Building Clinical Trial Capacity for Duchenne Muscular Dystrophy in the United Kingdom

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Patient organisations representing Duchenne muscular dystrophy (DMD) are concerned about lack of capacity for trials in DMD in the UK. Clinicians in larger centres are involved in multiple DMD studies and are reaching capacity, while smaller centres need support to develop their clinical trial capacity.

Table 1. Sites and Numbers of Participants for Ongoing DMD Studies in the UK

Site	Studies	Participants
Birmingham*	2	8
Cambridge	1	0
Glasgow	1	8
Leeds	1	8
Liverpool*	3	20
London*	9	95
Manchester*	2	4
Newcastle*	8	75
Oxford*	1	3

***Indicates Industry studies are taking place at this site.**

In July 2015 a workshop brought together patient organisations, clinical staff, representatives from the National Institute for Health Research (NIHR) and industry both to assess the current situation and to develop a strategy to improve capacity and better utilise resources.

The **Newcastle Plan** was devised to ensure that the UK builds clinical trial capacity for DMD and, by extrapolation, also other neuromuscular diseases.

The plan includes **three phases** of development:

- A **one year plan** aims to immediately boost capacity at existing UK centres of excellence.
- A **two year**, or medium term plan aims to build excellence and capacity at existing sites that have trial experience but need resource.
- A **five year** plan to ensure that all patients with DMD, including children and adults, have access to clinical research opportunities.

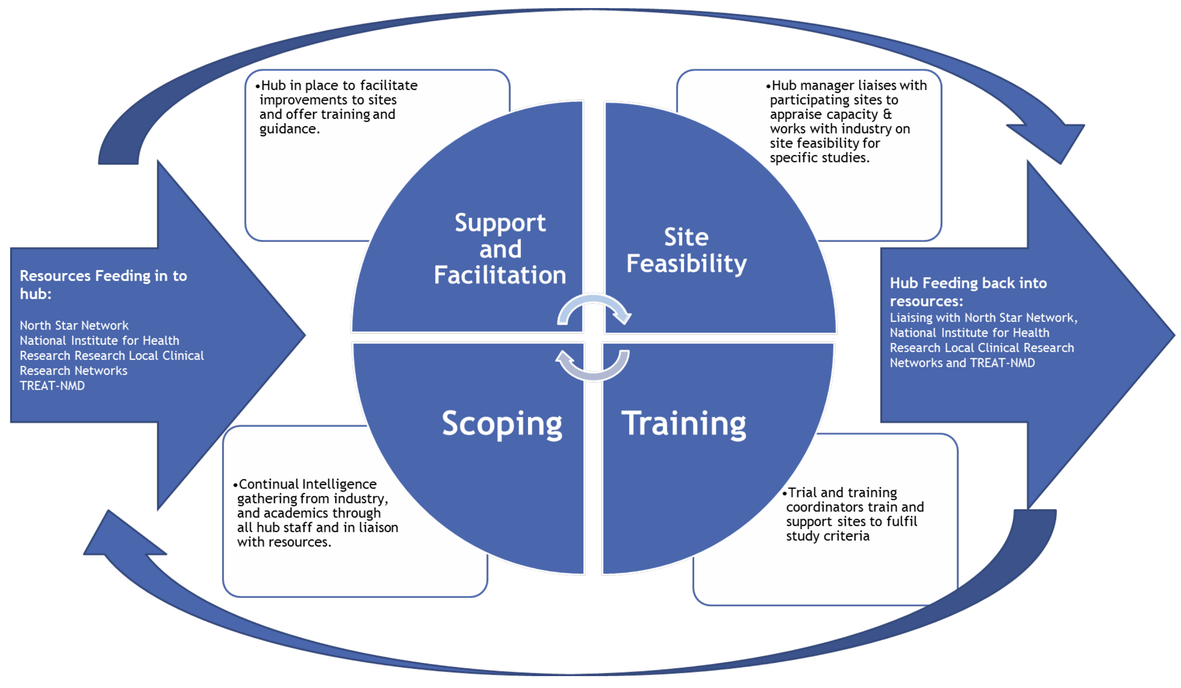
In order to fulfil the one year plan aims the patient organisations sent a questionnaire to sites undertaking the largest number of industry studies asking them the following types of questions about

- PATIENT POPULATION
- PERSONNEL AND EXPERIENCE
- CLINICAL TRIAL PIPELINE
- TRIAL CAPACITY
- BARRIERS TO CLINICAL TRIALS AT THEIR SITE
- THE IMPACT OF SUPPORT IF AWARDED
- OTHER SOURCES OF FUNDING SITES COULD ACCESS

After returning the questionnaire sites were invited to apply for funding for posts and site visits to those applying were conducted by the patient organisations. Funding decisions were made by the patient organisations based on applications and site visits and the results of the MDUK audit.

Development of a **clinical research hub** to aid in growing capacity for in clinical trials for DMD is also very much underway. The ultimate mission of the hub is to ensure that all patients with DMD, including children and adults, have access to clinical research opportunities. The hub would work towards the goals of the Newcastle plan by providing expert DMD advice to sites through the PI and working in conjunction with the facilities available including NHS trust and university staff, Clinical Research Facility (CRF) staff and Clinical Research Network colleagues.

DMD Clinical Research Hub



For more information about the Clinical Trial Capacity Workshop in July 2015, as well as an update on work done subsequently please go to: http://www.treat-nmd.eu/dmd/clinical-research/DMD-clinical-trial-capacity-workshop-2015/