

**Muscular
Dystrophy UK**

Fighting muscle-wasting conditions



Campaign

The newsletter for our supporters



Inside:
Here to explain the science
Update on the Gauder family
Fast Track to treatments

Spring 2018

BGC Charity Day

Last year on 11 September, global stockbrokers, BGC Partners held their 13th Annual Charity Day – one of the biggest events in the charity calendar. This annual event takes place in memory of the BGC staff who lost their lives during 9/11, and all money raised is donated to charities.

Last year, we were delighted that Vice President of MDUK Sir Alex Ferguson, Sam Allardyce and Sol Campbell represented us at the event at BGC's London office in Canary Wharf. The football stars visited the trading floor, and got involved in some trading themselves by speaking to clients over the phone — all to raise money for Muscular Dystrophy UK.

We'll hear the total amount raised by BGC Charity Day 2017 later this year, and we'll be sure to keep you posted.



Former Manchester United FC manager, Sir Alex Ferguson CBE



Former England international footballer, Sol Campbell



Families living with muscle-wasting conditions also attended this exciting day and got to meet the football stars



Everton FC manager, Sam Allardyce

Welcome

Welcome to this spring edition of *Campaign*, the newsletter that shows you how vital your support is.

You'll read how our new Clinical Trials Information Service is helping to answer questions for families who are considering taking part in clinical trials. And when you read how we fund world-leading research projects, you'll see how determined we are to beat muscle-wasting conditions more quickly.

You may remember hearing from the Gauder family before Christmas last year. We've had a terrific response to Emma's letter about her son Jack and on page 4 and 5 you'll read what they've been up to since then.



Sue Barker

Sue Barker OBE

President, Muscular Dystrophy UK

PS Please help us make our newsletter even better and tell us what you think of *Campaign*! Just complete the short questionnaire enclosed.

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.

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

020 7803 4800
info@muscular dystrophyuk.org
www.muscular dystrophyuk.org

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

DMN/1804

On the cover: Abbi Bennett and her Dad, Gary, supporting MDUK's campaign for fast access to specialist treatments

Join us online

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



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



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



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



[instagram.com/muscular dystrophyuk](https://www.instagram.com/muscular dystrophyuk)
Share our pictures on Instagram.



Registered with
**FUNDRAISING
REGULATOR**



Update from the Gauders



The Gauder family: Jack (7), Emma, Matt and Harrison (2)

On behalf of the Gauder family, we'd like to say a big thank you to you, our supporters, for the generosity you've shown in response to Emma's recent letter. You've sent in over 1,000 donations to help us support individuals and families just like Jack's – and gifts are still coming in!

When Emma wrote to you, the family had just learnt that Jack had been accepted on to a clinical trial. This is to test an exon skipping drug – a potential type of therapy for Duchenne muscular dystrophy.

Jack has since started this trial and we're pleased to let you know that it's going well. His mum Emma tells us that he's been really getting into the swing of things and is enjoying the weekly trips to Great Ormond Street Hospital in London.

And there are more changes happening in the Gauder home. As Emma mentioned in her letter, she'd contacted the Muscular Dystrophy UK helpline for information about adapting their home for Jack. Our team had helped in discussions with the council.

We're pleased to report that with their advice, the family is now adding a downstairs bedroom and bathroom especially for Jack. Jack's condition makes climbing stairs difficult, so this will make things much easier for him.

Emma says,
"There are still builders everywhere but hopefully by spring it might even be finished. And then Jack can start thinking about how he'd like to decorate his new bedroom!"

Your support enables us to continue supporting families just like Jack's on a range of areas from housing adaptations to helping them find the information they need to decide whether to take part in a clinical trial.

And on page 8, you can read about Muscular Dystrophy UK's new Clinical Trials Information Service.

- ▶ **If you haven't read Jack's story, you can do so here:**
www.muscular dystrophyuk.org/christmas



Jack during one of his weekly visits to Great Ormond Street Hospital for his clinical trial

How we fund research

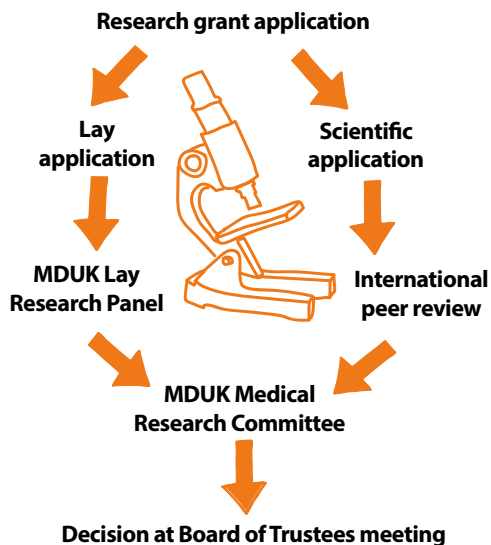
Thanks to the generosity of our supporters, Muscular Dystrophy UK is able to fund a number of world-leading research projects every year.

