



**DMD HUB**  
EXPANDING TRIAL CAPACITY

**IMPACT REPORT**  
2020

# The DMD Hub: changing lives by expanding DMD clinical trial capacity in the UK

“ It is impressive to see how the DMD Hub has evolved over the last 5 years - starting from developing a network of trial ready sites to actively facilitating the delivery of gene therapy trials in DMD, which we expect to see recruiting in the UK in 2021.

Dr Michela Guglieri, Senior Clinical Lecturer, Newcastle University

“ It's not an understatement to say that the DMD Hub has transformed the clinical trial landscape for DMD in the UK – providing a one stop shop for clinical trial support.

Our innovative funding “pump priming” model is part of Duchenne UK’s 360 degree approach to research.

Emily and Alex Co-Founders of Duchenne UK

## WHAT IS THE DMD HUB?

The DMD Hub, funded by Duchenne UK, is a network of hospitals delivering clinical trials and facilitating access to treatments for the DMD community in the UK.

The DMD Hub is a ground-breaking multi-stakeholder collaboration between Duchenne UK and the neuromuscular centres of excellence, in Newcastle and London, The John Walton Muscular Dystrophy Research Centre (JWMDRC) and Great Ormond Street Hospital (GOSH), NHS clinical trial sites, and the National Institute of Health Research (NIHR), to deliver trials in Duchenne muscular dystrophy (DMD) in the UK.

The COVID-19 pandemic has had a significant impact on the clinical trial landscape in the UK. Studies that were in the process of being set up were paused and recruitment to ongoing studies was halted. The DMD Hub has been working closely with sites, patients, and industry to help manage the situation and provide a united UK-wide response.

Our focus this year has been to prepare the UK for the arrival of Gene Therapy. Trials are expected to arrive in early 2021 and the DMD Hub has

been instrumental in bringing all the key stakeholders together to help best prepare the field: identifying and understanding how to overcome the barriers, clarifying institutional readiness at sites across the UK, working on developing a fair and equitable recruitment procedure for patients and engaging with companies conducting the trials.

Even with the challenges of COVID-19 and gene therapy, our core mission to expand clinical trial capacity in the UK remains unchanged.

Over the last five years, through the DMD Hub, Duchenne UK and its partners have funded 30 posts, and have brought companies, and income, to the NHS through clinical trials at 11 different sites across the UK. The DMD Hub has also helped to recruit more than 300 boys to take part in DMD trials.

Our innovative funding structure (pump-priming) for posts continues to enable income generated from clinical trials to

be invested back into neuromuscular teams. We are also very proud of our Clinical Trial Finder (CTF), which we created and built to help patients access clinical trials. It's success in engaging with the DMD community has led to greater attention by other organisations, both in the UK and internationally.

The DMD Hub is committed to addressing the challenges and difficulties facing clinical trials in the UK, and will continue to bring together patients, sites and industry to make the UK a competitive destination for clinical trials.

“ The DMD Hub has built a clinical trial machine in the UK that I can easily say is unparalleled in any other country.

Eric Hoffman, CEO, Reveragen



### WHO IS DUCHENNE UK?

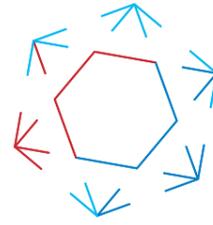
Started by families affected by the disease, Duchenne UK has one clear aim – to end Duchenne.

We're doing this by funding research that's focused on getting treatments to those affected now – as well as pushing for an effective treatment in the future.

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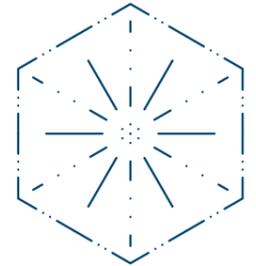


**30**  
POST  
FUNDED

**£1.6M**  
COMMITTED  
FOR THE NEXT  
4 YEARS



**OVER  
300  
PATIENTS  
RECRUITED**



**RECRUITMENT  
COORDINATION**

**3**  
STAFF NETWORKS

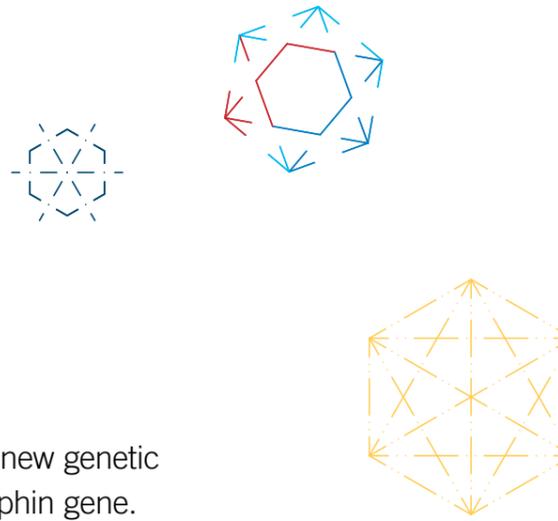
**9**  
DMD TRIALS  
IN THE UK

**12**  
POTENTIAL  
TRIALS  
COMING TO  
THE UK

**11**  
DMD HUB  
SITES



- 2016**  
Emergency posts funded at GOSH and Newcastle
- 2017**  
DMD Hub launched with first two additional sites, Alder Hey and Leeds
- 2018**  
Launched Hub website with clinical trial finder
- 2019**  
200 patients recruited
- 2020**  
Preparing the field for Gene Therapy
- 2020**  
COVID-19 trial response coordination



## THE ARRIVAL OF GENE THERAPY IN THE UK

Gene Therapy is a potential treatment for DMD which delivers new genetic material to cells, to overcome the errors/deletions in the dystrophin gene.

