



IMPACT REPORT

2018





We are a highly focused, ambitious and lean charity with a clear vision: to fund and accelerate treatments and a cure for Duchenne muscular dystrophy.

What is Duchenne muscular dystrophy?

Duchenne Muscular Dystrophy (DMD) is a devastating muscle wasting disease and is the most common genetic killer of children worldwide. Children will be totally paralysed by their teens and won't live beyond their 20s.

There is no treatment or cure. But thanks to recent breakthroughs, we believe we can save them. The disease almost always affects boys, and 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.

What are we doing about it?

We are committed to continuing to drive momentum to deliver treatments to help

THIS generation of those with DMD. We have invested millions of pounds in both research, clinical trials and supporting trial centres in the UK to run clinical trials, helping us move ever closer to effective treatments for DMD. We have an innovative approach to funding. Not only do we fund basic research, but we fund clinical trials. We fund the doctors and nurses in the UK to deliver those trials: and we look at where we can accelerate research and work with industry and regulators to get drugs approved.

Please read through our report to find out how we are making an impact in every area we possibly can to find treatments and a cure for DMD.

We have a growing army of supporters, without which none of this would be possible, so thank you to you all.



We would like to thank HRH The Duchess of Cornwall for everything she does to help the charity in her role as President.

“As President, The Duchess of Cornwall works to support children and young adults with life-limiting conditions. The Duchess supports The Charity's efforts to research treatments and find a cure for Duchenne muscular dystrophy, a devastating disease for which there is currently no cure.”



A MESSAGE FROM OUR CO FOUNDERS

Thank you for taking the time to read this impact report. It's something we are very proud of because for the first time, we have gathered together in one place the many things we have been working on over the past six years to end Duchenne.

In this financial year, we've spent £1.7million on accelerating research; that's double what we spent last year. And since we set up our foundations in 2012, we have together spent and committed more than £10 million on research.

We have devoted our lives completely and utterly since our sons were diagnosed, to fighting this disease. Thanks to the support of many dedicated researchers, clinicians, scientists, companies, families and fundraisers, we are proud to share with you the very real impact we are having.

Very few charities can claim to have achieved so much, with such low overheads, or to have addressed many of the challenges that exist in drug development with tangible, ground breaking and innovative initiatives that truly are delivering results. And in such a relatively short space of time.

One thing we never do, is take no for an answer!

We were told gene therapy would not happen in our lifetimes. But we did not accept that. So along with Joining Jack and the Duchenne Research Fund we invested \$5million in Solid Biosciences who THIS year dosed their FIRST patient. A major leap forward.

We learned that Tamoxifen – a cheap and readily available drug – may be a potential treatment for DMD. So we helped fund a clinical trial and the first patient was dosed in June! We found out Great Ormond Street Hospital and Newcastle were turning trials away – so we launched the DMD Hub and we have

invested more than £1.5 million to date and have funded 16 posts for doctors, physiotherapists, nurses and trial co-ordinators running extra trials for Duchenne in the UK.

We campaigned for and won a change in the law to allow patients to access promising treatments through the Early Access to Medicines Scheme (EAMS). At the time of publication 46 boys with DMD in the UK are taking a new medicine called Raxone, under this scheme.

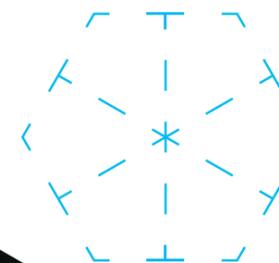
We realised that companies would struggle to get bodies like NICE to make decisions about whether or not to pay for new drugs, a challenge borne out of the limited data sets that come from dealing with a rare disease. So, we partnered with seven pharmaceutical companies to develop Project HERCULES, a ground breaking and innovative initiative, which is attracting global attention.

And we do this - all day, every day, all the time that our sons, our precious sons, are battling every day to live the best possible life in spite of this horrifically cruel disease.

And we say thank you to you all. Because without the support and the money you bring, we simply would not be where we are today. We would not have the power to say no – would not have the power to stare this disease down and fight the fight every day.

Emily & Alex

Emily Crossley and Alex Johnson
Co-Founders & Joint CEOs
of Duchenne UK



We are engaging at every stage of the drug development pathway:

BASIC SCIENCE RESEARCH



- **CRISPR/CAS9**
Toronto & UCL London
- **PEPTIDE PMOS**
Oxford
- **EXON SKIPPING FOR THE BRAIN**
Leiden
- **NOVEL ANTIFIBROTICS**
Newcastle & Geneva

PRE CLINICAL TESTING



- **SOY PROJECT (HAELAN)**
Sheffield
- **SIMVASTATIN**
University of Washington & Leiden
- **COMBINATION THERAPY**
London & Geneva
- **ELASTASE INHIBITORS**
Liverpool
- **NUTRACEUTICALS**
Reading
- **TAURINE**
University of Western Australia
- **DREAM CHAIR (WHIZZ KIDZ)**
London/Edinburgh

CLINICAL TRIALS



- **REVERAGEN- VAMOROLONE**
- **SOLID- SGT-001**
- **TAMOXIFEN TRIAL- TAMDM**
- **ARRHYTHMIAS**
Newcastle

TRIAL SUPPORT & REGULATORY APPROVAL



- **SUPPORT**
 - NOVEL BIOMARKERS
 - ACTIMYO
 - APARITO
 - **DMD HUB** - Expanding trial capacity
- **REGULATORY APPROVAL**
 - **OUTCOMES MEASURES WORKSHOP**

