







DUCHENNE UK

We are the leading Duchenne charity in the UK - we have one aim, to end the devastating muscle-wasting disease of our innovative and collaborative Duchenne Muscular Dystrophy (DMD).

Our mission is to bring together the best researchers in the industry, with the NHS, drug companies and families to change the lives of people with this condition.

We are dedicated to finding transformative treatments for this generation of people with DMD, and will not rest until we make the incurable, curable.

We are the largest funder of DMD research in the UK, and are proud approach, inspiring a family of ambassadors and philanthropists who empower us to change lives.

We are run by people who live with DMD every day, it is in our DNA. We unite families in a community of hope - and know that, one day, in the not too distant future, we will make this condition a thing of the past.

WHAT IS DUCHENNE **MUSCULAR DYSTROPHY?**

Duchenne Muscular Dystrophy (DMD) is a devastating muscle-wasting disease. It is the most common and severe form of Muscular Dystrophy. Diagnosed in childhood, it mainly affects boys.

They tend to be diagnosed before the age of 5. In the UK, there are around 2,500 boys affected and around 300.000 worldwide.



OUR APPROACH

We focus on five key, integrated priority areas:



Accelerating progress in DMD research, leading to transformative treatments.

Enabling more families to take part in clinical trials around the UK.



Providing information and practical/ emotional support to patients and families.



Speeding up access to treatments and approvals by government bodies through Project HERCULES.



Investing in technology that can help young people with DMD.



LETTER FROM CO-FOUNDERS, EMILY CROSSLEY AND ALEX JOHNSON

Dear friends.

Thank you for taking the time to read this impact report.

If this year has taught us anything, it's that medical breakthroughs can and do happen – if there is funding, laser sharp focus and a collective will. The discovery of vaccines for COVID-19 marked a major turning point in 2020.

As we discover vaccines for COVID-19, so too will we find treatments for DMD. To do this we need to go further than anyone ever has. That means setting our ambition higher and finding ways to overcome every barrier. We're going to do it faster too, because this generation of boys can't wait.

Duchenne UK is so much more than a charity. We are a highly specialised and motivated Research and Development catalyser, using our knowledge and experience as patients, combined with our networks and funds - to accelerate research and bring treatments to patients. We are a driving force within the ecosystem of Research and Development in the UK. We know what patients need because we are the patients - living this disease on the front line.

And we are turning that lived experience into life-changing science. You'll read here about our work in gene therapy; how we are funding our own clinical trial looking at a repurposed medicine; how we've brought global pharmaceutical companies together in our award-winning Project HERCULES.

All the while, we've been supporting families through the terrifying challenges of COVID-19. At the same time, we launched DMD Care UK, a nationwide initiative to ensure everyone living with Duchenne in the UK has access to the best available care right now.

We are facing unchartered times. We know our income is going to be badly hit.

Because we are such a lean charity, we haven't been forced to furlough any staff. And we have enough funds to honour ALL of our outstanding research commitments. This means that NO projects will stop because of us cancelling funds.

The philosophy that has underpinned our approach - is to never take no for an answer and to never say no to a project we believe in. We seek out new projects all the time. But our freedom to fund research is now in question due to the huge downturn in revenue caused by the pandemic.

The impact on medical research has been devastating and projects are being delayed by six months or more. And when you're living with a progressive disease like DMD, 6 months can seem like a lifetime.

Duchenne is a rare disease – only 300,000 patients worldwide. If Duchenne UK can't fund the risky but promising early phase research, if WE don't have the funds to attract industry to trial sites in the UK because our infrastructure is lacking, then who will?

So, thank you for supporting us on our journey. The only way we'll do this is together. By joining forces with families, by bringing researchers together to share results, by engaging the health service to make more trials happen faster, by building a powerful community of engaged and committed supporters.

Join us and be part of the end of Duchenne.

Thank you for your support.



Emily Crossley and Alex Johnson Co-founders of Duchenne UK



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DRIVING FORWARD NEW ASSISTIVE TECHNOLOGY TO IMPROVE LIVES

RECRUITING PATIENTS FOR TRIALS

> **CAMPAIGNING FOR QUICKER ACCESS** TO NEW **TREATMENTS**

GROUND-BREAKING MEDIATION BETWEEN DRUG

GIVING FAMILIES A VOICE



DMD HUB POSTS FUNDED

LARGEST UK FUNDER OF DMD RESEARCH

SUPPORTING FAMILIES THROUGH COVID-19 AND BEYOND

> **BUILDING NEW CLINICAL TRIAL INFRASTRUCTURE**

INITIATING TRIALS











GENE THERAPY

One of the most promising and exciting areas of research at the moment is the use of gene therapy for DMD. In it, a harmless virus is used to deliver new genetic material to cells, overcoming the errors or deletions in the dystrophin gene, and theoretically allowing the muscles to grow and function normally. But it has many serious challenges.

