DMD Hub Central Recruitment Pilot Project
Principal Investigator/Data Controller: Dr Michela Guglieri,
Translational and Clinical Research Institute, Newcastle University

Information for Participants

You are being invited to take part in The DMD Hub Central Recruitment Pilot Project. Before you accept or decline the invitation, it is important for you to understand why this project is being done and what it will involve. Please read the following information and discuss it with relatives, friends and your clinician, if you wish. If there is anything that is not clear, or if you have any further questions, please ask us (contact The DMD Hub Central Recruitment Pilot Project Co-ordinator, Phillip Cammish (email: phillip.cammish@ncl.ac.uk / Tel: 0191 241 8621).

Take time to decide whether you would like to take part, or not.

You are being invited to take part in this project because you have Duchenne Muscular Dystrophy (DMD).

What is the DMD Hub Central Recruitment Pilot Project?

The DMD Hub Central Recruitment Pilot Project is looking to create a national contact list of children and adults with Duchenne Muscular Dystrophy who are interested in participating in research studies. Over the past 10 years, there has been an increased number of natural history studies and clinical trials taking place to better understand Duchenne Muscular Dystrophy. Due to the complexity of the disease and of setting up sites to conduct research studies, they often only take place at a limited number of sites across the UK. This can create some challenges to clinicians, people diagnosed with Duchenne Muscular Dystrophy and their families.

The objective of the DMD Hub Central Recruitment Pilot Project is to identify if there is a benefit to people diagnosed with Duchenne Muscular Dystrophy and trial sites in maintaining a centrally coordinated national recruitment contact list of children and adults with Duchenne Muscular Dystrophy who are interested in participating in research studies in the UK.

How do you register to participate?

Registration via the study website will be voluntary and initiated by a person with Duchenne Muscular Dystrophy or their parent/guardian (if under 16 years of age). This online database will contain information that will support clinical sites to identify potentially eligible candidates for research studies (e.g. clinical trials and natural history studies).

Participants will be asked to consent to take part in this project, and also to provide consent to contact their main neuromuscular clinic to confirm some of the provided information if required.

It is anticipated that the pilot study will be active for 9-12 months, but if useful we will explore the possibility to continue or even expand it.

How will you benefit from registering?

Although one of the main objectives of this project is to facilitate recruitment in research studiesfor people living with Duchenne Muscular Dystrophy regardless of their geographical location, participation will not guarantee that somebody will be recruited (or approached to be recruited) in a study. Recruitment in a research studywill depend on national recruitment targets, eligibility criteria and will remain a responsibility of the Principal Investigator for the study at each clinical trial site.

If a site is looking for potential eligible participants for a specific research study,a Health Care Professional at the site will be able to contact the DMD Hub to identify potentially eligible people based upon the information provided to the DMD Hub Central Recruitment Pilot Project. Potential study participants will be identified by the DMD Hub based on the information provided as part of the pilot study. Information provided by participants will be passed on to the Health Care Professional at the trial site for them to contact the individual and their local neuromuscular specialist to discuss the specific trial and potential recruitment. All people identified as potentially eligible for a particular study will then be assessed against the study specific inclusion and exclusion criteria to confirm eligibility. As sites often have limits on the number of people that they can recruit to a research study, any people registered on the DMD Hub Recruitment Pilot Project who match the recruitment criteria will be selected at random, to ensure a fair process for all.

This pilot study is looking to assess whether a centrally coordinated national recruitment contact list for people living with Duchenne Muscular Dystrophy is an effective tool in supporting recruitment to research studies for Duchenne Muscular Dystrophy in the UK. A survey will be conducted around 6-9 months after the launch of the pilot study of registered participants, to gather feedback on the user experience.

You will not receive any payment or any other financial benefit as a result of joining the database. The results of any future research arising from The DMD Hub Central Recruitment Pilot Project may have business potential, but you will not receive any financial benefits from such developments by your participation in this study.

What information will you be asked to provide?

You will be asked questions about how Duchenne Muscular Dystrophy affects you and to provide some details of the clinic you attend to receive care for Duchenne Muscular Dystrophy. There are also questions on your genetic diagnosis, motor function, wheelchair use, medication taken and preferences for participation in research studies (including type of study and travel preferences). You can view all the questions on the study website before taking part. If a new question/questionnaire is added this will always be optional and additional information specific to that questionnaire will be provided.

I want to be involved in a clinical trial. If you register, is this guaranteed?

There is no guarantee that registering your details will mean you will be automatically approached to take part in a research study. Health Care Professionals looking to recruit people to a study will have the opportunity to review the details you have given and if the study appears to be suitable for you, they may contact you to discuss potential recruitment. All people contacted regarding a particular study will then be assessed in greater detail and at this stage it may be clear that other developments in your health or details not recorded on the DMD Hub Central Recruitment Pilot Project mean that the study is not a suitable one for you.

Will information about me be kept confidential?

