

Impact report 2023

A landmark year of achievement and impact





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About Duchenne UK

Duchenne UK has one clear aim - to end Duchenne muscular dystrophy (DMD); a severe muscle-wasting disease, diagnosed in childhood.

As the leading 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, and we're doing it faster, by overcoming the barriers in the way of getting treatments to this generation of patients.

We are also here to support every family and to make sure they receive the best care.

We work with scientists, the pharmaceutical industry, the NHS and families 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



Message from our founders

This year has seen a breakthrough – the first ever drug approved for use on children with DMD, Vamorolone, designed specifically for their condition. It's reaching patients because Duchenne UK and our partner charities funded the early-stage clinical research to test this drug in patients, when no-one else would.

When we first met in 2012, we were broken by a diagnosis that had robbed our sons of their future. But through that pain, we never lost sight of our goal; to claw some of that future back for our sons and those in families like ours.

We were told the standard medication for children with DMD, steroids, would keep them independently mobile for longer, but with harmful side effects. We didn't think this was good enough, and invested in finding better treatments.

Now Vamorolone has been approved for use in people with DMD in the US and Europe, and we hope the UK will follow soon.

The way Duchenne UK has helped achieve this significant milestone towards an improvement in how DMD is treated is a perfect demonstration of what Duchenne UK is about. A new, better drug, successfully coming to market for children with DMD, is proof that our charity has impact, and that we get results for our community of families and the people who so generously support our cause through fundraising.

And that's not all.

Gene therapy

When our sons were diagnosed, we were told gene therapy would not be ready in their 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 clinical trials taking place right now in the UK.

Best practice in DMD care

We learnt that patients with DMD were dying too young and their quality of life negatively impacted because they were not getting the right care. DMD Care UK is driving best practice across all medical disciplines so that everyone living with DMD in the UK has access to the best care. Following the publication of our cardiac guidelines in 2022, this year we launched new guidance on how DMD affects bones, helping parents know exactly what children and adults with DMD need.

Innovative technology

We're driving technological advances, for the benefit of DMD children and others losing their mobility. We're leading a collaborative R&D project to develop the first ever arm assist device to support arm function. Its third version is undergoing testing now. It's a project that puts the needs and aspirations of DMD children at its heart, with the potential to bring benefit to other people with arm mobility issues.

All of this couldn't happen without the incredible, selfless support of our funding partners, our Family & Friends Funds and our individual fundraisers. We offer our sincerest thanks to every one of them, whose support means we can continue on our mission to find treatments for DMD.

With love and thanks,

Emily Reuben OBE and Alex Johnson OBE Duchenne UK founders

Research and access to new treatments

For more than a decade, Duchenne UK has been And we are proud of the work our DMD driving pioneering research into treatments for DMD. This year saw a breakthrough; the regulatory approval of the first ever treatment for all people with DMD, Vamorolone.

We are proud to have contributed to this milestone in making new treatment options available to people living with DMD.

Hub continues to drive to enable access to treatments.

A global trial, which was run in the UK through our DMD Hub, showed Givinostat, another drug seeking regulatory approval in DMD, can slow disease progression and delay loss of ambulation.

11 trial sites

Our DMD Hub's network now has DMD trial sites in Alder Hey, Birmingham, Bristol, Glasgow, Leeds, London (Evelina), London (GOSH), Manchester, Newcastle, Oswestry, and Oxford, which have been undertaking trials for people with DMD from across the UK in 2023. By the end of 2023, the DMD Hub has run 41 trials and 574 people with DMD have taken part in a DMD Hub trial since it was set up in 2016.

Research portfolio

Our extensive portfolio of research projects that we funded in 2023 includes research into stem cells and their ability to regenerate muscles in DMD, developing new electronic clinical outcome assessments that can be used in DMD clinical trials, and a research programme on nutrition and weight management in boys living with DMD.