Duchenne UK is committed to bringing gene therapy trials to the UK. We're making progress. We are preparing the infrastructure in hospitals and clinics, helping to train staff, investing in research tackling immunity challenges, and supporting our international partners in their endeavours.

Overcoming immunity challenges in gene therapy

Duchenne UK has invested \$500,000 in research at the Powell Gene Therapy Centre at the University of Florida to help overcome immunity challenges.

The viral carrier in gene therapy is key. The method involves hijacking a known virus to deliver the therapy - but this can be problematic as patients may already have natural immunity to the virus. If they do, the modified virus would be destroyed by the immune system before it could deliver its benefits. Separately, patients may develop immunity during their course of treatment.

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Our investment is trying to find solutions to both these issues.

Scientists are developing a newly designed piece of genetic code, to be delivered by the AAV virus. This will be tested in mdx mice using different regimes of immunosuppression devised by the Powell Gene Therapy Center team.

This project is an important milestone in the development of a successful gene therapy treatment that could dramatically improve the quality of life for patients with DMD of all ages.





Duchenne UK played an integral role in providing Solid Biosciences with a US\$5m investment six years ago to help fund pre-clinical work for their Gene Therapy programme. We are delighted to say that this investment is paying off. A number of clinical trials are now up and running in the US. 6 boys have now been dosed.

There was a setback this year when the FDA, the body responsible for

overseeing trials in America, paused the programme. This is a natural part of the clinical trial process, when assessments of unexpected responses to medicines or treatments have to be made. We are very pleased to report that the trial has now restarted. We are also closely monitoring trials underway in America run by Sarepta Therapeutics and Pfizer.







BRINGING GENE THERAPY TO THE UK

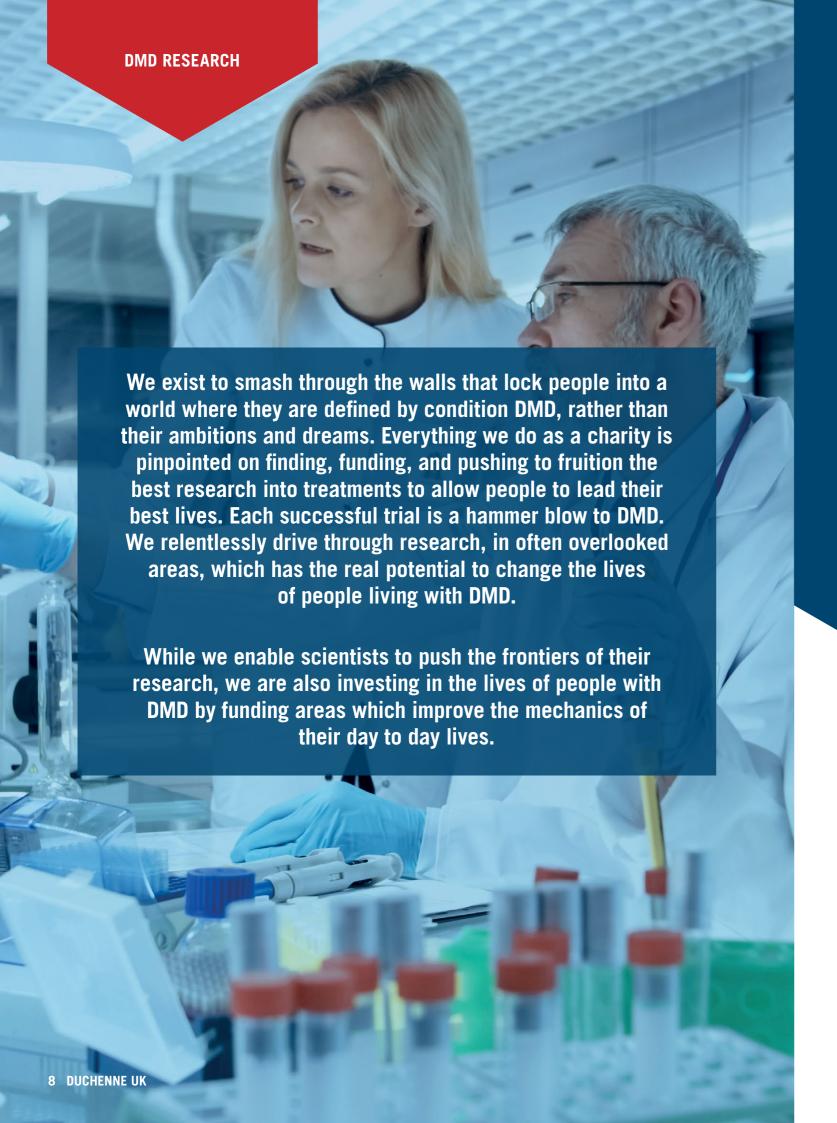
Building infrastructure through the DMD Hub

