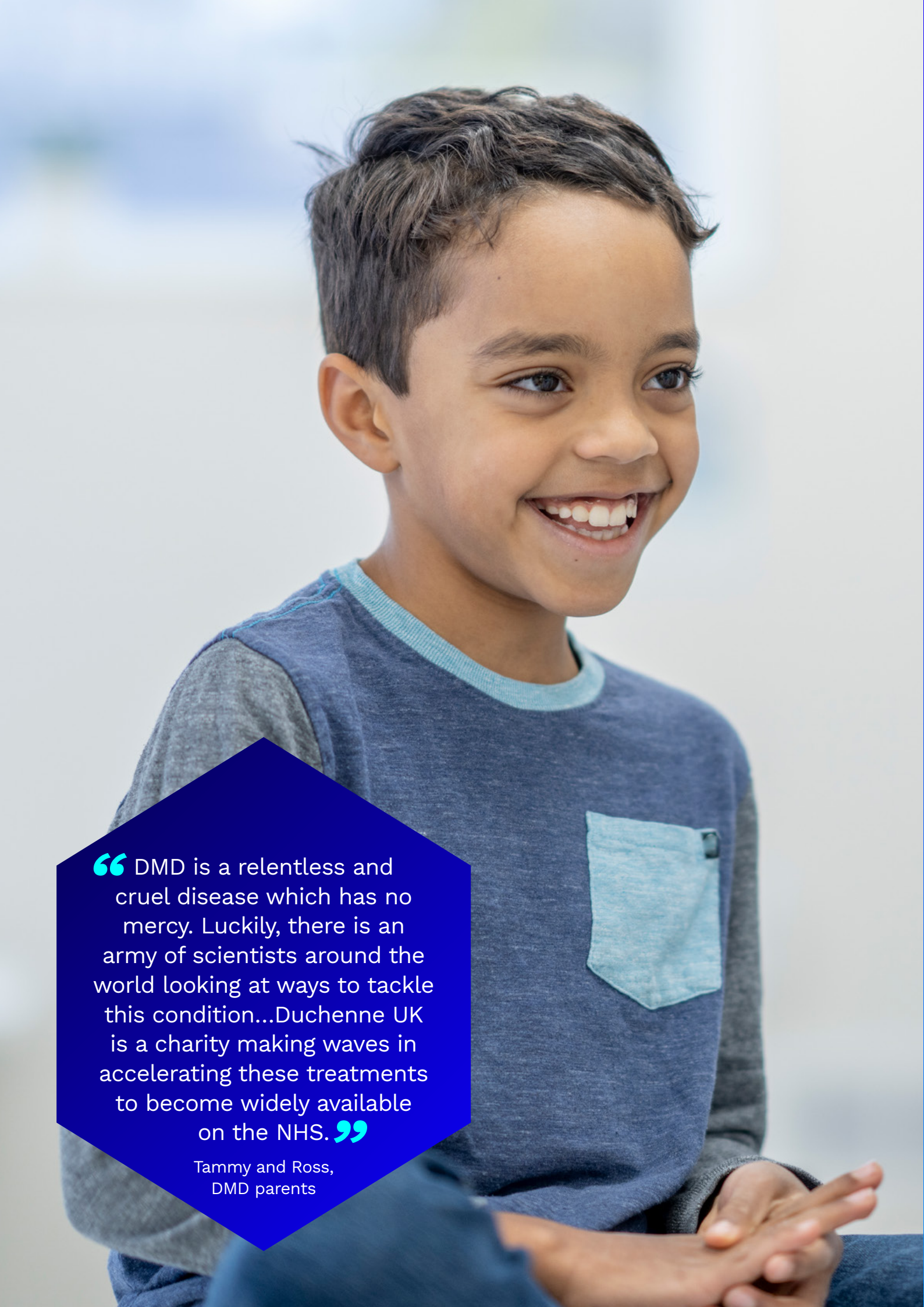
Duchenne UK

Impact report 2022

10 years of Duchenne UK



“DMD is a relentless and cruel disease which has no mercy. Luckily, there is an army of scientists around the world looking at ways to tackle this condition...Duchenne UK is a charity making waves in accelerating these treatments to become widely available on the NHS.”