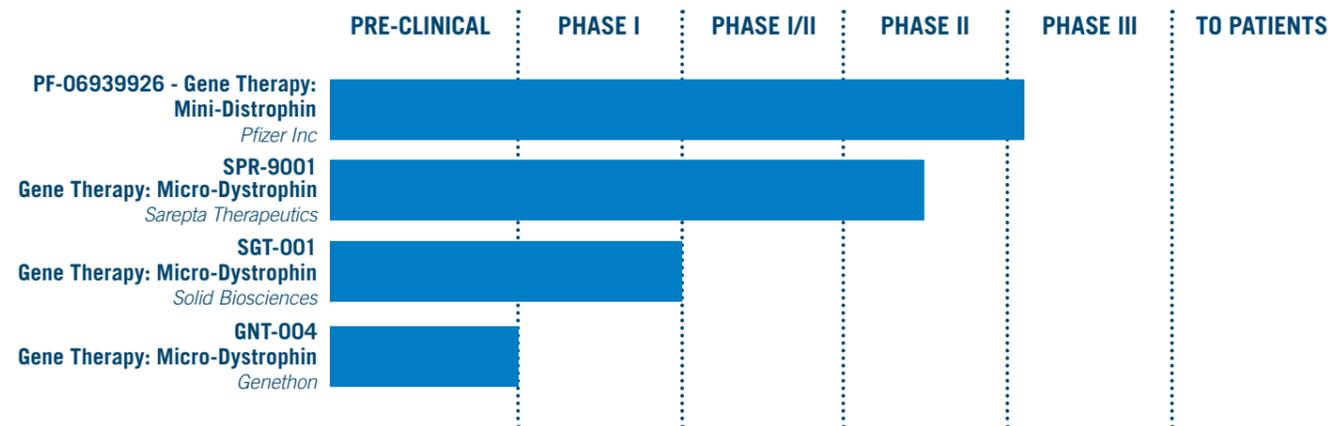
It is delivered through adeno-associated viruses (AAVs) which allows the genetic material to get into the cells. Gene Therapy is currently being used for other neuromuscular conditions such as Spinal Muscular Atrophy (SMA) in the UK. Gene therapy is not mutation specific, but the initial trials are limited to patients under 10 years old. However, there are trials planned for non-ambulant patients, which the DMD Hub is actively supporting.

There are four companies currently conducting gene therapy trials in DMD patients – Pfizer Inc, Sarepta Therapeutics, Solid Biosciences and Généthon. The Pfizer Inc, Sarepta Therapeutics and Généthon programmes have applied for approval to start trials in the UK in 2021. The Solid Biosciences' trial, which had been put on hold by the US drug agency, the FDA, received the approval to re-start in October 2020.

If you would like to understand more about Gene Therapy, watch the video in the code below:



### GENE THERAPY PROGRAMME PIPELINE



\*Based on Parent Project Muscular Dystrophy's (PPMD) Drug Development Timeline

## PREPARING THE UK FOR GENE THERAPY

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

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



The discussions and findings from the meeting have been included in a publication in *Neuromuscular Disorders: Gene Therapy in Duchenne muscular dystrophy: Identifying and preparing for the challenges ahead.*



<https://doi.org/10.1016/j.nmd.2020.10.001>

### CHALLENGES

#### PATIENTS

TRIAL AVAILABILITY

UNDERSTAND THE BENEFITS AND RISKS

ACCESSING CLEAR AND ACCURATE INFORMATION ABOUT TRIALS

#### SITES

LIMITED GENE THERAPY EXPERIENCE

CAPACITY ACROSS DEPARTMENTS

ADDITIONAL STAFF TRAINING

PHARMACY RESOURCES

#### INDUSTRY

PRODUCTION CAPACITY FOR VIRAL VECTORS

LACK OF EXPERIENCE IN APPROVAL BODIES

ISSUES WITH CONTRACTS

BREXIT

### ACTIONS

#### PATIENTS

ESTABLISH CENTRAL COORDINATION FOR RECRUITMENT

CONDUCT PATIENT SURVEY ON GENE THERAPY

KEEP COMMUNITY UPDATED AND INFORMED

#### SITES

DEVELOP DMD HUB GENE THERAPY TOOLKIT

IDENTIFY AND PROVIDE TRAINING OPPORTUNITIES

DEVELOP HUB AND SPOKE MODEL TO SUPPORT TRIAL DELIVERY

ESTABLISH CENTRAL COORDINATION FOR RECRUITMENT

CONDUCT DETAILED INSTITUTIONAL READINESS SURVEY

#### INDUSTRY

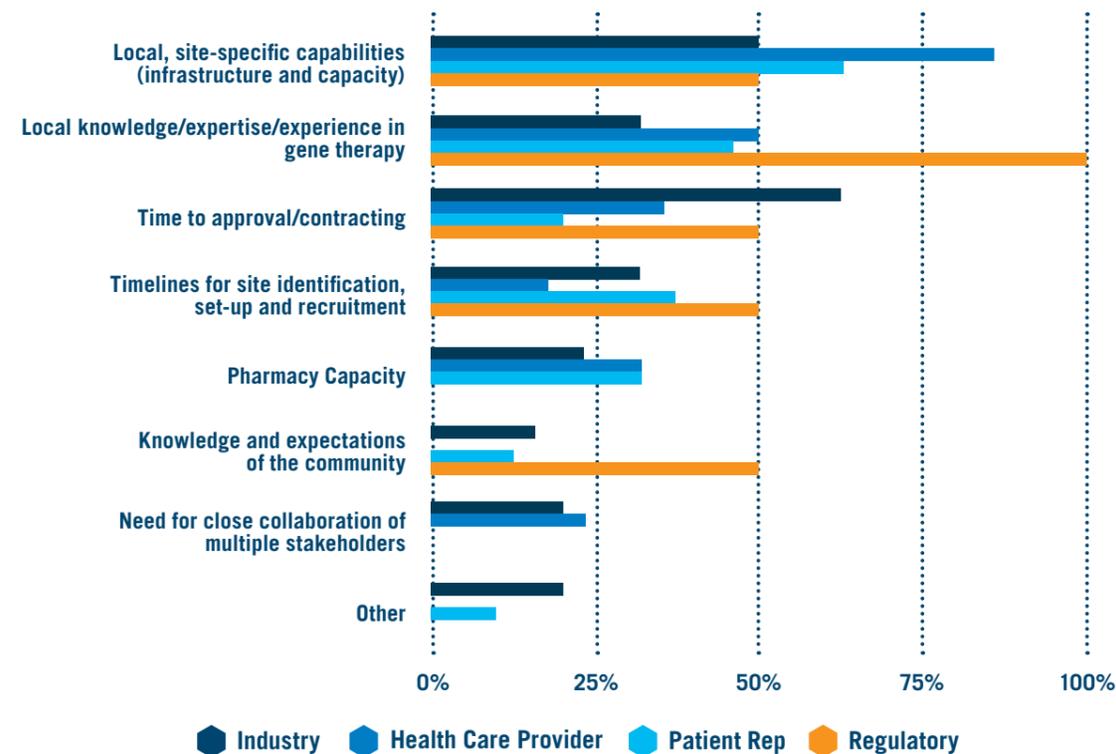
DEVELOP A MODEL FOR TRIAL DELIVERY IN THE UK