In November 2019, the DMD Hub gathered together more than 100 key stakeholders (patients, clinicians, regulators, industry representatives) to consider the likely barriers to gene therapy trials coming to the UK and how to overcome them, as well as the development of gene therapy as an approved and accessible treatment.

The UK is an attractive location for gene therapy clinical trials, with a significant amount of research ongoing in academic and clinical institutions, and some of the best DMD physicians in the world. However, there are several challenges to be addressed.







TAMOXIFEN UPDATE

Progress on a promising drug

We are delighted to say that the TAMDMD Tamoxifen trial, initiated by Duchenne UK, has now been fully recruited with 92 boys. The trial's looking into whether the breast cancer drug reduces fibrosis, slowing down the progression of DMD. Duchenne UK has committed £1.5 million over 4 years to the trial. By the summer of 2021, the patients will have completed 48 weeks of treatment and we can expect to see the results.



Rachel Williamson. Mum to Dexter:

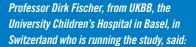
Our happy, funny, charming 7 year old Dexter started the Tamoxifen trial in December. Not only did Duchenne UK fund the trial, but thanks to the charity there was a trial site in Glasgow, which meant it was so much easier for us to access it. Thank you Duchenne UK - you are changing the world for these special boys.











Duchenne UK and the other patient organisations came in with funding at a very critical moment. Despite a positive evaluation and a recommendation by the E-Rare review process, this trial would not have been possible at all without the co-funding from Duchenne UK and the other patient organisations.

I am extremely grateful for their ongoing support, which has included not only financial help, but also help negotiating with regulatory authorities and other involved parties.





WORKING TO MAKE CLINICAL TRIALS EASIER TO TAKE PART IN

Plotting the progress of DMD as it advances is very important for treatments. Up until now the only way to do it has been through biopsies, which are intrusive and painful, and expensive MRI scans. We know our children need constant monitoring but we want to make this quick and easy so they can live their lives as normally as possible.

In 2018, Duchenne UK funded a small study into biomarkers, which are naturally occurring molecules, genes,

or signals found in blood and urine that reflect changes in the body. This year — in collaboration with the Muscular Dystrophy Association and Parent Project Muscular Dystrophy through a grant of \$686,500 to the University of California, Berkeley — we scaled up that study to conduct a trial in 150 boys. The results from the original study were very promising and we hope, one day soon, to develop a urine test to be used in diagnosis, treatments and clinical trials.



We felt it important to invest in this trial because cutting down on invasive hospital treatment for our sons - while still getting the doctors the information they need - will give our boys more time to be boys, rather than patients. Quality of life is so important.

Lisa Kuhwald, Mum to Felix



Working to make health checks quicker and pain free

GETTING ACCESS TO THE NEXT GENERATION OF NIGHT SPLINTS

Many young patients with DMD wear night splints to stop their leg muscles contracting and allow freer movement for longer. We wanted to see if there was a difference in two types of splints, one fixed and static (ankle foot orthosis) and one dynamic with a moveable ankle joint (contracture control devices). Health authorities have been reluctant to use the latter as there's no evidence they perform better, and they are more

expensive. Our children wear splints every night for many years of their lives so it's important that they provide the best support possible and are comfortable.

This year we gave a grant of £37,808 to the Neuromuscular Physiotherapy Team at the John Walton Muscular Dystrophy Research Centre in Newcastle to find out what works best.

