Muscular Dystrophy UK

Fighting muscle-wasting conditions

Campaign

The newsletter for our supporters

Inside:

New clinic roles

Update on the Rixon family

Half a century of royal support

Autumn 2017

Boosting access to Powerchair football

At the end of the first season of the Muscular Dystrophy UK Premiership and Championship, we are delighted with our partnership with the Wheelchair Football Association (WFA).

Powerchair football is the fastest growing disability team sport in the country with over 1,000 people playing on a weekly basis. More than half of the people participating have a muscle-wasting condition.

"We, at Muscular Dystrophy UK, believe that everyone living with a muscle-wasting condition has the right to live a full and independent life. The benefits of participating in sport are clear but sadly there are many practical and financial barriers that can prevent people living with muscular dystrophy from participating. This partnership with the WFA is the first of many steps that the charity will be taking to help to increase access and participation in sport."

Robert Meadowcroft, Chief Executive, MDUK

Welcome

With this autumn edition of *Campaign* you'll see how your support is helping Muscular Dystrophy UK beat muscle-wasting conditions.

You'll read about some exciting research updates, and new projects and resources to help improve quality of life for the individuals and families we support.

After Sian Rixon wrote to you about her little Emmie, we were overwhelmed with your generosity and kind words of support. Thank you ever so much. As you'll see on p4 and 5, Sian and Emmie have been busy keeping active during the summer since Sian's letter.

Enjoy reading this edition of *Campaign*. We're so grateful for the very real difference your support makes to so many lives.

Sue Barker, OBE
President, Muscular Dystrophy UK

PS Get a head start on your festive shopping! Shop online for your Christmas cards, and you'll find there are lots of new gift ideas for you too: www.musculardystrophyuk.org/shop

Our vision

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

Campaign newsletter for supporters of Muscular Dystrophy UK, written and designed entirely in-house.

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On the cover: Jennifer Sutton, part of Muscular Dystrophy UK's new clinic support team

Join us online

Make sure you keep up-to-date by joining our social media networks.

www.facebook.com/musculardystrophyuk
Join the **25,000+ community** on our Facebook page.

@MDUK_News
Keep on top of our breaking news by following us on Twitter.

www.youtube.com/user/MuscularDystrophy
Watch our videos on YouTube.

instagram.com/musculardystrophyuk
Share our pictures on Instagram.

Registered with FUNDRAISING REGULATOR

Update from the Rixons

(l-r) Allan, Finn (6), Emmie (5) and Sian

On behalf of Sian and Emmie Rixon, we'd like to say a big thank you to our supporters for the generosity we've received in response to Sian's recent letter – and the donations are still coming in!

Five-year-old Emmie Rixon and her mum, Sian, have myotonic dystrophy type 1*. When Emmie was born she was weak and floppy, and doctors whisked her away for a series of tests. When the results came back, Sian was shocked to learn that not only did her new-born daughter have a form of muscular dystrophy, but that Sian had it too and had unknowingly passed it on.

At the time Sian wrote her letter, she was awaiting a procedure on her heart called an 'ablation'. Owing to her condition, Sian's heart rate had been very fast, making it difficult for her to do any sort of physical exercise – even walking the family dog was a struggle. Sian has now had the procedure and is pleased to report that her recovery was surprisingly quick – she's now even started running again.

Meanwhile, Emmie has attended her first introductory day at a new school – and is excited to be attending the same school as her big brother Finn, who is six. Emmie was a little shy at first but soon made some new friends.

Emmie absolutely loves the water and Sian and her husband, Allan, are proud to say that she has recently passed her Stage 2 Learn to Swim award which, among other things, means she's able to swim 5 metres on her own. She is now making good progress preparing for Stage 3.

With your support, and the hard work of families like the Rixons, Muscular Dystrophy UK can continue to invest in groundbreaking research to find effective treatments and gain greater knowledge of muscle-wasting conditions. (And on p7, you can read why checks of the heart are so important for people like Sian who have myotonic dystrophy and how advances in research might assist in monitoring symptoms.)