Tammy and Ross,
DMD parents

Contents

About Duchenne UK

Duchenne UK has one clear aim - to end Duchenne: a severe muscle-wasting disease diagnosed in childhood.

As the leading Duchenne muscular dystrophy (DMD) charity in the UK, we want to bring an end to Duchenne's devastating impact. We're going further to find effective treatments by funding ground-breaking medical research. We're doing it faster, too, by overcoming the barriers in the way of getting treatments to this generation of patients. And we're here to support every family and ensure they receive the best possible care.

We're bringing scientists, the pharmaceutical industry, the NHS and families together to make real change happen.

Together, we will end Duchenne.

Accelerating access to treatments

Improving lives through technology

Funding medical research

Supporting the DMD community

Letter from our founders

Our battle against Duchenne muscular dystrophy is now ten years old. To reflect that milestone, this year's impact report reflects our achievements over the last decade.

In 2012, when Alex and I first met we were broken by a diagnosis that had robbed our sons of their future. By forming a charity, we hoped to claw some of that future back. As this report shows, we have achieved far more than we could have ever imagined. None of this could have been achieved without you. We are so grateful.

Since 2012, we have allied with families in the same predicament to raise £17 million. Our Family and Friends Funds have literally walked through fire, cycled across the UK and France, and carried a 150kg cask of whisky up a mountain. At Duchenne UK we promise these families control over where their money goes. It's an alliance that will only get stronger.

Ten years ago, we were told gene therapy would not be ready in our sons' lifetime. We disagreed. We were seed funders of Solid Biosciences, a start-up established to pursue this new technology, and boys are being dosed with gene therapy in this country now.

We invested in vamorolone - a drug that promised to cause less side-effects than the steroids currently prescribed.

We wanted more trials to come to the UK and more boys to have the chance to take part in new treatments. We conceived and funded the DMD Hub – a network of sites across the country meaning more trials are happening than ever before. An achievement that seems all the more remarkable given that trials in the rest of the UK are falling.

We were horrified to find there were potential treatments out there – but no-one willing to pay for them. We helped change the law to allow medicines to be approved more quickly – and formed Project HERCULES, a unique collaboration between patients, academics and drug companies to ensure more treatments get NHS funding.

We learnt that patients with DMD were dying too young because they were not getting the right care. DMD Care UK is establishing best practice across all the disciplines – and fighting to ensure all medical professionals and parents know exactly what children and adults with DMD need.

And we saw technological advances ignore the needs of our children. We are investing in the SMART Suit and a Dream Chair to make sure the innovation that has inspired the smart phone and the electric car also delivers for the disabled community.

We are ten years old, but feel we are just beginning. Please stay with us in our fight to end Duchenne.