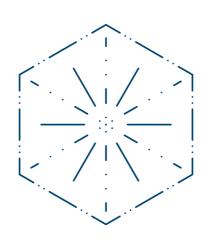


COMMITTED















HELPING THE HEARTS OF BOYS AND MUMS







Pushing research frontiers to make trial data more robust

New drugs are tested first on animals. If they are shown to be promising and safe, the drugs are then tested in humans. But some drugs which have been successful in mice with DMD have later been found to be ineffective in humans with DMD. We are trying to find ways to prevent the painful loss of hope and funds when trials fail, by investing in new technology.

In 2018, Duchenne UK began collaborating with a team based at Newcastle University which is using human heart muscle tissue in the

laboratory that mimics the biology of DMD. This has now successfully been used to test a drug which reduces fibrosis - a symptom of DMD which leads to cardiac failure, a major cause of death from the condition. The team have so far identified one approved medicine that could help reduce symptoms of heart disease in DMD. To allow them to build on this very successful start, this year Duchenne UK has awarded them a further grant of £163, 637.

£163k COMMITTED

> research into DMD. We want scientists with drive, determination and vision, to join us in our fight to end DMD. This year Duchenne UK — and the US charity Parent Project Muscular Dystrophy — are offering up to \$1,000,000 in funding to research

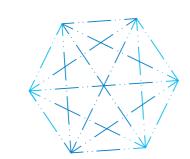
the immunological issues of gene therapy. Solving problems with immune responses to gene therapy will open up a gateway that offers huge potential in finding treatments for the condition. We recognise the creativity and talent of the scientific community and look forward to partnering with them on lifechanging projects.



Supporting mothers who carry the DMD gene

Mothers of Duchenne patients often carry the DMD gene. Up to 17% of Duchenne mothers may have mild symptoms of Duchenne. They are also at increased risk of developing cardiac and skeletal muscle weakness and dysfunction. This year we have committed an extra £53,000 to a study at Newcastle University into cardiac fibrosis, which is aiming to develop new ways to alert female carriers of the gene to heart issues.

While dealing with the devastating diagnosis that my son had DMD, I also learnt that I could have potential heart complications because I was a DMD gene carrier. This was a heavy blow when I was already on my knees. If there was a simple way to offer people like me reassurance, or help us monitor any heart conditions we might have, it would be such a weight off my mind. I'm so grateful that Duchenne UK is exploring this issue.











Duchenne UK is committed to funding

the best, cutting-edge, most effective

projects which focus on minimising







MAKING IT HAPPEN

Duchenne UK has completely changed the landscape of DMD trials in the UK through its ground breaking collaborative approach - the DMD Hub. The DMD Hub, funded by the charity, is a network of hospitals, specialist centres, and experts, all working to increase DMD trial capacity and access in the UK so that every patient who wants to take part in the latest research can.

In the past five years Duchenne UK and its partners have funded 30 posts. We started in 2 sites — we are now in 11.

Through our innovative 'pump priming' funding model, many of the posts we initiated have been taken on by the NHS and are now permanent. We offer training and mentoring to the staff at DMD Hub sites so that they can provide the best support in the running of trials and in helping patients and their families. Pharmaceutical companies now approach us when they want to get trials up and running in the UK because they know we will make it happen.

GLASGOW: ROYAL HOSPITAL • FOR CHILDREN

DMD HUB

No. of posts: 3 Year of funding: 2018 No. of current trials: 4 No. of potential trials: 2



NEWCASTLE: JOHN WALTON MUSCULAR DYSTROPHY RESEARCH CENTRE

No of posts: 6 Year of funding: 2015 No. of current trials: 8 No. of potential trials: 9

LIVERPOOL: ALDER HEY CHILDREN'S NHS FOUNDATION TRUST

No. of posts: 4 Year of funding: 2017 No. of current trials: 8 No. of potential trials: 3

LONDON: EVELINA LONDON

CHILDREN'S HOSPITAL

No. of posts: 2

LEEDS: LEEDS TEACHING HOSPITAL

• No. of posts: 4 Year of funding: 2017 and 2020 No. of current trials: 7 No. of potential trials: 3

MANCHESTER: ROYAL MANCHESTER CHILDREN'S HOSPITAL

No. of posts: 1 Year of funding: 2019 No. of current trials: 1 No. of potential trials: 1

LONDON: DUBOWITZ **● NEUROMUSCULAR UNIT, GREAT ORMOND STREET HOSPITAL**

No. of posts: 6 Year of funding: 2015 No. of current trials: 8 No. of potential trials: 6

DMD Hub Sites

KEY:

Centres of Excellence ondon-GOSH

Hub site

Alder Hey Birmingham Bristol Glasgow Leeds London - Evelina Manchester Oxford Oswestry

