Muscular Dystrophy UK's lifestyle magazine

Issue 1 – 2017

Target MD

Our new commitments to you

Investing £4.5m to accelerate clinical trials and drive better care and support

Meet our new Chair and Trustees

Target Research: news and updates

Muscular Dystrophy UK
Fighting muscle-wasting conditions

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The magazine for supporters of Muscular Dystrophy UK, written and produced entirely in-house.

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On the cover
Lilian and George Pegg with their sons, George (l, who has Duchenne muscular dystrophy) and John. Our new commitments are designed to boost clinical trials and improve overall care and support for families like the Peggs.

Hello

Hello and welcome to *Target MD* in 2017. It's a slightly different focus this time - with stories of some new commitments for the next few years. Our CEO, Robert Meadowcroft, tells you more about our strategic focus for these on page 4.

We also introduce you to some outstanding people, including our new Chair, Professor Mike Hanna, new Trustees Louisa Hill and Charles Scott, and Professor Alan Emery. You'll also read about some new opportunities for you to get involved in MDUK in the year ahead.

At MDUK, we are dedicated to support people with muscle-wasting conditions to live independently, and to have the best quality of life. We are constantly developing new services and resources to help do that, and we share some of those with you on page 29.

You may also wish to keep updated with new developments in the fight against muscle-wasting conditions, so please sign up to receive our regular eNewsletters. You can so on our website, or by emailing info@musculardystrophyuk.org

Have a look on p30 and see what #TeamOrange events are coming up. If you'd like to take part in any way, our events teams would love to welcome you. They always do a great job of supporting you too.

As always, please keep in touch and tell us what you'd like to read about in future editions. We want to bring you the magazine you want to read.

I'd love to hear from you.

Ruth

Ruth Martin, Editor, Target MD

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About us

Muscular Dystrophy UK supports 70,000 children and adults with muscle-wasting conditions to live as independently as possible. We accelerate the pace in development of effective treatments and cures.

Helpline

If you'd like to speak to someone about living with a muscle-wasting condition, please call our friendly care and support team.

They are available from 8.30am to 6pm Monday to Friday, on **0800 652 6352** (Freephone helpline) or **info@musculardystrophyuk.org**

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Photo © Anne-Marie Briscombe

Thank you for your ongoing support

Everything we do, as you'll read in the pages ahead, relies on the support of generous people like you. People who understand what it will take to beat muscle-wasting conditions.

Join us – we can do this, together.

Please contact our fundraising team to find out about all the ways you can get involved. Call **0300 012 0172** or email **fundraising@musculardystrophyuk.org**

Faster access to treatments, better care and support

As we move into April and thoughts turn to warmer days, all of us at the charity are keen to accelerate the steps being taken to secure faster access to treatments, better care and support for independent living.

There is encouraging progress in emerging treatments including the first licensed therapy to prevent inherited mitochondrial disease and also Translarna as the first available drug in the UK to treat eligible boys with Duchenne. These developments spur us on in raising the funds needed for further developments and support.

Indeed, our announcement of three large research programme awards and two other major initiatives reflects the altruism of those who generously left us legacies, as well as the commitment of our supporters in raising funds throughout the year. You can read about our new awards and the new programmes in the following pages.

You will also read about Professor Mike Hanna, the new Chair of Muscular Dystrophy UK, as well as Louisa Hill and Charles Scott who have taken on the roles of Trustee to oversee and guide the work of the charity. All three are making an excellent contribution, and Louisa as a mother of two boys (one of whom has Duchenne), brings her experience of the challenges of the diagnosis, attending hospital appointments and coping with the impact on the whole family. Like Charles, Louisa is also an amazing fundraiser working tirelessly in backing our Duchenne Research Breakthrough Fund. The outstanding contribution made to our field by Professor Alan Emery is also highlighted in this issue.

I started this piece by looking forward to the prospect of warmer days in the coming months. In looking at the promise of emerging treatments currently in trials and with the regulators, I have to say a cloud has appeared on the horizon. This is the policy change announced by NICE to impose a ceiling on the cost of new drugs, with the intention to limit the budgetary impact on the NHS.

We appreciate that drugs for rare diseases can be costly and, as further drugs are approved, the overall cost will be significant. However, it is essential a solution is found that does not delay or restrict access to treatments for muscle-wasting conditions. Our voice is strong when we act together and we will let you know of opportunities to support our campaign to ensure fast access to treatments. It is essential that potentially life-saving treatments are not blocked or delayed in the future.

Robert Meadowcroft, CEO

Our commitments for you

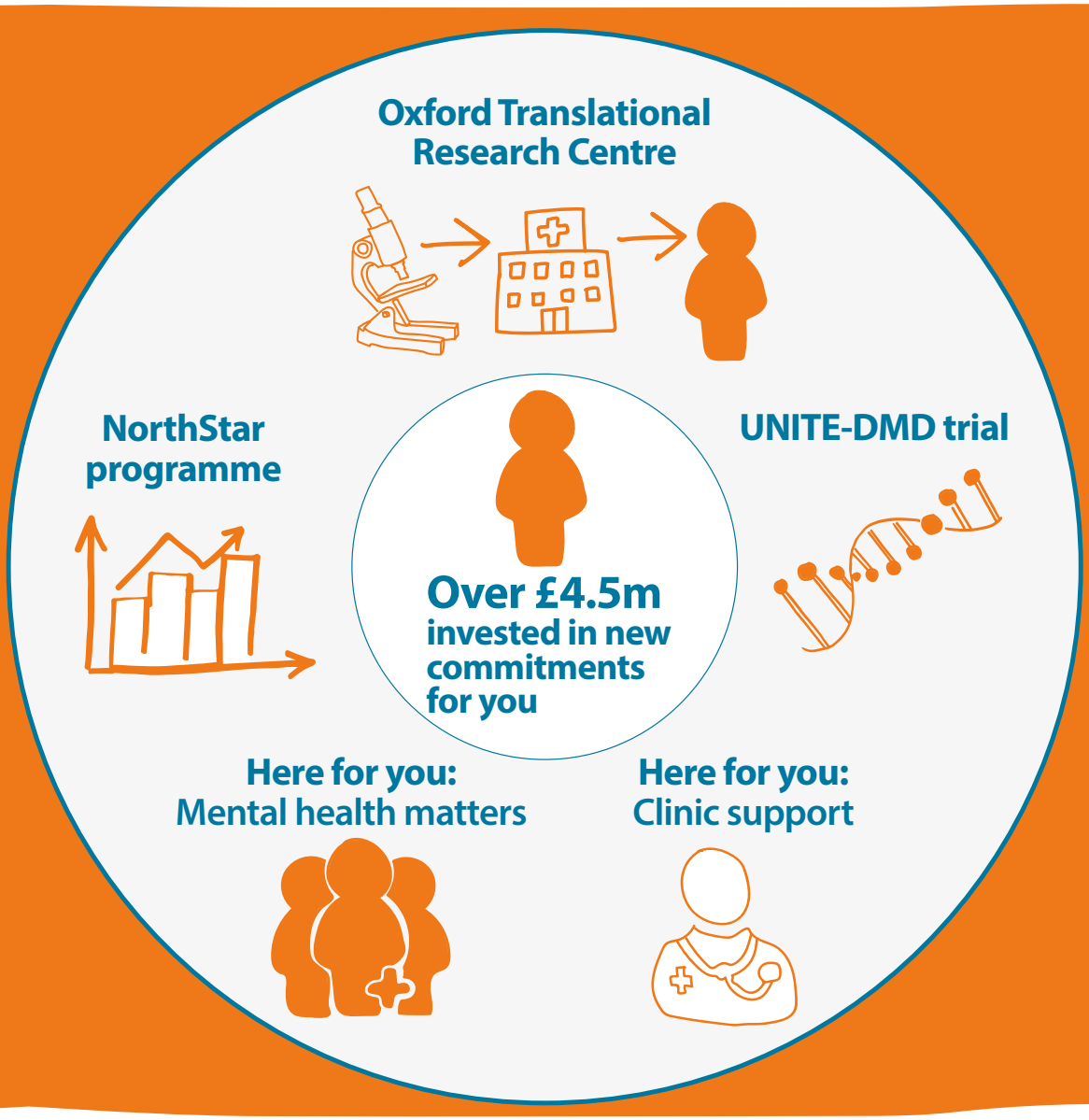
We recently announced an investment of over £4.5m into five new transformational commitments to provide everyone living with a muscle-wasting condition with choices – to live a life unlimited, understood and never alone.