Project HERCULES

In 2023, our Project **HERCULES** helped to support health technology assessments of new treatments for DMD such as Vamorolone, which is currently being assessed by NICE.

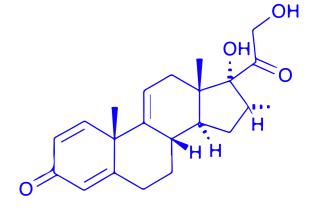
DMDhome digital platform

Our DMDhome digital platform that we are working on with Aparito has entered the validation phase. DMDhome uses video capture and computer vision analysis to measure limb function in DMD. The study will aim to validate this new approach for use in clinical trials and potentially in the approval of new medicines for DMD.

Breakthrough Vamorolone approval

Research funded by Duchenne UK produces the first ever drug for treatment of all children with DMD

It's not enough to discover and trial a new drug. It must be approved by regulators, too. Now this final step has taken place, it's expected that the first ever treatment for all DMD patients will become available in 2024. This breakthrough is the direct result of research and trials funded by Duchenne UK over the last decade.



win a £6 million grant from Europe's Horizon 2020 fund. Two years later, 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. Santhera Pharmaceuticals hopes to bring the drug to market in Europe in the first half of

A first in DMD medicines

Vamorolone, sold under the brand name Agamree, has been approved in the United States for patients from the age of 2 years old, and approved in Europe for patients aged 4 and up. In the UK, we are engaging the National Institute for Health and Care Excellence (NICE), which will decide if the new drug can be made available on the NHS in England, to support a decision in mid 2024.

Better treatment for our children

Currently, DMD patients are given steroids to reduce the progression of the disease. But this comes at a cost; weight gain, delayed puberty and osteoporosis. Duchenne UK wanted better for DMD children.

In 2015, Duchenne UK, Joining Jack and Duchenne Research Fund invested £750,000 towards a phase 1 clinical trial in boys with DMD, which then enabled ReveraGen BioPharma to



Gene therapy trials

Secondary endpoints met in EMBARK study, and we are funding new research into optimising gene therapy for people with DMD

Funding for gene therapy

DMD gene therapy aims to deliver a working version of the dystrophin gene, so that the body can produce functioning dystrophin, which is absent in people with DMD. The potential benefits of this include protecting the muscles and preventing the progression of DMD.

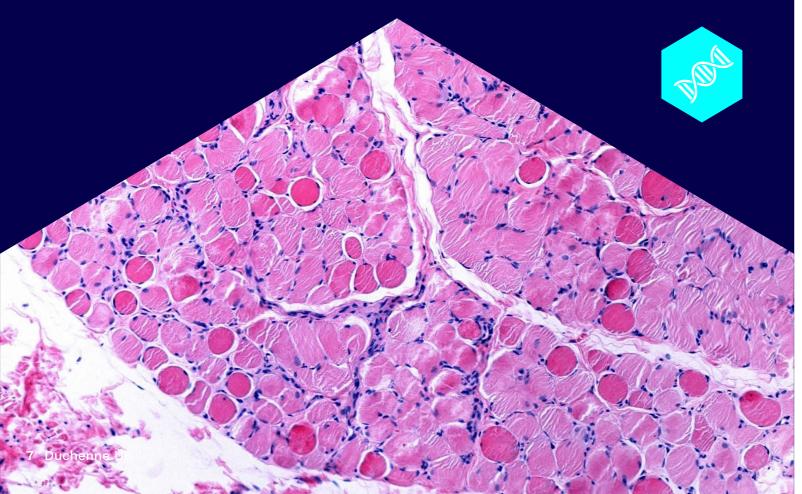
In 2014, Duchenne UK, Joining Jack and the Duchenne Research Fund gave £5 million in seed funding into gene therapy research, recognising its potential. Last year, the DMD Hub recruited the first boy to be dosed on a gene therapy trial (EMBARK) in the UK.