All potential projects go through a rigorous process to ensure we're funding the best-quality science relevant for people with muscle-wasting conditions.

The selection process

Once a year, scientists in the neuromuscular field apply for research grants. There are two parts to the application:

- 1. a scientific application:** a detailed, scientific description of the project. We send this to experts all over the world who review the applications for their scientific value.
- 2. a lay application:** this goes to our Lay Research Panel (most members are living with muscle-wasting conditions) to assess the project's relevance to people living with the condition. It's written so that people who aren't scientists can understand the research.

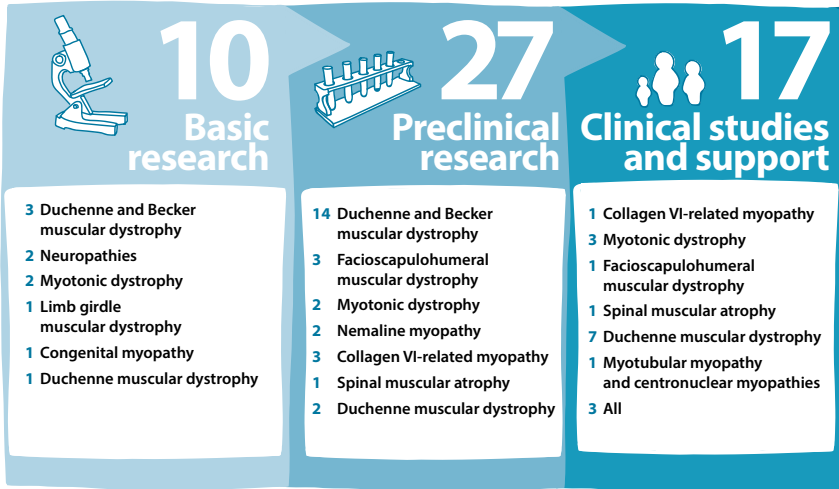


Our Medical Research Committee then considers the applications, taking into account the scientific and lay feedback. There are 10 – 12 scientists and clinicians, as well as two members of the Lay Research Panel on this committee.

The Committee meets once a year to discuss and score the applications. These scores form part of the final recommendations to the Board of Trustees about which projects Muscular Dystrophy UK should fund.

- ▶ **For more information about this process, please email research@muscardystrophyuk.org**

Muscular Dystrophy UK funds a range of research projects into potential treatments and cures for muscle-wasting conditions – all made possible by your generosity:



These numbers represent all active grants as at October 2017.

Basic research focuses mainly on understanding the cause of a condition and how this leads to muscle weakness and wasting. Researchers study cells and animal models in order to answer a range of research questions. Once they have these answers, they can then investigate potential treatments.

Preclinical research covers all the experiments to assess the safety and effectiveness of a potential treatment before testing it in human (clinical) trials. Again, this involves testing in cells and animal models. Scientists also look carefully for any potentially harmful side-effects of the treatment.

Clinical studies cover any form of research involving people. This includes understanding more about how a muscle-wasting condition is progressing, and assessing the safety and effectiveness of potential treatments in people. Clinical trial co-ordinators and patient registries need to be in place before clinical studies can happen.

Even if a new treatment gets through the lengthy process of clinical trials, there is still the final hurdle of making it available to patients. Through our **FastTrack campaign**, we are working hard to ensure that people living with muscle-wasting conditions in the UK get access to promising treatments, and as quickly as possible.

Here to explain the science



Dr Sofia Nnorom, Clinical Research Officer at Muscular Dystrophy UK

As more research takes place and an increasing number of therapies become available, more people are considering taking part in clinical trials.

It's not only a tough decision to make, but wading through all the information and terminology can also be overwhelming. For this reason, MDUK set up a new Clinical Trials Information Service at the end of 2017. Dr Sofia Nnorom manages the service, and she – along with her colleagues in the research team – is here to help you understand the science.

“Often I get enquiries about what therapies or trials are available for particular conditions. I’ll help by trying to explain the information in a way that is digestible,” says Sofia.