COORDINATE UK PROTOCOL REVIEW

FACILITATE SITE IDENTIFICATION AND PATIENT RECRUITMENT

ENGAGE WITH UK WIDE INITIATIVES TO PROMOTE UK SITES

CONSIDER POST-MARKETING DATA COLLECTION OPTIONS



During the meeting, a survey was conducted of all the stakeholders to assess the importance of the barriers they had identified. The results were further broken down by stakeholders indicating that all 4 stakeholders ranked the same 3 barriers with the most important:



## PUTTING PATIENTS FIRST

Since 2015, the DMD Hub has expanded the number of hospitals that run clinical trials and increased the number of available spaces for people to take part in clinical trials. To date, more than 300 patients have been recruited to take part in trials across the UK.

Before the DMD Hub, patients in the UK found it difficult to find out which trials were being run, where they were being held and how they could learn more about them. So we developed a state of the art Clinical Trial Finder (CTF) to make the search much easier for patients.

The CTF has been a key tool in improving recruitment at sites, as well as giving more families the opportunity to participate in research. Since January 2020, the page has been

viewed over 3,500 times, and has been cited as an excellent example of digital infrastructure in a report by the Association of Medical Research Charities to HM Treasury.

Internationally, the CTF has also been gaining recognition. After being approached by Dr Ian Woodcock, Melbourne Children's Hospital, we have been supporting the development of a similar model in Australia, that can be used across the different neuromuscular sites and potentially for different neuromuscular conditions.

Watch the DMD Hub Clinical Trial Finder in action



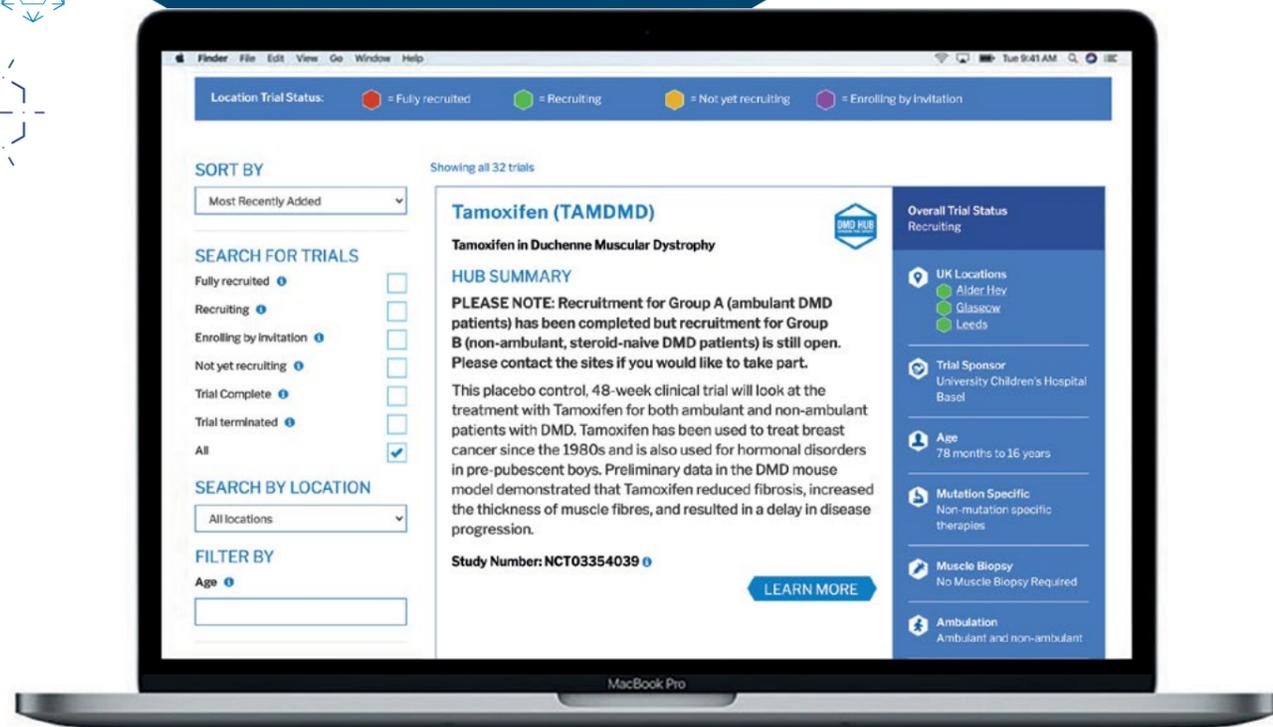
“ Ashley was recruited for the TamDMD clinical trial, at the Leeds site, following discussions with his consultant, Dr Childs. I had seen the trial was recruiting on the DMD Hub Clinical Trial Finder, which is a brilliant resource and so easy to understand.

