DMD Hub: Expanding Clinical Trial Capacity for Duchenne Muscular Dystrophy in the UK

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The DMD Hub is expanding clinical trial capacity for Duchenne muscular dystrophy in the UK. Working with key stakeholders, the DMD Hub is building on existing expertise to create a network of clinical trial sites.

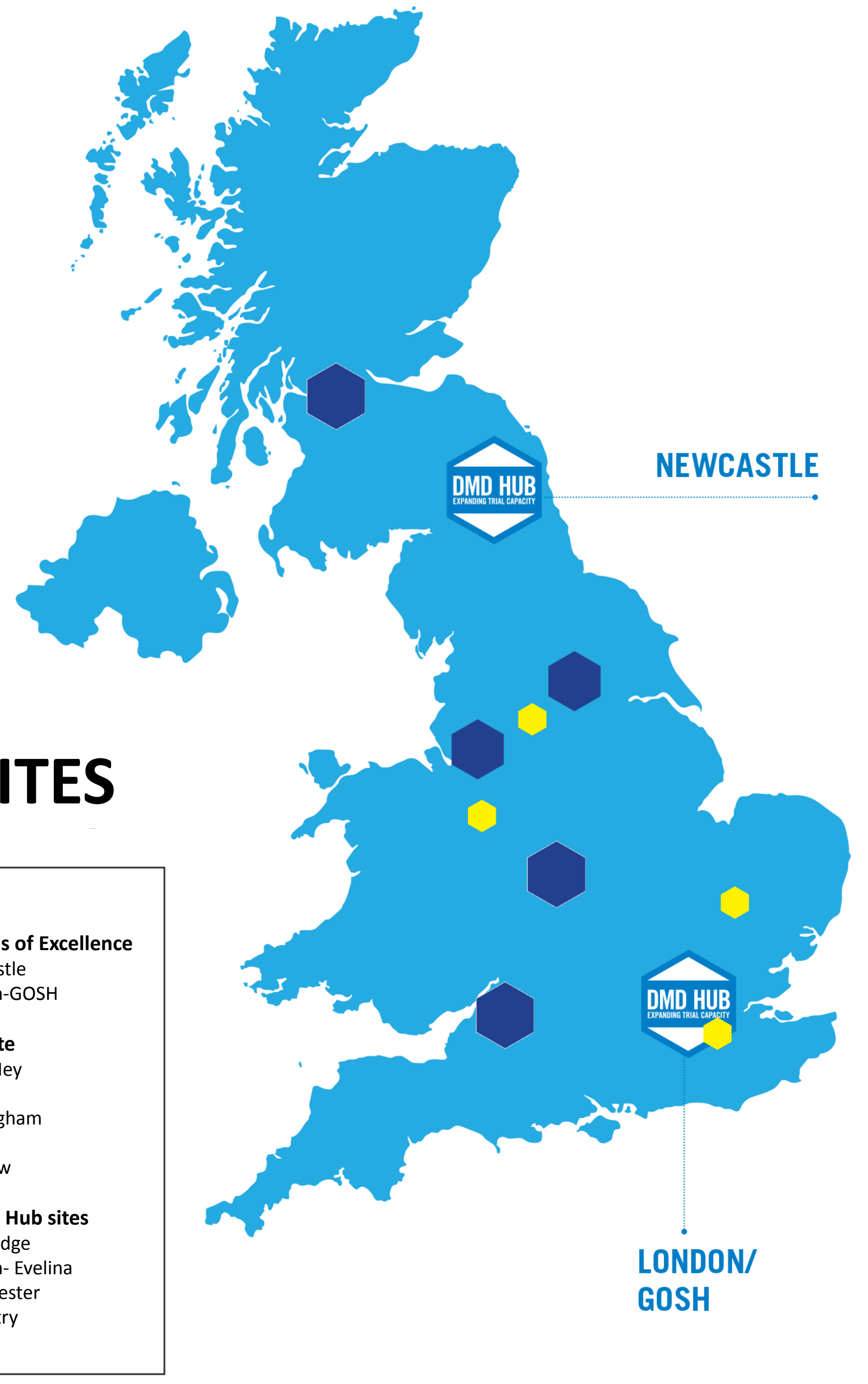
Our mission is that every child in the UK diagnosed with DMD is given the opportunity to take part in clinical research.