We believe our five-year investments will make a profound impact on lives, from the moment of diagnosis, improving quality of life, while also seeking to extend them.

These commitments are designed to increase the number of clinical trials taking place in the UK and to improve the overall care and support for all people living with a muscle-wasting condition.

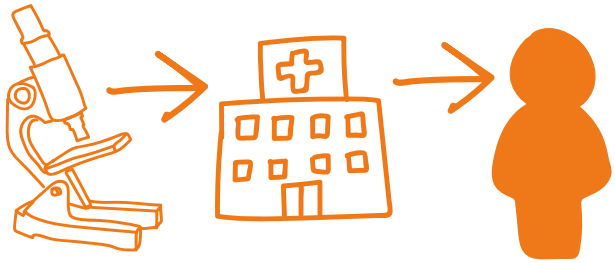
The biggest challenges in our field require multi-partner approaches to change the landscape. By working in partnership with world-renowned organisations we can share expertise to accelerate the path to treatments and improve clinical care.

Together, we can beat muscle-wasting conditions through accelerating research, driving access to the best of healthcare and enabling independence. Because everyone has the right to live their life to the fullest.



Find out more about these projects and how you can support them at
www.musculardystrophyuk.org/new-commitments

Oxford Translational Research Centre



Changing the landscape for clinical trials by developing a major centre in the UK

We are entering a new era of treatments for rare, genetic conditions like muscular dystrophy. Progress in the search for effective treatments and cures is being made in the UK and across the world.

But, as research continues to gain momentum – moving us closer to treatments – so does the demand for clinical trials.

The number of clinical trials being undertaken in the UK is increasing but inadequate capacity – such as a lack of infrastructure and expertise – means centres are struggling to meet demand.

We are aware that centres have been forced to turn down some clinical trials for muscle-wasting conditions in the UK.

Unless these capacity issues are resolved across the UK, we will see reduced opportunities for patients to be enrolled in clinical trials. In order for the UK to remain one of the key countries for clinical trials for neuromuscular conditions, additional capacity must be addressed.

We believe everyone has the right to live life to the fullest. That's why we're working with Oxford University and the John Radcliffe Hospital to establish a world-leading centre aimed at bringing new treatments to patients more quickly.

The multi-million pound Oxford Translational Research Centre will boost capacity for clinical trials. Working alongside the major centres in Newcastle and London, this third centre will be a game changer for individuals and families living with muscle-wasting conditions in the UK.

Professor Dame Kay Davies, Professor of Anatomy at the University of Oxford, says:
"This is a very important time in the history of research and therapy into muscle conditions. MDUK has been a major contributor to the basic research for more than two decades. The funding of a new neuromuscular translational centre at Oxford ensures that they can build on this investment and deliver on effective therapies for paediatric and adult disorders. I am very excited to be part of this initiative."

By driving research into an ambitious new era for clinical trials, this Centre's work will have a transformational impact and provide new hope to more people with muscle-wasting conditions.

What do we mean by a new centre?

The Oxford Translational Research Centre will not be a physical building but a partnership between two already existing sites in Oxford: the Oxford University's Medical Sciences Division and the John Radcliffe Hospital.

Why Oxford?

We have always funded world-class research in Oxford – including the work of our Vice Presidents, Professor Dame Kay Davies and Professor Matthew Wood. Their work alongside that of Professor Kevin Talbot, and the rest of the clinical care team, has truly moved forward the treatment options for neuromuscular patients. What is so exciting now about this new investment is that we are able to drive this work out of the laboratory and into the clinic.

How will the Centre increase clinical trial capacity?

Our investment in posts and other resources at the new Oxford Translational Research Centre will enable more potential treatments to be tested in clinical trials. The new resources include:

- a Chair of Paediatric Neurology to oversee the translational research programme
- a Consultant Paediatric Neurologist to lead clinical academic and clinical trial work
- a Clinical Trials Co-ordinator who will work with national and international clinical trial networks across adult and children's services
- a project manager, who will also source additional funding for the Centre and develop opportunities for public engagement
- start-up seed funding for research costs for clinical lecturers.

Find out more about these projects and how you can support them at www.musculardystrophyuk.org/new-commitments

UNITE-DMD trial

Investing in gene therapy to advance treatments for muscle-wasting conditions

We are proud to be a long-standing supporter of research into gene therapy. This technology has advanced significantly over the years and it's now time to take it to the next level: to test its safety in people living with muscle-wasting conditions in the UK.

UNITE-DMD is an international collaboration working on gene therapy. Once preclinical studies are complete, it will assess the safety of a gene therapy for Duchenne muscular dystrophy in a phase I/II clinical trial.

To date, no gene therapy trials for muscle-wasting conditions have been undertaken in the UK. The work of UNITE-DMD is therefore fundamental in evaluating the safety of this technique – something that we strongly support.

Although this particular gene therapy will be designed to treat Duchenne muscular dystrophy, its development will refine and improve the technique generally. This will help in the development of gene therapies for other muscle-wasting conditions in the future.

UNITE-DMD is a four-year project taking place in the UK and France. The UK investigators are Professor George Dickson at Royal Holloway, University of London; Professor Francesco Muntoni at the University College London Great Ormond Street Institute of Child Health; and Professor Volker Straub at the John Walton Muscular Dystrophy Research Centre, Newcastle University.

We are delighted to be the principal funder of the UK side of the project, in partnership with Action Duchenne. The French Muscular Dystrophy Association (AFM-Telethon) is funding the French arm of the project.

Professor George Dickson has studied gene therapy for over 20 years and is one of the leaders of the UNITE-DMD project.

"Our work has consistently been supported by funding from MDUK, providing the means to develop these new therapies. This new investment will help to move them into the clinic for the benefit of patients and their families."

By supporting world-leading research, Muscular Dystrophy UK is transforming treatments for all people living with a muscle-wasting condition. Because everyone has the right to live life to the fullest.

What is gene therapy?

Gene therapy is intended to be a single treatment for genetic conditions, such as muscular dystrophies. These conditions are caused by a mutation in a gene. The mutated gene does not produce the protein needed to keep the muscle healthy. Gene therapy introduces a new, healthy copy of this gene into the body. This then restores production of the protein that was missing.

How does this work for Duchenne muscular dystrophy?

Duchenne muscular dystrophy is caused by a faulty dystrophin gene. The UNITE-DMD project will deliver a smaller but functional copy of the dystrophin gene (known as micro-dystrophin) into people with Duchenne muscular dystrophy. This will increase the production of the micro-dystrophin protein in their muscles and could be a potential treatment for the condition.

A significant benefit of this approach is that it is not mutation-specific.

How does this gene get into the body?