Other sites Queens Square Temple Street

OSWESTRY: ROBERT AND AGNES HUNT HOSPITAL No. of posts: 3 Year of funding: 2019 No. of current trials: 2 **BIRMINGHAM: BIRMINGHAM HEARTLANDS HOSPITAL** No. of posts: 1 Year of funding: 2018 No. of current trials: 3 No. of potential trials: 1 BRISTOL: ROYAL HOSPITAL

OXFORD: MDUK OXFORD **NEUROMUSCULAR CENTRE**

FOR CHILDREN No. of posts: 1 Year of funding: 2018

No. of current trials: 2 No. of potential trials: 2



Year of funding: 2019 Year of funding: 2019 No. of potential trials: 7 No. of trials: 1 No. of potential trials: 1

No. of posts: 1

DMD HUB

figures may be lower than expected due to a number of months where sites were not able to recruit due to the COVID-19 pandemic. Clinical trial protocols are being adapted so as to be able to continue treatment in the event of further restrictions. Any trial that is in set-up is still undergoing approvals and has not yet been confirmed at the site.

*Please note that these





SUPPORTING PATIENTS

Trials don't just need infrastructure; they need participants too. We recognised that people who were keen Charities to HM Treasury. We have find out about what was available. So, we created the state of the art Clinical their own versions. Trial Finder (CTF) website. Since January 2020, it has been viewed more than 3,500 times. Our efforts have not gone unnoticed. The CTF has DMD trials. been cited as an excellent example

of digital infrastructure in a report by the Association of Medical Research to take part in trials were struggling to also been approached by international medical bodies who want to introduce

> We are incredibly proud that through the DMD Hub more than 300 boys have been able to take part in







Ashley was recruited for the TamDMD clinical trial, at the Leeds site, following discussions with his consultant, Dr Childs. I had seen the trial was recruiting on the DMD Hub Clinical Trial Finder, which is a brilliant resource and so easy to understand.

The Children's Clinical Research Facility (CRF) in Leeds has such a friendly team. Ashley became really fearful of blood tests prior to him being recruited to this trial but, with the help of the Leeds team, we found a solution and the routine visits for monitoring Ashley are now stress-free. Ashley completed the double-blind element of the trial during lockdown but, thanks to everyone pulling out the stops behind the scenes, Ashley was able to continue into the Open Label phase of the trial.

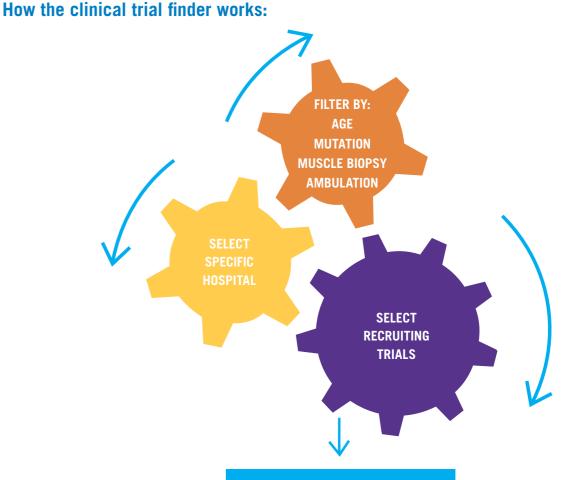
Our experience has been extremely positive with Ashley being engaged in the process throughout. We are very grateful to Duchenne UK and to the CRF team for supporting our family through the process and providing this opportunity."

Lesley Wegg, DMD mum









LIST OF AVAILABLE TRIALS

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DMD CARE UK - BEST CARE FOR ALL

In October 2020, we launched DMD Care UK and, with our partners Joining Jack and the Duchenne Research Fund, invested £130,000 in this nationwide initiative to ensure that every person living with DMD in the UK has access to the best care no matter where they live.

There is an international standard of care agreed by doctors for DMD. It covers things like the types of appointments children should have, and when they should start on drugs to support their condition. But some people in this country have not been receiving that care. We are working with expert clinicians and the patient community to communicate and implement these standards.