Clinical research in Duchenne muscular dystrophy (DMD) is at an unprecedented stage in terms of the number of possible therapeutic approaches coming to trials.

Pharmaceutical companies can be reluctant to consider clinical trial sites with no experience of running DMD trials, leading to bottlenecks at existing experienced sites which are reaching capacity.

The DMD Hub is working to make more clinical sites ready for trials by providing training, mentoring and resources to help them achieve the requirements set by industry.

The John Walton Muscular Dystrophy Research Centre in Newcastle and Great Ormond Street Institute of Child Health in London are two existing centres of excellence. The first DMD Hub sites launched in 2017 were Alder Hey and Leeds. In 2018 sites in Glasgow, Birmingham and Bristol have received support and the DMD Hub is working with the additional sites listed below to prepare them for upcoming industry and academic-led trials.



HUB SITES

KEY:

Centres of Excellence
Newcastle
London-GOSH

Hub site
Alder Hey
Leeds
Birmingham
Bristol
Glasgow

Future Hub sites
Cambridge
London- Evelina
Manchester
Oswestry

The DMD Hub is collaborating with existing infrastructures such as TREAT-NMD, the National Institutes of Health Research, the North Star Network and the DMD UK Patient Registry to develop resources whilst avoiding duplication of the significant work already achieved in this area.

The DMD Hub website (**www.dmdhub.org**) is a key resource for industry, clinicians and patients. It hosts an interactive clinical trial finder detailing clinical trial opportunities in the UK for patients, includes a Toolkit containing training and educational material for sites and acts as a one-stop shop for industry / sponsors interested in conducting trials in the UK.

OUR AMBITION IS THAT EVERYONE IN THE UK DIAGNOSED WITH DMD IS GIVEN THE OPPORTUNITY TO TAKE PART IN RESEARCH.

SEARCH FOR A CLINICAL TRIAL

Search the UK for existing and pending DMD clinical trials

- Clear information for you to make an informed choice.
- Clinical trial requirements, location and duration.

WORKING TOGETHER TO BRING TRIALS TO ALL.

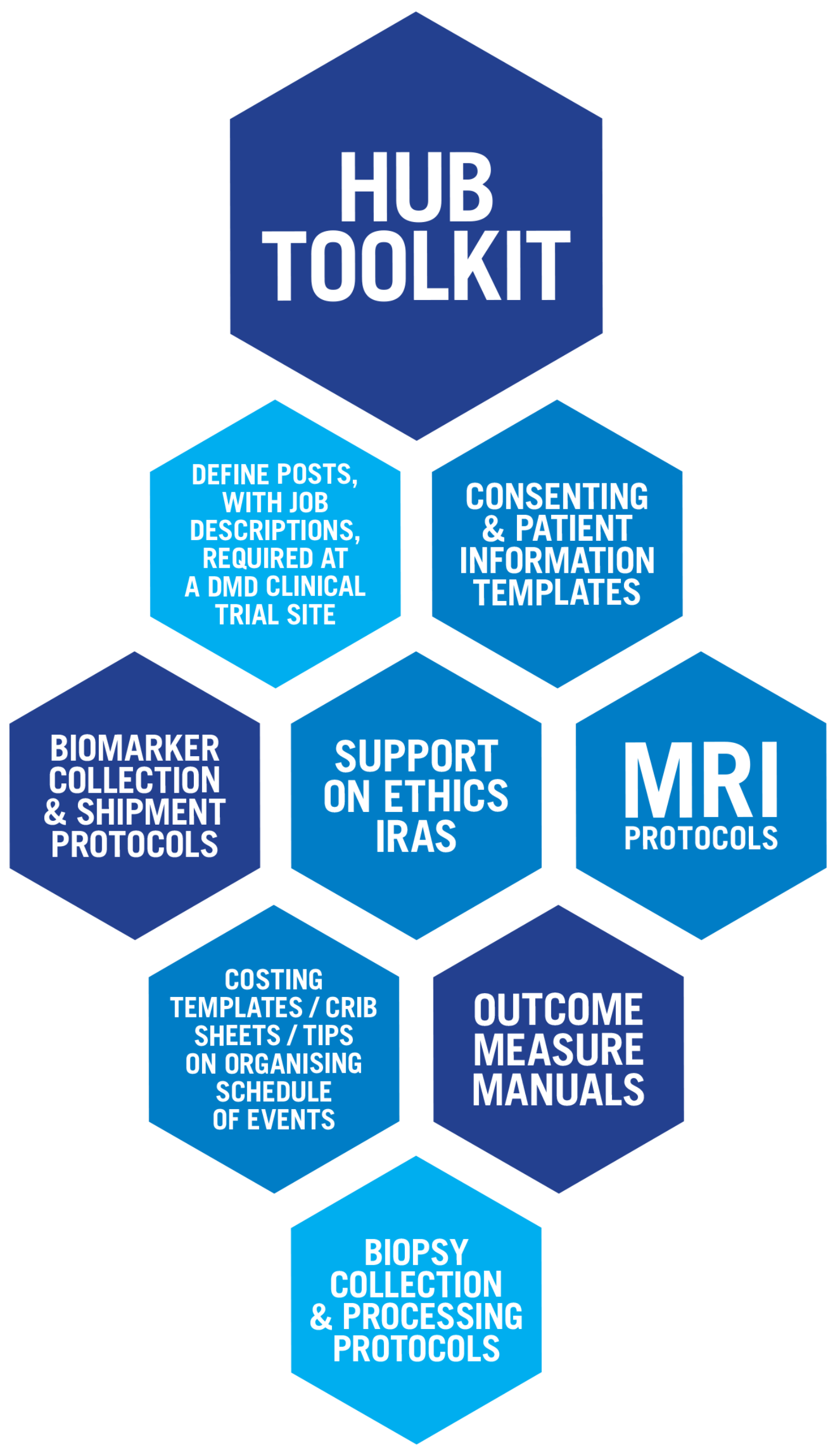
PATIENTS
All you need to know about clinical trials.