APPROVAL & ACCESS



- **ECONOMIC MODELLING WORKSHOP**
- **ECONOMIC MODELLING FOR REPURPOSED DRUGS**
Bangor
- **PROJECT HERCULES**
Phase 1
Phase 2



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MORE THAN
£3.5 MILLION
RAISED FROM THE
DUCHENNE DASH

90P IN EVERY £1
RAISED IS
ALLOCATED TO
RESEARCH

£10 MILLION
COMMITTED TO
MEDICAL RESEARCH

TOGETHER WITH
WHIZZ KIDZ WE WON A
£1 MILLION
GRANT FROM THE
POSTCODE LOTTERY
DREAM FUND TO CREATE
THE DREAM CHAIR

7
INDUSTRY PARTNERS
COLLABORATING
ON HERCULES

200+
PARENTS & CAREGIVERS
HAVE ATTENDED OUR
FREE PATIENT
INFORMATION DAYS

16
DOCTORS AND SUPPORT
STAFF FUNDED THROUGH
THE DMD HUB

46
PATIENTS ON RAXONE
AFTER WE LOBBIED FOR
EARLY ACCESS

3
CLINICAL TRIALS
FUNDED

89
PATIENTS
FUNDED ON
CLINICAL
TRIALS

57
PROJECTS
FUNDED

OUR ACHIEVEMENTS IN NUMBERS

In six years, Duchenne UK has had a major impact on the DMD field, using an innovative, evidence based and results driven approach to accelerate the search for treatments and addressing many of the challenges in drug development.



Note: These overall spend figures reflect the combined spend of Duchenne UK and its charity partner Joining Jack. From 2012 – 2016, Duchenne UK operated under the name of Duchenne Children's Trust.

FIRST PATIENTS DOSED WITH GENE THERAPY

Duchenne UK helps to accelerate Gene Therapy

We were told gene therapy would not happen in our lifetimes. But we did not accept that.

In 2014, Duchenne UK partnered with the Duchenne Research Fund and Joining Jack to commit \$5million to help fund pre-clinical work for Solid Biosciences' gene therapy programme.

Just three years later, their compound is **NOW IN THE CLINIC** being tested on boys with DMD!

We are extremely proud to have played a part in helping to accelerate the development of this gene therapy treatment. We await with hope the results of the study.

Solid Biosciences Gene Therapy timeline:

2013

Solid Biosciences was formed with the sole focus of solving DMD.

2014

We invested \$5million with Joining Jack and the Duchenne Research Fund to fund preclinical work for Solid Biosciences gene therapy compound.

2018

Following good preclinical data, Solid Biosciences successfully listed on NASDAQ, raising more than \$100m in its IPO.

2018

Clinical trial starts and first patients are dosed.

COMMITTED
\$5
MILLION
IN PARTNERSHIP WITH
DUCHENNE RESEARCH FUND
AND JOINING JACK

What is Gene Therapy & how does it work in DMD

Gene therapy offers hope as a potential treatment for DMD. DMD patients have a mutation in the dystrophin gene which means that they cannot produce the protein dystrophin. If their dystrophin gene (or more accurately, the working copy (mRNA) of the DNA) could be replaced with a healthy copy (or shortened section) then the hope is that their muscle cells would produce a functional dystrophin and any further damage caused by DMD would be much reduced.

Other Gene Therapy programmes in the clinic for DMD

SAREPTA

Sarepta Therapeutics has partnered with Dr Jerry Mendell of Nationwide Children's Hospital in Ohio to test their AAV microdystrophin programme. In June they shared positive data on 3 boys who have been dosed. All three patients showed robust microdystrophin expression at 76.2%, and two months after treatment, all three patients showed an 87% decrease in CK levels – an indicator of muscle damage.

PFIZER

In 2016 Pfizer acquired Bamboo therapeutics, and is now running a clinical trial to test their AwAV mini-dystrophin gene therapy. The first patient was dosed in March 2018. Early data is expected early 2019.

CANCER DRUG SHOWS PROMISE AS A TREATMENT FOR DMD

We co-fund a clinical trial to test if Tamoxifen can be used as a treatment for DMD

When we found out that Tamoxifen, a drug to treat breast cancer, could be an effective treatment for DMD, we wanted to do everything we could to help get this into a clinical trial.

We discovered a team in Switzerland, led by Professor Dirk Fischer, who had started planning a trial but they were struggling with the workload and needed funding. So we immediately paid for them to recruit a study manager. And we invested £575,000 to fund one European trial site and two in the UK.

We worked with the team on their regulatory strategy and Duchenne UK is now the holder of the Orphan Drug Designation (ODD) for Tamoxifen as a treatment for DMD.

The first patient was dosed in June 2018! This trial would not have come to the UK without our funding.

(The Tamoxifen trial is being jointly funded by Duchenne UK, E-Rare, Duchenne Parent Project and the Monaco Association against Muscular Dystrophy).

“Duchenne UK and the other patient organisations came in with funding at a very critical moment ... 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.”

Professor Dirk Fischer,
from UKBB, the University Children's Hospital in Basel, Switzerland



Duchenne UK and the team from Switzerland visiting the European Medicines Agency (EMA) for scientific advice

Tamoxifen is a cheap and readily available medicine, with a good safety record in adults. If this trial is successful, we could see a cheap, effective and readily available treatment for DMD in the very near future.

BRINGING THE TRIAL TO THE UK

Before Duchenne UK's involvement, this trial would not have been conducted in the UK. We are proud that our work through the DMD Hub will support two UK trial sites; Alder Hey Children's Hospital in Liverpool and Leeds Children's Hospital. Duchenne UK, along with Alex's Wish and Joining Jack, are also providing funding to support clinical staff at Alder Hey and Leeds to deliver the trial.