▶ **If you haven't yet read Emmie and Sian's story, please visit: www.musculardystrophyuk.org/Emmie**

***Both Emmie and Sian have this muscle-wasting condition. Emmie has congenital myotonic dystrophy – a form of myotonic dystrophy type 1 where symptoms appear from birth and are usually passed from mother to child in a more severe form.**

Research

Your continued support allows us to fund world-class research, helping us move ever closer to finding potential treatments and cures for muscle-wasting conditions.

Protecting motor neurons from SMA

MDUK-funded researchers have found that improving energy production in certain motor neurons with spinal muscular atrophy (SMA) could potentially protect them from damage. This could lead to the development of new treatments.

SMA affects the motor neurons (the nerves connecting the spinal cord to muscle), causing them to deteriorate and eventually die. However, certain groups of motor neurons escape this while others do not. With support from MDUK and the SMA Trust, Professor Tom Gillingwater and colleagues at the University of Edinburgh investigated why this is the case.

In mouse models with SMA, they found that genes involved with energy production were more active in protected motor neurons than in vulnerable ones. Protected neurons were also more efficient in generating energy.

They then treated a zebrafish model of SMA with an FDA-approved drug called terazosin, which activates a specific gene involved in energy production. Terazosin reduced the number of damaged motor neurons, suggesting it had a protective effect.

This study identified a way to potentially protect motor neurons with SMA. This is encouraging news, though further research is needed before terazosin can be used in people with SMA.

"We are very excited by these encouraging results and hope that our work will help contribute to the ongoing efforts to find successful and effective treatments for SMA and related conditions. We are very grateful to the SMA Trust and Muscular Dystrophy UK for supporting this research."
Professor Tom Gillingwater, University of Edinburgh

Blood biomarker may help to guide heart screening for people with myotonic dystrophy

Clinical research fellow Dr Mark Hamilton, who is based at the University of Glasgow and is funded by MDUK, has found that a particular blood test might be useful for flagging potential heart issues in people with myotonic dystrophy type 1.

Checks of the heart are important for people with myotonic dystrophy because the condition can affect the electrical system regulating the heartbeat and, less commonly, the heart muscle itself.

Doctors generally agree that an annual heart rhythm test should be offered to everyone with myotonic dystrophy. It's less clear if and when additional tests should be recommended, and so practice may vary between different centres.

Cardiac troponin (cTnI) is a heart protein that leaks into the blood if the heart becomes damaged. In this study, Dr Hamilton and colleagues have been investigating whether changes in cTnI levels can give useful information about the heart health of people with myotonic dystrophy attending an outpatient clinic.

One hundred and seventeen people with myotonic dystrophy type 1 participated in the study while attending routine appointments. About 53 percent of the participants had a very low cTnI level and of these, only one had any evidence of heart muscle impairment, which was mild. This suggests that low levels of cTnI may rule out significant problems with the heart muscle.

Though additional research is needed, this study suggests that blood biomarker tests, such as the cTnI test, may be a useful addition to other heart checks for people with myotonic dystrophy. They could also help identify those who should have more detailed heart checks or closer follow-up.

- **Stay up-to-date with the groundbreaking research that you help us fund: www.musculardystrophyuk.org/research**

Here for you: clinic support

Receiving a diagnosis of a muscle-wasting condition can be devastating, leaving a person feeling isolated. With your support, we've been able to invest in a project to offer local support exactly when it's needed.

Our *Here for you: clinic support* project sees a new team of health and social care professionals on our staff working alongside teams in clinics across the UK. Suni Narayan leads the team of four advocacy officers, who offer tailored support in local communities, so that people with muscle-wasting conditions can get access to resources and community support, as well as the best of specialist healthcare.

The advocacy officers offer MDUK services in clinics in Wales, Northern Ireland, Scotland and London, and will soon be joined by roles in Newcastle and Oxford.