DUCHENNE DMD CARE UK **BEST CARE FOR ALL**



PUBERTY & TESTOSTERONE TREATMENT & ADRENAL INSUFFICIENCY INFORMATION PUBLISHED



DMD Care UK Working Group Virtual Meeting, December 2020

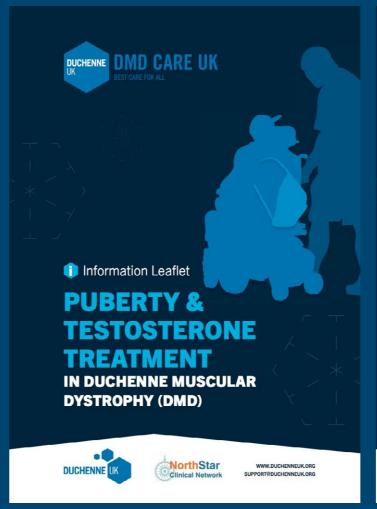


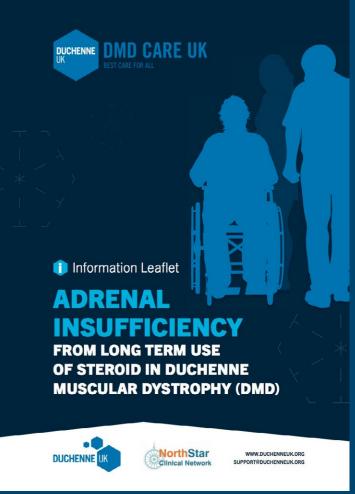






More information leaflets will be published in 2021



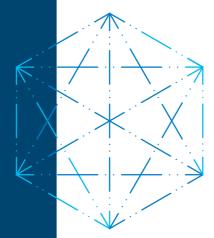






FAMILY FOLDER

We produce a free family folder to help families take control in the dizzyingly desperate first moments of diagnosis. It helps bridge the gaps in the coordination between medical, social and support services, so that the whole family get the best out of life. So far, we have handed out more than 600 folders, both directly to parents and through hospitals.







What a brilliant resource! It literally covers everything that's important around and after the time of diagnosis. There is so little info and support given to parents at the time of diagnosis; the folder really addresses that.

• Your folder has already given me so much valuable information which is helping me to understand and (slowly) come to terms with our situation.





This year every aspect of our lives have been upended by the devastating COVID-19 pandemic. This added burden of uncertainty and fear for families already struggling with DMD has been very hard. But at the heart of Duchenne UK is a strong supportive community. So we reached out to our

community offering support with weekly webinars. These looked at everything from how the coronavirus might affect someone with DMD, nutrition, the impact of the pandemic on clinical trials, and physiotherapy and exercise in lockdown.

2.5K **WEBINAR VIEWS**

I felt so out of control when the pandemic hit. But the weekly Duchenne UK webinars helped me reconnect and find strength in the DMD community, while offering really practical advice about how to cope with what was going on.

Feedback from parents

DECIPHA

This year we gave £30,000 to DECIPHA - a not-for-profit community interest company which offers free educational support to children and young adults with DMD. Managing any child through school and university is

a difficult process; managing a person with DMD — with their added physical, psychological and educational needs — is even harder. You are not alone. Together we can fight the corner for your children. DECIPHA gives you the information and the tools to do so. This year, as well as giving guidance on Education Health Care Plans, DECIPHA has offered support in COVID-19 related issues such as homeschooling and returning to school after lockdown.

£30k













AWARD WINNING PROJECT HERCULES

Project HERCULES is about collaboration, and getting smarter about the way drugs are assessed before they come to market.

Nobody in the entire landscape of drug development had tried this before us.

Getting drug companies - competitors - to collaborate and agree on ways to develop the best evidence to measure the value of a new drug. And engaging with hundreds of DMD families to really

find out what's important to them.



In just 2 and a half years, we have;

CREATED A NEW WAY OF MEASURING THE QUALITY OF LIFE FOR PEOPLE WITH DMD, ALLOWING US TO MEASURE WHAT **REALLY MATTERS** TO PATIENTS AND **FAMILIES**

CREATED A BETTER UNDERSTANDING OF DISEASE CYCLE, ALLOWING MUCH BETTER CLINICAL **TRIALS**



These discoveries allow us to help clinicians and charities deliver what matters most to families, and allow much more effective clinical trials, removing the delays in getting treatments to DMD patients.

Project HERCULES Industy sponsors:











From a clinician's point of view, what Project HERCULES has achieved and its contribution in the process of providing effective treatments to patients is amazing. We, as clinicians, have learnt and continue to learn from the project which will impact future clinical practice and clinical trials to ensure a fair drug assessment by regulators. It's unlike any other initiative I am aware of.

