



# DMD HUB

## Impact Report 2021

“In 7 years, the DMD Hub has grown from the creation of a network of clinical trial sites, to a pivotal contact point that is actively enabling and facilitating the setting up of clinical trials in the UK, including for the first time, gene therapy trials in DMD.”

Emily Reuben & Alex Johnson,  
Founders, Duchenne UK

“The DMD Hub has revolutionised the way the UK DMD clinical community communicate around clinical research.”

Professor Volker Straub, Professor of  
Neuromuscular Genetics at Newcastle  
University

The DMD Hub was set up in 2015 to expand Duchenne muscular dystrophy (DMD) clinical trial capacity in the UK: and with each passing year, our ambitions have grown.

The figures speak for themselves: before the DMD Hub was set up, the UK was running 13 clinical trials. Today, 20 trials are ongoing, with the DMD Hub helping to get 8 new trials open in 2021, including the first ever gene therapy trial for DMD.

The DMD Hub works because it has a laser-sharp focus and determination to bring clinical trials to the UK, and support the NHS to run them. It is an exemplar for how multi-stakeholder collaborations can deliver huge improvements to R&D and patients' lives – if enough focus, resource, dedication and expertise all work together for the same collective aim. The DMD Hub model has now been adopted in Australia, and is being used in other disease areas in the UK, like Facioscapulohumeral muscular dystrophy (FSHD) and the Brain Tumour Trust. Since 2015, through the DMD Hub, Duchenne UK and its partners have funded 34 posts, and the DMD Hub has brought companies, and income to the NHS through clinical trials at 11 different sites across the UK.

Our sustainable pump-priming funding model means that income generated from trials is invested back into the neuromuscular teams at the trial sites.

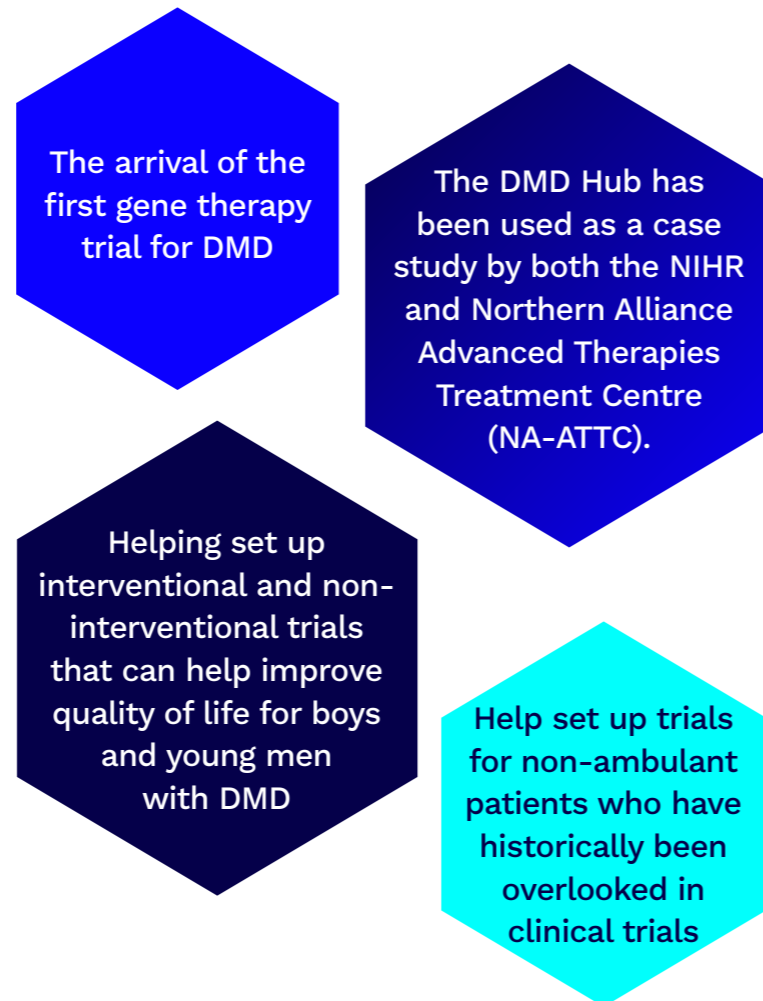
The DMD Hub is working effectively to coordinate with pharmaceutical companies, sites, and patients to ensure that the UK remains a competitive destination for gene therapy and other promising treatments.

The DMD Hub is committed to promoting access to research for all patients with DMD. In addition to gene therapy trials, we have supported the opening of other interventional trials, including a trial for non-ambulant patients as well as several in exon skipping. There have also been several

innovative non-interventional trials aiming to improve the quality of life for boys and young men with DMD. For example, the BIND study 'looking at the relation between dystrophin abnormalities and the central nervous system, as well as a study using immersive virtual reality for physiotherapy.

Our innovative Clinical Trial Finder provides the most up to date information about clinical trials to the DMD community and enables faster recruitment to trials. This year we are focusing on trying to make trial recruitment as fair as possible and we will be launching a pilot study this year to test a central recruitment procedure.

### Some of our highlights from this year include:



### What is the DMD Hub?

The DMD Hub, funded by Duchenne UK, is a groundbreaking 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), and NHS clinical trial sites to deliver trials in Duchenne muscular dystrophy (DMD) in the UK.



“We believe that collaboration has truly benefited both initiatives and will reduce duplication of effort in converting gene therapy into transformative new treatments”

(Jim Shaw, Professor of Regenerative Medicine, NA-ATTC Clinical Advisory Group Chair)

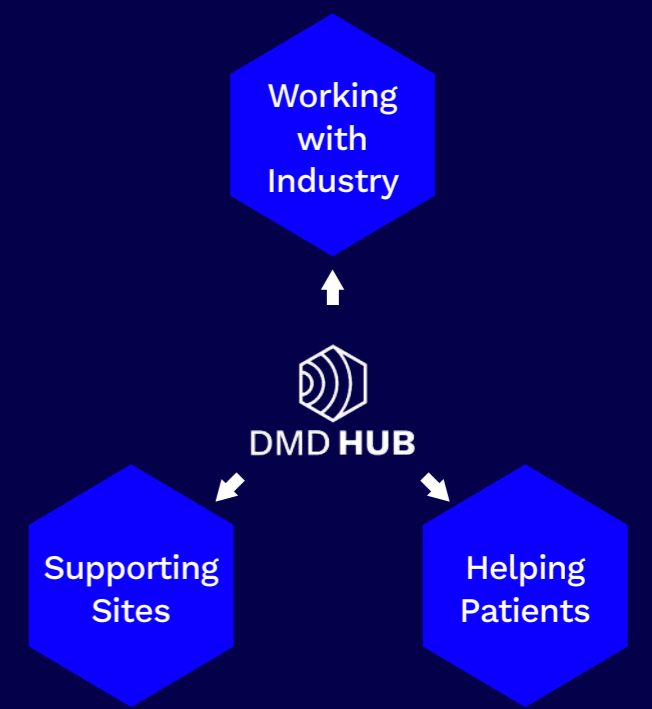
### Who is Duchenne UK

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

As the leading Duchenne muscular dystrophy (DMD) charity in the UK, we're going further to find effective treatments for DMD to end its devastating impact. We're doing it faster, too, by accelerating access to these treatments and therapies for this generation of patients.

