receive treatments while long-term data about the drug's effectiveness is gathered. In May, we learnt the agreement was being extended until January 2023, meaning new eligible patients can get access to Translarna during this time.

Since the MAA began in 2016, MDUK has represented families living with Duchenne as part of the NICE-led Managed Access Oversight Committee (MAOC) – the committee responsible for monitoring the implementation of the MAA and recommending actions to support its operation.

The news was a huge relief for Pedro and Joanne Fernandes, parents of 11-year-old Luca, who has Duchenne muscular dystrophy. Luca has been on Translarna since September 2016 and, while most boys with the condition need to use a wheelchair by the age of 12, Luca rides a bike, is learning to skateboard and surf, and can keep up with his three-year-old brother Micah.

Despite being told he'd need mobility aids from the age of eight, Luca has recently completed his cycling proficiency test – something his parents never thought they'd see him achieve.

Pedro said: **"The news that the MAA for Translarna has been extended is an enormous relief for my family. As it is for so many other children, Translarna is a lifeline for Luca because it helps keep him active and mobile and slows down the progression of his condition. I never thought I'd see my son learn to ride a bike – I can't**

**explain how powerful that moment was for my family, and we believe that's because of Translarna."**

Robert Burley, our Director of Campaigns, Care and Support, said: "While we welcome this update, it's vital that all parties continue working together to ensure the right decision on Translarna is made by the end of the extension period, and there's clear communication with the Duchenne community throughout the next 18 months. We'll continue to support families, while working as part of the MAOC."

**Visit www.musculardystrophyuk.org/fast-track to find out how we're helping to improve access to specialist treatments for people with muscle-wasting conditions.**

11-year-old Luca with dad, Pedro

# Providing support after lockdown

## MDUK Muscles Matter 2021
online seminar series

Last year, with the UK in lockdown, we were unable to meet in person for our annual national conferences and so instead, we brought people together through our first series of online seminars. Members of our community were able to get information from experts covering a wide range of topics, all from the safety of their home.

Attendees told us that they found the seminars helpful and informative, accessible and easy to attend. Building on the success of our 2020 series, the MDUK Muscles Matter seminar series will become a regular feature in MDUK's calendar. They won't replace but will run alongside our live events when we can host them again.

Our MDUK Muscles Matter 2021 online seminar series started in May and will run through to October. The sessions will focus on a range of general and condition-specific topics that past attendees found to be most useful. For example, sessions such as research, practical and financial support, and managing your condition.

### Upcoming sessions

**September**

- **Duchenne muscular dystrophy**
  Thurs 9 Sept, 1pm - 3pm
- **Education**
  Fri 17 Sept, 12pm - 1pm
- **Mitochondrial disease**
  Tues 21 Sept, 10am - 12pm
- **Limb girdle muscular dystrophy**
  Thurs 30 Sept, 10am - 12pm

**October (dates and times TBC)**

- Becker muscular dystrophy
- Charcot-Marie-Tooth disease

**For details of our upcoming seminars or to book your place, visit: www.musculardystrophyuk.org/musclesmatter**

## Muscle Group meetings

Our network of local groups, known as Muscle Groups, have always given individuals and families living with muscle-wasting conditions important opportunities to find out how MDUK can support them, to meet others living with these conditions and to hear about opportunities for support in their local areas.

But in 2020, the COVID-19 pandemic and restrictions on face-to-face contact with people meant we had to put our Muscle Group meetings on hold.

We're delighted to let you know that, with input from our fantastic volunteers who have experience of living with muscle-wasting conditions, we have designed new virtual Muscle Group meetings. These will bring people together online, offering vital support and information, as well as the chance to connect with other people living with muscle-wasting conditions.

As restrictions ease, we'll be reviewing whether we can return safely to face-to-face meetings in the future.

**Visit www.musculardystrophyuk.org/get-support to find out more about the ways that we're supporting people living with muscle-wasting conditions.**

# More people eligible for SMA drug

**More people with spinal muscular atrophy (SMA) will now be eligible for access to Spinraza (also known as nusinersen).**

This news follows a decision from NICE (the body that advises what treatments should be available on the NHS in England) after reviewing data collected as part of the Managed Access Agreement (MAA) for this treatment.