Our other repurposing work



FOCUS ON REPURPOSING

Repurposing is an exciting approach to drug development; it involves looking at existing medicines, and testing them to see if they could be effective in treating Duchenne Muscular Dystrophy. The advantage of this approach, compared to the development of an entirely novel compound, is that the development time has the potential to be cut dramatically, because these medicines are already approved as safe for use in humans.

Metformin

Metformin is an approved drug for the treatment of type II diabetes. We have been working closely with Professor Dirk Fischer at the Children's Hospital in Basel, Switzerland (UKBB), to help plan a clinical trial to examine the safety and efficacy of Metformin and L-Citrulline as a combination treatment for DMD. Duchenne UK received Orphan Drug Designation for Metformin/L-Citrulline in March 2018.

Combination therapy

Tamoxifen and metformin, with L-citrulline. We granted £200,000 to Dr Olivier Dorchie at the University of Geneva to run a 2-year pre-clinical study into a combination

therapy for DMD. The study is investigating the effect of combining two repurposed drugs, tamoxifen and metformin, with L-citrulline, a nutraceutical. The study is also looking at the effect of combining these drugs with prednisone, the most commonly prescribed steroid for DMD.

Simvastatin

A 2015 paper from the University of Washington showed that Simvastatin, a drug commonly prescribed to reduce cholesterol, could be effective for the treatment of DMD. Duchenne UK have given funding to Solid Biosciences and the University of Leiden to carry out further pre-clinical work to determine if we could take simvastatin forward into a DMD clinical trial.

OUR MAJOR ACHIEVEMENTS

BRINGING MORE CLINICAL TRIALS TO THE UK

Duchenne UK set up the DMD Hub

A clinical trial is often a family's only hope of accessing potential new treatments that might one day become approved medicines. It offers a lifeline and it offers hope.

So when we learnt that clinical trials were being turned away from UK hospitals, because of a lack of doctors, nurses and physiotherapists to run them, we collaborated with other patient organisations to change this.

In July 2015, we brought together all the patient organisations in the UK and together we funded 13 emergency posts at Newcastle in the UK to fund at Newcastle and GOSH.

However, we didn't want to stop there – the landscape for DMD research has transformed and so many more trials are now coming to the clinic.

We need to make sure no trial is turned away.

So we launched the DMD Hub, and through it we fund the doctors, nurses and support staff to run DMD Clinical Trials, as well as providing training, mentoring and resources to the hospitals.

The mission of the DMD Hub is to ensure all patients with DMD, both children and adults, have access to clinical research trials

The DMD Hub has already helped increase the number of UK sites which run DMD trials.

£1.5 MILLION COMMITTED SO FAR

5 NEW HUB SITES

16 POSTS FUNDED

DMD HUB
EXPANDING TRIAL CAPACITY

“No studies have been turned away since this award. Actually having the staff in place has allowed us to expedite many preparatory processes of assessing new studies for adoption whereas in the preceding two years the team had turned down 10 novel experimental approaches.”

Professor Francesco Muntoni
Director of the **Dubowitz Neuromuscular Centre, Great Ormond Street Hospital for Children.**

“Working with Duchenne UK has been absolutely fantastic. I think they are a very creative organisation. They are really aspiring to do the best for patients and to bring high quality research to those patients and families. I can honestly say they are one of the most inspirational groups I've ever worked with.”

Matthew Peak
Director of Research
Alder Hey Children's Hospital

89 PATIENTS NOW ON CLINICAL TRIALS

“As the DMD Hub Manager I can see first-hand the impact we are having nationally to increase capacity, enabling additional sites to take on trials and facilitating the established sites to recruit additional patients to trials. With more than 6 hub sites being supported by the DMD-Hub in the first year and a half it is rewarding to see how we are working together as a community to make a real difference to patients and their families wishing to take part in trials.”