All information we receive from you will be treated confidentially. The information that you enter into The DMD Hub Central Recruitment Pilot Project online platform about yourself will be stored on a secure server located **in the UK**. Only members of the DMD Hub Central Recruitment Project team will be given specific permission and will be allowed to look at this information. If we publish any research or other documents based on information from this project, this will not identify you by name.

A key aspect of the DMD Hub Central Recruitment Pilot Project is that we are able to share your information with clinical trial sites within the UK, in order to help with recruitment to DMD studies that you have notified us that you are interested in. In order to do this, we will be required to share your information (including your personal information and information about your condition) with these trial sites. To do this we will verify the trial sites requesting to utilise the DMD Hub Central Recruitment Pilot Project and then use a secure file drop off service between the University and a trial site. You will have the opportunity to give your permission for sharing your data in this way on the participant consent form.

If you would like more information about how we manage personal data more generally, including your rights under law, and the contact details of the University's Data Protection Officer, please see our website: http://www.ncl.ac.uk/data.protection/

Who is running the DMD Hub Central Recruitment Pilot Project?

The DMD Hub Central Recruitment Pilot is a project between the John Walton Muscular Dystrophy Research Centre (Newcastle University) and Duchenne UK. Both organisations are working together on the pilot phase of this study, with coordination being managed by the DMD Hub team at Newcastle University.

Post pilot study

If the DMD Hub Central Recruitment Pilot project is successful it may continue as an open-ended study. The person/organisation who manages your data on the DMD Hub Central Recruitment Pilot Project may also change if the study continues past its pilot phase. For the pilot phase, Dr Michela Guglieri and Newcastle University are responsible for managing the study and your data.

Once a decision has been made regarding what will happen after the pilot phase of this study, you will be notified.

Do you have to join the study and can you withdraw if I change my mind?

Joining the DMD Hub Central Recruitment Pilot Project is voluntary. Should you wish to withdraw your information from the study you will be free to do so at any time without having to provide any explanation. If you wish to withdraw, you should contact the staff in charge. Contact details are provided below. Joining or leaving the study will in no way affect the care you receive for your condition and will not preclude you from participating in clinical research and trials.

How will my details be updated?

You can amend your details at any time.
We will contact you six months after you have registered and completed the study questionnaire, to ask you to update your information so that is up to date and accurate. We will

do this using the email that you provide when first registering. If you need to update your details at any other point you can do so by contacting the study team.

Who is funding the study?

The project is funded by Duchenne UK and is part of the DMD Hub. Launched in 2016, the DMD Hub is a collaboration between the two neuromuscular centres of excellence (The John Walton Muscular Dystrophy Research Centre (Newcastle University) and Great Ormond Street Hospital (London) and Duchenne UK). The DMD Hub is a network of trial sites with trained staff which are funded to carry out clinical trials for Duchenne Muscular Dystrophy. It uses existing UK clinical trial expertise, to provide a central resource offering advice, guidance and training to sites less experienced in running Duchenne Muscular Dystrophy clinical trials. Ultimately, the mission of the DMD Hub is to ensure all people with Duchenne Muscular Dystrophy, both children and adults, have access to clinical research trials.

Who has reviewed this project?

This study has been reviewed and approved by the relevant ethics committee at Newcastle University, to ensure we are not doing anything harmful to you or your data in this project.

What if you have any concerns?

If you have any concerns or other questions about this study or the way it has been carried out, you should contact the principal investigator:

Dr Michela Guglieri
Tel: +44 (0) 191 241 8606
Email: Michela.Guglieri@newcastle.ac.uk

Thank you for taking the time to read this information sheet

Informed Consent (Initials to be given against each item)

- ☐ I confirm that I have read and understand the information sheet for the study. I have had the opportunity to consider the information, ask questions and have had these answered satisfactorily.
- ☐ I understand that my participation is voluntary and that I am free to withdraw at any time, without giving any reason, without my medical care or legal rights being affected.
- ☐ I give consent for the storage of data on myself in the DMD Hub Central Recruitment Pilot Project.
- ☐ I give consent for the sharing of my personal information with DMD Hub clinical trial sites regarding recruitment to Duchenne Muscular Dystrophy research studies.
- ☐ I understand that the storing and sharing of data will allow contact to be made with me if a suitable research study becomes available.
- ☐ I understand that allowing my data to be stored in this database does not mean that I will automatically be recruited (or approached to be recruited) into a research study.

- ☐ I understand that participation in this pilot study may not have any direct benefit for myself or my family.
- ☐ I confirm I am happy for the clinicians in charge of my medical care to be contacted by the DMD Hub Central Recruitment Pilot Project and clinical trial sites in order to obtain additional information about my condition/add relevant information to my database entry on my behalf.
- ☐ I am happy to receive regular information and updates about Duchenne Muscular Dystrophy via the DMD Hub Central Recruitment Pilot Project
- ☐ I am happy to consent to be included in the DMD Hub Central Recruitment Pilot Project

This study was approved by the Faculty of Medical Sciences Research Ethics Committee, part of Newcastle University's Research Ethics Committee. This committee contains members who are internal to the Faculty. This study was reviewed by members of the committee, who must provide impartial advice and avoid significant conflicts of interests.