Best wishes,
Emily Reuben and Alex Johnson
Duchenne UK founders

Our impact so far

2012

Duchenne Children's Trust founded

2013

First 10 projects funded

2014

Vamorolone Phase 1 trial funded

ReveraGen BioPharma

2015

Duchenne Children's Trust partners with Joining Jack to becom Duchenne UK

Duchenne UK

SOLID BIOSCIENCES

Seed funding for gene therapy company

2016

DMD Hub founded to expand clinical trials in UK

DMD HUB
Expanding Trial Capacity

2017

Project HERCULES launched to accelerate access to new treatments

HERCULES

SOLID BIOSCIENCES

Exoskeleton suit project funded

Committed £17 Million to research, access and support

2018

Project to design dream wheelchair launched

DREAM
Chair

2019

Stem cell research funded

2020

DMD Care UK launched to ensure the best care for all

DMD CARE UK
Best Care For All

Projects to address gene therapy immunity challenges funded

2021

National trial recruitment database launched

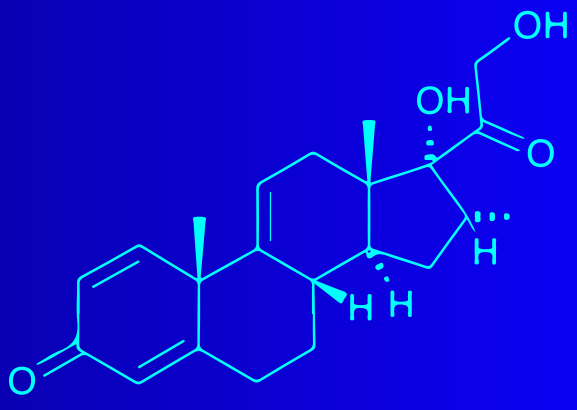
2022

Grant secured for Smart Suit development

SMARTSuit

- First UK patient dosed in gene therapy trial
- NICE review of vamorolone

Discovering new drugs – vamorolone



Duchenne UK has been a driving force in the development of new, better treatments for DMD patients - our work with vamorolone is a prime example.

The current standard of care for DMD, steroids, comes at a cost - such as weight gain, delayed puberty and osteoporosis. We need better for our children.

Finding new treatments

Our founders travel the world meeting people working in the DMD field. At an international conference, they were approached by the founder of the company ReveraGen BioPharma who were developing a potential steroid alternative and wanted to test it in DMD patients. We agreed to provide £750,000, together with our charity partners, Joining Jack and Duchenne Research Fund, to fund a Phase 1 trial.

Making trials happen

We helped to set up the Phase 1 trial through the DMD Hub, offering ReveraGen assistance and support. The data showed the drug to be safe, and the company was able to go to Phase 2 trials - again supported by Duchenne UK — which were also successful.

Getting drugs approved

It's not enough to discover and trial a new drug – it must be made available to the people who need it. We are now working with the Medicines and Healthcare Products Regulatory Agency (MHRA) in the UK to seek approval as quickly as possible.

7-year-old Edward was on the vamorolone clinical trial

"The obvious ideal is a cure, however until this is achieved, anything to enhance life is beneficial! Vamorolone will hopefully have less side effects than a standard steroid, making the best of Edward's and other boy's lives. The trial provided the opportunity for us to help make a difference for boys with Duchenne"

David, Edward's dad

Gene therapy – making it happen

When our founders were diagnosed, they were told that gene therapy wouldn't happen in their sons' lifetimes.

We refused to accept that, and backed some of the pioneering research aimed at tackling DMD at its root cause. Our determination paid off when the first child was dosed in a gene therapy trial in the UK last year.

What is gene therapy?

DMD is caused by a fault in the dystrophin gene. This gene is responsible for producing dystrophin, a protein which protects the muscles.

DMD gene therapy aims to deliver a miniature version of the dystrophin gene carried into the body through a virus (a 'viral vector'), so that the body can produce functioning dystrophin.

Gene therapy and Duchenne UK:

