Muscular Dystrophy UK

Fighting muscle-wasting conditions

Campaign

The newsletter for our supporters

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Increasing clinical trial capacity

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Meet the care and support team

Spring 2016

Our new ambassador in Scotland

Gordon Smith, football pundit, former football player and Scottish FA chief, has joined Muscular Dystrophy UK as an official charity ambassador. A long-standing supporter of the charity in Scotland, Gordon was moved to get involved as an Ambassador after he met 11-year-old Rangers fan, Robbie Martin (pictured below with Gordon), from Lanark. Robbie has Duchenne muscular dystrophy.

"It's been a privilege to meet young people like Robbie. I feel honoured to become an Ambassador, and I hope my involvement will help raise awareness and make a difference in fighting these conditions, which are affecting many lives today," said Gordon.

"We think it's absolutely fantastic Gordon is joining the charity as an ambassador. He's a kind and genuine person, who has really taken the time to get to know Robbie. Gordon can do so much to help make sure more people know about muscle-wasting conditions," said Barbara, Robbie's mum.

Welcome

Welcome to this spring edition of *Campaign*. You'll read about the exciting developments and events across the UK to beat muscle-wasting conditions.

The research projects that the charity funds are hugely important in the fight to beat these devastating conditions. Clinical trials are vital to this research, so it's interesting to read how the charity works in partnership to build clinical trial capacity in the UK.

You'll see how your support enables Muscular Dystrophy UK to ensure the UK infrastructure for clinical trials works effectively. It also enables the charity's frontline staff to be there for people needing to talk, or to find out more about their muscle-wasting conditions.

I hope you enjoy reading *Campaign*, and seeing how your generosity helps Muscular Dystrophy UK's programmes of support. Thank you from me and from everyone at the charity for the huge, positive difference you make.

Sue Barker, OBE
President, Muscular Dystrophy UK

PS Thinking of taking on a challenge in the summer? You'll find lots of events to choose from at **www.musculardystrophyuk.org/events**

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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Join us online

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

www.facebook.com/musculardystrophyuk
Join the **20,000+ community** on our Facebook page.

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

www.youtube.com/c/musculardystrophyuk
Watch our videos on YouTube.

instagram.com/musculardystrophyuk
Share our pictures on Instagram.

On the cover: Archie Hill (10), who is on a clinical trial for Duchenne drug, Translarna

Thanks from the Twines

(l to r) Josh, Mia and Ethan Twine

On behalf of the Twine family, we'd like to say a huge thank you for your generous response to Charmaine's Christmas letter. Over 1,220 of you responded with gifts totalling more than £33,234.

Your generosity could help fund pioneering research, and could enable families to meet together for peer support and advice. All of that means the world to Charmaine and her family.

"Ever since Josh and Ethan were diagnosed, Muscular Dystrophy UK has been just a phone call away. As I've struggled to navigate the healthcare bureaucracy, trying to do what's best for my boys, I can't tell you the difference it has made to have someone on my side, who really knows the system, and cares about me," said Charmaine.

"I also know that scientists are making real progress towards treatments and cures for muscular dystrophy. I want to do all I can to help.

"I am so pleased our story managed to raise that money. I wish life was different, but it's not so I'm pleased we could make a difference. Thank you," said Charmaine.

Charmaine and husband, Alex, have three children – Josh (8), Ethan (6) and Mia (3). Josh and Ethan both have Duchenne muscular dystrophy, a devastating muscle-wasting condition that causes their muscles to weaken and waste over time, leading to increasing disability.

With your support, Muscular Dystrophy UK can continue to support families like the Twines to live as independently as possible and enjoy the best quality of life.

Together, we can beat muscle-wasting conditions more quickly. If you haven't had the chance, there's still time to respond to Charmaine's letter. Please visit: **www.musculardystrophyuk.org/twine**

Cancer drug shows promise for Duchenne

A drug commonly used to treat leukaemia is showing potential as a treatment that could slow the progression of Duchenne muscular dystrophy.

This potential has been investigated in an innovative research project at the University of Sheffield, led by Muscular Dystrophy UK-funded researcher, Professor Steve Winder. It is your generous support that has enabled Muscular Dystrophy UK to fund this project, with joint funding from the Medical Research Council and the Duchenne Parent Project NL.