Secondary endpoints met

The results of the EMBARK gene therapy Phase 3 trial showed that the drug didn't achieve a statistically significant difference in the key measure chosen to test its effectiveness, known as the primary endpoint. This endpoint is the NorthStar Ambulatory Assessment, which measures motor function in people with DMD. However, other measures of the trial, known as secondary endpoints, were met.

Our new research into improving gene therapy

While gene therapy brings a lot of hope, there is still a lot more that we need to understand about it, and how it can be optimised as a treatment. We are also funding further research into overcoming the limitations of gene therapy, such as the production of antibodies against it, so that more people can potentially benefit.



Our DMD Hub central recruitment database

Making sure everyone with DMD has access to clinical research trials

When Duchenne UK's founders started the charity, they were shocked to learn how limited the opportunities to take part in DMD clinical research trials were for people living with DMD in the UK.

To solve the issue, in 2016 they created the DMD Hub. It's a collaboration between Duchenne UK and neuromuscular centre of excellence; The John Walton Muscular Dystrophy Research Centre at Newcastle University. The DMD Hub is a network of clinical trial sites that have dedicated, trained staff, funded to carry out research studies for DMD.

As the number of clinical trials has increased in recent years, so the DMD Hub Central Recruitment Pilot Project takes the DMD Hub's original mission a step further. Funded by Duchenne UK since 2022 and coordinated by Newcastle University, this project has established a national contact list of children and adults with DMD who are interested in taking part in research studies.

The database contains information that supports clinical sites to identify potentially eligible candidates for research studies, as well as the contact details for their main neuromuscular centre and clinician. It means people with DMD are now able to take part in research studies, regardless of where they live.

By the end of 2023, 208 DMD patients were registered with it. Eight studies have used the platform to recruit people for trials and 22 people have been recruited as a direct result of

using it. Every commercial DMD trial opened in the UK since the establishment of the Central Recruitment Database has recruited patients using it.





Promising results

Our global reach means that our DMD Hub is involved in the world's leading drug trials, all focused on finding treatments for DMD patients as soon as possible.

Our DMD Hub recruited patients in the UK for a worldwide trial of Givinostat.

The trial was delivered at four of the eleven DMD Hub sites; Newcastle, Alder Hey, Oswestry and Great Ormond Street Hospital.

The phase three evaluation of Givinostat demonstrated that it can slow DMD progression in boys aged six and up. Givinostat is a histone deacetylase (HDAC) inhibitor that is produced by the Italian pharmaceutical company Italiarmaco.

Getting treatment to patients

The European Medicine Agency (EMA) is now beginning its approval process, and US regulators are doing the same.

This is good news for patients in the UK. If Givinostat is approved by the EMA, its producer can use this approval to seek a quicker approval by the Medicines and Healthcare products Regulatory Agency (MHRA) in the UK.

We aim to work with Italfarmaco to support the next steps towards UK regulatory approvals to ensure that everyone living with DMD who could benefit from this treatment has access to it.

Care for DMD patients and families

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 NorthStar network of neuromuscular experts, we created DMD Care UK, which continues to improve the standards of care for DMD patients and their families.

Our DMD Care UK psychosocial programme developed a referral pathway for children with DMD and patients started to be referred to it in 2023. This year, DMD Care UK also launched a guide on bone care and health for people with DMD and their families.

Bone guidance

Our DMD Care UK programme published a new bone health guide for people with DMD and their families in 2023.

Diet and nutrition

We launched a new project to drive better care for nutrition and weight management in DMD. The aim is to develop guidance and materials to support better diet and nutritional management in people with DMD and to gather evidence, through research, of the impact of steroids on weight.

Psychosocial help

Our DMD Care UK programme is developing best practice in supporting people with DMD.