UNITE-DMD will deliver the micro-dystrophin gene by packaging it into viruses called adeno-associated viruses (AAVs) and injecting these into the bloodstream. Once inside the body, the viruses carry the gene into the muscle cells.

Find out more about these projects and how you can support them at www.musculardystrophyuk.org/new-commitments

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NorthStar programme

Paving the way for trials, treatment and care

To make a profound impact on people's lives, experts need to understand more about conditions and develop best-practice for care and treatments.

Our new commitment of £1.45m will boost the world's largest natural history study for Duchenne muscular dystrophy, known as NorthStar, and improve the quality of life for children and adults living with the condition.

This investment will help to accelerate the development of treatments, by providing data to improve the design and evaluation of clinical trials. It also offers a unique platform to influence best practice in the care of Duchenne patients nationally.

In 2004, we helped establish NorthStar, and since then thousands of records have been added.

Emerging trend-data from the study is already helping improve quality of life. By showing the benefits of steroid treatments, the NorthStar study led clinics across the country to use steroids in treating Duchenne patients. As a result, children with Duchenne are now walking for around 3.5 years longer than in the previous decade. This was identified as one of the most impressive achievements ever demonstrated at a national level.

Improvements in care over the last decade mean people with Duchenne muscular dystrophy are living longer. But these adults have less access to clinical trials than children.

To address this, NorthStar will now be expanded to include the collection of information from adults, in order to develop clinical trials, and to establish guidelines for best-practice care and support.

Professor Francesco Muntoni, Paediatric Neurologist at Great Ormond Street Hospital, says:

"The NorthStar study is a unique and effective consortium of all the UK neuromuscular paediatric centres. It is now expanding to also include young men and adults with Duchenne. This will help the implementation and monitoring of optimal standards of care for this particular group, which is the pre-requisite for clinical trial readiness."

NorthStar has the potential to transform the lives of the 2,500 children and adults living with Duchenne in the UK.

What is a natural history study?

A natural history study follows a group of people over time, measuring how their condition progresses and how it affects them. Healthcare professionals collect this information at every clinic appointment.

What is included in this investment?

NorthStar consists of two elements: a natural history study, and a network of consultants, physiotherapists and other healthcare professionals to support the study across the UK.

This programme focuses on a major upgrade to improve the collection of data for clinical trials. This includes investment in following roles:

- a full-time clinical co-ordinator to manage NorthStar information and work with pharmaceutical companies to share the data
- a network co-ordinator who will manage the Network
- a physiotherapist who will update and develop physiotherapy protocols and training modules
- administrative support to the 23 NorthStar Centres
- a PhD research co-ordinator who will lead on the collection of data for adults.

This programme will also see the development of the first-ever clinical care guidelines for adults with Duchenne. Improved standards of care and support could have a positive effect on life-expectancy and quality of life for men with the condition.

Find out more about these projects and how you can support them at www.musculardystrophyuk.org/new-commitments

Here for you: Mental health matters

Supporting people, from the moment of diagnosis, to be understood and never alone.

Living with a rare, progressive muscle-wasting condition can be challenging. But when you're denied – or not offered – basic support, for simple things like emotional wellbeing, it can leave you feeling isolated and alone. And that's hard.

Long-term prognosis is improving and there are exciting treatments on the horizon. But muscular dystrophy and its wide-ranging impact can affect every area of life, including mental health. If left unsupported, these negative experiences can have a detrimental effect and can ultimately lead to devastating consequences.

Professional psychological support is essential to improve emotional wellbeing. It doesn't make sense for this support to be forgotten and ignored. But we know this is happening, leaving many out in the cold to support themselves or seek information from non-professional sources including the internet.

Pushing for access to specialist psychological care and support from the moment of diagnosis to end-of-life, Here for you: Mental health matters is our commitment to making tangible changes to the current offering for all individuals and families living with a muscle-wasting condition.

We will be investing in better support for individuals and families, better support and training for healthcare professionals and putting pressure on the Government for NHS improvements.

There are many stages from the moment of a suspected diagnosis where you, and those around you, may experience devastation and loss. There will also be times when you will have to make some difficult decisions about your life. All of these moments can weigh heavily on your mental health.

By investing in Mental health matters, we want to make sure that you feel understood and never alone, because we believe you have the right to live your life to the fullest.

"Being diagnosed with muscular dystrophy is a bit like bereavement. People need psychological support to help them through that isolating process. It's important that support is there for you as soon as you're diagnosed, so you realise you're not alone and someone will be with you on that journey. We at Muscular Dystrophy UK will help you lead a fuller life as soon as possible and help you get on with your lives without the condition taking over."
Sheila Hawkins, Muscular Dystrophy UK Trustee, who has FSHD

What will the Here for you: Mental health matters programme of work involve?

There are six streams of work included within this programme:

1. setting up an expert working group to ensure that access to psychological support is addressed
2. holding a psychological support workshop to share learning and experience on a European level
3. developing free information resources and support for individuals and the wider family, including siblings, partners and parents
4. providing resources, better support and training for healthcare professionals to improve knowledge. Additionally, ensuring the NHS provides the right support for every individual or family receiving a diagnosis at a muscle centre or clinic
5. influencing Government strategies – rare disease, mental health and suicide prevention – to meet the needs of individuals and families with rare, long-term and progressive conditions, ensuring they do not fall through the emotional safety net
6. working in partnership with Professor David Abbott, and other Duchenne charities, to review and address the needs of adults with Duchenne, including end-of-life decisions.

Who will be involved?

- The new expert working group will bring together clinicians, care advisors and psychologists, as well as parents, young people and adults living with the condition.
- The European workshop will be held at the European Neuromuscular Centre (ENMC) with experts from across Europe.
- Reviewing the needs of adults with Duchenne will see the collaboration of researchers, patient groups, healthcare professionals and adults living with the condition.

Find out more about these projects and how you can support them at www.musculardystrophyuk.org/new-commitments

Here for you: Clinic support

Providing personal support to improve quality of life and independent living

From the moment of diagnosis your life will have changed. Being faced with a whole new world of medical understanding, while also needing emotional support and practical information about your new daily life.

We're here to support you, and your family, along this journey. To help, listen, and connect you with others who understand what it means to live with a muscle-wasting condition.

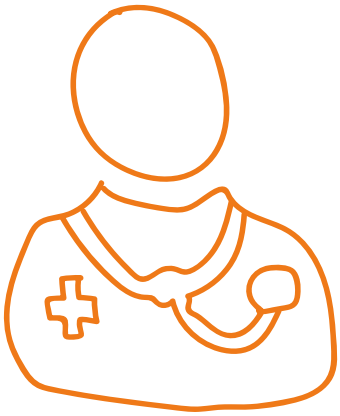
Our new Here for you: Clinic support programme addresses a growing need for support in local communities, such as help accessing disability benefits, signposting to relevant services and understanding the realities of living with a muscle-wasting condition.

This new commitment will see our information and advocacy services sitting alongside the expertise being offered in clinic by healthcare professionals to give individuals and families living with a muscle-wasting condition rounded support in all areas of life.

"When my son Archie was diagnosed, the support I had wasn't great. That isolating time is something I won't forget. I was just given a leaflet put under my nose – I wish I had personal support. You need to know you can walk out the door of the clinic where you've been diagnosed and pick up the phone for support."
Louisa Hill, Muscular Dystrophy UK Trustee and mum of Archie (11), who has Duchenne muscular dystrophy

By offering tailored, region-specific support to clinics, our teams will be able to call on the complementary services we offer and join up your care. This will ensure you have access to all the resources and community support you need as well as the best of specialist healthcare.

Our new commitment will also see development in clinical research and quality of life initiatives.