“Sometimes people will feel overwhelmed with the information their clinician has given them about a clinical trial. I can help develop questions that they can ask their clinicians. I know it’s a big decision to take part in a clinical trial, so we’re here to offer a relaxed, informal environment to answer questions and help people make informed decisions.”

After doing her PhD in gene therapy, Sofia worked as a research scientist for seven years before joining MDUK in 2017 as a Clinical Research Officer.

"I've always enjoyed explaining how a drug works. Many people have questions about the science, perhaps wanting to know about how the drug works, for example. We also offer unbiased information on current clinical trials."

Here's how the clinical trials information service is here for you:

- ▶ the research team will answer your science- or research-related questions – over the phone or by email
- ▶ on our website, you can learn more about clinical trials or use the clinical trials finder tool to find trials that are relevant
- ▶ we'll keep you updated about progress in international research into neuromuscular conditions.

"We're here to answer any research or science-related questions. There is no such thing as a silly question – if it's bothering you, please ask us."

The Clinical Trials Information Service is just one of the ways Muscular Dystrophy UK supports people with muscle-wasting conditions to improve their quality of life and live more independently.

So whether someone wants to find out more about a condition, or help accessing equipment or the financial assistance they're entitled to, our specialist helpline and free information are here for individuals and families living with muscle-wasting conditions.

In 2016-17, we gave information and support to someone in need **every 10 minutes**

During the past year (2016-17) we've received **56,000 requests** for support and information including by phone, email, via our website and through our online forum – two-thirds more than in the previous year

- ▶ **If you'd like more information about clinical trials, do get in touch with Sofia and the research team on 020 7803 4813 or research@muscular dystrophyuk.org**

FastTrack to treatments

For people with muscle-wasting conditions, every day counts. That is why we launched our FastTrack campaign, in October 2017. It focuses on removing the barriers that stop people with muscle-wasting conditions getting access to new treatments quickly.

This is an encouraging time as there are many clinical trials in development, and several treatments are either already available or emerging on the horizon. The first one, Translarna, a drug to treat Duchenne muscular dystrophy, is now approved across the UK.

However, Exondys 51, another drug to treat Duchenne, and Spinraza, a treatment for spinal muscular atrophy, are still awaiting approval in the UK.

As part of FastTrack, we've supported a petition by Claire Liggett, mum to eight-year-old Charlie who has Duchenne muscular dystrophy. They are asking for the Prime Minister's support in speeding up access to treatments for muscle-wasting conditions.

The petition has now received over 20,500 signatures and gained the support of their local MP, Julian Lewis.

Earlier this year, families came to Parliament to meet with over 20 MPs from across the political spectrum. The families had a chance



Claire Liggett and her son Charlie meeting their MP, Julian Lewis, at Parliament at the end of 2017

to tell MPs why faster access to treatments was important to them.

As part of MDUK's FastTrack to treatments campaign we work with health bodies and pharmaceutical companies to identify and remove barriers to accessing new and effective treatments for muscle-wasting conditions. We're also committed to making sure the voice of those living with muscle-wasting conditions is heard. With progressive muscle-wasting conditions, it's important patients get access to treatments as quickly as possible, while they can still benefit from them.

- ▶ **Find out more about the FastTrack campaign and how you can get involved here:** www.musculardystrophyuk.org/fast-track/



Josh Langley meets Vince Cable MP at Parliament



Abbi Bennett and her Dad, Gary, show support for the campaign at our National Conference



Lucy Frost and her son, George, at our Parliamentary event

Improving independence and quality of life

At Muscular Dystrophy UK, we want to make every day count for everyone with muscle-wasting conditions. That's why we're co-chairing the Changing Places Consortium, giving young people the space to talk about issues that are important to them, and sponsoring Powerchair Football in Scotland and England.

MDUK co-chairs Changing Places Consortium

In November 2017, MDUK took on a leading role campaigning for fully accessible toilets across England, Wales and Northern Ireland. As new co-chair of the Changing Places Consortium, along with PAMIS who lead this activity in Scotland, we want to ensure people with severe disabilities can use public toilets safely and with dignity.

More than a quarter of a million severely disabled people UK-wide need access to Changing Places toilets. Without these, disabled people either can't go out or have to get changed on a toilet floor. These are not reasonable options.

What is a Changing Places toilet?

Larger than a standard accessible toilet, a Changing Places toilet has an adult-sized height-adjustable changing bench, a toilet designed

for assisted use, and a hoist. It is about the size of a single car parking space.

By creating greater public awareness, MDUK wants to press for legislation to make these toilets mandatory in new public buildings, and to double the number of registered Changing Places toilets UK-wide.