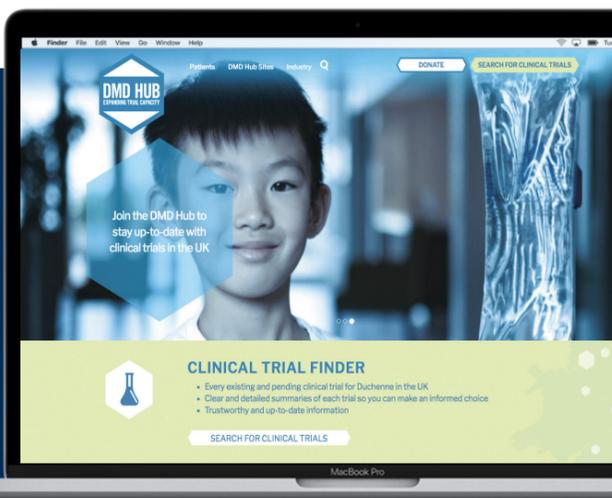
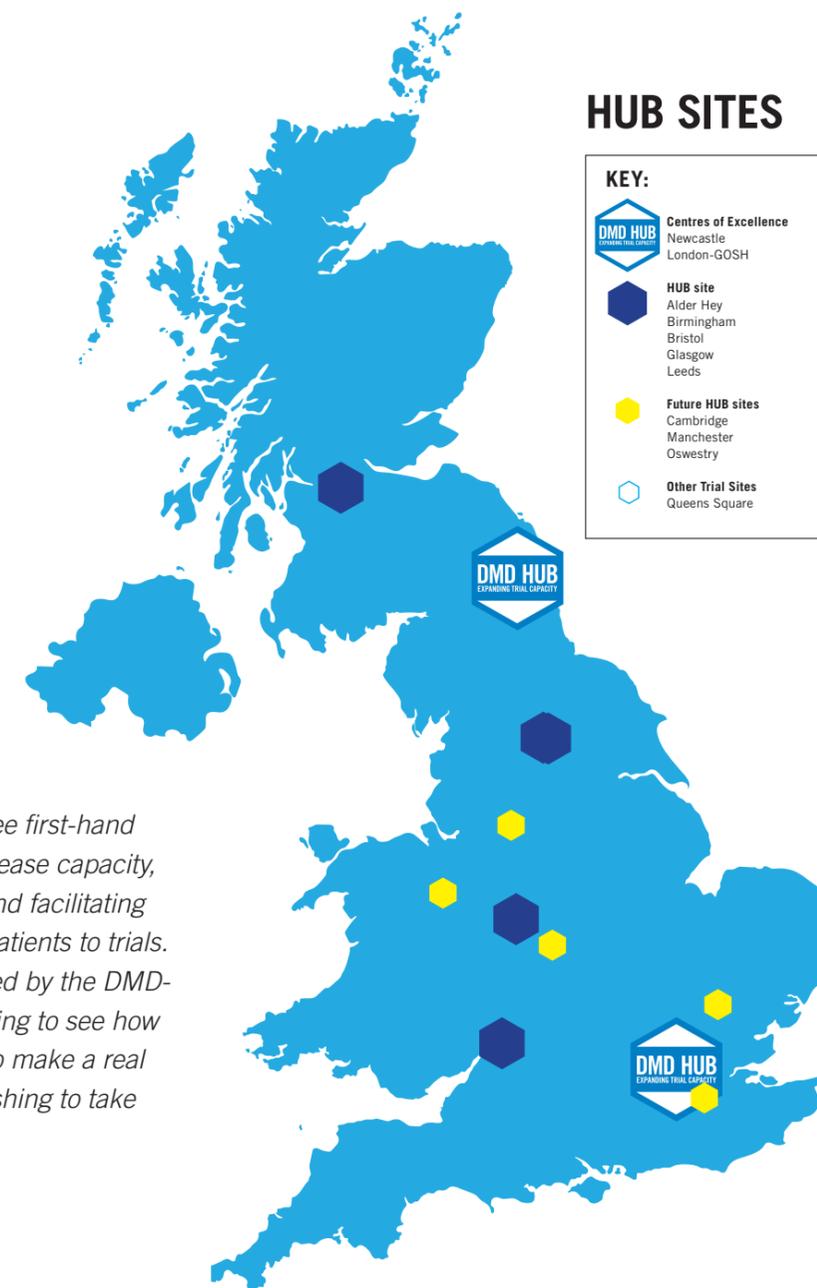
Emma Heslop,
DMD Hub Manager
The John Walton Muscular Dystrophy Research Centre at Newcastle University

The DMD HUB website DMDHUB.ORG

We responded to the needs of patients who were struggling to find out information on clinical trials and where they were happening. So we launched the DMD HUB WEBSITE, to provide patients with trustworthy and up to date information about clinical trials in the UK.

The website provides FAQs on what to consider before joining a trial, there is also an interactive map with information on all the DMD Hub sites.

The website also has sections for hospitals and drug development companies to support them to setup and run clinical trials.



UNITING PHARMA IN GROUND BREAKING COLLABORATION

Duchenne UK launch project Hercules to expand access to treatments

We are trying to tackle the long delays between effective drugs for DMD being approved and them being funded by the NHS.

Through Project Hercules we have brought together seven leading pharmaceutical companies to try to speed up getting effective and innovative medicines to DMD patients.

This ground breaking collaboration is working with seven pharmaceutical companies, multidisciplinary advisers leading universities and experts to develop data and tools to support Health Technology Assessments and reimbursement processes in the UK and globally.

HERCULES.

DMD: HEALTH RESEARCH COLLABORATION UNITED IN LEADING EVIDENCE SYNTHESIS

THE PROBLEM

In order to get new treatments reimbursed, the full benefits of the treatment need to be demonstrated. This relies on data about the quality of life of those with DMD and the burden and costs associated with the disease. At the moment there is limited data in this area and this leads to delays and difficulties in ensuring access to new treatments.

THE SOLUTION

Project HERCULES aims to create data and tools to support pharmaceutical companies and reimbursement decision makers to better understand DMD, this should help decision makers have all the data they need to assess reimbursement when a new treatment becomes available, so patients can access the treatment without the delay.

Duchenne UK is also at the forefront of a new wave of collaborative programmes bringing together rival drug firms to tackle some of the toughest challenges in medicine, from rare disease to antibiotic resistant superbugs. The charity has achieved a rare thing in the pharmaceutical world - it has got seven competitor companies to work together.

Quote from article in *The Telegraph*, February 2018

How is the project run?

The project is led by Duchenne UK with support from Josie Godfrey, a former Associate Director at NICE. There is a quarterly multi-stakeholder Steering Group chaired by Fleur Chandler who worked in Value Evidence and Outcomes at GlaxoSmithKline, and a Duchenne parent who also sits on the Parent Advisory Board of Duchenne UK. The Steering Group is also attended by the pharmaceutical companies, patient organisations, advisers and academics.



Josie Godfrey

Fleur Chandler

The seven pharmaceutical partners for phase 1 of the project are:

- Pfizer Inc
- PTC Therapeutics International Ltd
- Roche
- Sarepta Therapeutics, Inc
- Solid Biosciences
- Summit Therapeutics plc
- Wave Life Sciences USA, Inc

How is the project funded?