▶ **Read more about our work in clinics at: www.musculardystrophyuk.org/clinic-support-team**

Suni Narayan, Head of Clinical Development (who also works clinically as a physiotherapist and care advisor based in University Hospital Southampton NHS Foundation Trust):

"I am leading on this exciting new venture to develop these roles to provide support and advocacy services locally. Over two years our aim is to evaluate the support provided by the advocacy officers and based on the success of this, roll it out to other centres."

Jackie Munro, Advocacy and Information Officer, trained as a social worker and is based in Scotland:

"I will be working with the teams across Scotland, getting to know individuals and their families and offering advocacy support and information about MDUK."

Rebecca Brown, Advocacy and Information Officer, based in Wales:

"My role is unique because it's so varied and I get to help people with problems daily. Often it just helps to know that you are not alone. I have a muscle-wasting condition myself and I remember feeling very isolated and not knowing who to go to for information or support when I was diagnosed. I want to help change that."

Jennifer Sutton, Advocacy and Information Officer, based in London at Great Ormond Street Hospital (GOSH):

"I have experience working directly in health and social services for individuals with disabilities. I will be spending time in clinics at GOSH where I am available to offer advocacy support and information about Muscular Dystrophy UK to help people overcome barriers and to support them by liaising with other services."

Demelza Stuart, Advocacy and Information Officer, is an occupational therapist based in Northern Ireland:

"Muscle-wasting conditions are rare. We know that many people feel isolated because there just aren't the community services available, and some healthcare professionals may not always understand muscle-wasting conditions. An important part of my role is to bridge that gap – provide practical support to families where required and assist with the upskilling of healthcare professionals."

Green light for SMA drug

In April, the European Medicines Agency (EMA) recommended approval for Spinraza as the first-ever treatment for spinal muscular atrophy (SMA).

This approval means Spinraza can be marketed in Europe for children and adults with SMA types 1, 2 and 3.

Spinraza addresses the underlying genetic cause of SMA, by increasing SMN protein (which people with SMA don't have enough of).

The drug manufacturer set up an Expanded Access Programme (EAP) to make the drug available for free to infants with SMA Type 1.

However, the drug is intensive to administer, requiring spinal injections to be given by trained staff and monitoring afterwards. Because of this, there are additional costs associated with the drug and few infants have been able to begin treatment.

NHS England has now agreed to fund these additional costs for SMA Type 1 patients being treated with Spinraza.

We worked with SMA Support UK, SMA Trust and Treat SMA to press for this agreement. It means all eligible infants with SMA Type 1 should now receive treatment through the EAP.

Roisin and Eunan O'Neill's daughter Katie (pictured) was diagnosed at four

months with SMA Type 1, and began treatment last December 2016.

Roisin said: **"We follow many children online who have been on Spinraza longer than Katie and have had more time for the drug to work, and their progress is amazing. This has given us such hope."**

- **Keep up-to-date with campaign news at: www.musculardystrophyuk.org/campaign-news**

Making your house a home

People often tell us of the challenges they face in adapting their homes: thinking of current and future needs, and understanding what is possible.

Our newly published second edition *Adaptations manual: for children and adults living with muscle-wasting conditions* aims to address many of these challenges.

Occupational therapists who work closely with families living with muscle-wasting conditions helped to update the guide.

It is full of examples and practical information about the adaptations process, what new and helpful equipment is available, as well as legislation and policy updates.