And we're here to support every family affected and ensure that they receive the best possible care.

Together, we will end Duchenne.



# Contents

- 3** Introduction
- 7** Supporting industry
- 13** Supporting DMD Hub trial sites
- 15** The DMD Hub and gene therapy
- 19** Training and resources for DMD Hub staff
- 23** Pump-priming model and the long-term sustainability of the DMD Hub: Case studies
- 27** Working for patients, improving recruitment to clinical trials in the UK
- 35** DMD Hub steering committee



**13** more boys participating in interventional clinical trials since 2020



**£3m** committed since 2015

**9** trials in set up

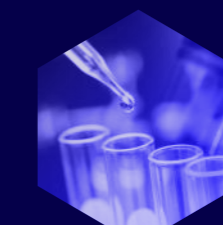
**34** posts funded

**11** DMD Hub sites

**437** boys on trials

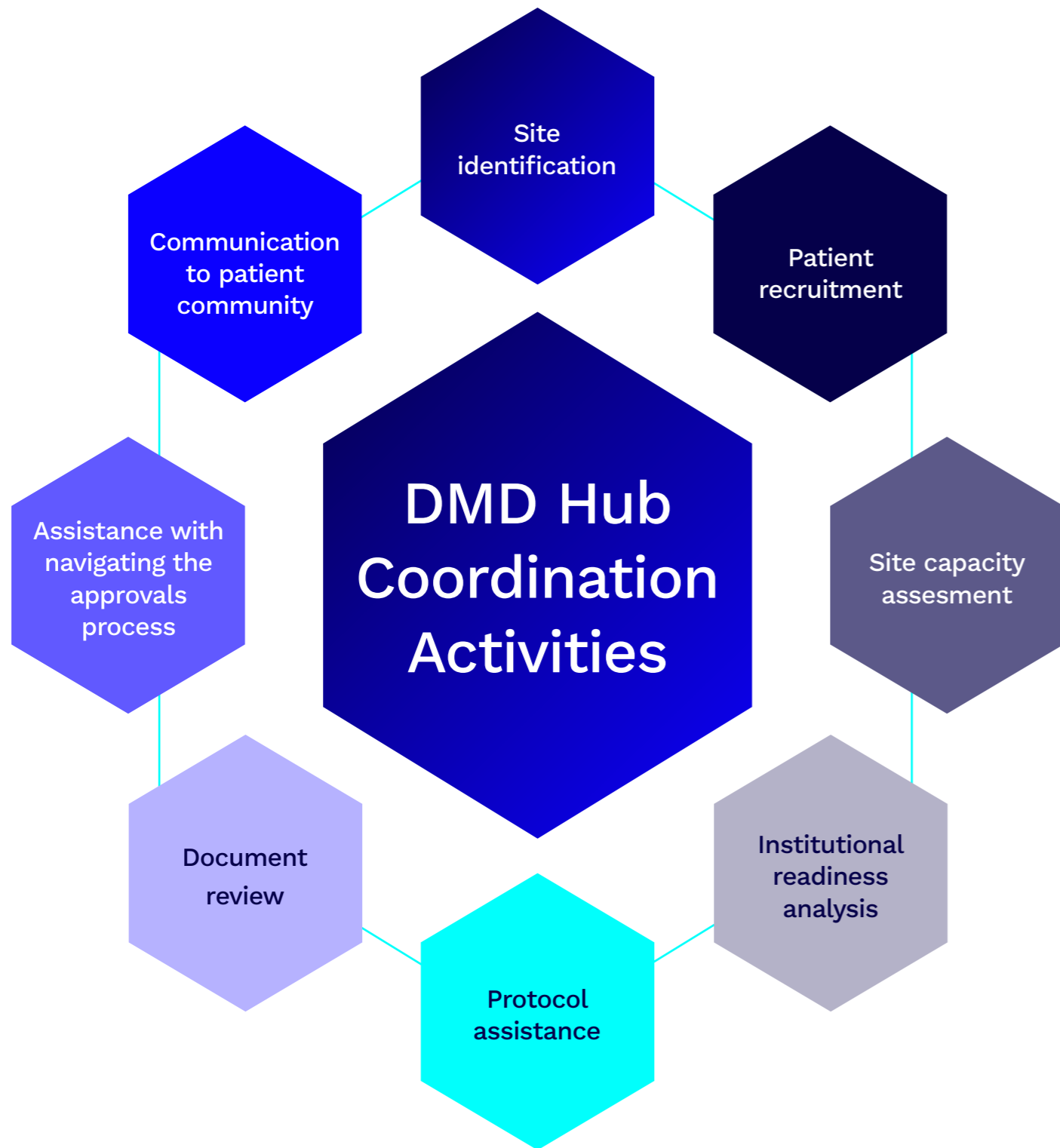
**8** trials opened in 2021

**20** trials ongoing in the UK

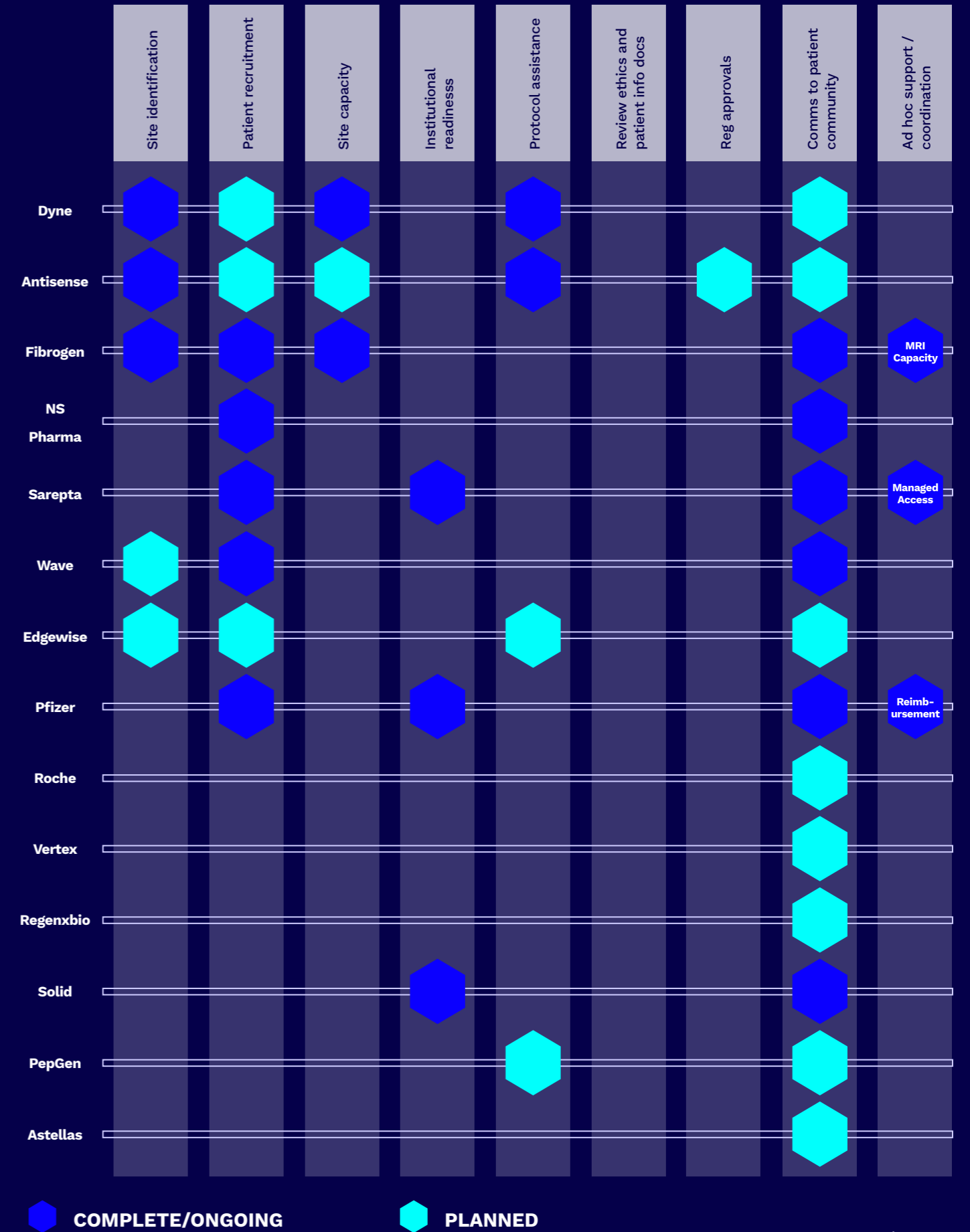


## Engaging with and supporting companies running DMD trials in the UK

Because the DMD Hub works closely with hospital sites and clinical staff we are able to act as a one-stop shop for pharmaceutical companies wanting to run DMD trials in the UK. In 2021 we were able to advise 14 companies. We offer the following services, and are able to offer ad-hoc support as required.



## In 2021, we assisted the following companies through:



Supporting  
Industry

Patient  
recruitment

Protocol  
assistance

## Facilitating faster patient recruitment

As soon as the DMD Hub is informed that a site is recruiting for a trial in the UK, an email alert is sent out to people who have requested to hear about clinical trial updates. Currently, there are over 200 parents and caregivers from the UK on the mailing list. The e-mail contains information about which sites are recruiting and contact details for them so families can get in touch directly.

“The DMD Hub has been tremendously helpful in sorting out site activation impediments and in communicating the trials out to the DMD community across the UK. Without their assistance we would not have been able to activate sites on time and meet enrolment goals.”

Christina Jobson, Associate  
Director, FibroGen Inc.

## Providing protocol review through the DMD Hub Principal Investigator network

The DMD Hub Principal Investigator (PI) network was established in 2020. It consists of at least one PI from each of the DMD Hub sites and meets regularly to discuss ongoing issues including upcoming trials and recruitment. This year, the DMD Hub PIs have provided protocol review assistance to companies planning to run DMD trials in the UK, where the company can submit their protocol for review and ask questions specific to the practicalities of running DMD trials in the UK.

The review allows the PIs to discuss the UK patient population and site capacity, and helps identify any possible issues to ensure they are tackled early in the process and avoid unnecessary delays.

All discussions are held under a confidentiality agreement and a written report is provided to the company after the meeting. To date, the network has carried out two reviews, and feedback indicates that both have been exceptionally helpful for the companies.