Duchenne UK is investing £200,000 in the initial stages of the project. The seven pharmaceutical companies are also investing in the project.

EARLY ACCESS TO MEDICINES

Changing the law to give patients early access to medicines

We are fighting on all fronts. For everyone with DMD.

In 2014 we campaigned for – and won – a change in the law to allow patients with life limiting conditions to access as yet un-approved drugs. It was called the Early Access to Medicines Scheme (EAMS).

Today – 46 boys with DMD in the UK are now taking a drug called Raxone because of that scheme which is helping to improve and stabilise their lung function.

Thanks to the hard work of Duchenne UK, the Duchenne community and other UK Duchenne patient organisations, we are delighted to see the scheme being used for a treatment for DMD.

“ Translarna was approved by European regulators in May 2014 but it took 23 months for Britain’s National Institute for Health and Care Excellence (NICE) to agree to fund it. In the time it took to get the drug reimbursed, boys stopped walking and were no longer able to access the drug. I realised at that moment that there is actually something worse than having no drug for your disease – it’s having a drug that’s approved but is sitting on a shelf because no one can agree how to pay for it. ”

Emily Crossley,
Co-founder and
Co-CEO of
Duchenne UK



Our founders, Emily and Alex, pictured with George Freeman MP and Lord Howe, were instrumental in lobbying for the introduction of Early Access to Medicines Scheme (EAMS).

TIME LINE FOR ACCESS TO RAXONE

JUNE 2013

Emily and Alex lobbied with Empower for the introduction of the Early Access to Medicines Scheme

MARCH 2014

Change in the law to introduce EAMS

MAY 2016

Santhera applied for marketing authorisation for Raxone for DMD.

JUNE 2017

Raxone granted positive EAMS Scientific Opinion from the MHRA

SEPT 2017

First patient given Raxone under EAMS September 11th 2017

JAN 2018

Santhera was denied market authorisation for Raxone from the EMA.

JUNE 2018

The MHRA renewed the Raxone positive opinion for a further 12 months.

RESEARCH INTO NEW COMPOUNDS

Duchenne UK funds two new exciting projects

Elastase Inhibitors

During inflammation, a protein called Neutrophil elastase is released. Patients with DMD have increased inflammation in their muscle tissue, therefore neutrophil elastase is increased as well. Neutrophil elastase has been shown to increase muscle damage and prevent muscles from regenerating.

By blocking the effects of Neutrophil elastase with an inhibitor we could potentially counteract the muscle

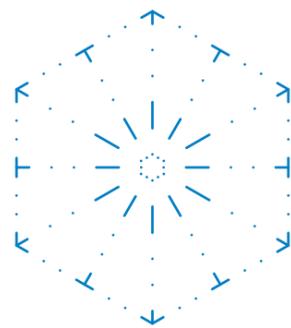
damage it causes. In partnership with Joining Jack and Charley's Fund we are co-funding a study into the use of an elastase inhibitor to treat the symptoms of DMD. This pre-clinical project is testing the use of an elastase inhibitors in the DMD mouse model. If the study is successful, the treatment could be taken forward into a clinical trial on patients with DMD.

Elastase inhibitors are already used to treat other diseases such cystic fibrosis

and Chronic Obstructive Pulmonary Disease COPD. They have therefore been through a lot of testing already, so we know they are safe and have very few side effects. Consequently, the timeline to deliver Elastase inhibitors to patients with DMD could be faster than developing a brand-new treatment.

Duchenne UK has committed £44,865 towards this project. We are co-funding this study into the use of elastase inhibitors to treat the symptoms of DMD in partnership with Joining Jack and Charley's Fund.

The research is also being supported by a grant from the Medical Research Council.



Bowman-birk Protease Inhibitor (BBI): A soy product which could treat DMD

In August 2016 we funded Professor Steve Winder at The University of Sheffield to investigate a nutraceutical soy product called Haelan 951, as a treatment for Duchenne muscular dystrophy.

Some DMD families give their son Haelan 951 because there is anecdotal evidence that it helps slow down disease progression BUT it has never been tested in clinical trials. We are funding this study to understand whether there is any scientific truth to the anecdotal evidence.

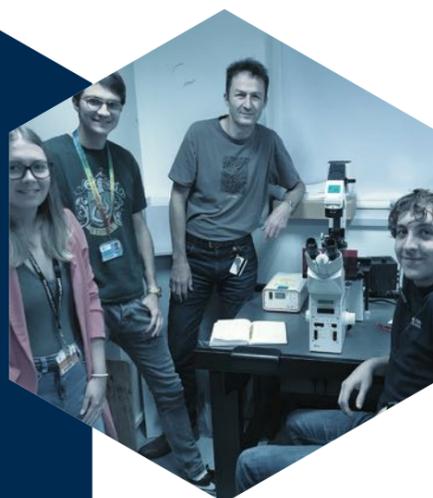
The study compared the effects of Haelan 951, genistein (an isoflavone) and Bowman Birk Inhibitor (BBI) in mdx mice. Genistein and Bowman Birk Inhibitor are both components of Haelan 951. The study showed that the diet supplemented

with BBI, significantly improved holding impulse (grip strength) but no significant benefits were found from any of the other components found in Haelan 951 that were also tested.

Based on these findings we will now focus on BBI, so we are funding a further study to enable Professor Winder and his team to carry out a dose-escalation study.

We hope to find out whether BBI displays a dose-dependent effect in slowing disease progression in a DMD mouse model, with the aim of finding the most effective dose.

We have now invested more than £135,000 towards Professor Winder's investigation.