We are here for you at every stage, from the moment of diagnosis, to improve your quality of life and independence. Because everyone has the right to live their life to the fullest.

How will this commitment be delivered?

We have already established work in clinics in London, Wales, and Northern Ireland. We will be investing in four further Advocacy and Information Officer roles at Great Ormond Street Hospital, Oxford, Newcastle and Scotland.

Dr Chiara Marini Bettolo, Consultant Neurologist, John Walton Muscular Dystrophy Research Centre, said: "The role here in Newcastle would provide essential support to our patients and their families. It is vital that patients receive the necessary care and assistance at home and in the community. An Advocacy and Information Officer role is pivotal to ensuring our users receive the highest quality health, education and social care services."

What will these roles do?

Advocacy and Information Officers:

- discuss practical and financial support that people might be entitled to
- support with grant applications, for example DWP benefit forms, housing grants
- connect people with others in a similar situation
- offer support through our Freephone helpline and email
- organise support group meetings and information events for individuals and families
- support applications to overturn unsuitable care packages, if required
- signpost medical questions to healthcare professionals.

Find out more about these projects and how you can support them at www.musculardystrophyuk.org/new-commitments

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Target Research

Welcome to the research pages of *Target MD*.

I'm delighted to share with you some of the top research news stories from over the last six months. There were some exciting advances in the neuromuscular field, including the first ever treatment for spinal muscular atrophy being approved in the United States (US). An exon skipping drug for Duchenne muscular dystrophy has also gained approval in the US.

Although the approval process in Europe is different from that in the States, I very much hope that the European regulators will agree with their American peers, and that we will see these potential treatments entering UK clinics in the near future.

I'd also like to update you on the outcome of last year's research grant round. We received a record number of applications and ended up funding the top 10 projects. This wouldn't have been possible without the generosity of you, our supporters, so many thanks for helping us to fund this high-quality research.

I wish you all the best!

Dr Jenny Sharpe,
Editor, *Target Research*

End-of-life care

Adults with Duchenne muscular dystrophy who wish to discuss end-of-life care need better psychological and practical support.

A new study, published in the scientific journal Neuromuscular Disorders, suggests that more can be done to pay attention to these issues. Adequate psychological and social support services, as well as training and support for clinicians, need to be available.

The Duchenne Forum-funded qualitative study was led by David Abbott, Professor of Social Policy at the University of Bristol, and Helen Prescott, Consultant Clinical Psychologist and Head of Lifetime Service at Sirona Care & Health. They interviewed 15 men, aged 20 to 45 years old, with Duchenne muscular dystrophy.

"Having a good life remained central to the men in our study and talking about death and dying was not something that dominated their thinking at all. Providing opportunities for good quality conversations at the right time is an important task and challenge for clinicians – and perhaps the wider Duchenne community – to think about." Professor David Abbott and Dr Helen Prescott, who led the study.

The Duchenne Forum is a group of charities established to speed up progress in the search for treatments and better care for Duchenne muscular dystrophy: Muscular Dystrophy UK, Joining Jack, Harrison's Fund, Duchenne Children's Trust, Duchenne Research Fund, Alex's Wish.

What are the next steps?

A working group will be set up to share the findings and find ways to apply them. The group will include a palliative care consultant, a neuromuscular psychologist, a neuromuscular care advisor and an adult with Duchenne muscular dystrophy.

Professor David Abbott, one of the leaders of the study

SMA Spinraza

Late last year, the US Food and Drug Administration (FDA) announced it had approved Spinraza as a treatment for SMA. (Spinraza is the brand name for the drug, nusinersen.)

This approval is for a broad licence, meaning that Spinraza can be marketed in the US for children and adults with SMA types 1-3.

Although the approval process in Europe is separate from that in the US, it's a major step forward that the drug may soon be available in clinics in the US.

Spinraza's manufacturer, Biogen, has applied to the European Medicines Agency (EMA – the body responsible for approving potential treatments in Europe and the UK) for a broad licence. The EMA is currently reviewing this for a decision later this year.

We hope that the outcome will be similar to that in the US, and a broad licence will be approved. Muscular Dystrophy UK, SMA Support UK and The SMA Trust have written jointly to the EMA urging a fast approval for Spinraza in Europe.

What are the next steps?

If the EMA approves Spinraza, the following need to decide whether or not to fund the drug in the UK:

- the National Institute for Health and Care Excellence (NICE)
- NHS England
- the Scottish Medicines Consortium
- other authorities in the devolved nations.

This would usually be in line with the terms of the licence set out by the EMA.

Scoping process

NICE has already started what is called a 'scoping' process. It is gathering evidence about SMA and Spinraza to help decide whether to review the drug should the EMA grant Biogen a licence.

Muscular Dystrophy UK and representatives from SMA Support UK and The SMA Trust will be advocating for a review that will consider broad approval for all types of SMA. If a review does go ahead there will then be an opportunity for families to have their say.

Keep an eye on our website for further updates.

Read more at: www.musculardystrophyuk.org/progress-in-research/news

It is your generous support that enables MDUK to fund pioneering research. We cannot do this without you. Find out more at www.musculardystrophyuk.org/get-involved

A life-changing decision

Parents at risk of having a child with mitochondrial disease may soon have the chance of having a healthy, genetically-related child. This is a life-changing decision. The IVF procedure could be available for women in the UK as soon as spring 2017.

The Human Fertilisation and Embryology Authority (HFEA) announced at the end of 2016 that they had approved mitochondrial IVF for use in clinics. With clinics now able apply for a licence to offer the procedure, scientists at Newcastle University have received the first licence from the HFEA to perform the technique.

What is mitochondrial IVF?

Mitochondrial IVF allows DNA to be transferred from an egg with damaged mitochondria to a healthy, empty egg. It can prevent eligible women affected by mitochondrial disease from passing the condition on to their future children.

The technique is relevant to patients for whom pre-implantation genetic diagnosis (PGD) would be inappropriate, or unlikely to succeed. While IVF does not guarantee the prevention of mitochondrial disease, prospective parents can make informed reproductive choices.

Professor Sir Doug Turnbull and Professor Mary Herbert at Newcastle University developed technique. Muscular Dystrophy UK funded their early-stage research into mitochondrial donation IVF for more than 10 years.

"We are delighted by today's decision as it paves the way offering mitochondrial donation as part of an NHS-funded package of care for families. Newcastle is a major UK referral centre for women with mitochondrial DNA mutations. We will be aiming to treat up to 25 carefully selected patients a year with the mitochondrial donation technique as a clinical risk reduction treatment. We will also provide long-term follow up of any children born."
Prof Sir Doug Turnbull

"This is a historic decision. We know of many women who have faced heartache and tragedy, while trying to start their own family. This gives them new hope and choice for the first time.

"We recognise this approach is not without some uncertainty, and, in any trial, success cannot be guaranteed. However, it is important that women are able to make informed choices," said Robert Meadowcroft, CEO of Muscular Dystrophy UK.

"Although it is tempting to rush ahead with new treatments, the UK approach of testing public opinion, putting the issue to parliament and carefully monitoring laboratory research has proved to be the most responsible and sustainable of introducing new, cutting edge treatments into the clinic. Such an approach has allowed us to balance innovation with safety, maintaining public trust as we go."
HFEA Chair, Sally Cheshire

Professor Sir Doug Turnbull developed mitochondrial donation IVF with initial support from Muscular Dystrophy UK

Exondys 51, the FDA and the EMA

The Food and Drug Administration (FDA) in the USA has given the green light to a new drug for Duchenne muscular dystrophy.