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

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

Lesley Wegg, DMD mum

## HOW THE CLINICAL TRIAL FINDER WORKS



## STANDARDISING TRIAL RECRUITMENT TO MAKE IT FAIR FOR ALL

Participating in a clinical trial is not an easy thing, and getting on to a trial can be a very difficult process. Competition for spaces is high, and this will be even more so when gene therapy trials arrive in the UK.

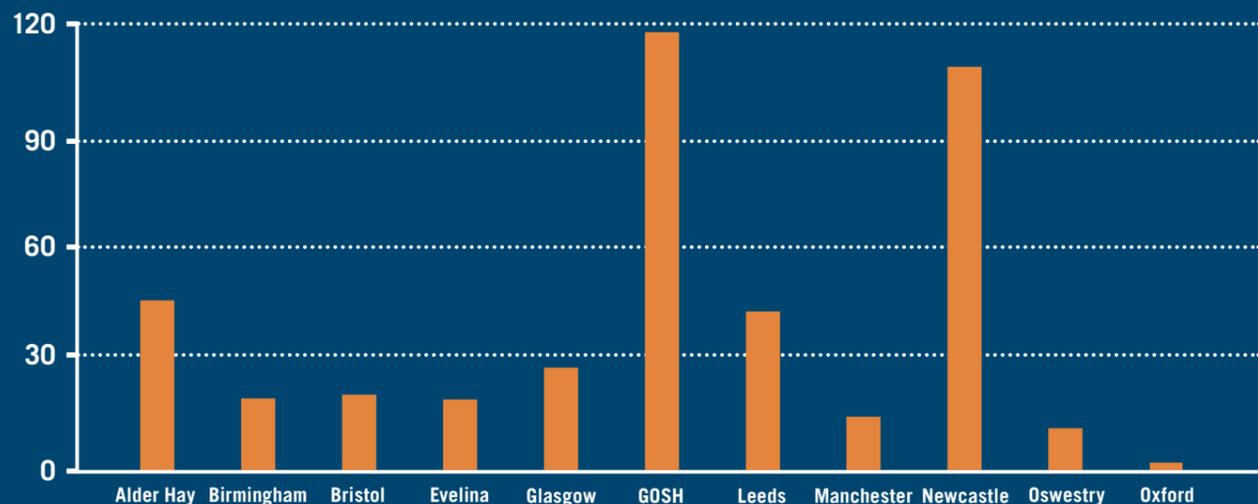
As such, the DMD Hub is working with sites and patients to establish a procedure to help provide eligible patients with fair and equitable access to trials.

“We found out about the Tamoxifen trial at a Duchenne UK Patient Information Day. It was quite a while before they were actually recruiting and I immediately contacted Jack’s consultant to express our interest. We then kept checking the DMD Hub website for any new information. Jack had a screening to see if he fit all the relevant criteria and we started the trial.”

Claire Sowden, DMD mum

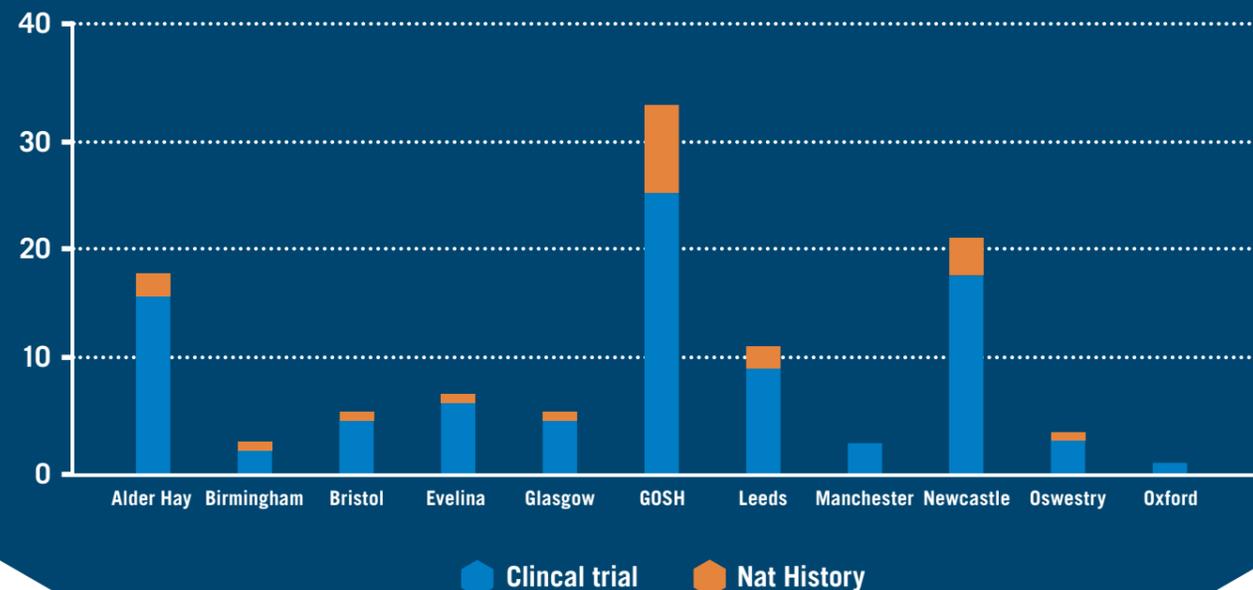


## Number of Boys on Clinical Trials at DMD Hub sites since 2015



\*not including natural history but including open label extensions

## Number of Clinical Trials and Natural History studies at DMD Hub sites since 2015



## Creating an Adult Hub for non-ambulatory trials

Whilst many of the current trials in the UK are for young patients who are still able to walk, there are an increasing number of potential trials looking at treatments for those patients who are no longer walking. These include gene therapy trials for Pfizer Inc and Sarepta Therapeutics, as well as Fibrogen’s Lelantos trial.

As these trials are looking at an older patient population than the ambulatory trials, the DMD is engaging with sites that care for older patients, to facilitate their recruitment to the non-ambulatory clinical trials and, that if patients do transition to an adult site whilst on a clinical trial, their treatment can continue.