Professor Steve Winder with his team at The University of Sheffield

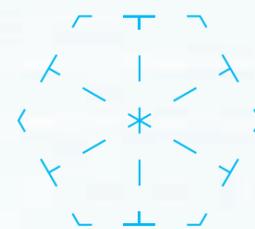


BUILDING THE DREAM CHAIR

We partnered with Whizz-Kidz and the University of Edinburgh to win £1 million from the People's Postcode Lottery Dream Fund to develop a dream chair for wheelchair users

Duchenne UK has joined forces with Whizz-Kidz and the University of Edinburgh to collaborate on the Wheels of Change project which hopes to improve current technologies for wheelchair users.

The project will research and develop new technology, design & innovation to integrate with and enhance mobility equipment.



Wheels of Change 1 MILLION



FREE PATIENT INFORMATION DAYS

We are acutely aware of the difficulties parents face when trying to find accurate information to educate themselves on everything they need to know to support their child with DMD.

This is why we run FREE Patient Information Days.

The patient information days are free for parents and caregivers to attend. During the one day meetings we cover a range of topics including research, standards of care and how to support the education of children and adults living with DMD.

We also have a panel session where people are invited to submit questions to parents of children with DMD to discuss issues that arise from living with DMD and taking part in research.

When asked by parents and caregivers what is the best thing they you can do for their child with DMD, our answer is EDUCATE YOURSELF on everything there is to know from the standards of care to what support is available in schools. And remember: WE ARE HERE TO HELP YOU.

OUR PREVIOUS FREE INFORMATION DAYS

July 2017
Liverpool

February 2018
London

September 2018
Newcastle

UPCOMING
March 2019
London



Feedback from our information day in February 2018:

“ I thought the entire day was perfectly paced, incredibly informative and I'm very glad I came. I've learnt a lot. I've only been part of this community for a few weeks but this day has inspired me to fight as fiercely as you guys fight. Thank you for all you do for our boys. ”



FREE EDUCATION SUPPORT

DECIPHA

We realised that parents weren't getting the right information and support to help their child access the best education for them, all the way from primary school to applying to University.

We have directly helped 70 families by funding, Decipha's Road Map for Life, a project run by Nick Catlin to provide special educational needs support and advice about school, Further Education, home adaptations, mobility equipment, clinical trials and more.

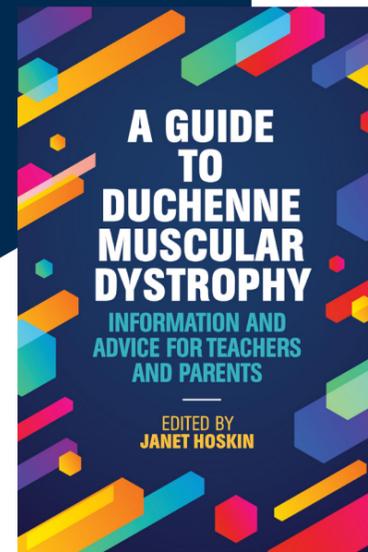
Our funding enables them to help families free of charge.

We have granted £60,000 so far to Decipha and will continue this partnership so that all families who need support can get it.

Decipha's RoadMap for Life Programme can help to identify key outcomes required for Education Health and Care Plans for young people living with DMD.

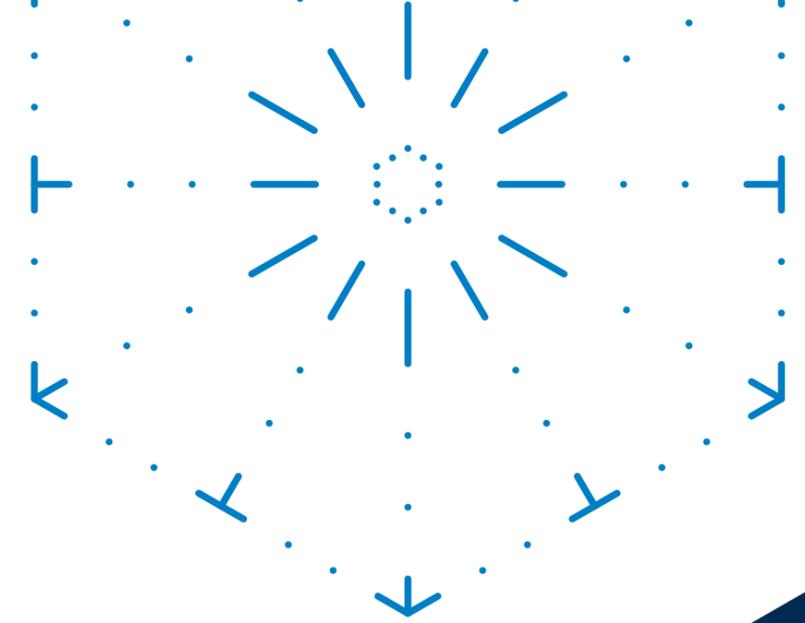


DECIPHA



We have given out hundreds of FREE copies of Janet Hoskin book: 'A Guide to Duchenne Muscular Dystrophy: Information and Advice for Teachers and Parents' to the Duchenne community.

This is a really useful resource which brings together chapters by a range of experts in DMD aimed at supporting young people and their families as they move through school and college.



DMD PATHFINDERS

We are working with DMD Pathfinders to ensure that treatments and therapies developed for DMD are relevant and available to adults with DMD, as well as children.

DMD Pathfinders is the first user-led organisation run by and for adults with DMD themselves, and provides the unique perspective of adults who have lived with the condition all their lives. As well as providing advice and information, DMD Pathfinders are determined to ensure adults are not left behind in the race for a cure.