Dr Michela Guglieri, John Walton Muscular Dystrophy Research Centre, Newcastle University

I see this from both sides, as the mother of a Duchenne child, AND as an assessor of drug efficiency for a big pharma company, and when we started Project HERCULES, I don't think anyone thought we could achieve what we have. It has been an amazing success, that will improve the lives of hundreds of Duchenne boys and their families.







HERCULES is a great piece of work in the Duchenne space. There's now a good base for companies to demonstrate the effects of their treatment and its impact. It should speed the whole process up tremendously and I think it's just a shining exemplar to other rare diseases and so I'm hoping to see more of this kind of collaboration.

Professor Ron Akehurst, BresMed



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THE WHEELCHAIR REVOLUTION

It's taken two years and £1,000,000 but on the 9th of November we and our partners Whizz-Kidz took delivery of our Wheels of Change prototype wheelchair, on time, and on budget despite the global pandemic. Our partners at the University of Edinburgh have designed and built a fully functioning wheelchair now known as the "DREAM" which, as we promised, has a set of advanced features not found on any other wheelchair currently in production. Why is this project so

important to us? We know that for a person who can no longer use their legs a wheelchair is an extension of who they are. Current wheelchairs are clunky, old fashioned and awkward. Rather than helping users engage in society and feel included, they act as a barrier. We are so excited to show you our wheelchair revolution - and look forward to unveiling the DREAM in the New Year. Thank you so much to the People's Postcode Lottery Dream Fund for their backing.









D = A = A M





46 YOUNG PEOPLE WITH DUCHENNE & THEIR SIBLINGS TOOK PART IN DUCHENNE UK'S ONLINE **GAMING TOURNAMENTS THIS YEAR.**

Thank you to everyone involved!













DUCHENNE UK CONNECTS PEOPLE LIVING WITH **DUCHENNE THROUGH GAMING**



Neil Robinson, dad to Thomas:

Thank you for organising the tournament - it was lovely to know that all these youngsters who took part have been made to feel special, and despite their own unique problems and circumstances, they had a safe Minecraft place they could go to, and despite not knowing each other, every player shared a common bond. 99

THE DUCHENNE DASH GOES VIRTUAL DURING LOCKDOWN

COVID-19 forced us to cancel our premier fundraising event of the year, the VIP 24 hour bike ride from London to Paris, the Duchenne Dash. Undeterred, we took the event virtual and launched the Duchenne Dash AT HOME. People walked, ran, took to static bikes, or cycled the city streets or country lanes of their neighbourhoods - solo or in teams - to tot up the miles to Paris, using their social networks to spread the news and raise money.

We were overwhelmed by the response. It was a poignant reminder of how the

amazing Duchenne community can pull together when life takes a turn for the worse. 350 people took part - mums, dads, brothers, sisters, grandfathers and grandmothers, friends and neighbours.

And to top it all, the English Rugby Union International squad joined in with the challenge led by their incredible captain, Owen Farrell. Players including Elliot Daly and Jamie George got on their static bikes and offered much needed encouragement to all the people taking part in the Duchenne Dash AT HOME.









THANK YOU TO EVERYONE WHO GOT INVOLVED!



Alex Latham. Dad to Jamie

Jamie's Dashers sent 2 unsuspecting souls to do the Dash indoors on static bikes, cycling 300km to Paris via a virtual Alpe d'Huez in early June. It was much tougher and much hotter than the real thing! 20 other Dashers organised our own DIY Dash London to Brighton and back cycling 170km in early July. We arranged a mechanic and a broom wagon and we even had fish and chips and a beer waiting at the end. We missed the landmarks of the proper Dash - from the Premier Inn at Newhaven to the Champs Elysee - but we didn't miss the dreaded

overnight ferry journey!

The result was beyond our wildest dreams. We raised a phenomenal £500,000!!

























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START STOP CHALLENGE

We started a new fundraiser this year - the START STOP Challenge. People were encouraged to start something new and / or stop something they've been trying to cut out of their lives for the 31 days of October. We had people STARTING running, doing push ups, reading each day, eating more vegetables and STOPPING eating chocolate and crisps, drinking alcohol or fizzy drinks, or overdosing on sugar. It was a challenge whole families could take part in - and our 50 supporters raised more than £13,000.

Thank you to everyone who took part or donated!

We wouldn't have STARTed our mission to end Duchenne without the love and support of the families involved and we won't STOP until DMD is a thing of the past.

