The DMD Hub is working with the John Walton Muscular Dystrophy Research Centre, Queen’s Square, the Adult North Star Network and DMD Pathfinders to help develop this work.



## HOW THE DMD HUB IS SUPPORTING PATIENTS WHEN TRIALS TERMINATE

Drug discovery is challenging, and new treatments are found through ground-breaking research, but not all of it is proven safe or effective. Only one in ten\* products that start off in clinical trial make it through the process to become a licensed medicine. The DMD community has had its share of failed clinical trials. Late in 2020, both Santhera Therapeutics' study, SIDEROS and Catabasis Pharmaceuticals' study, Polaris DMD were stopped because they failed to meet their primary endpoint.

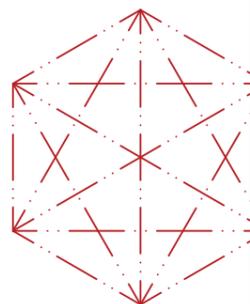
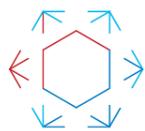
The DMD community is no stranger to this news. Prosensa Therapeutics/GlaxoSmithKlein, Summit Therapeutics, WAVE Life Sciences, F. Hoffman-La Roche Ltd, and Pfizer Inc have all seen trials end.

Taking part in a clinical trial is a huge commitment, not just for the patients but for their caregivers. It's not just the time, but the physical demands.

It's also the emotional investment parents make: the investment in hope; the investment in the glimmer of light that they have now been given a little bit of control over this disease, and that their child may be one of the first to have been given a chance to have a different life, the one they imagined for him before DMD.

Unfortunately, many patients find out about trials discontinuing through social media.

This is because many of the pharmaceutical companies running clinical trials are what's known as listed companies, and so they are obliged by financial regulation to release news of trials publicly. It's for this reason that clinicians are not able to be contacted in advance, so they often find out about trial results at the same time as patients.



## The DMD Hub plans to:

1. PROVIDE TRAINING FOR NURSES TO BETTER SUPPORT FAMILIES THROUGH THE CLINICAL TRIAL PROCESS

2. ADDRESS THE LACK OF PSYCHOLOGICAL SUPPORT AVAILABLE BY EXPLORING FUNDING MENTAL HEALTH NURSES AND PSYCHOLOGISTS

3. EDUCATE MORE PATIENTS ON THE CHALLENGES AROUND CLINICAL TRIALS AND MANAGE EXPECTATIONS

4. ENGAGE WITH REGULATORS AND INDUSTRY TO FIND SOLUTIONS TO THE CHALLENGES ASSOCIATED WITH THE TERMINATION OF CLINICAL TRIALS

\*<https://www.bio.org/press-release/bio-releases-largest-study-ever-clinical-development-success-rates>

# TRIAL SITE CAPACITY

## What is a DMD Hub site?

A DMD Hub site is a hospital where the DMD Hub has provided funding and support to increase capacity to run DMD clinical trials.

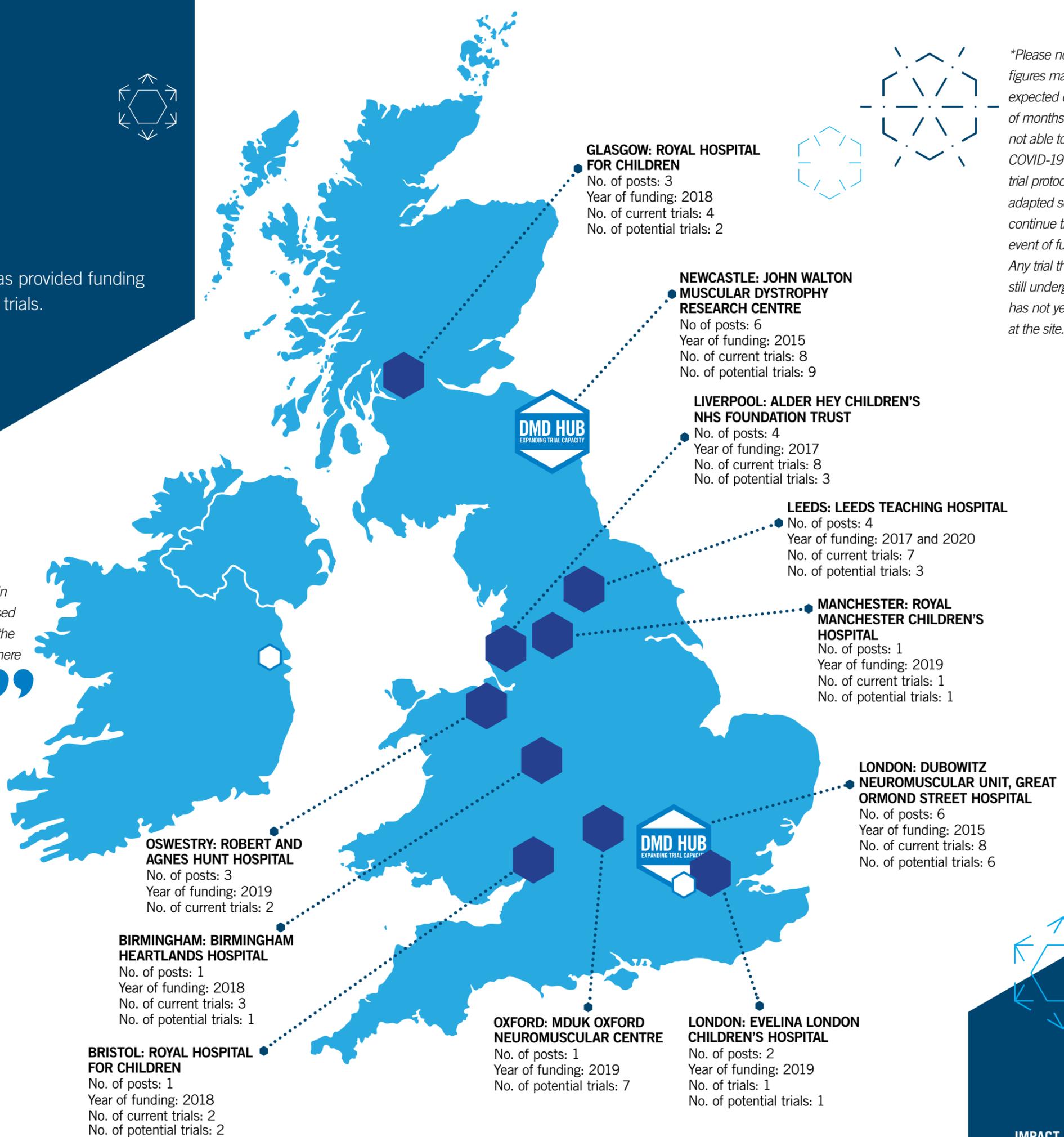
“ The DMD Hub is now an integral part of the DMD community in the UK. With the development of the DMD Hub network and the increased capacity at the sites, we have the confidence that DMD trials coming to the UK will not be turned away. This additional capacity will be needed, as there is a still lot of trial activity and interest from industry.