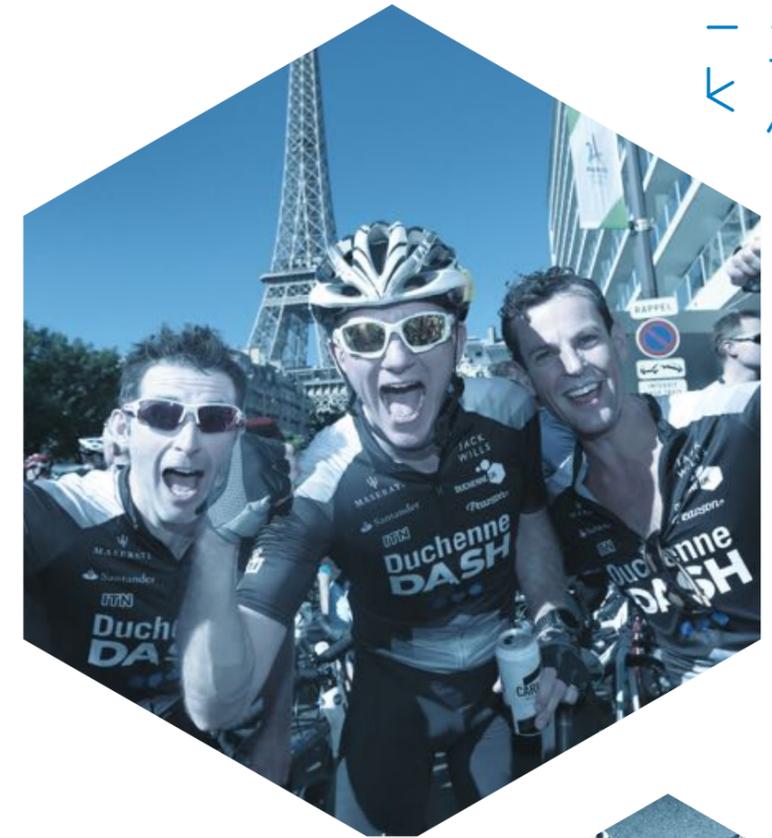
This project is spearheaded by CEO Dr Jon Hastie and run as a joint project between Duchenne UK and DMD Pathfinders.

We are engaging with pharmaceutical companies, research funders and other stakeholders to push for adults to gain access to life-changing treatments.



DUCHENNE DASH

The Duchenne Dash is a an exhilarating 24-hour bike ride from London to Paris in which our supporters take on an amazing challenge to help children with DMD. We start in London and arrive triumphant, a day later, under the Eiffel Tower.



CHARITY PATRON KRISHNAN GURU-MURTHY

We couldn't have achieved what we have without the support of our wonderful patrons.

Krishnan Guru-Murthy, newsreader for Channel 4 News, has been a dedicated patron of the charity since we launched.

In 2013 Krishnan came up with the idea of the Duchenne Dash when he and a small group of friends, cycled 300km from London to Paris in 24 hrs.

The Duchenne Dash has grown to become Duchenne UK's biggest and most challenging event of the year with 160 cyclists taking part each year.



THE DASH HAS
RAISED MORE THAN
£3.5
MILLION
SINCE 2013

FOR MORE INFORMATION, VISIT
DASH.DUCHENNEUK.ORG

FAMILY AND FRIENDS FUNDS

We know that the people who are most passionate about raising money to find treatments and a cure for DMD are those who live with it every day.

Our Family and Friends Funds provide vital support to Duchenne UK, by fundraising for us and helping us to advocate to accelerate research.

They share our ambition to fund the most promising research, no matter where it is in the world, and to do so with a vision to help bring that research out of the laboratory and into the clinic where it can help patients.

“ There is hope now because of what Duchenne UK are doing as a charity. They give us the strength to carry on and believe that there will be a treatment one day to end Duchenne. ”

Rachel Halpin, Mother of Harry, Set up Help Harry family fund with Duchenne UK



Thank you to our family funds - our army of supporters fighting to End Duchenne

- Archie's March
- Chasing Connor's Cure
- For Felix
- Help Harry
- Lifting Louis
- Jack's Aim
- Jack's Mission
- Jacobi's Wish
- Team Dex
- Team Felix
- The Lygo Family Fund
- Muscles for Mitchell
- Project GO
- Williams Fund

PARTNER CHARITIES

We are so pleased to work with many DMD charities both in the UK and internationally to fund research and raise awareness for DMD.



Alex from Alex's Wish starred in our World's Strongest Boys film.

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.



“ Alex's Wish love working with Duchenne UK to co-fund clinical projects and clinical trial capacity. They enable us to focus on fundraising and raising awareness, whilst they work relentlessly to find out where the money is best placed. Emily and Alex are incredibly professional and knowledgeable and it is a pleasure to work with them to end Duchenne for all.

Emma Hallam



“

We've been working alongside Alex and Emily from Duchenne UK for many years, and I am blown away by the progress they have made. They have attended hundreds of meetings, and have put many quality projects together that Duchenne Now and others have supported. I feel that it is in the best interests of the community for us to join forces, because together we will be stronger.