Exondys 51 treats boys with a gene mutation amenable to exon 51 skipping. It could slow down the progression of Duchenne. Three years of clinical trials showed that boys on the drug could walk 151 metres more in six minutes than boys not on it.

Claire O'Hanlon, Chair of MDUK's Northern Ireland Council, went to the FDA's open hearings last year. Claire, whose son Luke has Duchenne, joined other families to put forward their views on the drug, ahead of the decision.

Muscular Dystrophy UK has backed the development of exon skipping technology for over 20 years. This is the first exon skipping drug to be approved. But the FDA's decision doesn't apply to Europe.

Manufacturer of Exondys 51, Sarepta Therapeutics, has applied to the European Medicines Agency (EMA). The application has been validated for assessment during 2017. MDUK is working with other patient groups to push for fast access to the drug here.

T-Jay Roe, from Nottinghamshire, could benefit from Exondys51

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Saturday 7 October 2017
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Visit www.musculardystrophyuk.org or call 020 7803 4800 to find out more.

Meet our new Chair

Professor Mike Hanna, a Consultant Neurologist, is the new Chair of Muscular Dystrophy UK's Board of Trustees. A Trustee since 2013, Mike was appointed last October, when outgoing Chair Bill Ronald completed his term of office.

Director of the London-based National Hospital for Neurology, and of the MRC Centre for Neuromuscular Diseases, Mike leads an active research programme and has published over 150 peer-reviewed papers.

"This is a really exciting time for the charity, the patients, and families. I want to prioritise research, continue to lobby the NHS for access to treatments, and continue the campaign for independent living. Bill has done a fantastic job over the last six years and has left the charity in great shape. I'm looking forwarding to continuing his work."

Outgoing Chair, Bill, held the position for six years. His leadership saw increased research funding, the strengthening of NHS services and improved care for patients. In Bill's final year, the charity celebrated a landmark event after the first-ever drug for muscular dystrophy – Translarna – was licensed by the NHS. Bill leaves the charity with solid foundations for containing the fight on behalf of the 70,000 children and adults living with muscle-wasting conditions.

With several clinical trials underway, and new treatments on the horizon, Mike's experience and authority will no doubt prove invaluable in guiding the charity towards a new era.

Chief Executive, Robert Meadowcroft said:
"We must give Bill huge thanks for his leadership and support during his time as Chair. Bill leaves the charity in a very good position, stronger and focused on accelerating progress in research. I am delighted Professor Mike Hanna will provide the leadership and support to help us take the charity forward."

"Mike has huge expertise in neuromuscular conditions – in research and as a clinician. He also brings extensive senior management experience. I am sure he will make a huge contribution to all aspects of our work as we look to achieve our goal of beating muscle-wasting conditions."

Outgoing Chair Bill Ronald (l) hands over the baton to Muscular Dystrophy UK's new Chair, Professor Mike Hanna

Louisa Hill

Louisa (pictured above with her family) became a Trustee in October 2016. A strong supporter of the charity, a successful fundraiser and an experienced media spokesperson, Louisa is passionate about helping families living with Duchenne muscular dystrophy.

She was also heavily involved in the successful campaign for Translarna, even getting to meet David Cameron and Theresa May.

"There is no better person than a mother to give a mother's perspective. I feel very honoured to be a Trustee – it's my way of helping other families going through the same things I've been through, and dealing with new diagnoses."

A mother of two sons – Leyton (14) and Archie (11) – Louisa is an NNEB-qualified Nursery Nurse. When Archie was diagnosed with Duchenne muscular dystrophy seven years ago, the family was told there was nothing they could do.

"An inner strength comes out from time to time. I've done things I never thought I'd do. Being in the public eye, and in the news, speaking at the Microscope Ball and the Sports Quiz about our journey with Archie.

"I want people to be part of Archie's life and remember him for everything he does. Our Action4Archie Fund has loads of people supporting us, even people we haven't met."

Along with husband Gary, Louisa has raised well over £100k for the charity. She has run her first-ever marathons – she's now up to her sixth – and taken part in the 100k walking challenges.

"MDUK has become a real part of my family, and the charity team is fantastic. I'm passionate about raising awareness about MDUK – I want to be remembered for making a difference."

Charles Scott

Charles was appointed to the Board of Trustees in October 2016. He brings with him a wealth of experience in finance, as well as a long-standing connection with the charity.

"I was good friends at university with Mark Reynolds, and was a founding supporter of Q Trust, which Mark founded. I very much enjoyed fundraising with the remarkable Mark and the equally remarkable Jemima, and so I have been associated with Muscular Dystrophy UK for more than 15 years."

A law graduate from Oxford University, Charles started his career in finance with Wood Gundy in Toronto. He joined Morgan Stanley in 1985, ran European equity sales and research, and left the firm as Chief Operating Officer of Morgan Stanley UK in 2007. He served as a Trustee of the Morgan Stanley International Foundation from 1992-2007, and as Chairman from 1993-2000. He is a Director of Polar Capital Funds, and has a number of other Trusteeships.

Charles is Chairman of the successful appeal for his village (Shalbourne in Wiltshire) Sports Pavilion and is very supportive of local causes in his community.

"We Trustees have an important role to play in determining how the charity should be spending its money. This is particularly important in an environment where research into the various muscle-wasting conditions and clinical trials has been so intense. We are excited about how the charity is contributing to this progress.

"There has been much encouraging progress in research, and real advances being made. It is really exciting and makes the charity's work ever more important: we may be close to a real breakthrough."

"As a Trustee, I'm also here to help with fundraising. I enjoy getting out and about and meeting as many families as possible, particularly at the AGM and events."

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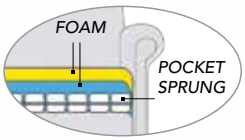

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Reflections on an eminent career

"I'll never know how I got here."

These are the words of Emeritus Professor Alan E H Emery MD DSc (Manc) PhD (Johns Hopkins) FRCP FRCPE FLS FRS(E) FRS(SAf) FRSA, an eminent, world-renowned, multi-award-winning clinical researcher. I spent an afternoon with him at Oxford University's Green Templeton College, where he is an Honorary Fellow. He shared with me, with trademark humility and candour, the highlights of his astounding and successful career.

His achievements include myriad degrees, qualifications, honours, awards, academic distinctions, appointments, published works and books. There is one award, however, that stands out for Alan: the honorary doctorate he received two years ago from the University of Chester.

Alan was born and raised in Gorton. As he stood to receive the honorary doctorate in 2014, his childhood in Gorton suddenly felt very far away.

"Given my background, this award has been the most meaningful for me," he said.

Born in the very poor area of Gorton, Manchester, Alan was raised in his early days by his grandparents. They couldn't read or write, but sent Alan to school. After being evacuated for a year when war broke out, Alan returned to Manchester and left school to work in a dye factory. After a call-up to the military, Alan met a colonel in the uniformed army education corps, who saw his potential. He encouraged him to study and to go to university.

Alan did so, and emerged from Manchester University with a double first in zoology and botany in 1952, and first-class honours in medicine in 1960. As house physician to Professor Sir Robert Platt at the Manchester Royal Infirmary, Alan was encouraged to think about studying genetics.

"None of us understood or realised the relevance of genetics. I applied for and was awarded a grant to study genetics with world-renowned geneticist Victor McKusick at Johns Hopkins University in Baltimore. I found the research in this unit did not spark my interest at the time. Then someone told me about muscular dystrophy – I'd never heard of it before," he said.

In 1961, Alan met two Baltimore families from vastly different socio-economic backgrounds, both of whom had sons with Duchenne muscular dystrophy. It was when he met the family living in downtown Baltimore that something changed.