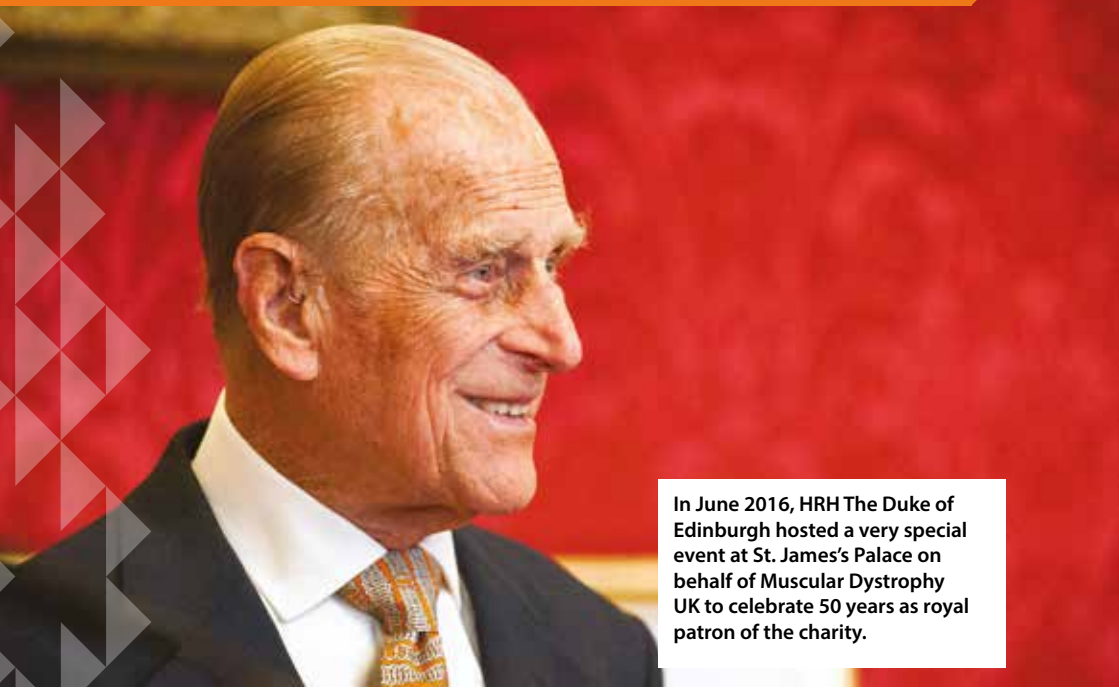
The College of Occupational Therapists reviewed and endorsed the guide, assuring its quality.

"MDUK's Adaptations manual has proven to be an invaluable resource to my family as we prepare to make our home more accessible for our son, Luke, as his needs change."
Claire O'Hanlon, MDUK Regional Development Manager for Northern Ireland, whose son has Duchenne muscular dystrophy.

With your generous support, we are able to create relevant information resources like this to support people with muscle-wasting conditions to live as independently as they wish.

- **To order your copy or to find out more, contact us at info@musculardystrophyuk.org**

Half a century of royal support



In June 2016, HRH The Duke of Edinburgh hosted a very special event at St. James's Palace on behalf of Muscular Dystrophy UK to celebrate 50 years as royal patron of the charity.

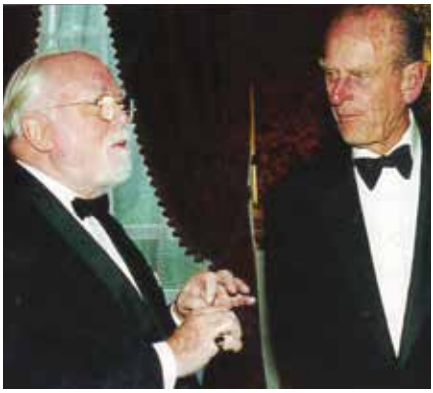
His Royal Highness The Duke of Edinburgh will be retiring from public engagements from autumn this year, Buckingham Palace has announced.

The Duke of Edinburgh is Patron, president or a member of over 780 organisations, including Muscular Dystrophy UK. Although he will no longer play an active role, he will continue to be associated with us.

Since he became Royal Patron in 1966, the Duke of Edinburgh's support has enabled Muscular Dystrophy UK to play a vital role in leading the fight to beat muscle-wasting conditions.