Professor Winder's team found that a drug called *dasatinib*, which works by blocking certain chemical signals that stimulate the growth of cancer cells, would also switch off similar signals in a protein implicated in Duchenne muscular dystrophy. This protein, called *dystroglycan*, has a part to play in maintaining healthy muscle tissue.

The team tested the drug in zebrafish bred to carry Duchenne muscular dystrophy, and recorded a 40 percent improvement in the condition of the fish. Those treated with *dasatinib* were able to swim further and for longer than those in a control group.

Combining the drug with other treatments currently under development could improve their effectiveness even further. The results have been published in the journal *Human Molecular Genetics*.

"*Dasatinib* clearly has promise as a treatment for Duchenne muscular dystrophy," said Professor Winder.

"From our understanding of how the drug works, we believe it could be effective in slowing muscle deterioration, prolonging patients' ability to walk and also protecting their heart and respiratory muscles.

"There is the potential that if the drug were taken immediately upon diagnosis, the disease progression could be dramatically reduced."

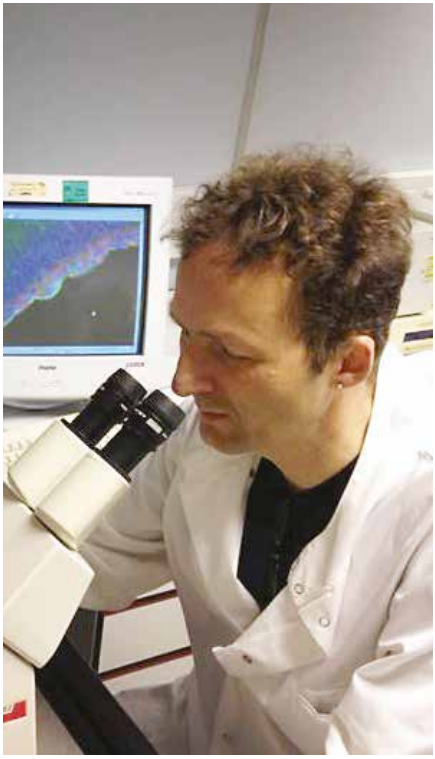
Because *dasatinib* is already cleared for clinical use, researchers hope that progress can be made more quickly towards trialling the drug in humans.

Experiments have already begun in mice, with promising results. Professor Winder's team is also investigating other drugs that work in a similar way to *dasatinib*.

With your support, Muscular Dystrophy UK is able to fund a wide range of research projects like this, from basic science to translational research (moving promising technology into clinic) and clinical pilot studies.

Keep up-to-date with news of the research your support is helping fund at **www.musculardystrophyuk.org/research**

Duchenne muscular dystrophy is a life-shortening muscle-wasting condition, caused by the lack of a vital muscle protein called dystrophin. The condition affects mainly boys. It causes muscles to weaken and waste over time, leading to increasing and severe disability. It currently affects around 2,500 people in the UK. About 100 babies in the UK each year are born with the condition, and few currently live to see their 30th birthday.



Professor Steve Winder

Paving the way for new clinical trials

Archie Hill (10), who is on a clinical trial for Duchenne drug, Translarna

Earlier last year, Muscular Dystrophy UK joined forces with a number of Duchenne charities, as well as Great Ormond Street Hospital (GOSH) charity, as part of the 'Newcastle plan' initiative. This followed concerns from clinicians and patient groups that trials were being turned down owing to a lack of capacity and a need for more specialists in post.

As a result, a major investment of over £1.2m was announced in December, to fund 18 key roles at three neuromuscular Centres of Excellence. Muscular Dystrophy UK's initial contribution is set at £361,000 over the coming two years and will fund four of the new roles across these centres.

Muscular Dystrophy UK's is funding the following roles:

- in Liverpool, a clinical trial co-ordinator at Alder Hey Children's Hospital
- in Newcastle, a part-time research physiotherapist at the Newcastle Muscle Centre
- in London, two senior research roles at GOSH.

This builds on Muscular Dystrophy UK's existing commitment in Newcastle and London, where we fund two clinical trial co-ordinator posts, as well as the North Star natural history database for Duchenne muscular dystrophy and spinal muscular atrophy (SMA).