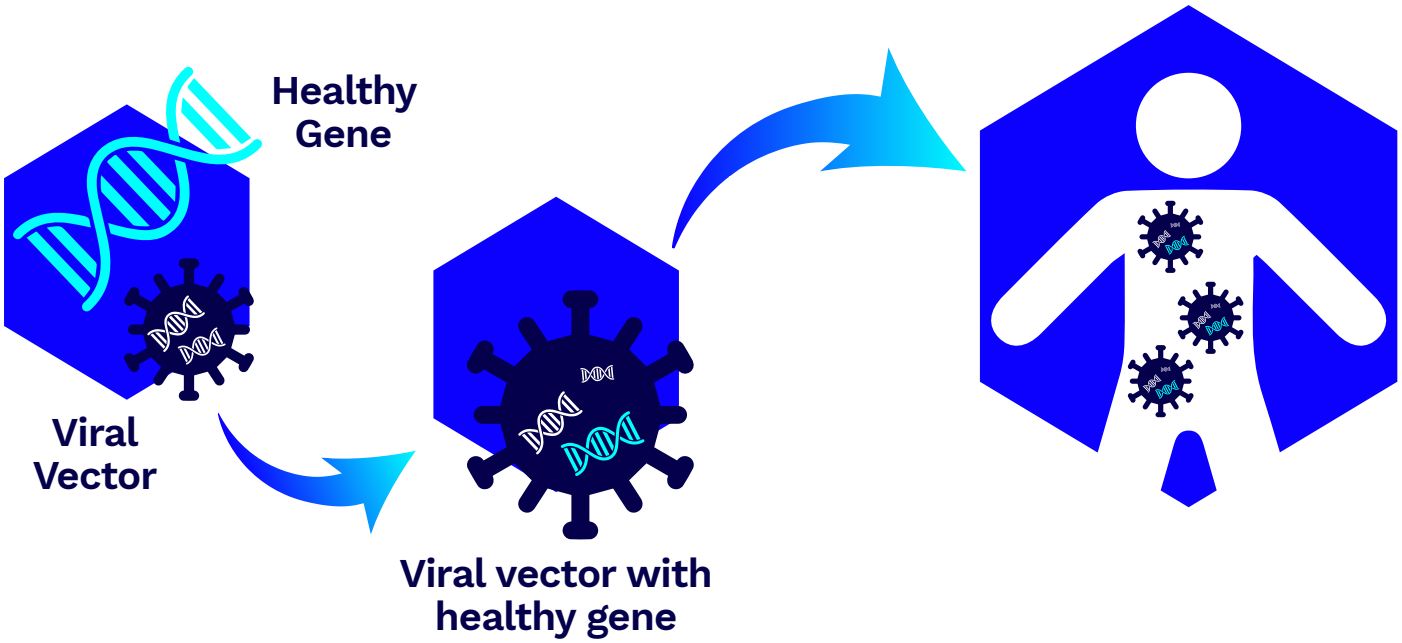
We met parents Annie and Ilan Ganot in 2012. When their son was diagnosed with DMD they moved from London to Boston, to pursue their dream of setting up a pharmaceutical company dedicated to uncovering a gene therapy treatment for DMD.

We recognised their potentiaL, and — along with Joining Jack and the Duchenne Research Fund — gave £5M in 2014 as seed funding. Two years later they went on to raise more than £40m in funding and have since set up Solid Biosciences — expanding to employ 100 staff. The first trial took place in 2017 in the US.

Other drug companies have since joined the field, such as Pfizer and Sarepta. This year, Sarepta's EMBARK trial and Pfizer's CIFFREO trial began dosing boys in the UK.

As well as funding research, we have invested in infrastructure and staff so that there is capacity for these trials to take place in the UK through the DMD Hub.

We are also funding further research into overcoming the limitations of gene therapy, so that the treatment is safer and more people can benefit.



Changing the law – Early Access to Medicines Scheme

Shortly after Duchenne UK was founded, we realised that funding research was not enough.

We were shocked to learn that potential treatments that could have kept children with DMD walking for longer were shelved while long funding negotiations took place.

Our CEO Emily Reuben explains the anguish this can cause:

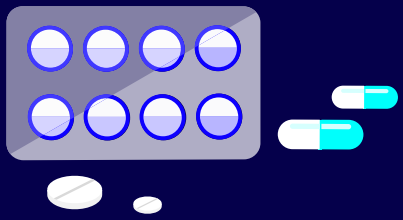
“There is one thing worse than having no drug for your son’s disease. And that’s having a drug that’s approved for use but sitting on the shelf because no one will pay for it”

By the time funding was agreed, precious time had been lost and children who had been waiting for access had lost the ability to walk and were no longer eligible.

So, in 2014, our founders campaigned and won a change to the law — the Early Access to Medicines Scheme (EAMS). This allows patients with rare and life-limiting conditions to have early access to safe but unapproved drugs.

Since 2014, more than 1,200 people living with rare diseases have benefited from the scheme.

Duchenne UK has shown that it is prepared to take on the establishment and win, to get the best for this generation of children, and for all people living with rare and life-limiting conditions.



Increasing access to clinical research - DMD Hub

In our decade fighting DMD — creating the DMD Hub has provided a catalyst to getting treatments to this generation of children.

A clinical trial is often the only hope of getting access to new, ground-breaking medicines, but DMD trials were being turned away from the UK because there wasn't the capacity to run them. We set about finding a solution and came up with the DMD Hub.

It's been a huge success story. The number of clinical trials for ALL diseases in the UK has fallen since 2017 – but thanks to the DMD Hub, DMD trials have defied that trend.