Robert Meadowcroft, Chief Executive of Muscular Dystrophy UK, said:

"His Royal Highness The Prince Philip, Duke of Edinburgh has been our dedicated Patron for over 50 years. We cannot thank him enough for his active support, which has been a precious asset in the fight against muscle-wasting conditions. We also wish him a well-deserved and happy retirement."



Clockwise starting top left:

HRH The Duke of Edinburgh joins delegates from Muscular Dystrophy UK on a lab visit with scientist Professor George Dickson watched by the late Professor Lord John Walton (c. 1980)

Honorary Life President of Muscular Dystrophy UK, the late Lord Richard Attenborough CBE, with HRH The Duke of Edinburgh (c. 2000)

HRH The Duke of Edinburgh with Lord Heyworth and Lord John Walton at the premiere of *The Sand Pebbles* in aid of the charity, at the Metropole Cinema in London (1967)

HRH Duke of Edinburgh hears at first-hand about living as a young person with muscular dystrophy (1970)

Ways to get involved

Christmas card competition

We're getting into the Christmas spirit here at Muscular Dystrophy UK and on 2 October, we'll be launching our annual Christmas card competition!

The Christmas card competition is open to under 18s and we need your entries by midnight on Monday 13 November. So send us your designs and your artwork could feature on one of Muscular Dystrophy UK's Christmas cards next year!

Money raised from the sale of all of our Christmas cards helps us to support the 70,000 individuals and families in the UK who are living with muscle-wasting conditions.

- **For information on how to enter and full terms and conditions, please visit: www.musculardystrophyuk.org/competition**

Last year's Christmas card competition winner Archie Kay (11) with brother Bertie (6)

go orange for a day!

Help make 2 February the brightest day of the year and go orange for a day for MDUK!

Last year over 35,000 people dressed in orange for Muscular Dystrophy UK, raising awareness and funds for research and support.

You can even combine the day with some extra fundraising events! Why not sell some cakes or hold a lunchtime quiz? And don't forget to check out the **#TeamOrange** section of our online shop where you can get everything you need to go orange!

To make sure you're part of this fun event in 2018, sign up your school, workplace or club to 'go orange for a day'.

- **Register for free at www.musculardystrophyuk.org/go-orange**

Cambridge Town and Gown

Whether you're trying to beat your personal best or running for fun with friends, help us turn Cambridge orange on 22 October in this chip-timed run and fundraise for Muscular Dystrophy UK.

Our Cambridge Town and Gown is a closed-road and scenic 10k run where you can embrace the stunning autumnal sights of Cambridge and the green spaces this wonderful city has to offer. There's a Junior 3k for 9- to 15-year-olds too.

Book your place today – it costs just £22 for adults and £12 for juniors.

- **You can register at: www.townandgown10k.com/register** (registration closes midnight Wednesday 20 October)

Shopping that makes a difference

New

We've added some great new products to the MDUK shop! Whether you're treating yourself or a loved one to one of our brand new stripy aprons, a trolley coin keyring or buying this year's Christmas cards, your money helps us support individuals and families with muscle-wasting conditions around the UK.

- **You can order using the enclosed catalogue or view the range and purchase online at: www.musculardystrophyuk.org/shop**

Congratulations to our Spring Raffle winners!

1st prize: £3,000
M Learmonth, Bo'ness
2nd prize: £250
J Usher, Ilkley
3rd prize: £50
N Canton, Southampton

- **Our Christmas Raffle will open in October. If you'd like to receive raffle tickets in the post to buy or sell to family and friends, please contact us on 0300 012 0172 or raffle@musculardystrophyuk.org**

Muscular Dystrophy UK

Fighting muscle-wasting conditions

Weekly Lottery

Play our Weekly Lottery and you could win £10,000!

Here's an exciting new way for you to join the fight against muscle-wasting conditions and help people like Luke – play the MDUK Weekly Lottery!

Playing is easy – from just £1 a week you can have the chance to win some great cash prizes from £5 to £1,000 – and a whopping first prize of £10,000!

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

Luke (4) who has Duchenne muscular dystrophy