“The UK DMD Hub provided a unique opportunity to have a dialogue with the leading Duchenne research institutions in the UK all at one time, and we would highly recommend this to other sponsors. The process for engagement was clear and there was excellent communication with the coordinator. The DMD Hub investigators were clearly very knowledgeable and experienced in conducting clinical trials, and provided valuable feedback on our planned clinical program in Duchenne.”

Ash Dugar, Global Head of Medical Affairs and Kristina Johnson,  
Head of Clinical Operations, Dyne Therapeutics



Supporting  
Industry

Communication  
to patient  
community

# Opportunities to interact with the patient community

In October 2021, Duchenne UK were able to host their first Parent Information Day for nearly two years and included 100 parents and caregivers. One of the sessions was an update from the companies conducting gene therapy trials – Pfizer Inc, solid Biosciences and Roche, who are collaborating with Sarepta Therapeutics. Both companies and parents welcomed the opportunity to ask questions and better understand each other's concerns and priorities.

**98%** of parents  
agreed or strongly  
agreed that the gene  
therapy information  
session was useful



# 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 trials'*

\*Please note that any trial that is in set-up has not been included. Data correct as of October 2021



**GLASGOW: ROYAL HOSPITAL FOR CHILDREN**  
 Posts: 3  
 Year of funding: 2018  
 Current trials: 4  
 Potential trials: 3

**NEWCASTLE: JOHN WALTON MUSCULAR DYSTROPHY RESEARCH CENTRE**  
 Posts: 8  
 Year of funding: 2015 and 2020  
 Current trials: 11  
 Potential trials: 8

**LIVERPOOL: ALDER HEY CHILDREN'S NHS FOUNDATION TRUST**  
 Posts: 4  
 Year of funding: 2017  
 Current trials: 7  
 Potential trials: 6

**LEEDS: LEEDS TEACHING HOSPITAL**  
 Posts: 4  
 Year of funding: 2017 and 2020  
 No. Of current trials: 5  
 Potential trials: 3

**MANCHESTER: ROYAL MANCHESTER CHILDREN'S HOSPITAL**  
 Posts: 1  
 Current trials: 2019  
 Potential trials: 2

**LONDON: DUBOWITZ NEUROMUSCULAR UNIT, GREAT ORMOND STREET HOSPITAL**  
 Posts: 6  
 Year of funding: 2015  
 Current trials: 13  
 Potential trials: 6

**LONDON: EVELINA LONDON CHILDREN'S HOSPITAL**  
 Posts: 2  
 Year of funding: 2019  
 Current trials: 1  
 Potential trials: 3

**OXFORD: MDUK OXFORD NEUROMUSCULAR CENTRE**  
 Posts: 1  
 Year of funding: 2019  
 Current trials: 2  
 Potential trials: 6

**BRISTOL: ROYAL HOSPITAL FOR CHILDREN**  
 Posts: 1  
 Year of funding: 2018  
 Current trials: 2  
 Potential trials: 4

**BIRMINGHAM: BIRMINGHAM HEARTLANDS HOSPITAL**  
 Posts: 1  
 Year of funding: 2018  
 Current trials: 3  
 Potential trials: 1

**OSWESTRY: ROBERT AND AGNES HUNT HOSPITAL**  
 Posts: 3  
 Year of funding: 2019  
 Current trials: 3  
 Potential trials: 2



**Centres of Excellence**

London - GOSH  
 Newcastle



**Hub site**

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



**Other Site**

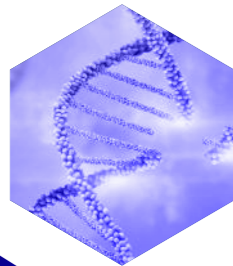
Queens Square  
 Temple Street

# Gene therapy arrives in the UK

One of the key priorities for the DMD Hub over the next five years continues to be preparing UK sites to deliver gene therapy trials.

In May 2021, the first patient was dosed with gene therapy in the UK at the John Walton Muscular Dystrophy Centre in Newcastle Upon Tyne. As such, the DMD Hub is now able to learn from the experience of the Newcastle team and help other sites which will be opening for this trial and similar gene therapy trials in the future.

