



Duchenne
UK



**DMD Hub Central Recruitment Pilot Project
Parental/Guardian Information and Informed Consent
Principal Investigator/Data Controller: Dr Michela Guglieri,
Translational and Clinical Research Institute, Newcastle University**

Information for Participants

To make an informed decision about your child's participation in the DMD Hub Central Recruitment Pilot Project, it is important that you understand what is involved and what will be done with the information that you provide. This form contains the answers to the questions that you might have. At the end of the form there are text boxes for you to initial to confirm that you agree to your child participating. If you have any questions after reading this form, please contact The DMD Hub Central Recruitment Pilot Project Co-ordinator, (email: dmdhub@ncl.ac.uk / Tel: 0191 241 8621).

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 (DMD) 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, parents/guardians 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 the parent/guardian. 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).

If you agree to your child taking part in this project, you should read this information and place your initials in each of the text boxes on the consent section. This confirms that you agree to your child participating. Then you should complete the online questionnaires, in which we ask you for some of your child's personal data and some information about their condition. The information that you provide will be entered into The DMD Hub Central Recruitment Pilot Project, which is governed by the study Steering Committee. Your child's data will be stored securely and no unauthorised persons will be able to gain access to any information about your child.

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 my child benefit from registering?

Although one of the main objectives of this project is to facilitate recruitment in research studies for people living with Duchenne Muscular Dystrophy, regardless of their geographical location, participation will not guarantee that they will be recruited (or approached to be recruited) in a study. Recruitment in a research study will depend on national recruitment target, 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 you about your child will be passed on to the Health Care Professional at the trial site for them to contact you and your child's 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 and parents/guardians, to gather feedback on the user experience.

You or your child 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 your child and how Duchenne Muscular Dystrophy affects them. You will also be asked to provide some details of the clinic your child attends 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 my child to be involved in a clinical trial. If you register, is this guaranteed?

There is no guarantee that registering your child's details will mean that 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 about your child and if the study appears to be suitable for them, they may contact you to discuss

potential recruitment. If you are contacted regarding a particular study, your child will require to be assessed in greater detail. At this stage it may be clear that other developments in your child's health or details not recorded on the DMD Hub Central Recruitment Pilot Project, mean that the study is not a suitable one for them.

Will information about my child be kept confidential?

All information we receive from you will be treated confidentially. The information that you enter in to The DMD Hub Central Recruitment Pilot Project online platform about yourself and your child 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 or your child by name.

A key aspect of the DMD Hub Central Recruitment Pilot Project is that we are able to share information about your child 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 information (including personal information about you and your child and information about their 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 data about yourself and your child 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.

Does my child have to join the study and can you withdraw them if you change your mind?

Joining the DMD Hub Central Recruitment Pilot Project is voluntary. Should you wish to withdraw information about your child and yourself 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 DMD Hub Central Recruitment Pilot Project Co-ordinator, (email: dmdhub@ncl.ac.uk / Tel: 0191 241 8621). Joining or leaving the study will in no way affect the care your child receives for their condition and will not preclude them from participating in clinical research and trials.

How will my details be updated?

You can amend details about your child or yourself at any time.

We will contact you six months after you have registered and completed the study questionnaire, to ask you to update information about your child and yourself 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.

What happens after my child's 16th birthday?

In the UK, once a child becomes sixteen, they are able to choose for themselves whether or not they would like to participate in studies such as The DMD Hub Central Recruitment Pilot Project. It will no longer be their parents' decision. Following your child's sixteenth birthday, the study team will contact you and your child to ask if they would like to continue participating in the study.

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 DMD 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 on behalf of my child for the study. I have had the opportunity to consider the information, ask questions and have had these answered satisfactorily.
- I understand that my child's participation is voluntary and that I am free to withdraw at any time, without giving any reason, without his/her medical care or legal rights being affected.
- I give consent for the storage of data about myself and my child in the DMD Hub Central Recruitment Pilot Project.
- I give consent for the sharing of personal information about myself and my child with DMD Hub clinical trial sites regarding recruitment to Duchenne Muscular Dystrophy research studies.
- I understand that the storing and sharing of data about myself and my child will allow contact to be made with me if a suitable research study becomes available for my child.
- I understand that allowing data about myself and my child to be stored in this database does not mean that my child 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 child.
- I confirm I am happy for the clinicians in charge of my child's medical care to be contacted by the DMD Hub Central Recruitment Pilot Project and clinical trial sites, in order to obtain additional information about their condition/to add relevant information to my database entry on behalf of myself and my child.
- 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 on behalf of my child 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.