DMD Care UK launches new recommendations focused on delivering the right bone care at the right time

The best for all

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 NorthStar Network, we launched DMD Care UK in 2020. It's a nationwide initiative aiming to ensure that every person living with DMD in the UK has access to the best care, no matter where they live. The project brings together expert clinicians and the patient community to agree, communicate and implement DMD standards of care recommendations.

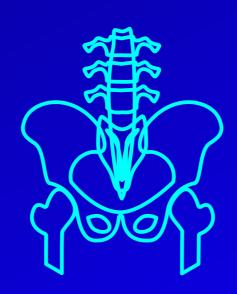


Guidance on care for bones

This year, guidance was published for patients on the best standards of care regarding bone health in DMD.

It is crucial that people with DMD receive the highest standards of care for their bones as they are at high risk of developing osteoporosis, where bones become weak and fragile and break or fracture more easily. The guidance recommends a range of measures to keep bones healthy for longer.

The guidelines aim to ensure that every DMD patient in the UK can access the recommended care through the NHS. A library of guidelines for the correct care of people with DMD are available, including recommendations on cardiac care.





Improving mental health outcomes in DMD

Our DMD Care UK programme is working with the DMD community to develop best practice on meeting the psychosocial needs of patients and their families

It is possible to live a happy and fulfilling life with DMD. Duchenne UK is supporting that ambition by launching a new workstream to identify and tackle the biggest issues affecting the mental health and learning of people with DMD.

Psychosocial care needs

As well as physical needs, children and young people with DMD may experience an impact on their mental, psychological or social wellbeing - also known as psychosocial health. While they may benefit from specific support, these difficulties are often overlooked or misunderstood, and it can be hard to get professional assessments, support and interventions.

Improving care

A new DMD Care UK programme is addressing this shortfall through its psychosocial work stream, chaired by Professor Volker Straub. The Duchenne Research Fund and Joining Jack have funded the key team members for three years to carry out research and develop guidelines on best practice and assessments for psychosocial care in the UK.

The project will keep track of the unmet need from the DMD community, looking at how many referrals and what types are requested. This information will be used to show the level of need to the NHS and make a better case to fund these kinds of posts across the UK.

Alongside the development of clinical recommendations, assessments and consultations with patients have begun. A referral pathway for children with DMD has been developed with patients now being referred to it.



Technological advances

Technology is advancing at an accelerating rate, but disabled people are often the last to benefit. As DMD steals our loved ones' strength and mobility, modern technology should be there to help them. That's why we're investing in the development of cutting-edge equipment to prolong strength, dignity and independence – and putting DMD children at the heart of the research process.

Reimagining wheelchairs

We are undertaking extensive research with young people with DMD to completely rethink how wheelchairs can help them live their lives.

Prototype developed

A third SMART Suit prototype undergoes user testing ahead of further development with the DMD community in 2024.

Innovative technology

We have grown our technology team this year and will be seeking to dramatically change the technology landscape for people with disabilities.



Keeping our children moving

How young people with DMD are helping to develop the latest innovations in mobility

Applying tech to disability

Despite recent advances in robotics, there is no technology available to help people with disabilities with the mobility of their upper bodies. But that is changing, thanks to a project led by Duchenne UK, in collaboration with Spinal Muscular Atrophy (SMA) UK and the University of Liverpool's Inclusionaries Lab.

Together, we are creating the SMART Suit; a discrete, wearable exoskeleton controlled by the wearer. People with DMD need this kind of assistance to extend their independence for as long as possible, as their limbs become weaker with age.

Users driving development

People with DMD are right at the centre of this project. It's their testing of prototypes, and feedback on what works, that is informing the design direction of this project.

Our collaboration brings together Duchenne UK and SMA UK's lived patient insight with the University of Liverpool's world-leading expertise in user-centred design research.

Alongside our project partners, we've created a team of delivery partners with skills and expertise spanning user engagement, technical, engineering, service provision, health economics and market access.

We engage with the people who will benefit from the SMART Suit at every stage, to ensure that the final design is both functional and desirable; it's got to be something potential users will want to wear.