This represents a huge leap forward, and our mission is to increase the number of gene therapy trials in the UK.



Bringing together key stakeholders around long-term access to gene therapy

In 2019, the DMD Hub conducted a survey looking at the capacity and capability of sites to run gene therapy trials. In 2021, a further survey was designed to gain a more detailed understanding of the experience and interest that UK sites have in delivering these trials. Results of the survey will help the DMD Hub to identify gaps in capacity, processes, training and facilities that need to be addressed before sites can take on these trials. The survey was completed by all DMD Hub sites and the DMD Hub will present the findings in Q1 2022.

Following on from the 2019 DMD Hub gene therapy workshop, we will be hosting a workshop focused on **“Advancing gene therapies for Duchenne muscular dystrophy: what we have learned and where we go next”**, in 2022.

This whole day workshop will bring together key stakeholders to share and reflect on the key learnings in DMD gene therapy to date, and discuss the current challenges and barriers in advancing gene therapies through the development and market access pathway. As a community, we will discuss how we can work together to ensure these potentially transformative treatments can reach and benefit as many patients as possible, as quickly as possible.



1. Heslop E, Turner C, Irvin A, Muntoni F, Straub V, Guglieri M. Gene Therapy in Duchenne muscular dystrophy: Identifying and preparing for the challenges ahead. *Neuromuscular Disorders* Vol. 31, Issue 1 (P69-78) Jan 21 <https://doi.org/10.1016/j.nmd.2020.10.001>



## Gene Therapy: tackling limited experience and facilitating additional staff training

In October 2021, the DMD Hub hosted a face-to-face gene therapy workshop in Newcastle upon Tyne with 25 staff from 8 of the 11 DMD Hub sites, as well as Temple Street, Dublin. The workshop was organised in response to the needs identified by the nurses and clinical trial coordinator networks for greater understanding of gene therapy, and specifically to share learnings between sites around set-up, management and delivery of trials.

The day consisted of many important talks and discussions including:

Gene therapy overview

Psychosocial impact



PI experience

CTC and nurse case studies

Parent experience of screening

Gene therapy training resource



# Gene Therapy

## Some important action points that came from the workshop were:

- Facilitate additional training opportunities for the staff networks
- Provide further Cognitive Behavioural Therapy training
- Develop gene therapy educational material for DMD Hub website
- Explore hub and spoke model for gene therapy delivery at UK sites
- Reviewing consent process for gene therapy trials
- Improve communications with sites, patients and industry to manage expectations
- Support DMD Care UK psychosocial programme

**“Having the opportunity to network with other sites”**

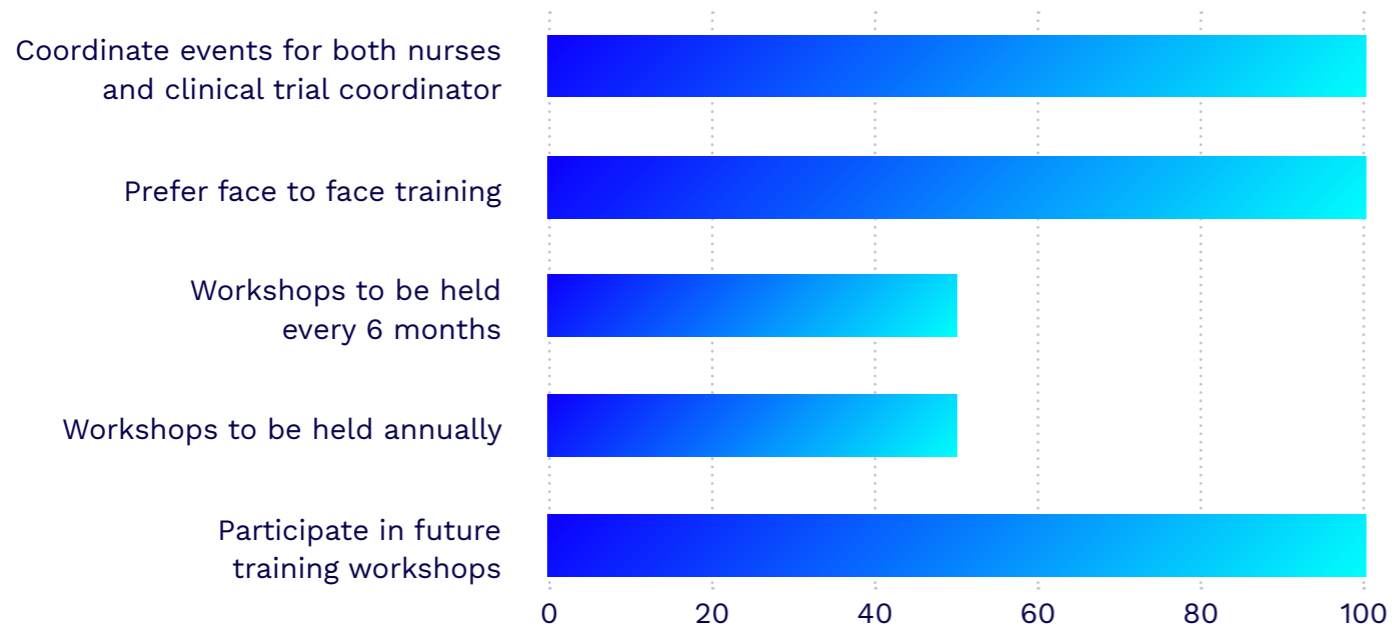
Lizzie Vella, Nurse, GOSH

**“Great location, good facilities, well organised, and good range of topics”**

Sarah Meadowcroft, Nurse, Leeds

The workshop received excellent feedback and the results will help inform the development of the future training and education work within the DMD Hub.

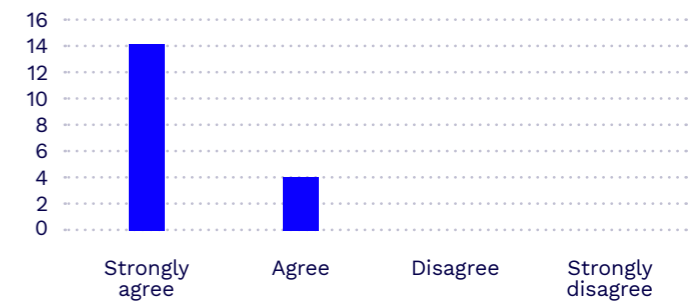
## Future Training Preferences



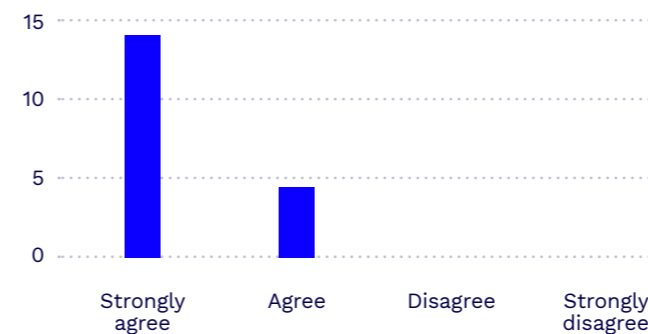
**“Interactive sessions with practical knowledge”**

Helen Sutherland, Clinical Trial Coordinator, Newcastle

Did you feel the talks were relevant to you?