To find out more about how we're helping lead the campaign for fully accessible toilets, please visit: www.musculardystrophyuk.org/changing-places-consortium

MDUK launches well-being camps

Young people with muscle-wasting conditions don't always have the opportunity to talk about issues that affect them as they move into adulthood. MDUK's 'Here for You: Mental Health Matters' initiative has been made possible thanks to your support and as part of this, we'll soon be holding two residential well-being camps for young people with muscle-wasting conditions.

This is the start of a new pilot project supported by the Bupa UK Foundation.

The purpose of these well-being camps is to provide a relaxed and comfortable environment for young disabled people to have these discussions.

Run in collaboration with the Robert Jones and Agnes Hunt Orthopaedic Hospital, the well-being camps will be open to anyone aged 18-25 who has a muscle-wasting condition and attends the hospital.

We hope this pilot project proves a success and will be the first of a number of well-being camps for young people.

- ▶ **To find out more about our 'Mental Health Matters' project, visit: www.muscular dystrophyuk.org/mental-health-matters**

Supporting the beautiful game in Scotland

Thanks to your support, Muscular Dystrophy UK now sponsors the Scottish Powerchair Premiership for the 2017/18 season. After sponsoring the Premiership and Championship of the English Powerchair football leagues in 2016/17, this new development underlines our commitment to the fastest-growing disability team sport in the UK. The sponsorship includes funding to develop new teams in Scotland.

“We’re delighted to receive this sponsorship, which is vital for the running and growth of Powerchair football in Scotland. The sponsorship will benefit the Premiership immensely and the development grant is so important to help more people access Powerchair football and enjoy the physical and physiological benefits of team sport.”

Garry Johnstone, Scotland Powerchair Football National Team Coach and former Chair of the Scottish Powerchair Football Association

- ▶ **To find out more visit: www.muscular dystrophyuk.org/powerchair-football**



Around 1,200 people play Powerchair football each week in the UK

Ways to get involved

Enter the Great Muscle Raffle today!

Our Great Muscle Raffle 2018 is now open and by buying tickets you could be in with a chance to win an amazing first prize of £3,000!

Taking part is a fun way to support individuals and families across the UK who are living with muscle-wasting conditions.

- ▶ **Entering couldn't be easier – simply call our Raffle Hotline on 01628 201 289 or buy your tickets online at www.musculardystrophyuk.org/raffle**



Congratulations to our Christmas Raffle winners!

- ▶ **First prize:**
Ms Wells, Colchester
- ▶ **Second prize:**
Miss Scholz, Salisbury
- ▶ **Third prize:**
Mr Cockings, Southampton

And thank you to everyone who took part.

Spring catalogue out now!

Step into spring with our latest catalogue! It's full of great products and some fun new items that'll be sure to tempt you, including our new MDUK-branded Rubik's Cube keyring.

When you buy any item from our shop, all the proceeds go directly towards beating muscle-wasting conditions.



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



Tell us what you think about Campaign

We'd really appreciate it if you could answer our short questionnaire to help us make *Campaign* even better.

You don't need to include your name and we'll keep your feedback confidential, and use it to fill *Campaign* with the kinds of things that you want to read.

You can do this by:

- ▶ **completing our questionnaire (enclosed inside this edition of *Campaign*) and posting it back to us using the Freepost envelope provided**
- ▶ **visiting www.muscular dystrophyuk.org/YourThoughts to complete the online version of our questionnaire.**

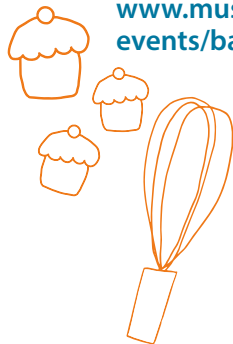
Bake a difference



Get your apron on and unleash your inner star baker this October by taking part in Bake a Difference!

You can host a bake sale at your school, office or home and the money you raise will help us continue to fund groundbreaking research and support those with muscle-wasting conditions in the UK.

- ▶ **To sign up to 'Bake a Difference' or for more information, please visit: www.muscular dystrophyuk.org/events/bake-a-difference**



Get your own MDUK apron for just £12.99 by visiting our shop at: www.muscular dystrophyuk.org/shop



**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.muscular dystrophyuk.org/lottery
or by phoning our Weekly Lottery hotline on
01628 821 983 (Mon-Fri, 9am-5pm)



Luke (4) who has Duchenne muscular dystrophy


**£10,000
JACKPOT**