Collaborative approach

SMARTSuit

The project has raised £2 million, with £1.25 million of this being thanks to an award from players of People's Postcode Lottery. We are following an iterative development approach, building prototypes and testing them with members of the DMD and SMA communities. We have just built and tested our third development prototype and will continue to work closely with our partners and the community as we enter the next phase of product development.

Our technology projects are quite unique – as a patient organisation, our primary motivation is delivering products that meet the needs and desires of disabled people. We continue to work alongside other organisations who share our values to drive progress in this area. We hope to find a commercial partner in 2024 to help us take the SMART Suit to market by 2025.



Developing the Dream Chair

In 2018, People's Postcode Lottery funded a collaboration between WhizzKidz, Duchenne UK and the University of Edinburgh to develop the first prototype of the Dream Chair. The outcome of the project was the mark 1 prototype, which had lots of unique features.

In 2021, we took on the Dream Chair project and explored manufacturing options for the mark 1 design concept.

Could the concept be made using existing manufacturers and production lines?



Prototype

We explored what's possible using current production methods by working with a wheelchair manufacturer to create the mark 2 prototype. We invested our own money in doing this.

We then took the prototype back out to the community and clinical stakeholders for review in January 2023. The feedback from the DMD community was mixed, so we decided to dig deeper into the underlying issues and commissioned some user research into powered wheelchairs.

This research included observing people with DMD using their powered wheelchairs in different environments and speaking to them about their experiences of being a powered wheelchair user. We learnt about the challenges of stability over rough and uneven terrain, the annoyance of the beeps from the controller, the importance of how it looks and much more.

We will be taking these learnings forward into three concept visions - opportunities for innovation that would transform the market and answer the expectations of the community.

We'll conduct a benchmarking exercise to ensure that our chosen vision for the project is transformative when compared to current wheelchairs on the market.

We will be engaging the community at every stage, and working in collaboration with organisations who share our vision and values.



Duchenne UK's fundraising community

Our impact in treating DMD could not have been achieved without our community of funders. From the children raising money through Duvet Days to the Family & Friends Funds energising communities to donate to our funding partners who see the potential of a large grant. People's Postcode Lottery, our major donors, grant funders, corporate donors, people who make regular donations, and those who leave money in legacies and in memory of someone.

They are all united behind our mission to end DMD.

To everyone who has helped us this year, a huge THANK YOU!

£27 million

raised over the charity's lifetime

Our flagship event,
the Duchenne
Dash, raised
more than
£832,000
over one weekend

Support from Her Majesty

Her Majesty The Queen has been one of our most steadfast supporters since we began the fight to end Duchenne, and we really appreciated her support in 2023.

From holding an event for us in February, to celebrating our Director of Research, Dr Alessandra Gaeta, on International Women's Day for her leadership of our SMART Suit project.

Thank you to Her Majesty The Queen, for your support this year.





Our partner charities

Alex's Wish and the Duchenne Research Fund, are key funders of Duchenne UK. Our incredible partnership is founded on our determination to transform care and treatment for people with DMD. You can see what has been achieved in 2023 thanks to this special partnership. ??

Alex Johnson OBE, Founder of Joining Jack and Duchenne UK



gives us the motivation and hope to continue our important work, we could not thank them enough for their support and encouragement over the years. We regularly talk and have meetings ensuring that we can effectively collaborate and work as efficiently as possible. We look forward to continuing our work with them in 2024. ??

Emma Hallam, Founder of Alex's Wish



Rugby legends breakfast Martin Offiah MBE, Andy Farrell OBE and Shaun Edwards OBE, gave their time to take part in an exclusive panel discussion to raise money for our vital work in assistive technology, specifically the SMART Suit. They took time out to support Duchenne UK at the event, ahead of the Rugby World Cup.