The workshop increased my knowledge on gene therapy



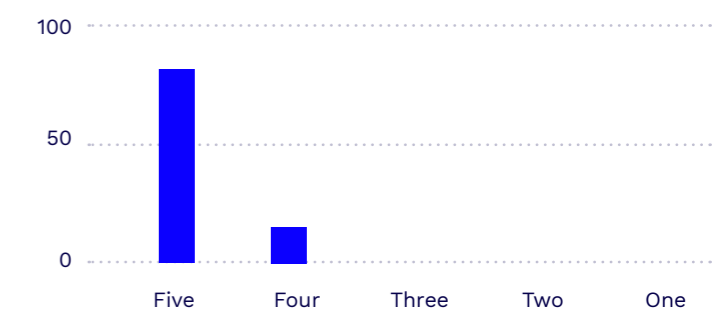
**“I found the parent perspective and panel discussion with nurses and CTCs really informative”**

Ruth O’Neill, Nurse, Dublin

**“Great chance to hear experience gained from gene therapy sites, and highlighted the key challenges involved in the delivery.”**

-Tracey Langan, Clinical Trial Coordinator, Glasgow

Please rate your experience of the workshop out of 5 (5 being excellent and 1 being poor)



## Gene Therapy Roadmap

In collaboration with the NA-ATTC, we are creating a template roadmap for nurses on how to set up and run a gene therapy trial. The DMD Hub will then take the template and enhance the information to make it applicable to nurses and clinical trial coordinators involved in the setting up and running of a DMD gene therapy trial.

The DMD Roadmap will be available on the DMD Hub website and will be disseminated through the DMD Hub networks.

# Providing important training resources to staff at DMD Hub sites

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.

In 2021 the DMD Hub focused on developing tools to help prepare sites for setting up and delivering gene therapy trials, which are highlighted in green.



[View Toolkit Here](#)

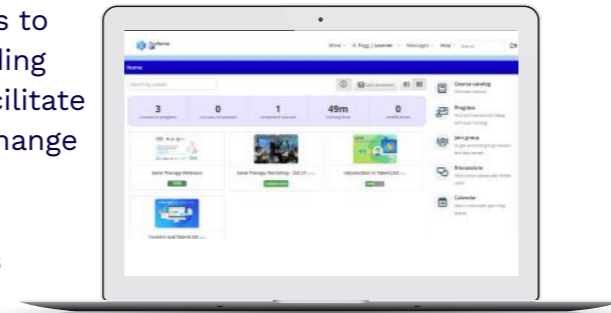


# Increasing accessibility of training through online platform

The next step in the development of the DMD Hub Toolkit is the development of the DMD Hub Online Training Platform, which was launched in November 2021. This is an interactive learning platform to upskill DMD Hub site staff, initially for nurses and clinical trial coordinators. The content of the platform is tailored to training needs identified by the sites, and future developments will

integrate additional tools to help benefit staff, including discussion forums to facilitate communication and exchange knowledge with peers.

One of the first modules to feature are the recorded presentations from the Nurse and Clinical Trial Coordinator Gene Therapy Workshop.



## Engagement and Collaboration

The DMD Hub works collaboratively with organisations to facilitate the sharing of knowledge and to reduce duplication of effort.

Since 2020, we have worked with the Northern Alliance Advanced Therapy Treatment Centre (NA-ATTC) to address the need for training and education to support implementation of gene therapy clinical studies and subsequent rollout. DMD Hub Principal Investigators Prof Volker Straub and Dr Michela Guglieri are key contributors to the NA-ATTC Clinical

Advisory Group (CAG) and Trial Coordination Unit (TCU), with the TCU forum endorsing the Safety Task Force, conceived by Dr Guglieri, as an example of sharing best practice. The DMD Hub made valuable contributions to the creation of the Advanced Therapies Clinical Trial Toolbox, and provided more specific Institutional Readiness data related to gene therapy medicinal provision for the overall benchmarking of Institutional Readiness levels exercise.



The DMD Hub also contributed a case study to the NA-ATTC Harmonisation document and has plans to continue the collaboration in 2022.

The DMD Hub and NIHR have been collaborating to ensure trials in DMD are appropriately costed. The DMD Hub guidelines have now been supported by NIHR and are available on the NIHR website alongside the NIHR costing tool.

In addition to collaborating with existing initiatives, the DMD Hub has also shared knowledge and experience of the tools developed with other countries looking to set up a similar network (Australia) and other diseases groups keen to replicate the DMD Hub model (FSHD and Brain Tumour).

“ We are very grateful to be introduced to the DMD Hub and its innovative projects. There are several elements of the DMD Hub that are relevant and useful to apply to the brain tumour community including the Clinical Trial Finder and the impressive way of networking and collaborating through the DMD Hub. We are excited about staying in touch with the project team and exchanging ideas and knowledge to benefit rare disease treatment. ”

Nicky Huskens, Director,  
Tessa Jowell Foundation



## Site Exchange Visits

One of the most important ways for sites to learn from each other is to take part in exchange visits to understand how experienced sites set up and run DMD clinical trials. Sites who have started to run DMD trials will host staff from sites yet to/in the process of setting up the same trials. This will allow experienced sites to share their knowledge and experience to facilitate the set-up of trials in newer sites.

This has been made possible thanks to funding by the John Coates Charitable Trust, PTC Therapeutics, Roche and Sarepta Therapeutics. Covid-19 has unfortunately meant our plans have been delayed - however we are planning for these to resume in 2022.

“ At the time we spoke to the DMD Hub, we were just starting to explore how we might make our brain tumour Clinical Trial finder better. Being able to look at the DMD Hub and being able to talk to Emma about the network of relationships they have developed and maintained over the years which has made it possible to curate and maintain the data that underpins the DMD Hub Clinical Trial Finder was incredibly useful. Thank you for your help and insight. ”

Fiachra Woodman, Chief Technology Officer, The Brain Tumour Charity



# Pump-priming model and the long-term sustainability of the DMD Hub: Case studies

In the DMD Hub, we have an innovative funding model for posts at sites, which helps ensure their sustainability. In combination with a tapered funding model, we work with NHS trusts to support neuromuscular teams and with industry to promote sites as attractive destinations for DMD trials.

Several of the posts previously funded by the DMD Hub have been taken on by NHS Trusts. Our funding model has also enabled staff to develop in the neuromuscular field and take on substantive roles. By funding key junior doctor roles for clinical trials, the DMD Hub is playing a crucial role in developing the next generation of consultants. New appointments such as these demonstrate how our funding

model is providing sites with long-term capacity to run clinical trials in the UK.

Dr Emily Whitehouse was appointed as the Clinical Research Fellow at Royal Manchester Children's Hospital (RMCH) in 2019, funded by the DMD Hub. This is a position which provides support to the neuromuscular consultant, such as by running patients' assessments to measure how well a trial drug is working. In June 2021, she was appointed to a substantive neuromuscular consultant post, funded by the trust, which will allow her to lead on DMD clinical trials and all aspects of the care of patients with neuromuscular conditions.