Professor Kate Bushby, from the John Walton Muscular Dystrophy Research Centre at Newcastle University, said, "I am delighted that we have already received such tangible results in such a short space of time and hope that these posts will be the start of building real and sustained trial capacity, and will help to keep the UK at the forefront of exciting trials coming through for Duchenne muscular dystrophy."

In addition to working closely with other charities in establishing the need for these posts, Muscular Dystrophy UK set up an expert group to run a UK-wide audit to investigate clinical trial capacity in the UK. Chaired by Professor Martin Bobrow, the audit group helped to guide Muscular Dystrophy UK in making this significant investment at these three centres.

Manoj Thakrar, who together with his wife Sejal set up the Smile with Shiv Family Fund after their son Shiv was diagnosed with Duchenne muscular dystrophy, said, "It's really encouraging that Duchenne charities and families like mine can work towards one clear goal and mission. A significant investment has now been made to improve clinical trial capacity, which will help ensure more trials can start and more patients can be recruited.

"As a member of the clinical trial audit group, and from what has been discovered and highlighted in work done in the past by all the charities, I've seen the challenges facing many centres. It's clear that we need to continue pushing to ensure those neuromuscular specialists have the resources they need."

Thank you to all our supporters for making this investment possible and helping us to provide these real steps closer to treatments.

- **Read more about clinical trials, and the research projects you are helping fund: www.musculardystrophyuk.org/research/current-grants**

- **Keep abreast of breaking news in research at: www.musculardystrophyuk.org/breaking-research-news**

Meet the team on the front line

When you're living with a muscle-wasting condition, it's vital to have access to the right information and advice. With your support, Muscular Dystrophy UK is able to be here for people at their point of need, with a specialist helpline and free information and advice.

Manjula Gohil got in touch with the charity when Vivek was diagnosed with Duchenne muscular dystrophy.

"I've learned so much about Duchenne muscular dystrophy and how to manage life with it. MDUK has given me tools to cope with many situations I've faced, in a positive and constructive way."

Every day, people like Manjula call Muscular Dystrophy UK. The helpline team knows what it means to be the first port of call for people in need of vital information. Not only do they offer support and advice, but they also love what they do.

"It's a pleasure to help different people every day. Hearing everyone's story and letting them know they aren't alone is the best part," says Maddy Rees, Care, Information and Advocacy Manager.

The team across the UK offers support to people at their point of need. They organise and run Muscle Groups, information events, and refer people to our network of peer support volunteers or advocacy ambassadors. Talking to others who understand what you are going through can really help.

"We can offer support in applying for benefits, or provide information on housing, for example. We also have some great networks to put people in touch with others with their condition," says Joel Rackham, Advocacy Development Officer.

In the past year the team responded to **2,900** emails, **236** advocacy cases, and **480** queries through social media. More than **25,000** information factsheets were downloaded from our website.

"We send out information factsheets in response to what people call us about, and provide information about peer support volunteers and care advisors.

"We're here to listen and to give individuals and families access to the resources and information they need and are entitled to," says Emilie Shore, Information and Engagement Officer.

"We're here to help people who have questions about research, practical and financial support. As an example, I recently helped someone with a rare form of muscular dystrophy finally move into a house that would suit her needs – a real home in which she could move about independently," said Shivani Handa, Senior Campaigns, Advocacy and Information Officer.

Demelza Stuart, Advocacy and Information Officer/Occupational Therapist in Northern Ireland, supports people on a range of issues such as housing and adaptations, financial support and benefits, and enquiries about local support.

Rebecca Brown, Advocacy and Information Officer for Wales, gives people information about their muscle-wasting conditions, and offers help with benefits and housing adaptations.

"People tell us it helps knowing someone is there who understands, and can help with issues they are facing," says Rebecca.

Mariana Pierotti, Scotland Advocacy and Information Officer, often speaks to people who are looking not only for advice but also for someone to talk to.

"People like to know the charity is part of their support network. We can also give them information about how to manage living with a muscle-wasting condition," says Mariana.

Your valued support ensures our frontline team is here for those who want to find out more about a muscle-wasting condition and the practicalities of living with it.

Here for you:
0800 652 6352 (Freephone helpline, open 8.30am to 6pm, Monday to Friday)
info@musculardystrophyuk.org

Creating the best tomorrow

Lilian Pegg, pictured with husband, George, and sons George (l) and John

Our work to beat muscle-wasting conditions relies almost entirely on voluntary donations. And more than a quarter of our research projects are made possible by gifts in Wills to Muscular Dystrophy UK.