Patient groups – MDUK, SMA UK and TreatSMA – have been working for nearly two years, in partnership with clinicians, so that people with SMA Type 3, who are unable to walk, can have access to Spinraza too. Previously, this group had not been eligible to access the drug.

NICE concluded that it was appropriate to extend the clinical eligibility criteria for Spinraza to this group. Their review has also removed the rule that meant those who lost the ability to walk after 12 months of treatment would no longer be eligible for further treatment.

NHS England is developing its service capacity to deliver Spinraza to this wider group and, as COVID-19 restrictions ease, they will be able to expand this capacity further. Where there are already services in place, some people will be able to have access to the treatment immediately.


Alongside SMA UK and TreatSMA, we worked with NICE, NHS England and the Managed Access Oversight Committee to update the MAA document to reflect the changes. These changes are now published on the NICE website.

**Find out the latest news in research by visiting: www.musculardystrophyuk.org/news**

# Meet your local MDUK support team

**Our Care and Support team are committed to providing vital information and support to individuals and families living with muscle-wasting conditions. In addition to the team in London who are available to provide support no matter where you live, we also have dedicated regional support managers in Scotland, Northern Ireland and Wales and we'd like to take this opportunity to introduce them.**

## Sam – Regional Information, Advocacy and Support Officer for Wales

I live just outside the beautiful harbour town of Aberaeron on the west coast of Wales. I enjoy going for walks and learning Italian – my dream is to retire in southern Italy!

I've worked in the charity sector for 16 years and have particular expertise in welfare benefits. I've worked at MDUK for two years and no two days are ever the same.

Whether it's chairing a Muscle Group meeting, taking part in a Wales Cross-Party Group meeting to campaign for better services, or meeting people attending clinic, my goal remains the same: to support those living with a muscle-wasting condition to the best of my ability.

**I recently helped someone who wanted to apply for Personal Independence Payments (PIP) but who felt overwhelmed by the process. We completed his PIP form together and I wrote a letter of support on his behalf, providing specialist information about his condition. When it came time for his assessment, I joined him on the call to offer extra support. He felt so relieved when, a few weeks later, he got his letter confirming he'd be getting this financial help.**

**The rapport and trust we'd built meant he was able to open up to me about his wish to live independently – something he never thought was possible. After giving him information about additional financial support he could be entitled to, including help with housing costs, I helped him through the application process. In June this year, he moved into his own flat and was delighted to finally have his independence.**

### Jackie – Regional Information, Advocacy and Support Manager for Scotland

I live just outside Glasgow with my family and dog, and love travelling around Scotland and discovering new places.

I've worked for MDUK for nearly four-and-a-half years and love what I do. My background is in social work and this knowledge and experience contributes towards achieving positive outcomes for people living with muscle-wasting conditions. I especially enjoy meeting individuals and their families and I value the time I get to spend with each of them.

After such a difficult year, I'm looking forward to the future and am focusing on re-developing the advocacy and support service in Scotland, following the pandemic.

### Demelza – Regional Information, Advocacy and Support Manager for Northern Ireland

I live in Belfast with my husband, two kids and our pet pooch. I love days out in the countryside and around our gorgeous coastline.

I've been part of the team for nearly seven years and love supporting people from all over Northern Ireland. I am an Occupational Therapist by background, and this healthcare experience has helped me in understanding people's needs and how potential gaps in services can have a detrimental effect on quality of life. I'm looking forward to getting back out to meet the people I help face-to-face in the year ahead.

**Visit www.musculardystrophyuk.org/autumn21 and donate and help us continue providing vital information and support to individuals and families.**

# Making a difference to Jodie

**For Jodie Murphy from Falkirk, the road to diagnosis for her seven-year-old son, Frazer, was long, difficult and, at times, very lonely. But when she reached out to MDUK, her local Information, Advocacy and Support Officer, Jackie, made sure she got the support she needed when she needed it the most.**