"I saw this young boy in a wheelchair. He was holding a piece of string that was attached to the doorknob so he could pull the door open. I realised at that moment I wanted to study this condition."

This new career direction included Alan's being the first to delineate the condition that came to be known as Emery-Dreifuss muscular dystrophy. Its

protein, emerin, was named after him too, as was Emery-Nelson syndrome.

After observing some mothers of boys with Duchenne muscular dystrophy, Alan's paper, *Clinical Manifestations in Two Carriers of Duchenne muscular dystrophy,* was published in The Lancet in 1963. This was among the first of his more than 400 scientific papers to be published.

While in the US, Alan met John Walton (the late Lord Walton of Detchant, a founder of Muscular Dystrophy UK), who encouraged him to return to England to research Duchenne muscular dystrophy. He did so in 1964, and went on to establish the Medical Genetics Departments at Manchester University (1966) and later at Edinburgh University (1968). In 1989, he established the European Neuromuscular Centre (ENMC).

"The support of a number of eminent neuroscientists throughout Europe ensured the ENMC's success. I wanted people to collaborate and agree on diagnostic criteria for each neuromuscular condition, then to look at the gene defects and think about therapies. That is ongoing now," he said.

"The research landscape has changed significantly over the years. In the 1950s and 1960s, we started to develop the base of creatine kinase (CK) testing, and identified the carriers of Duchenne muscular dystrophy. John Walton's work in defining the different types of muscular dystrophy was groundbreaking too.

"With the development of DNA studies, the genes for almost all the neuromuscular conditions have been identified. There are now more than 50 different forms of muscular dystrophy, each with their own gene defects."

Alan first became involved with Muscular Dystrophy UK in 1964 after meeting Richard Attenborough. The charity funded a number of his research projects over the next 30 years, and he is a Vice President of the charity. He also received our 2012 Lifetime Achievement Award.

"When I look back through my whole career, one of the most important aspects was talking to parents and families. A clinical diagnosis is important, but you also need to take time to make sure families know and understand what you're telling them. I never saw patients just as people in a clinic; I needed to see them in their own homes and get a sense of what their whole lives were like. I visited 90 percent of the families of patients I saw.

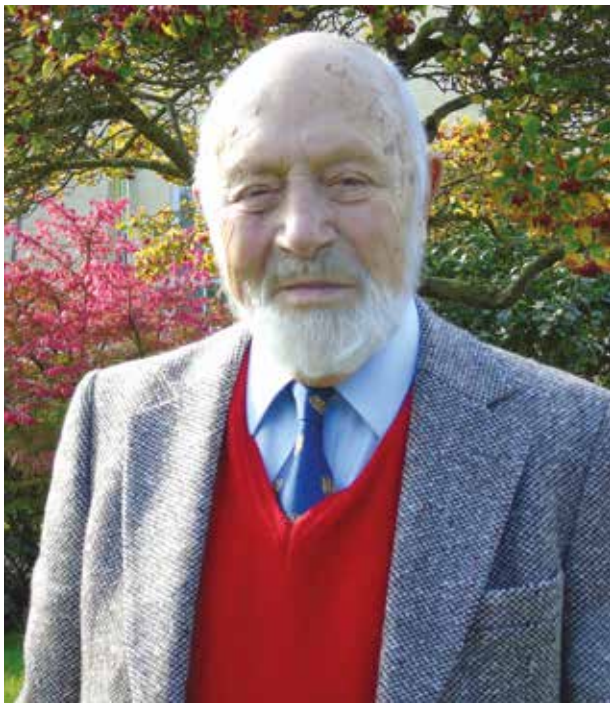
"There are also other aspects of life with muscular dystrophy that need to be looked at. I introduced the role of family care officers, to support families through the key milestones of school, needing a wheelchair and needing ventilation. Things are infinitely better today than they were."

Alan wrote *Muscular Dystrophy (The Facts)* and has recently published the fourth edition of *Duchenne Muscular Dystrophy (Oxford Monographs on Medical Genetics),* that he wrote with Muscular Dystrophy UK-funded researchers, Dr Ros Quinlivan and Professor Francesco Muntoni.

After a full and distinguished career, Alan now divides his time between his college in Oxford and his home in Devon. His current interests include natural history, oil painting (he's had several exhibitions) and the relationship between the humanities and medicine. He and his wife, Marcia, have published several books on medicine and art, and he is a published poet. Alan is also listed in, among others, the *International Who's Who, Dictionary of International Biography and Outstanding Intellectuals of the 21st Century*.

Not sure about you, but I think I know how Professor Emery got here. A generous helping of natural talent, hard work, astonishing curiosity and extraordinary compassion and kindness. I'd say that's how.

Ruth Martin, Editor



Campaigning updates

UK research investment commitment

The UK Government has highlighted investment into science and research as a top priority. This, as it launched its Industrial Strategy Green Paper in January.

The Green Paper outlines the Government's research and development commitments, including:

- increasing research and development spend by £4.7bn – an extra £2bn per year by 2020-21
- creating a new Industrial Strategy Challenge Fund
- investing in new research and development facilities.

What could this mean for people with muscle-wasting conditions?

MDUK welcomes the spending commitment on research. The Government is now consulting on where this money should be spent. A new body – UK Research and Innovation (UKRI) – will consult further later this year.

MDUK will push to keep research into rare diseases – and adequate funding for it – on the agenda.

Trailblazers improving access to rail transport

Trailblazers' concerns on accessibility on the rail network were highlighted in questions for the Department for Transport in the House of Commons in February. These included the inaccessibility of many train stations and difficulties with the advanced booking system.

Nick Thomas-Symonds MP challenged the Government on what action they were taking to ensure disabled passengers had equality of access to rail services.

"Has the Minister had a chance to read the Muscular Dystrophy UK Trailblazers' End of the line report, in which young disabled people identify problems with accessibility to train stations … and the advance booking system? Will the Government commit to looking at both issues with a view to finding a solution?"

In response, Rail Minister Paul Maynard MP reflected positively on his former role as Chair of the All Party Parliamentary Group for Young Disabled People:

"Probably the most rewarding period of my time as a Member of Parliament has been spent chairing the Muscular Dystrophy UK Trailblazers all-party group and challenging and cross-examining the industry, so I am well aware of the report. It is worth pointing out that Passenger Assist bookings are increasing by seven percent, year on year. The challenge for the industry is to ensure that passengers who wish to just 'turn up and go' get the same service as those who book through Passenger Assist. More than that, the industry should ensure that when Passenger Assist does not work properly, people have adequate recourse to an ombudsman's system to get redress. That is not currently the case."

Three-quarters of respondents to Muscular Dystrophy UK Trailblazers' *End of the line* transport survey would prefer a 'turn up and go' service rather than booking 24 hours in advance. As well as ensuring that advanced booking systems work effectively, Trailblazers are also keen to make sure that spontaneous travel is supported by the rail industry and that a 'turn up and go' type of service is in place across the country.

Nick Thomas-Symonds will be writing to Paul Maynard to highlight these issues and to ensure that progress is made by the rail industry.

MDUK represented on Minister's Young Disabled People Forum

With representation on a new Forum, MDUK will have the opportunity to put issues that affect young disabled people directly to the Minister for Disabled People, Work and Health.

Nic Bungay, Director of Campaigns, Care and Information, is among the leaders in the disability charity sector who have been invited to sit on the Minister's new Young Disabled People Forum.

"I'm delighted to be a part of this forum and have the opportunity to bring to the Minister the views and experiences of young people with muscle-wasting conditions. Through our Trailblazers network, we know that young disabled people still don't have access to the same opportunities as their non-disabled peers, and we look forward to working with the Minister to change this."