*Prof Volker Straub, Professor of Neuromuscular Genetics at the Institute of Translational and Clinical Research at Newcastle University*

## DMD Hub Sites

**KEY:**

-  **Centres of Excellence**  
London-GOSH  
Newcastle
-  **Hub site**  
Alder Hey  
Birmingham  
Bristol  
Glasgow  
Leeds  
London - Evelina  
Manchester  
Oxford  
Oswestry
-  **Other sites**  
Queens Square  
Temple Street



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



## UPSKILLING DMD HUB SITES TO SUPPORT DMD PATIENTS

The DMD Hub Toolkit is a repository of information developed in collaboration with key opinion leaders and expert centres. It includes standard operating procedures, protocols, manuals and check-sheets. It is aimed at upskilling sites and offering pragmatic support in trial set-up.

We offer tailored support to sites at every stage of clinical trial development from initial studies to the trial process itself.

### Collaborating with National Institute of Health Research (NIHR) to improve accuracy in costings

The NIHR costing template has proved a valuable resource for DMD Hub sites. Alongside this template, the DMD Hub, together with the John Walton Muscular Dystrophy Research Centre and Great Ormond Street Hospital, have developed specific guidance for the costing of a DMD clinical trial.

This has been rolled out across DMD Hub sites and has improved the accuracy of costing estimates, helping to ensure that there are no unexpected costs for either sites or industry.



### New updates to the Toolkit

Throughout 2020, we have been increasing the number of resources available to sites through the toolkit, developing two new sections:

- COVID 19
- Gene Therapy



Visit the toolkit here:



## SHARING KNOWLEDGE AND EXPERIENCE BETWEEN DMD HUB SITES

### Coordinating COVID response with Principal Investigators

The PI (Principal Investigators) strategy group was established in 2020 initially in response to the additional coordination required to plan for the impact of the COVID-19 pandemic on DMD clinical trials.

However, the benefits of having the option to discuss and plan clinical trial activity on a national level soon became apparent and the PIs agreed to continue with the calls.

The calls provide the PIs with an opportunity to discuss the pipeline of upcoming trials, to help identify potential gaps and address capacity issues. They will also be able to agree national statements on behalf of neuromuscular PIs running DMD clinical trials.

### Supporting Clinical Trial Coordinators in the DMD Hub Network

The DMD Hub Clinical Trial Coordinator (CTC) Network was set up in January 2020 and clinical trial coordinators at the 11 DMD Hub sites are involved, including Anna Irvin and Agata Robertson, from the John Walton Muscular Dystrophy Research Centre - Newcastle, Tracy Langan, from the Royal Hospital for Children - Glasgow and Mashal Asif, from the Bristol Royal Hospital for Children (all pictured below). It is led by experienced coordinators from the John Walton Muscular Dystrophy Research Centre.

The aim of the network is to provide a forum for clinical trial coordinators across the UK to support each other and share their experiences of running clinical trials.

Based on needs identified by the CTCs themselves, the DMD Hub plans to run a program of tailored training and education, starting in 2021 and covering key topics relevant to their roles such as consistent costing of DMD clinical trials and gene therapy trial set-up.

The DMD Hub is also working to facilitate shadowing opportunities for CTCs, to share knowledge, experience and good practice between sites.

### Expanding training opportunities for Research Nurses at DMD Hub sites

In response to the ever-expanding portfolio of trials within DMD, the DMD Hub and several research nurses from DMD Hub sites decided to establish a network to help improve patient care, increase access to trials and share best practice between sites.

Asyah Chhibda, from Leeds Children's Hospital and Tim Henderson, from Alder Hey Children's Hospital (both pictured right) are coordinating the network with the DMD Hub Manager, Emma Heslop, through quarterly teleconference

calls with research nurses involved in delivering DMD trials at DMD Hub sites.

The overall aim of the network is to facilitate learning, and help members gain valuable skills in various aspects of trial delivery. Activities for this year include Cognitive Behavioural Therapy training to support patients and their families through a clinical trial, as well as shadowing visits for research nurses at sites with more experience of running DMD trials. They will also be a crucial part of the DMD Hub's central coordination of recruitment.



### HOW THE DMD HUB SUPPORTS THE CLINICAL TRIAL PROCESS

DMD Hub engagement with Industry and clinical trial sites



Industry approach / approve site



Feasibility

Hub funding, training and education

Ethics, costing and local approval

Standard Operating Procedures

Physio training

Trial start

Recruitment

On-going support for the site staff

## DEVELOPING OUR INNOVATIVE “PUMP-PRIMING” FUNDING MODEL TO BOOST LONGEVITY OF HOSPITAL POSTS

We have developed an innovative funding practice based on a tapered funding model. Several other organisations have a similar model, but the DMD Hub is more effective due to the connections we have with sites, government bodies and industry. This collaboration has really improved the results we see.