Fundraising was brought to a virtual halt this year by the pandemic. When the London Marathon was cancelled, the organisers launched the 2.6 challenge instead. People took part in activities based around the numbers 2, 6, or 2.6. Thank you to everyone who took part for Duchenne UK. We were overwhelmed by the incredible response from our supporters. Together they raised more than £33,000 for Duchenne UK.















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THANK YOU

Thank You To Our Supporters:

Scientific Advisory Board, Patient Advisory Board, Trustees, Patrons, Dashers, Individual Givers, Corporate Partners, Trusts and Foundations.



Thank you to our Family Funds:

Action for Arvin	Help Harry
Action for Zach	Helping Hayden
Archie's March	Henry's Hurdles
Backing Jack	Hope for Gabriel
Ben vs Duchenne	Hope for Harry
Chasing Connor's Cure	Jack's Mission
Cure 4 George	Jack's Aim
Defending William Against Duchenne	Jacobi's Wish
Edward Steam Team	Jayden's Army
Family Saul	Joe's Journey
Following Felix	Lifting Louis
For Felix	Love for Leon

Lygo Family Fund

Mission Jensen

Moving Muscles for Marcus

Muscles for Mitchell

Project GO

Team Dex

Team Felix

Team Oscar

William's Fund

Ralphy's Fund

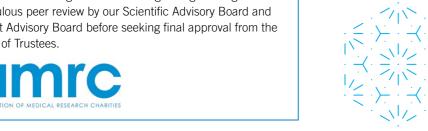
Smile with Shiv

Standing with Jack

Strength for Stanley

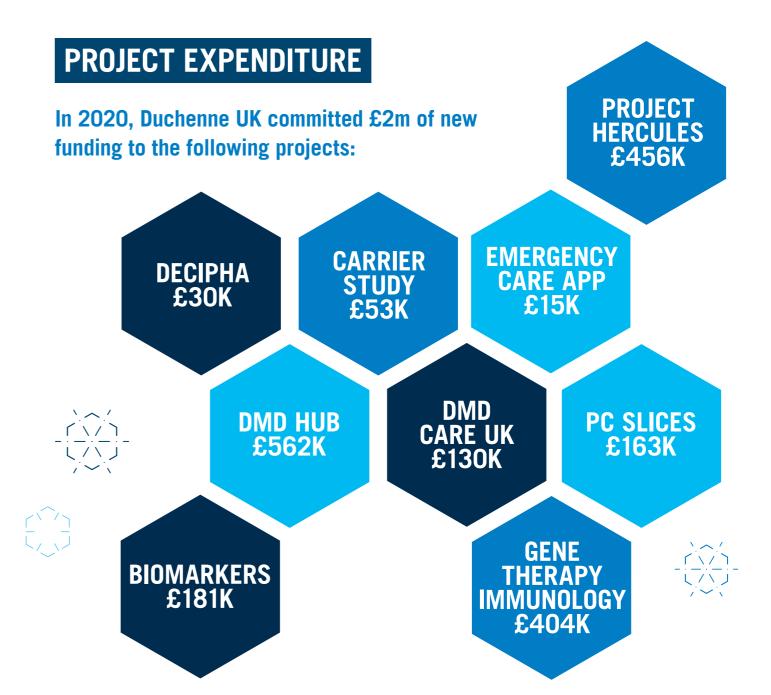
Thank you to our Partner Charities:

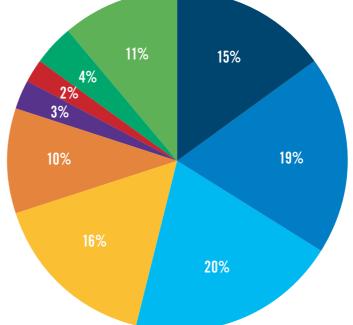
Duchenne UK is proud to be a member of the Association of Medical Research Charities (AMRC). Our grant giving policy conforms to their high standards. All grants go through a meticulous peer review by our Scientific Advisory Board and Patient Advisory Board before seeking final approval from the Board of Trustees.











DUCHENNE UK PROJECT COMMITMENTS TO DATE (2013-2020)





20% Expanding Trial Capacity

Gene Therapy

Approval & Reimbursement

Community

Technology

Nutraceuticals

11% **Other**

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Duchenne UK has one clear aim – to end Duchenne,

a devastating muscle-wasting disease. As the leading Duchenne charity in the UK, we connect the best researchers with industry, the NHS and families to challenge every stage of drug development to make the incurable, curable.

Together, we will find treatments and cures for this generation of patients with Duchenne.