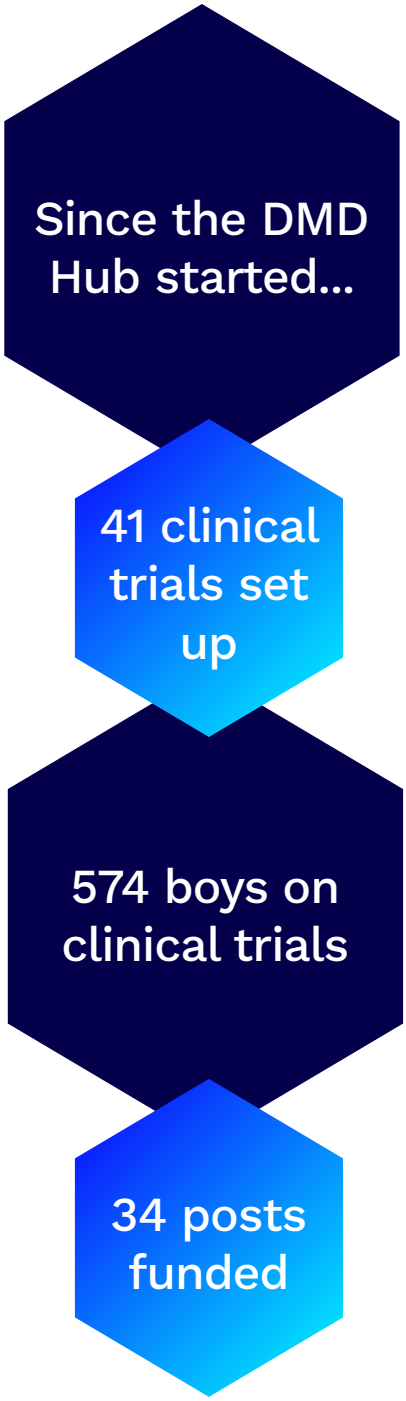
In 2022 we have launched a new project to make access to clinical research fairer and more inclusive — the Central Recruitment Pilot Project. 145 patients and caregivers have already registered on the platform.

Expanding capacity

In 2016 there were only two places where DMD clinical research could be carried out, Great Ormand Street and Newcastle Hospital. Now, thanks to Duchenne UK there are 11 sites, including in Manchester, Liverpool, Glasgow, Bristol and Birmingham.

Funding staff posts

We fund doctors, nurses and support staff to run trials and provide training, mentoring and resources to the hospitals. Many of the positions we created with our funding are now self-sufficient and paid for by the NHS.



Engaging patients

Trials cannot run without participants. Families were finding it hard to find out their options for trials. So in 2016 we launched dmdhub.org. As well as a database of current trials and studies, the site offers support and advice to families and information for hospitals and pharmaceutical companies on setting up and running successful trials.

This year, 4-year-old Charlie from Aberdeen was selected to take part in the EMBARK gene therapy trial. He was recruited through the DMD Hub Central Recruitment Project.

> “After realising Charlie may be eligible for some studies I had contacted the relevant sites throughout the UK but as Charlie received his clinical care here in Aberdeen, out nearest trial site in Glasgow is still 150 miles away, it was unlikely he would be recruited to a site. The DMD Hub’s central recruitment project was possibly the only wat that Charlie could have had a chance at participating in a trial.
>
> Registering was simple. It took around 10 minutes to complete – 10 minutes well spent. Had it not been for this project, the recruiting site at Newcastle NHS Trust would not have had Charlie’s details. He would not have been recruited to the trial.
>
> Knowing that Charlie has played just a small part in this, whatever the outcome, thanks to the opportunity the DMD Hub has given him, fills us with pride in our brave little boy and hope for the future for all those affected by Duchenne”
>
> Jennifer - Charlie’s mum

Accelerating access to innovative treatments - Project HERCULES

Project HERCULES is an award-winning initiative created by Duchenne UK, bringing together competing drug companies and regulators with the aim of getting new drugs approved faster.

In 2017, we persuaded seven pharmaceutical companies and health organisations to come together to create a consistent method of assessing evidence from DMD trials. Where there is clear-cut evidence of a treatment's effectiveness, applications for funding from regulators are much more likely to be successful.