“We are delighted that Dr Whitehouse has been appointed to join our neuromuscular consultant team and extremely grateful to the DMD Hub for supporting the post of Neuromuscular clinical research fellow, which gave her invaluable experience in clinical trials and all aspects of management of patients with neuromuscular disease, cultivating her interest to continue in this speciality.”

Dr Imelda Hughes, Paediatric Neurology Consultant at Manchester University NHS Trust



“Funding from DMD Hub allowed me to gain experience in an area of paediatric medicine which I would not otherwise have had the opportunity to do. The post gave me a wide exposure to neuromuscular disease including working with boys with DMD and their families and understanding the clinical research process. The model of increasing access to research for families is vital in improving future care of our patients and I am incredibly grateful for the opportunity given to me.”

Emily Whitehouse, Neuromuscular Consultant, Manchester Children's Hospital



# DMD Hub continues to expand neuromuscular teams and develop new areas of focus

During 2021, the following members of staff have joined the DMD Hub:

Amy Wyatt	Research Physiotherapist	Robert Jones and Agnes Hunt Hospital (RJAHH), Oswestry
Chloe Perry	Clinical Trial Coordinator	Robert Jones and Agnes Hunt Hospital (RJAHH), Oswestry
James Jones	Advanced Nurse Practitioner	Robert Jones and Agnes Hunt Hospital (RJAHH), Oswestry
Katie Pegg	Training and Education Coordinator	John Walton Muscular Dystrophy Centre, Newcastle
Phillip Cammish	Central Recruitment Manager	John Walton Muscular Dystrophy Centre, Newcastle

Oswestry have become the latest site to be funded by the DMD Hub with 3 posts. The Advanced Nurse Practitioner role will serve as a pilot to assess the impact of such a role on the ability of a team to deliver clinical trials.



## James Jones - Advanced Nurse Practitioner

James completed his paediatric nurse training in 2005 in Manchester and spent 3 years working at the burn's unit at Manchester Children's Hospital. He then spent time working on neonates before working for 6 years full time at Hope House Children's Hospice providing specialist palliative care to children and young adults. In 2015 James took on a new research post and spent 5 years developing the paediatric portfolio as well as supporting many other specialities within research. He continued and still continues to work one day a week at hope house clinically and is passionate about supporting families clinically and driving research. He joined the DMD Hub team at Oswestry in late 2021.



## Amy Wyatt - Research Physiotherapist

Amy qualified as a physiotherapist in 2013 from Birmingham University. Amy joined the DMD Hub and the Oswestry Neuromuscular Team part time in May 2021. She also works in the outpatient physiotherapy service specialising in neurological conditions for 2 days a week.

Amy is involved in the screening and assessment of patients taking part in research trials, and reviews patients attending the neuromuscular clinic for routine reviews. She has a special interest in rehabilitation for patients presenting with neurological and neuromuscular conditions, and respiratory monitoring and preventative treatment options.

## Networks

In 2020, we established networks for nurses and clinical trial coordinators so they can share experiences and provide updates about trial activity to the DMD Hub. This year the networks have focused on opening trials, recruitment and preparing for gene therapy.

We have 20 trial coordinators and 21 nurses from all sites who actively participate in the online meetings.

In early 2021, the DMD Hub coordinated a 2-day cognitive behavioural therapy training course for research nurses to better equip them to help families through the demands involved in taking part in a trial, including consenting, screening, and trial termination.

Feedback from the nurses who participated in the course was positive and there is



## Chloe Perry - Clinical Trial Coordinator

Chloe joined the DMD Hub as a Clinical Trial Coordinator in May 2021. Her role as a CTC involves the day-to-day management of the trials. She works closely with patients and their families, clinicians, and pharmaceutical companies to deliver trials as per GCP and protocol standards.

“Through the funding of our 3 posts, we can consider to take on more clinical trials in Oswestry, expanding our portfolio of DMD studies. It also allows us to become more accommodating to eligible patients to join the trials and working within the DMD Hub network helps give every patient a chance to join a clinical study. We plan to open more DMD studies in the future and support the DMD Hub network as much as we can.”

Chloe Perry

demand for similar courses to be made available to nurses and other healthcare professionals in the future. All participants we received feedback from stated that objectives were clear, presenters were engaging, content was organised, questions were answered well, and the course length was appropriate.

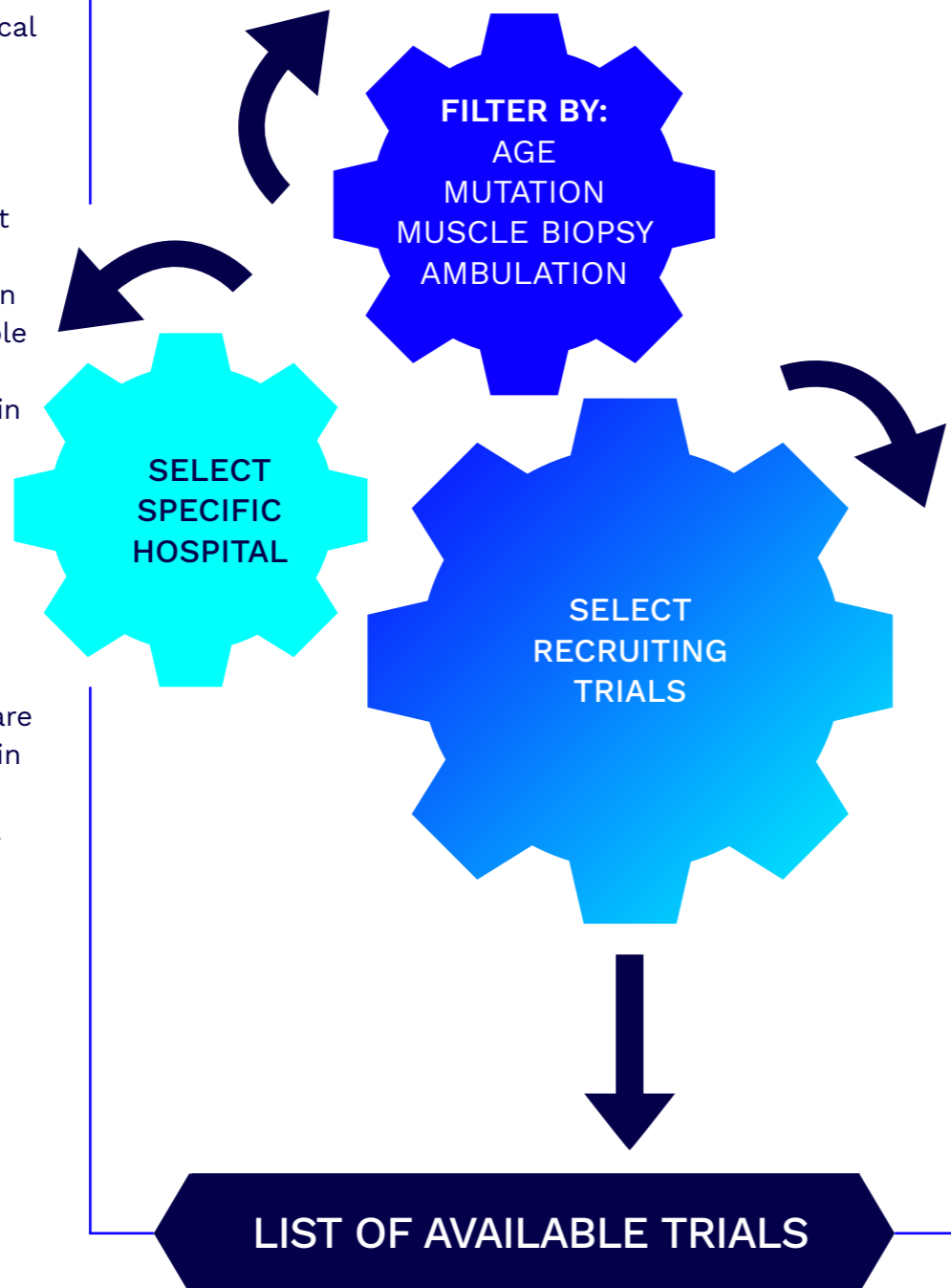
# Improving recruitment to clinical trials in the UK

Since 2015, the DMD Hub has expanded the number of hospitals that run trials in DMD and increased the number of opportunities for people to take part in clinical trials.