Writing a Will is an important step in planning for the future. It does not have to be expensive or complicated. With a Will in place, you'll have peace of mind knowing you are in full control of who will inherit your belongings. Everyone will know what your wishes are, and that you've looked after the people and causes closest to your heart.

"Muscular Dystrophy UK's dedication to funding research to find treatments and cures gives us real hope for George's future," says Lilian Pegg, whose son George has Duchenne muscular dystrophy.

When you choose to leave a gift in your Will to Muscular Dystrophy UK, you'll be creating the best tomorrow for everyone in the UK born with a muscle-wasting condition.

Dr Huw Thomas from Aberystwyth passed away in October 2014. He became involved with the charity when he was diagnosed with limb girdle muscular dystrophy, and was a long-standing supporter and active member of Aberystwyth Branch. Huw generously left a share of his estate in his Will to Muscular Dystrophy UK and a further share to Aberystwyth Branch, to help the vital work of the charity to continue.

We are enormously indebted to Huw, and to everyone who leaves a gift in their Will to Muscular Dystrophy UK.

A wonderful gift of £100 enabled us to provide a family access to a specialist physiotherapist who showed them valuable muscle-stretching techniques to keep their child walking for longer. A gift of £5,000 enabled us to fund a full month of world-class research to find treatments and cures for muscle-wasting conditions.

Please call Catriona Parker for a confidential chat, or to order a copy of our new booklet: *Create the best tomorrow: a guide to leaving a gift in your Will.*

You can reach Catriona on 020 7803 4834 or legacy@musculardystrophyuk.org

Shop with us

The winner of our Christmas card competition

A huge thank you to everyone who entered our Christmas card design competition. With some truly fantastic entries, our Chief Executive, Robert Meadowcroft, had a very difficult choice to make in deciding a winner. However, he was very pleased to announce that nine-year-old Scarlett Parker's entry, *Family Scene*, won the competition – congratulations to Scarlett!

Keep an eye out for Scarlett's card in our 2016 Christmas card range, available from August.

Non-stop shop

We are excited to announce that you'll now be able to show your support of Muscular Dystrophy UK all year round, with our extended range of branded items available for you to buy.

New items include water bottles, notelets and T-shirts. Every time you purchase any of these products, you'll be helping beat muscle-wasting conditions.

Treat yourself or a loved one to some of these new items today. You can use the order form in the enclosed catalogue or view the range and purchase online at: **www.musculardystrophyuk.org/shop**

go orange for a day!

On Friday 22 January, people across the UK made the day a bright orange one. Taking part in the charity's first 'Go orange for a day' awareness and fundraising event, more than 15,000 people turned their offices and schools orange. Together they raised £16,000, which will fund research and support for people with muscle-wasting conditions.

If you'd like your school, company or club to 'Go orange for a day' on Friday 27 January 2017, register at **www.musculardystrophyuk.org/events/go-orange**

Run in our 35th Oxford Town and Gown 10k

This year marks the 35th anniversary of our chip-timed, closed-road 10k Oxford race. Join **#TeamOrange** on **Sunday 15 May** and run through the streets of Oxford city centre, finishing in the beautiful University Parks. There's also a Junior 3k Run.

- **Get your place today – it costs just £25. www.townandgown10k.com/tgoxford**

- **Find out more at www.musculardystrophyuk.org/events or email events@musculardystrophyuk.org**

Muscular Dystrophy UK

Fighting muscle-wasting conditions

"Keeping fit is something that is very important to me and that is why I am championing the charity's Move a Mile for Muscles summer fundraising campaign and I want YOU to get involved.

"There are so many ways you can Move a Mile – from swimming in your local pool, doing a mile in a wheelchair, or you could try something completely out of the ordinary."

Jack Wilshere, Arsenal and England footballer, Muscular Dystrophy UK Ambassador

Join us between May and September, and help fund research and support for families living with muscle-wasting conditions.

Move a Mile for Muscles

FRSB FundRaising Standards Board

www.musculardystrophyuk.org.uk/move-a-mile

020 7803 2884 / events@musculardystrophyuk.org / @MDUK_News

#MusclesMatter