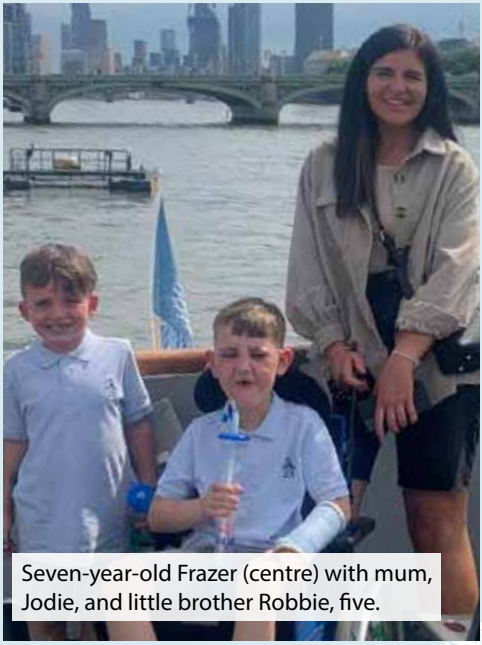
"From the moment Frazer was born, I could tell something wasn't right. As time went on, he struggled to meet his milestones – he couldn't lift his head or pull himself up when he was lying down.

"I went to what seemed like countless appointments with GPs, doctors and neurologists but was told, time and time again, that I was worrying about nothing.

"Finally, in 2019, Frazer was referred to a neuromuscular specialist who rapidly identified a muscle condition but was unable to specify what the condition was. At this point, I started doing my own research online and found MDUK. I emailed Jackie, explaining my situation and, within a week, she'd visited my house to meet Frazer and put me in contact with a neuromuscular advisor.

"From that moment, I felt as though I had the support I'd been missing. It has been a long road to getting a diagnosis for Frazer – it took nearly seven years until finally, in April this year, he was diagnosed with nemaline myopathy. Some days felt almost unbearable, but Jackie was there to support me at hospital appointments and school meetings, bringing her authority and expertise.

"Through MDUK, I was also able to attend Muscle Group meetings and meet other parents of children with neuromuscular conditions. Hearing them speak and knowing I was no longer alone really helped boost my confidence to advocate for my son."



Seven-year-old Frazer (centre) with mum, Jodie, and little brother Robbie, five.

# Get involved

## MDUK shop

Head to the MDUK online shop to discover this year's selection of Christmas cards, gifts and homewares.

With 12 new fun and festive card designs to choose from, as well as stocking fillers starting at just £1, you're sure to find the perfect gift this Christmas!

**Shop online at shop.musculardystrophyuk.org**


## MDUK Raffle

**Congratulations to our Great Muscle Raffle winners, drawn June 2021.**

**1st prize £3,000 Mrs Way, Isleworth**

**2nd prize £250 Mr and Mrs Gerry, Swanage**

**3rd prize £50 Mrs Hughes, Benfleet**

Thank you to everyone who took part and do keep an eye out for the MDUK Christmas Raffle, which will be launching soon. It's such a fun and easy way to support MDUK and you could even win a whopping £3,000!

**From early October, you can enter the MDUK Christmas Raffle by visiting www.musculardystrophyuk.org/raffle**

Roundups

We have partnered with Roundups to give you an easy way to donate vital funds to MDUK as you spend, using your bank card. Through the Roundups app, you can round up your card payments and donate the pennies to MDUK and receive updates on the amazing impact you're making.

You can choose how much you want to roundup your card transactions by – 10p, 50p or £1. You can also set a weekly cap, to keep you in control of how much you donate. Each time your rounded-up donations accumulate to £5, MDUK will receive a £5 donation from your account.

**Visit www.roundups.org/mduk to find out more.**

## Gifts in Wills

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

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

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

**For more information about gifts in Wills, visit: www.musculardystrophyuk.org/giftsinwills or phone 0300 012 0172.**

Friends of
Muscular
Dystrophy UK
Fighting muscle-wasting conditions

## Join for just £15* a year

Our lifestyle magazines sent to your home

Discounts and priority booking on MDUK events

Discounts on specialist holidays

Exclusive offers in the MDUK online shop

# Become a Friend of MDUK

Join us in the fight against muscle-wasting conditions, while enjoying a whole range of great benefits for you and your family.

To join today, go online at **www.musculardystrophyuk.org/friends** or call us on **0300 012 0172**. And as a little thank you for joining, we'll send you an MDUK-branded trolley coin keyring.

*£15 a year when you join by Direct Debit. £18 a year when you join by debit/credit card or cash

**Join or renew before 30 September 2021 and we will automatically enter you into a draw to win a fantastic prize:**
two nights' accommodation for two in a UK/European destination, courtesy of Handiscover accessible breaks!

**(Terms and conditions apply)**