Joining our fight - Family & Friends Funds

As a charity set up by two mothers of DMD boys, 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 50 Family & Friends Funds. Together they have raised more than £3.6 million, and raised just over £330,000 during 2023 alone!

There are so many fundraising activities we could mention but here are a few that caught our eye this year: Henry's Hills held a gala dinner in November which raised £20,000, fundraiser Jimmy Jackson walked a million steps for Moving Muscles for Marcus, and Oscar's Duchenne Challengers completed the 45km Big Black Mountain challenge.

Interested in finding out more about Family & Friends Funds?

Get in touch with familyfunds@duchenneuk.org

The Duchenne Dash -London to Paris in 24 hours

Duchenne UK's flagship fundraising event is the Duchenne Dash, now in its 11th year. 158 riders cycled from London to Paris in 24 hours, raising £832,831. Our founders, Emily and Alex, and their partners, Nick and Andy, all took part, along with our Chair, Krishnan Guru-Murthy. Keen supporters who couldn't join the ride contributed via the Duchenne Dash AT HOME, cycling locally to contribute almost £40,000 to the total. Since its inception, committed Duchenne Dashers have helped to raise £7.3 million towards our mission.

The Duchenne Dash 2024 rolls out from London on the 17th May 2024, arriving in Paris on Saturday 18th May for the riders' gala dinner in the shadow of the Eiffel Tower. Join us for the challenge at duchenneuk.org/dash/



A special thank you to the Dash Ride Captains and volunteers who make the ride possible!





Our fundraising campaigns

Duvet Days – this was our cosiest fundraiser, perfect for the cold days of February. Families raised money by taking time out to 'Du' nothing, but still raising £28,998.

Duchenneber – our annual advent calendar with festive photos of our families' boys and siblings, which are shared on social media.



Taking on a challenge for Duchenne UK

We are so thankful to our incredible community of fundraisers who always amaze us with their creativity and commitment to Duchenne UK. This year they've hiked up Mount Kilimanjaro, walked on the wings of an aircraft in flight and taken their kilts out for a stroll – to name just a few of their achievements!

Congratulations and well done to our team of dedicated runners who have taken on challenges for Duchenne UK this year, including raising £19,030 on the Royal Parks Half marathon, £2,750 on the London Marathon and £3,950 on the Great North Run.





Thank you to everyone who has supported us this year!



TEAM ... **CALLUM**

Thank you to our Family and Friends Funds and partner charities

Access to Life Action 4 Arvin Action for Zach Aidan's World Archie's Army Archie's March **Backing Jack** Ben vs Duchenne Bruce's Battle Squad Changing Charlie's Future Chasing Connor's Cure Cure4George Defending William Against DMD Doing it for Dexter Edward Steam Team Elliot's Endeavours Fight for Finn Following Felix For Felix Help Harry Helping Hayden Henry's Hills Henry's Hurdles Hope for Harry Jack's Aim

Jacobi's Wish Jayden's Army Joe's Journey to end Duchenne Life & Hope for Lenny Lifting Louis Love for Leon LucasFightingDuchenne Lygo Family Fund Mac My Day Matthew's Mighty Mission Mission Jensen Moving Muscles for Marcus Muscles for Mitchell Oscar's Duchenne Challengers Project GO Ralphy's Fund Smile with Shiv Standing with Jack Strength for Stanley Team Callum Team Dex Team Felix Team Oscar Together for Rhys William's Fund





Jack's Mission









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Thank you to our donors and funders

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People's Postcode Lottery Priory Foundation/Saracens Rockcliffe Charitable Trust Sinclair Trust Smart Water (Coca Cola) The Brother's Trust The Garfield Weston Foundation The Patrick Trust The PM Society The Tyburn Tom Wickert (Passenger Bikes)

> UKRI WinTrust With Intelligence

UCC Coffee

27 Duchenne UK













































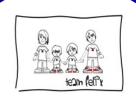






























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