The Clinical Trial Finder (CTF) has been a key tool in improving recruitment at sites and in 2021, we have been looking at how we can further develop this valuable tool to give more patients opportunities to take part in research.

The DMD Hub Central Recruitment Pilot Project will establish a 'national recruitment contact database', for people diagnosed with DMD who are interested in participating in DMD specific clinical trials taking place within the UK.

## How the clinical trial finder works



## Providing greater access to trials for patients across the UK

Due to the complexity of DMD and of setting up trial sites to conduct clinical studies, they often only run in a limited number of centres in the UK. This creates challenges for clinicians, patients and their families.

In a survey we carried out to gather patient and caregiver perspectives on DMD clinical trials we found that 91% of patients surveyed said they DID want to take part in research, but 68% mainly used social media to find out about studies and were most likely to get onto a trial through continued emailing of clinicians and trial co-ordinators. 29% said that they had tried to enrol but were not able to. The survey highlighted the sense of desperation felt by patients and caregivers who were unable to access research.

**"absolutely devastated"**

**"hopeless and a feeling that we are missing out"**

**"Disappointed with an unfair system"**

“ It was important to us that when we set up the DMD Hub that we created a system that gave the 91% of the people surveyed a fair and equitable chance to take part in trials and we created information for patients and their families that made this process easy and clear. The central recruitment pilot for trial recruitment and Clinical Trial Finder are helping us achieve these objectives ”

Alex Johnson, co-founder and joint CEO of Duchenne UK

## What will the DMD Hub Central Recruitment Pilot Project do for registered participants?

The pilot study will establish a centrally coordinated national recruitment contact database with information about people diagnosed with DMD, including genetic diagnosis and preferences for participation in clinical trials (e.g. types of trials and distance willing to travel). Registration via the study website will be voluntary and

initiated by the person with DMD or their parent/guardian. This information will support clinical sites in identifying potentially eligible candidates for trials.

Recruitment to a clinical trial will not be guaranteed if a patient or parent decides to participate in this pilot. This will still depend on national

recruitment targets, eligibility criteria and sites will still be selecting participants.

However, the study will ensure that sites look at patients from outside of their local area, so even if the patient or parent is not seen at the site, they could have the opportunity to take part.

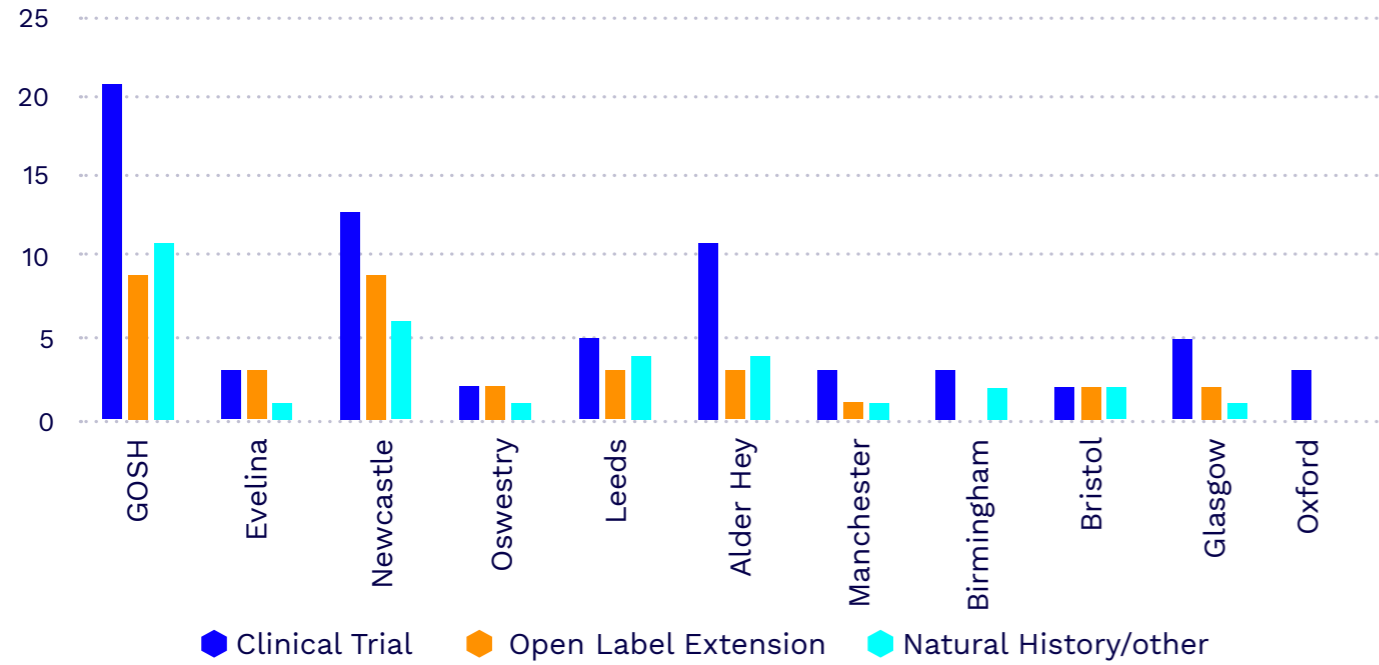
## When will the DMD Hub Central Recruitment Pilot Project be available?

The central recruitment database will launch in early 2022 and will be initially active 12 months. We will engage the patient community both through the DMD Hub mailing lists and working with other networks to ensure that as many families as possible have the opportunity to register.