By 2019, another pharmaceutical company had joined. We were awarded the prestigious Eurodis Black Pearl Award in recognition of our innovation.

In 2021. we launched the quality of life tool DMD QoL. It measures the outcomes of treatments in a way that is patient-centred, considering aspects such as mental health and the ability to socialise, to help regulators make decisions on drug approval.

This year, we've been working on a toolkit of resources to promote awareness and understanding of how HERCULES works in our clinical and patient communities. We are also publishing a natural history model which shows how DMD progresses without medical intervention - an essential baseline which can be used to show the effectiveness of treatments.

"Experience of companies trying to get payment from health services for new treatments for DMD showed that important tools for demonstrating the value of new pharmaceuticals through Health Technology Assessment agencies, such as NICE in England, simply did not exist. In particular, the understanding of the experience of the typical patient and family; the effectiveness and costs of current interventions; the proper way to capture the value of improvements in health and survival and the best way to demonstrate the effects of treatment over time were all poorly understood. Project HERCULES has addressed these deficiencies through a remarkable collaborative effort between patient organisations, companies, reimbursement agencies and universities. It will have made bringing an effective treatment for DMD to patients quicker and easier and provides an example for other severe rare diseases."

Dr Ron Akehurst, Professor of Health Economics at University of Sheffield

HERCULES

So far, HERCULES has delivered

- A natural history model (a model of how the disease progresses without treatment) for DMD and new mortality data analyses
- A bespoke quality of life measure for DMD, the DMD QoL
- A burden of illness study to map the impact of DMD on patients, families and carers
- An economic model, which can be adapted by individual companies, to support the assessment and reimbursement of DMD treatments
- A review of the burden of side effects from long-term steroid treatment
- Analysis of patient data to inform the natural history and burden of illness workstreams.

Harnessing innovation – DMD tech

There have been huge advances in technology in the ten years since Duchenne UK was set up - but disabled people are often at the back of the queue to benefit from them.

As DMD steals our loved ones' strength and mobility, modern technology should be there to help them.

That's why we're investing in cutting-edge equipment to prolong strength, dignity and independence.

The SMART Suit - harnessing robotics to help people with DMD

This year, we were delighted to secure a large grant to help make our dream project a reality.

The idea is to create an exoskeleton suit which straps to the upper body, allowing people to carry out day-to-day activities when the upper limbs have become too weak to do so.

Solid Biosciences initiated the project, which we helped to fund in 2019. This year, Duchenne UK has taken on the development of the suit. We won a £1.25m grant with fellow charity, Spinal Muscular Atrophy UK and the University of Liverpool from the People's Postcode Lottery to continue developing the product and bring it to market. We recruited a project manager, appointed an industrial design project partner, and a market strategy partner.

Research at the University of Liverpool has now begun to uncover the needs of people living with DMD and SMA, to make sure that the product puts disabled people first.

SMARTSuit

Creating a wheelchair fit for modern life

Wheelchair users want to do the things that others take for granted — to live a full and active life. But sometimes their chairs hold them back.

Wheelchair design hasn't changed in decades. In 2018, we started our wheelchair revolution, partnering with Whizz-Kidz and the University of Edinburgh to win £1m from the People's Postcode Lottery Dream Fund to develop a new chair.

We created a prototype which puts young wheelchair users at the centre - for instance, allowing them to rise to adult standing height so they can talk to people face to face, giving the chair the ability to mount curbs and allowing it to play music.

This year, we have worked on creating a second version of the prototype – a key step to bringing our dream chair to market.

The best care for all – DMD Care UK

We passionately believe that everyone in the UK should get the best standard of care for DMD, wherever they live and whatever their circumstances. Families told us that this was not the case - so together with Joining Jack, the Duchenne Research Fund, and the North Star network of neuromuscular experts, we launched DMD Care UK in 2020.

Our co-founder Alex Johnson explains:

> “Parents were coming to us worried and confused about what care their children were being given - which varied wildly across the country and the world. For instance, some doctors were refusing to prescribe things that were standard in other countries. We decided to take a stand, to find consensus by working with medical teams to determine what the best care should be for Duchenne patients in the UK – we want to ensure no one misses out.”