We work closely with sites, to encourage support for the neuromuscular teams, and DMD trials, within the hospital infrastructure.

Our work with the NIHR and the Centres of Excellence to develop DMD trial-specific guidance also ensures that trials are costed accurately and that any surplus can be re-invested

back into the neuromuscular team.

Our relationship with our industry partners increases the visibility of DMD Hub sites and highlights the UK as an attractive destination for DMD trials.

All these factors make posts sustainable in the long term and ensures viable locations for DMD trials in the UK.

### Case Study: LEEDS TEACHING HOSPITAL

Since 2018, the DMD Hub has been funding a Neuromuscular Clinical Research Fellow, Dr Cristina Martos Lozano (pictured right) at Leeds Teaching Hospital.

Cristina has been instrumental in developing Leeds’ clinical research team, including undertaking the majority of clinical assessments and developing procedures to assist in recruitment from outside the region.

The extra revenue generated from the trials that Leeds have undertaken, thanks partly to having Cristina on the team, has been invested back into the department. Having demonstrated the importance of Cristina’s work, her role is now fully paid for by the Trust. She has also been promoted to Consultant.



### Case Study: ALDER HEY CHILDREN’S NHS FOUNDATION TRUST

Tim Henderson (pictured right) is the Lead Research Nurse in DMD at Alder Hey hospital, whose post has been funded by both Duchenne UK and Joining Jack.

As with Cristina, now that the funding for Tim’s role has finished, the Trust have taken on his salary. Tim is now planning to work towards gaining the qualifications required for an Advanced Nurse Practitioner (ANP) role.

An ANP role would allow him to take on additional responsibilities within the delivery of clinical trials, having the additional benefit of relieving some of the time for the principal investigator.

This is a model we are exploring within the DMD Hub to contribute to addressing the issue of recruiting clinical fellows to work on trials in the UK and an ANP pilot trial will start in 2021 in Oswestry.



“ The initial support we received from the DMD Hub was critical in enabling us to develop neuromuscular research in Leeds. Subsequent DMD Hub ‘pump-primed’ posts have been essential in expanding our research capacity, allowing us to recruit additional patients to existing studies, including families from outside our region, as well as being able to open new interventional studies in DMD.

Dr Anne-Marie Childs, Principal Investigator, Leeds Teaching Hospital (pictured below)



“ Having additional PI time, thankfully funded by DMD Hub, helped us to keep VISION DMD trial running with modifications during the pandemic. It also helped us to keep in touch with other sponsors and to finish up capturing the data needed for FOR-DMD and to continue the STRIDE Patient Registry data collection.

Dr Zoya Alhaswani, Principal Investigator, Birmingham Heartlands Hospital

### DMD HUB PUMP-PRIMING FUNDING MODEL



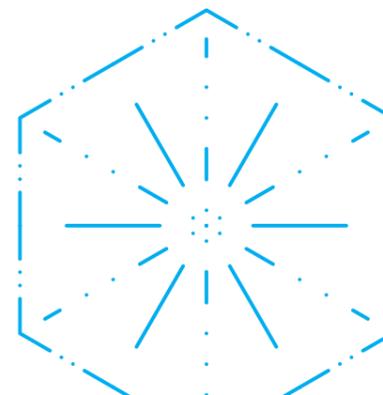
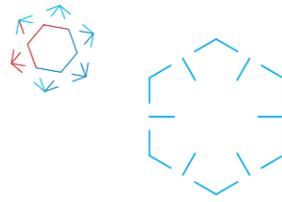
## PROMOTING DMD HUB SITES BY WORKING WITH INDUSTRY

In addition to expanding clinical trial capacity in the UK, the DMD Hub works collaboratively with industry partners to act as a one-stop-shop, providing advice and support as they set-up and run DMD clinical trials.

Industry partners also have privileged access to our DMD Hub toolkit,

including downloadable templates.

We have a flexible approach, evolving and developing additional tools as required by the DMD community, to provide tailored support as detailed below. We are always happy to discuss any specific support needs with industry.



### Communication to Patient Community

As a parent-led organization, Duchenne UK is committed to putting patients at the heart of research. At our last Patient Information Day in September 2019, we brought together representatives from Solid Biosciences, ReveraGen, Sarepta Therapeutics and Catabasis Pharmaceuticals to talk about their trials with 100 parents and family members.

### Patient Recruitment

In February 2020, the DMD Hub supported Sarepta Therapeutics to increase the number of patients recruited to the Essence 53 trial. This involved contacting DMD Hub sites running the trial, assessing their capacity for extra patients, and then pairing them with eligible patients from other DMD Hub sites.



*“ The UK is a crucial part of Sarepta’s mission to develop transformative treatments for DMD and we hugely value the work the DMD Hub has and continues to do to improve clinical DMD research in this country. ”*

*James Richardson, Country Medical Director, UK and Ireland, Sarepta Therapeutics*

### On-going Support

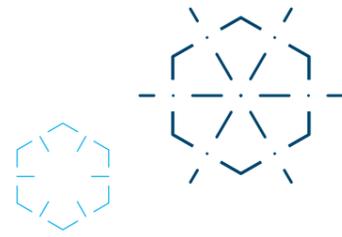
In 2018, the DMD Hub worked with the trial organisers of the TAMDMD, a collaboration between academics and charities, which is looking at the effects of Tamoxifen in DMD patients.

The DMD Hub supported the trial organisers by discussing protocol

issues with sites, as well as advising on ethics and IRAS approvals.



In 2020, the DMD Hub continued to provide support, assisting with the recruitment for the non-ambulant arm of the trial, and promoting the trial through our DMD Hub newsletter, which has around 400 families registered.