Tony Levene, founder and trustee of Duchenne Now

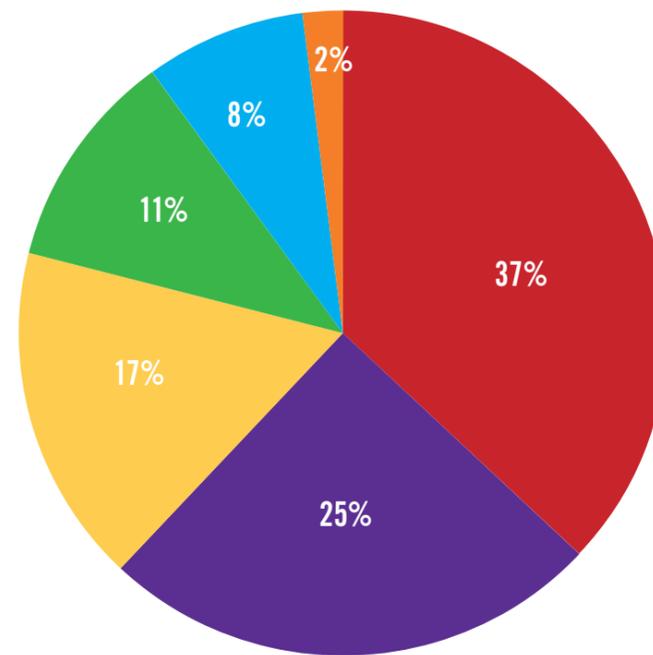
Joining forces with Duchenne UK is the next logical step – to share our knowledge, our advocacy experience, our funds and expertise to accelerate the fight for a cure.

Vici Richardson founder and trustee of Duchenne Now



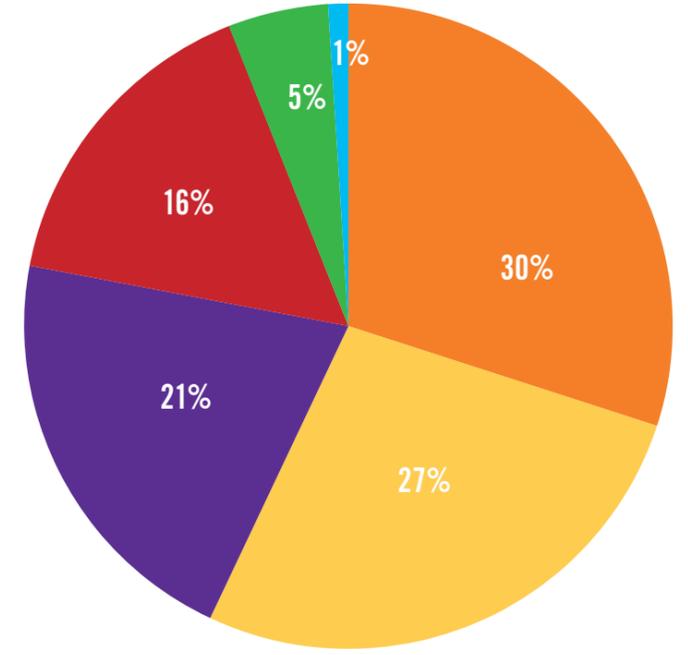
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SUMMARY OF SPENDING



FY ENDED APRIL 2018

- 37% Repurposing drugs
- 25% Expanding Trial Capacity
- 17% Research Into New Drugs
- 11% Approval and Reimbursement
- 8% Other
- 2% Gene Therapy



2012-2018

- 30% Gene Therapy
- 27% Research Into New Drugs
- 21% Expanding Trial Capacity
- 16% Repurposing Drugs
- 5% Approval & Reimbursement
- 1% Other

90P

IN EVERY £1 RAISED IS ALLOCATED TO RESEARCH

£1.4 MILLION

SPENT ON 41 PROJECTS IN FY18

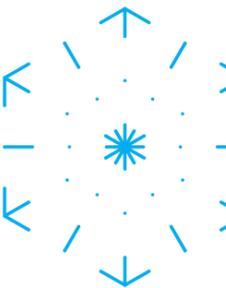
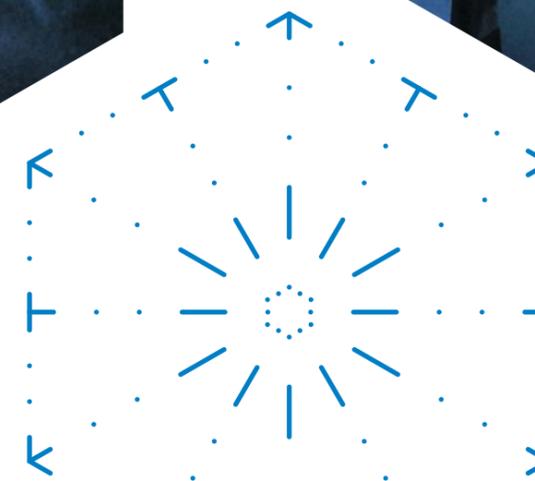
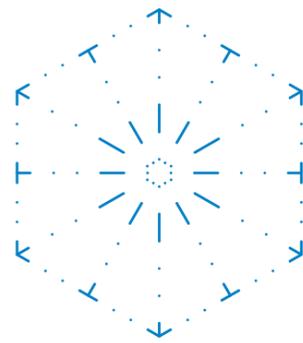
THANK YOU FOR YOUR SUPPORT

We could not have achieved what we have without your support

Owen Farrell is a prolific Saracens and England fly half. He has been part of helping promote awareness of DMD since the start of his friend Jack Johnson's diagnosis.

“ My dad and Jack's dad played rugby together at Wigan and our families are very close. The charity has been a huge part of our family's lives. Everyone who donates or who tells their friends and they then go on to spread awareness or raise money, then they are helping. If I can help spread awareness by doing the salute when I kick a goal, it's the least I can do. ”

Owen Farrell





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Join us to end Duchenne

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