Last year our first recommendations were published on bone and endocrine care. This year we published our guidelines on cardiac care which were endorsed by the British Cardiovascular Society and published in the BMJ Open Heart journal. Children on corticosteroids have delayed puberty so we've also created a puberty and testosterone guidance booklet. We are now working on respiratory care guidelines.

Our work has potentially life-saving results. Many families and medical professionals are unaware of the specific needs that people with DMD have in emergencies, so we launched steroid wristbands and the In Case of Emergency app. The app gives medical professionals access to essential information in emergencies to ensure DMD patients get the right treatment.

Alex Johnson knows
just how vital it can be.

“My husband had to take to Jack to A&E because he had fallen. It turned out that Jack had fractured his ankle and the medical team in A&E were really pleased to have the information that the app provided. It gave them all Jack’s relevant patient information, how they needed to treat him because he takes daily steroids, that they needed to watch for things like fat embolism syndrome, what anaesthetics he could (and couldn’t!) have, how to treat his fracture, who to contact, a list of his medications and his recent clinical results.

Most doctors in A&E rarely come across Duchenne patients so having this information in an easy to digest format can be invaluable. It can help clinicians, result in reduced stress for families, better outcomes for patients and ultimately, it could save lives.”

The newly published cardiac care recommendations are already have a positive impact on families’ experience of care:

“My son has DMD and I’m a carrier. I knew that as a carrier taking care of my heart is important, so had been asking about getting checks for three years. When the new recommendations came out, suddenly there was clear evidence and guidance for what I needed – I managed to get a scan booked in straight away!”

Sabrina, DMD mum

Joining our fight - Family & Friends Funds

As a charity set up by two mothers, we know the power and potential of families.

All too often patients and families are left feeling utterly helpless, forced on a DMD journey with no say about what will happen.

To empower parents, we launched Family & Friends Funds - a simple way to raise money under their own child's name without the headache of setting up their own charity. And families can choose which Duchenne UK projects they want to invest in.

Our first funds were set up in 2016. We now have more than 40 Family & Friends Funds. Together they have raised more than £3.2 million!

There are so many fundraising activities we could mention but here are a few that caught our eye this year: Project GO raised more than £70,000 with a Kiltwalk through Glasgow and a climb to the summit of Lochnagar accompanied by a 150kg whisky cask! A supporter of For Felix swam the 39KM Loch Lomond, raising more than £7K. And Jacobi's Wish raised more than £19K with their regular golf day.

"We are able to look at research projects and decide we would like to put X amount of money into that. For the people that support us and the people who do events for Duchenne UK, it's important for us to be able to say to them: 'Actually with the money you've raised, we have contributed it to this clinical trial, and this is what stage it's at.' It makes a difference for people to think I am doing something to support research specifically for DMD."

Kirsty, mum to George and Owen (pictured right)

The energy, enthusiasm and sense of community is awe-inspiring. Thank you to these families — the backbone of our organisation — whose dedication allow us to drive through change for those we love with DMD.

And a very warm welcome to Fight for Finn, Life and Hope for Lenny, Doing it for Dexter and Matthew's Mighty Mission who join us this year!

Interested in finding out more about Family & Friends Funds?

Get in touch with
funds@duchenneuk.org

> "Duchenne UK is a charity making waves in accelerating these treatments to become widely available on the NHS. We are so proud to add Matthew's Mighty Mission as a Family & Friends Fund for this amazing cause."

Ross and Tammy, Matthew's parents (pictured right)

London to Paris to End Duchenne - Duchenne Dash

What started as a novelty fundraising suggestion to cycle from London to Paris by the Channel 4 News anchor Krishnan Guru-Murthy ten years ago has gone on to raise more than £6.5M for Duchenne UK.

The journey of the Duchenne Dash echoes that of Duchenne UK. A passionate and committed individual was moved by the injustice and heartbreak of people affected by DMD to take targeted action, with life-changing results. It started with just 30 riders. Now, a decade later around 160 riders take part each year.