HUB SITES
What the DMD Hub can provide.

INDUSTRY
How the DMD Hub can help you.

The Hub is a network of trial sites - and staff - trained and funded to carry out clinical trials for DMD.

The DMD-Hub Toolkit is aimed at upskilling existing sites. It can offer pragmatic support in trial set up and aims to result in the establishment of a common UK approach to running trials, offering industry reassurance. The Toolkit will evolve over time, as additional resources are identified by patients, sites and industry.



HUB TOOLKIT

DEFINE POSTS, WITH JOB DESCRIPTIONS, REQUIRED AT A DMD CLINICAL TRIAL SITE

CONSENTING & PATIENT INFORMATION TEMPLATES

BIOMARKER COLLECTION & SHIPMENT PROTOCOLS

SUPPORT ON ETHICS IRAS

MRI PROTOCOLS

COSTING TEMPLATES / CRIB SHEETS / TIPS ON ORGANISING SCHEDULE OF EVENTS

OUTCOME MEASURE MANUALS

BIOPSY COLLECTION & PROCESSING PROTOCOLS

Innovative funding models are being implemented at DMD Hub sites to ensure sustainability of the funded posts. Expansion of the model to other rare neuromuscular diseases and other countries is being explored.

HUB AIMS

FIRST YEAR ACHIEVEMENTS

- **Establish a network of clinical trial sites in the UK, providing core support to undertake DMD trials**
- **Set up Alder Hey Children's Hospital in Liverpool and The General Infirmary at Leeds as the first DMD Hub sites capable of running academic and industry led studies**
- **Work with industry to promote DMD Hub site capabilities**
- **Utilise existing resources to enhance the work already achieved collaborating with TREAT-NMD, North Star Clinical Research Network, the National Institute of Health Research and Action Duchenne UK DMD Patient Registry**

SECOND YEAR

- **Establish and launch a DMD Hub website for sharing resources and information**
- **Coordinate UK industry enquiries**
- **Set-up 3 additional Hub sites to run industry and academic led trials**
- **Expand focus to non-ambulatory population**
- **Use the DMD Hub to inform and engage the community about taking part in research**
- **Establish the DMD Hub as a training resource, facilitating training for sites including physios, clinical trial coordinators and clinical research associates**

LONGER TERM

- **Publish a sustainability model**
- **Expand the model to other rare neuromuscular diseases and other countries**

The Hub is a collaboration between leading neuromuscular experts and patient organisations.
The DMD Hub is funded by Duchenne UK.