New disability sector champions

MDUK also welcomes the news that Penny Mordaunt MP, Minister for Disabled People, Work and Health, has appointed 11 industry leaders as disability sector champions.

The new roles will be filled by leaders in the retail, music, leisure, tourism, hotels, media, advertising, airports, buses, banking and gaming sectors. With an aim to increase representation of disabled people in fashion, gaming and television, the industry leaders will all champion the value of providing an inclusive and accessible service to disabled customers.

"These industries must become fully inclusive. Not being able to access the high street, products and services, transport or simply to access a loo jars with our national values: it must change."
Ms Penny Mordaunt MP, speaking to the BBC

Taxis to be fined for breach of Equality Act 2010

Taxi drivers could face a fine of up to £1,000 if they refuse to transport wheelchair users or attempt to charge them extra.

Transport Minister Andrew Jones announced recently that legislation would come into effect on 6 April, when sections 165 and 167 of the Equality Act 2010 are commenced.

After years of campaigning by disabled people and Trailblazers, the change will mean taxi and private hire vehicle drivers will be obliged by law to:

- transport wheelchair users in their wheelchair
- provide passengers in wheelchairs with appropriate assistance, and
- charge wheelchair users the same as non-wheelchair users.

Extending ambulance action for people with muscle-wasting conditions

Last year MDUK launched an exciting new partnership with the London Ambulance Service. In the 'Ambulance Action' scheme, calls to emergency services from people with muscle-wasting conditions are 'flagged', allowing paramedics to prioritise them effectively.

This is an important step forward for people living with rare muscle-wasting conditions, which paramedics may not often come across.

Since the launch in London we have been working with other ambulance services to ensure that everyone with a muscle-wasting condition has access to a similar level of emergency care across the UK.

We need your help to improve the care and support on offer. Let us know your views and experiences of emergency care including using ambulances the UK. Please get in touch with Lloyd Tingley at l.tingley@musculardystrophyuk.org or on 020 7803 4804.

My experience of going through PIP

Jane Bloxham (47) from Oxfordshire, who has limb girdle muscular dystrophy, shares her personal experience

"My experience of being assessed for Personal Independence Payments (PIP) was very scary and left me feeling totally helpless. I was glad to have a team around me, including the Muscular Dystrophy UK helpline, who helped when I felt like I was at rock bottom. I will always be thankful for the advice and guidance I received.'

It all started with a letter telling me my Disability Living Allowance (DLA) was coming to an end and being replaced by PIP. I was told I needed to contact PIP to claim for it.

I received a 40-page document to complete, with lots of questions, most having nothing to do with your medical condition. I was panicked by the timescale, but I got it completed as soon as possible and sent off.

I got a telephone call on a Tuesday, telling me to be at an appointment on the Friday. Again, worrying about time lines, I accepted the appointment and was told the paperwork would not reach me in time. It arrived the day after my assessment.

The assessment

My husband took time off work to get me to my early morning appointment. I was very nervous, but I knew this was an extremely important assessment and concentrated on listening to everything my assessor said. She went through the questions like it was just a tick box exercise. She seemed to know nothing about my disability and how it impacts on me and my life.

Then came the unexpected physical! When I was asked to get out of my wheelchair and balance on one leg, then on the other, then squat on the floor, I had to explain that if I did this the assessor would have to pick me up off the floor as I can't get myself back up. She said I didn't have to do it.

The result

Seven weeks passed and I got my letter to say I had been refused PIP. My worst nightmare had come true, and now I was 22 days away from losing my mobility car.

I emailed my neurologist to see if he could help, and for the first time ever, I contacted the Muscular Dystrophy UK helpline. I spoke to someone who was reassuring and very helpful – I will always be thankful for that – and was advised to call CAPITA to ask for a reconsideration.

I had one month to appeal, from the date of the refusal letter. My neurologist wrote a letter explaining my condition, someone from the advocacy team at MDUK wrote to CAPITA directly, and I wrote a letter, challenging the refusal letter.

Losing my Mobility car

In all of this process, the staff at Motability were very helpful as they have the worst end of the job to have to deal with. My car is basically my legs, so I felt like I had no option but to get a loan and buy my Mobility car or become housebound. I know I could not afford like for like if I tried to buy one from a garage.

I eventually received a letter to say I'd been awarded PIP. This was wonderful news but I still felt extremely angry about the whole process. What's the most worrying is that I will have to go through it all over again in 2021.

Our advocacy team is here to help if you are struggling with an application, or would like to dispute your PIP application. Contact us on 0800 652 6352 (Freephone) or info@musculardystrophyuk.org

Pride of Britain honours for Louise

After winning Pride of UTV for their fundraising efforts, Louise James and #TeamEvan went forward for the ITV Fundraiser of the Year at the 2016 Pride of Britain national awards.

Louise and #TeamEvan, from Derry in Northern Ireland, raised over £15,000 for Muscular Dystrophy UK. Just two months after losing five members of her family in a tragic accident, Louise took on this incredible fundraising feat in honour of one of her sons, who had Duchenne muscular dystrophy.

In response to an ITV regional news programme call, Louise was nominated as an inspirational fundraiser in her region. Seventeen finalists – including Louise – were selected by judges from each ITV region, all competing for the overall winner award.

UTV said: "It was the young mother's ability to transform her personal tragedy into a feat of courage, and her generosity of spirit, that stood out to the judging panel, including gold medal-winning Paralympian Michael McKillop."

Louise James said: "My involvement with the charity started when Evan was diagnosed with Duchenne muscular dystrophy at the age of three. We never let Evan have an illness; he was Evan, he was his own wee character and his illness never ever got him down.

"This award is not for me, it's for this town and it's for my son and my family. I feel like I'm not the only person grieving, everybody is grieving with me and I will fight for as long as I can to help other boys with Evan's condition."

A rare disease in a TV soap opera spotlight

Early last year, our press team took a call from a producer at national soap, Coronation Street. They wanted to explore the impact of a genetic diagnosis on an entire family, and wanted to know if we could help. They had heard about myotonic dystrophy, and got in touch with us to ensure an honest portrayal in both script and performance. From our point of view, we think they did. Mostly.

Why a soap opera?

We decided to get involved, to achieve three key aims:

- to bring myotonic dystrophy – and other genetic muscle-wasting conditions – to the attention of a whole new section of the UK public
- to provide information, support and factsheets to people who may be affected by the storyline
- to promote the Myotonic Research Breakthrough Fund (MRBF).

When the storyline finished airing at the end of October, there were some positive comments in the press and on social media, and some criticism. Overall, it raised a huge amount of awareness of genetic muscle-wasting conditions.

Our involvement

We advised ITV's scriptwriters and production team by:

- supporting two of researchers over a three-month period and answered questions about the impact of myotonic dystrophy on families, how these types of conditions were diagnosed and who would give families the news
- putting them in touch with a family living with myotonic dystrophy, who have had some difficult decisions to make
- advising them to speak to a genetics counsellor – which they did
- having our helpline number on the advice line after the programmes aired.

Raising awareness through a soap opera

It can be difficult to cut through the media noise out there and get your message heard. It's not enough to tell people who we are or what we do – we need to make sure they're listening and engaging too.

Working with a TV soap – where the storylines are interesting, addictive, personal, heartfelt – helped us reach a large number of loyal viewers who may never have heard of muscular dystrophy. These are people who know the characters like old friends, who care about them, and who might want to do something to help people in similar situations.

Myotonic dystrophy and related issues were featured in 10 episodes of Coronation Street. With nine million viewers per episode, the amount of awareness raised was, surely, priceless.

"I thought that MD is discovered when you're a kid. Must look that up."