Thank you to the generosity of the corporate sponsors, volunteers and the courageous cyclists who battle through the weary miles, a sleepless night on the ferry and the cobbles of Parisian streets to experience the glory of riding to the Eiffel Tower on this very special bike ride. And thank you to our patron and chair of trustees, Krishnan Guru-Murphy, for his constant support – and willingness to risk ridicule by doing it himself.

> “I knew that everyone I know would find the idea of me cycling to Paris in 24 hours preposterous – they would understand it would involve a lot of training and a lot of pain. SO it would definitely raise money. And it did.”
>
> Krishnan Guru-Murthy

This year the Dash raised more than £650,000

From this

2013

30 plucky cyclists with a map and one support car and a doctor picking their way to Paris

To this

2022

150 plus cyclists taking part in a gruelling challenge, fully catered, with bike engineers and medics on hand - ending with a gala celebration in Paris.

For our 10th year — and our first back to Paris after the pandemic — CEO Emily Reuben took on the Dash for the first time: "I've cheered from the sidelines so many times, so to mark a decade of the Dash I had to join in! I found it really challenging, but so worthwhile. I was swept along by the camaraderie of the Dashers and amazed at how everything was laid on to make it the best possible ride for the cyclists. It was hard work but so much fun - I will never forget the sense of achievement as we rode up to the Eiffel Tower!"

And at the gala dinner Emily's son Eli, and Krishnan's son Jay performed for the riders with their band Alcatraz – the perfect way to mark a decade of the Dash.

Support from the rugby world - Saracens

The rugby world has rallied around Duchenne UK from the start. The sport's contribution to raising awareness of DMD has been immeasurable. England and Saracens rugby star Owen Farrell is a family friend of our founder Alex Johnson, whose son Jack has DMD. Every time he scores a penalty for club or country, he links his fingers together to make the iconic Joining Jack salute.

"Congratulations Duchenne UK on a decade of amazing work helping people like my friends the Johnson family living with DMD. You've showed real grit and determination fighting for a better future for Jack and people like him. You have my full support."

Owen Farrell

And to mark his 100th cap for England, he invited Jack and his younger brother James to join him on the pitch at Twickenham for the singing of the national anthem.

The three-time champions of Europe, Saracens Rugby Club, came on board with the charity in 2019. Since then the club has raised tens of thousands of pounds, organising regular dinners, celebrity auctions, prize draws at games, placing adverts in programmes, and adding donations to goods bought at its shop. Thousands of rugby fans have now heard of this rare disease.

Sport can feel exclusive if you have DMD, so the club hosted a gaming day at their home stadium StoneX in London in 2019. Dozens of children with DMD were invited and had a truly memorable day.

In 2022 Saracens hosted a Showdown against Bristol Bears at Tottenham Hotspur Stadium, with Duchenne UK as its featured charity. More than 40,000 people attended to see Saracens prevail 27-23!

"Our partnership with Duchenne UK has been so rewarding. Working with such a passionate group of people is really inspiring. We know that every pound we raise will be spent well, helping to find treatments for DMD. Saracens is a family club and Duchenne UK feels part of that family."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BRITISH AIRWAYS

Thank you to everyone who has supported us this year!

To our Family and Friends Funds and partner charities:

Access to Life
Action 4 Arvin
Action for Zach
Archie's Army
Archie's March
Backing Jack
Ben Vs Duchenne
Changing Charlie's Future
Chasing Connors Cure
Cure4George
Defending William Against DMD
Doing it for Dexter
Edward Steam Team
Elliot's Endeavours to End Duchenne
Family Saul
Fight for Finn
Following Felix
Help Harry
Helping Hayden
Henry's Hurdles
Hope for Gabriel
Hope for Harry
Jack's Aim
Jack's Mission
Jacobi's Wish
Jayden's Army
Joe's Journey to End Duchenne
Life & Hope for Lenny
Lifting Louis
Lygo Family Fund
Matthew's Mighty Mission - to end Duchenne
Mission Jensen
Moving Muscles for Marcus
Muscles for Mitchell
Project GO
Ralphy's Fund
Smile with Shiv
Standing with Jack
Strength for Stanley
Team Dex
Team Felix
Team Oscar
Together for Rhys
William's Fund
Alex's Wish
Joining Jack
Chasing Connor's Cure
Decipha
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