## ON-GOING SCOPING

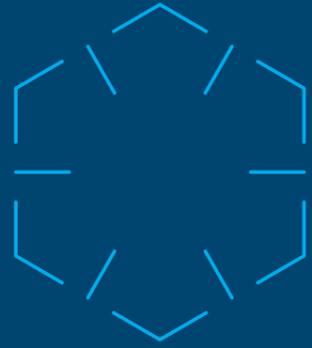
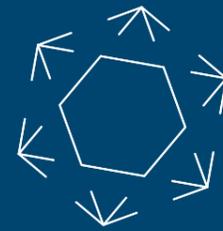
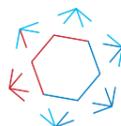
The DMD Hub provides advice and assistance to industry and is currently engaged with the companies listed below:

COMPANY AND TRIAL/PROGRAMME	PHASE I	PHASE II	PHASE III
<b>ANTISENSE THERAPEUTICS</b> – ATL1102	[Progress bar]		
<b>FIBROGEN</b> – LELANTOS	[Progress bar]		
<b>NS PHARMA</b> – RACER53	[Progress bar]		
<b>PFIZER</b> – PF-06939926	[Progress bar]		
<b>SAREPTA</b> – MOMENTUM	[Progress bar]		
<b>SAREPTA</b> – MIS510N	[Progress bar]		
<b>SAREPTA</b> – SRP-9001	[Progress bar]		
<b>SOLID BIOSCIENCES</b> – SGT001	[Progress bar]		
COMPANY AND TRIAL	PHASE I	PHASE II	PHASE III
<b>REVERAGEN</b> – VISION-DMD	[Progress bar]		
<b>DUK/EU COLLABORATION</b> – TAMDMD	[Progress bar]		
<b>CATABASIS</b> – POLARISDMD	[Progress bar]		
<b>CATABASIS</b> – GALAXYDMD OLE	[Progress bar]		
<b>ITALFARMACO</b> – GIVINOSTAT	[Progress bar]		
<b>SAREPTA</b> – ESSENCE	[Progress bar]		
<b>SAREPTA</b> – CASIMERSEN/GOLODIRSEN OLE	[Progress bar]		
<b>SAREPTA</b> – SRP-4053	[Progress bar]		
<b>ROCHE</b> – WN402227	[Progress bar]		
<b>PTC</b> – ATALUREN OLE	[Progress bar]		
<b>PTC</b> – ATALRUEN PHASE 3	[Progress bar]		



Over the last 5 years we have also actively engaged with the following companies and trials.

TRIAL	NATURAL HISTORY/OBSERVATIONAL
TESTOSTERONE FOR DMD	[Progress bar]
TESTOSTERONE EXTENSION	[Progress bar]
FOR-DMD	[Progress bar]
<b>PTC</b> – STRIDE PATIENT REGISTRY	[Progress bar]
<b>GENETHON</b> - PREU7-53	[Progress bar]
<b>GENETHON</b> – GNT-004	[Progress bar]





## THE DMD HUB STEERING COMMITTEE



**Emily Crossley and Alex Johnson** Emily and Alex are co-founders of Duchenne UK and are passionate about advocating for the rights of DMD patients and their families. Both have sons with DMD and established their own charities before merging to form Duchenne UK in 2016.



**Emma Heslop** Emma Heslop is the DMD Hub manager and is funded by Duchenne UK. She has been part of the John Walton Muscular Dystrophy Research Centre at Newcastle University since October 2006, when she joined the TREAT-NMD Neuromuscular network of excellence.



**Professor Volker Straub** Professor Straub is the Deputy Dean, Harold Macmillan Professor of Medicine and Professor of Neuromuscular Genetics at the Institute of Translational and Clinical Research at Newcastle University. One of Professor Straub's main interests in muscle diseases is around translational research. He was the co-founder of the EU FP6 funded network of excellence for genetic neuromuscular diseases, TREAT-NMD, which he coordinated together with Kate Bushby.



**Professor Francesco Muntoni** Professor Muntoni is a Paediatric Neurologist with an interest in clinical, pathological and molecule aspects of neuromuscular disorders. He is the director of the Dubowitz Neuromuscular Centre, a leading clinical and research institution for children affected by neuromuscular disorders.



**Dr Michela Guglieri** Dr Guglieri is a Senior Clinical Lecturer and Honorary Consultant at Newcastle University. She leads the clinical research team which is currently involved in over 30 studies over the past 5 years. Duchenne UK collaborated with 5 patient organisations to award a 5-year lectureship to Dr Guglieri in 2015.



**Dr Anne-Marie Childs** Dr Childs has been working as a Consultant Paediatric Neurologist in Leeds since 2001 when she became the Lead for Children's Neuromuscular Services. She is committed to delivering high-quality care to all patients with neuromuscular disorders in Yorkshire. Her clinical research interests include MR imaging and rare disease phenotyping, although her predominant research involvement is now in relation to DMD and SMA.



# DUCHENNE UK

Duchenne Muscular Dystrophy (DMD) is a devastating muscle-wasting disease. It is the most common and severe form of muscular dystrophy. Diagnosed in childhood, it mainly affects boys. There is currently no cure. **Started by families affected by the disease, Duchenne UK has one clear aim – to end Duchenne.**

We're doing this by funding research that's focused on getting treatments to those affected now – as well as pushing for an effective treatment in the future.

We connect leading researchers with industry, the NHS and patients to challenge every stage of drug development, from research to clinical trials to drug approval. We connect families with each other to create a network of mutual support and to pool our resources, knowledge and experience. We connect brilliant people and generous philanthropists to grow our impact and help them be part of something incredible – the end of Duchenne.

Our heart and soul, and our reason for carrying on, are our boys and men, and rare girls, living with DMD.

This collaborative approach means that we are able to focus on ending Duchenne as quickly and effectively as possible. And it means you can be part of it too. Together, we will end Duchenne.





**THANK YOU NHS**



**[WWW.DUCHENNEUK.ORG](http://WWW.DUCHENNEUK.ORG)**

**[WWW.DMDHUB.ORG](http://WWW.DMDHUB.ORG)**

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