"Thanks alot for info I have heard of it, but wasn't overly sure what it was. Definitely didn't realise it was passed on."

"I learnt that in the second episode. Never heard of it before. Great work Corrie on raising awareness."

"Well done @itvcorrie showing not all neuromuscular conditions present at birth/ childhood."

What impact did this storyline have?

An increase in sign-ups to our support information and newsletters as well as:

- a huge spike in traffic to our website: on the first night of airing, over 5,000 new visitors to and during the month, a 70 percent increase in visitors
- a 30 percent increase in visitors to the myotonic dystrophy condition landing page
- a 70 percent increase in downloads of the myotonic dystrophy factsheet
- a 180 percent increase in downloads of the inheritance factsheet
- an 875 percent increase in downloads of the carrier testing factsheet
- a 1,400 percent increase in visitors to the Diagnosis and Family Planning web page, and
- 900 percent increase in visitors to the Genetic Counselling and Family Planning web page.

Resources and services to support people living with muscle-wasting conditions

When you're facing a new diagnosis, new challenges as your condition progresses, or challenges you hadn't anticipated, the last thing you want to feel is alone. You're never alone.

With emotional and practical support and advice for you, we at MDUK are here for you. Our range of support, services and resources is available for you, wherever and whenever you might need it.

Understanding your condition

Our range of factsheets about muscle-wasting conditions are Information Standard-accredited, which means it's relevant, up-to-date info that you can trust. You might want to find out about a condition you or someone close to you has been diagnosed with, or about how the condition is likely to progress. Or you might want to print the factsheet out and give to your child's school, community health professional or take it with you to a PIP assessment.

Alert cards – help in an emergency

In times of medical emergency, it helps to have the vital information about your condition close at hand. Our range of alert cards - designed to fit in your wallet – do just that. You might also need more detailed information, which you can have at hand in a care plan.

Someone to talk to

When you need to speak to someone who can relate to what you might be going through, we can put you in touch. All of our peer support volunteers have direct or indirect experience of living with a muscle-wasting condition. You can talk via email or on the phone. You can also join one of the many conversations – or start your own – on our online forum, with people who may have been through similar experiences.

New challenges ahead?

Looking for an accessible school or uni? Needing to adapt your house? A PIP assessment coming up? Here's how we can support you with any issues like this:

- speak to someone in our advocacy team – they can advise you, support you with letters or appeal on your behalf to your local authority
- download or order one of our practical guides on benefits and financial assistance available for you
- get in touch with one of our volunteer Advocacy Ambassadors, who can support you with information or advice, or visit our website and download some of their top tips

For healthcare professionals

Some GPs may never come across some of the rarer muscle-wasting conditions, so you can share with yours the eLearning module we developed with the Royal College of General Practitioners. It helps GPs understand the tell-tale signs of the condition, so they can help people manage living with it. We have developed a similar module for physiotherapists.

To find out more about any of these resources, contact us on 0800 652 6352 (Freephone) or info@musculardystrophyuk.org or visit www.musculardystrophyuk.org

When someone intervenes on your behalf, by speaking to a decision maker, they are called an advocate. At Muscular Dystrophy UK, we call the service that does this our Advocacy service.

Our Advocacy service is here to help anyone with a muscle-wasting condition who is having difficulties getting the care and support, services, benefits and equipment they are entitled to.

If you are struggling to access services, we can help by providing advice or intervening on your behalf. Remember, early intervention is important so get in touch with us as soon as you receive a decision you aren't happy with. The sooner you contact us, the quicker we will be able to help.

Our friendly Advocacy Officers can support you to access a variety of services, some of which are outlined below. Please call the team to find out how we can help.

You can get in touch with us by calling our Freephone helpline on 08006526352 or by emailing us on info@musculardystrophyuk.org

Fundraising updates

Orange all over the UK!

Thanks to your overwhelming support, 35,190 people took part in our Go orange for a day fundraising event in January. People in 182 schools, clubs and workplaces across the UK dressed in orange, raised more than £35,400 and huge awareness of the fight to beat muscle-wasting conditions. It was an orange day to remember, and a colourful day to repeat: look out for details of next year's event taking place on Friday 2 February 2018.

Make Today Count 2017

Our amazing 211 skydivers – and the 19 people who indoor skydived for us – this year will have raised close to £130k towards our research.

Annjanet Mckerral (pictured) skydived from Hinton. She wanted to raise awareness for muscular dystrophy, as her mum and several family members have OPMD (oculopharyngeal muscular dystrophy).

"Just want to say thank you for such an amazing experience – absolutely loved every second of it. Roll on next year!"

Fancy joining in next year? You can Make Today Count on 23 February 2018, at a range of skydiving sites across the UK. By the time you land, you'll have funded nine hours of groundbreaking research.

Last Christmas

Long-standing supporter, Christine Ogden, held her 12th fundraising concert in November at the Bridge Centre in Bolton. With entertainment from the Parkside Colliery Male Voice choir and Matthew Chadbond, a young violinist, 230 people – including six local mayors – came to the event. A total of £8,500 was raised on the night and from donations during the year.

"All funds will go towards Professor Dickson's research [see p7] into Duchenne muscular dystrophy. We're doing our best here in the north west to make more people aware of muscular dystrophy and the charity's work," said Christine, whose grandson has the condition.

Pedal, Paddle, Peak

Congratulations to the 32 participants who successfully completed our inaugural Pedal, Paddle, Peak on 18 June 2016. What a brilliant day it was: a one-day multi-activity team event set in the beautiful Lake District. The weather was perfect, the teamwork was excellent and together, along with the generosity of sponsors McBains Cooper, teams raised an incredible £37,000. Our thrill-seeking group cycled 30 miles, canoed for two miles, (taking in the breathtaking beauty of Ullswater Lake), and then climbed the third highest peak in England, Helvellyn Mountain – all in a bid to conquer muscle-wasting conditions.

Pledge Dinner

In January, a group of supporters from the property industry raised £33,000 at our Property Pledge Dinner at the Mandarin Oriental Hotel in London. After being wined and dined, guests were treated to a fascinating Q&A, (hosted by the BBC's Ben Thompson), and business insights and personal anecdotes from property legend Sir John Ritblat and his son Jamie. Thanks to Committee members Andy Martin, Simon Knights and David Sinclair for ensuring the evening's success.

Microscope Ball

Our 33rd Microscope Ball last year was a golden affair which exceeded all our expectations. Our esteemed guests from the commercial property industry dug deep and raised a phenomenal £315,000. Louisa Hill, Trustee of MDUK and Mum of Archie (who has Duchenne), spoke about the family's experiences. Special guest Archie, and brother Leyton, raised the roof when they came on to say hello and thank you. Thank you to the Hill family, our Chairman David Morris and the rest of the Microscope Ball Committee for all their support and hard work.

Cambridge Town and Gown 10k

Over 1,800 runners braved the Cambridge rain last October to take part in our sixth Town and Gown in the city, raising a phenomenal £60,000 for MDUK. The closed-road run began on Midsummer Common and, for the first time, around 50 nine – 15-year-olds took part in our brand new 3k junior race. Thanks to all those who took part and came along to cheer on the day despite the weather!

London Marathon

Huge good luck to our mighty team of Marathon runners who will be lacing up their trainers on 23 April this year. Not only have they been busy training, but they have also been hard at work fundraising: together #TeamOrange is on track to raise a massive £250,000 for the fight against muscle-wasting conditions! Thank you to you all for your commitment and support.

Keep up-to-date with all of our upcoming events. Call 0300 012 0172 or visit www.musculardystrophyuk.org/get-involved and join in the #TeamOrange fun!

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