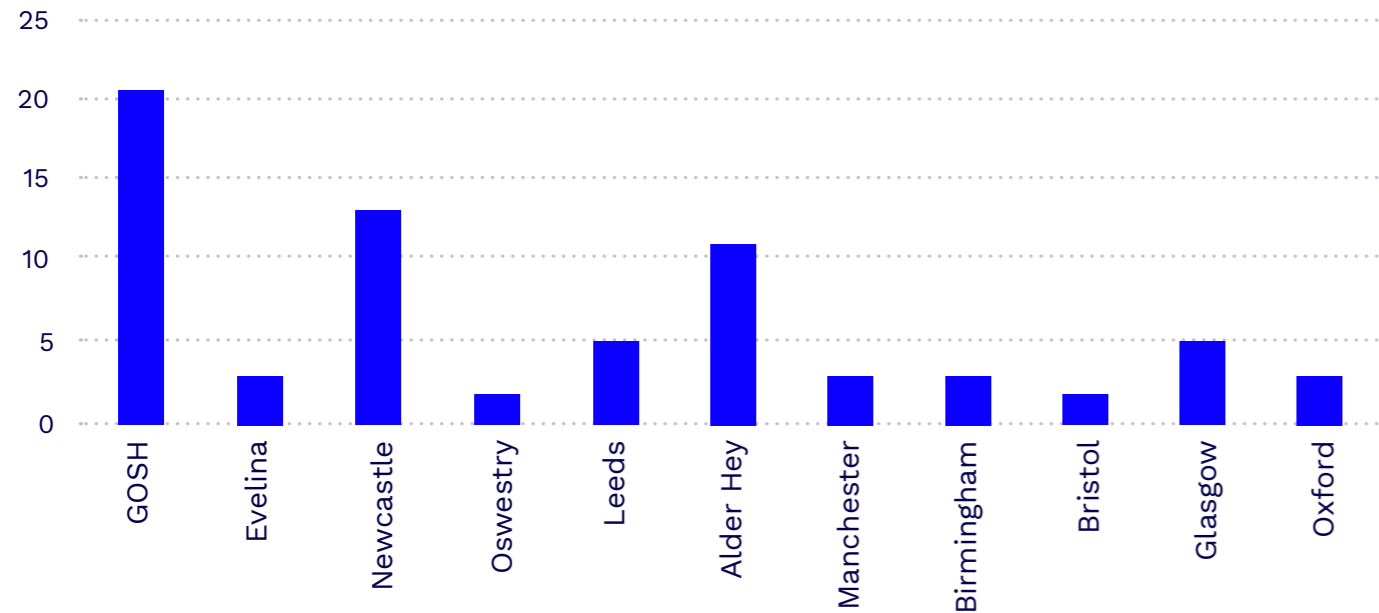


## Clinical trials

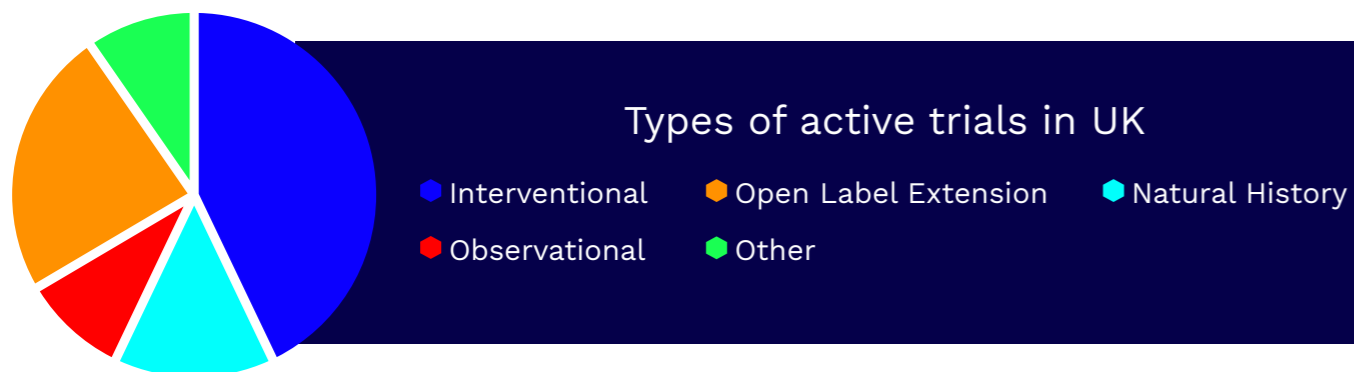
Types of trials at DMD Hub sites since 2015



Number of boys recruited to interventional clinical trials since 2015



Types of active trials in UK



“ Patient data is extremely important to ensure the recruitment process of any clinical trial is efficient. As a parent of a child with Duchenne, I am really pleased to see the effort that has been made by the DMD Hub and the DMD Hub trial sites to make headway in the development of this national recruitment process. This project will be addressing key concerns associated with the recruitment of patients for Duchenne clinical trials and is certainly a positive step forward for the DMD community.”

Sejal Thakrar, Central Recruitment Pilot steering group member



# DMD Hub Webinars

Engaging and communicating with parents and caregivers about key issues around trials

In collaboration with Duchenne UK, the DMD Hub ran two webinars looking at the important issues of **what happens when trials end**, as well as **what patients need to know about gene therapy**.

The webinars brought together parents of DMD boys, clinicians and industry representatives to talk about these important issues, especially the challenges companies face trying to communicate trial failure to patients and the upset caused to families during the lengthy gene therapy screening and consenting process. These have been viewed more than 100 times and are now stored on the DMD Hub toolkit and on the Duchenne UK Youtube channel.



[View Duchenne UK Youtube channel](#)

## Collaborating with existing initiatives to promote improved psychological support for patients and families

The DMD Hub has also been collaborating with Duchenne UK's DMD Care UK Project, which ensures that everyone in the UK has access to the best care, no matter where they live. Emotional and psychological support for patients and their families, particularly whilst taking part in clinical trials, has been limited to date, and the DMD Hub is committed to finding ways to address this lack of psychological support available.

The DMD Care UK Project has recently announced a Psychosocial programme, funded by Duchenne Research Fund, to address the current lack of understanding of, provision for and consensus around psychosocial care and needs, and make recommendations for improvement. The DMD Hub will work with the DMD Care UK Project to encourage these recommendations are implemented at all DMD Hub sites.



[View DMD Care UK Website](#)

## Case Study – Consenting for Gene Therapy Trials



*'Louis was brought into the [consent] process for the gene therapy trial before any samples were taken, but once we found out that he had antibodies, he wasn't able to take part. He still asks about "when is the trial going to start" and "when will the medicine make my muscles better" so he can do the same things as his brother. To not do anything was not an option, but I wish I could have saved him this.'* **Helen Graham, DMD parent**



# DMD Hub Steering Committee



Emily Reuben  
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 2006, when she joined the TREAT-NMD Neuromuscular network of excellence.



Professor Volker Straub

Professor Straub is the Harold Macmillan Professor of Medicine, Professor of Neuromuscular Genetics, Director of the John Walton Muscular Dystrophy Research Centre and Deputy Dean at the Institute of Translational and Clinical Research, 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 disease, TREAT-NMD, which he coordinated together with Professor Kate Busby.



Dr Anne-Marie Childs

Dr Childs has been working as a Consultant Paediatric Neurologist in Leeds since 2001 when she became the Lead of 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.



Dr Michela Guglieri

Dr Guglieri is a Senior Clinical Lecturer and Neuromuscular Consultant at Newcastle University. She leads the clinical research team which have been 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.



Professor Francesco Muntoni

Professor Muntoni is a Paediatric Neurologist with an interest in clinical, pathological and molecular 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.





**DMD HUB**

[duchenneuk.org](http://duchenneuk.org) | [dmdhub.org](http://dmdhub.org)



**Duchenne  
UK**

[info@duchenneuk.org](mailto:info@duchenneuk.org)

Registered Charity No. 1147094

A non profit company limited by guarantee.

Registered in England No. 8030768