DMD Hub Central Recruitment Database
Parental/Guardian Participant Information Sheet
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Information for Participants

To make an informed decision about your child's participation in the DMD Hub Central Recruitment Database (CRD), 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 Database Project Manager, Phillip Cammish (email: phillip.cammish@ncl.ac.uk / Tel: 0191 241 8621.

What is the DMD Hub CRD and why has it been set up?

The DMD Hub CRD is a national resource for children and adults with DMD who are interested in participating in clinical research studies.

Over the past 15 years, there has been an increased number of natural history studies and clinical trials taking place to better understand DMD. Due to the complexity of the disease and of setting up of sites to conduct research studies, they often only take place at a limited number of centres across the UK. This can create some challenges to people diagnosed with DMD and their families who want to participate in clinical research and to the clinicians who want to recruit participants in these research studies.

People with DMD have previously reported that they often do not know how to access research opportunities. They feel that they do not have the same chances to be involved in research studies depending on where they live and if they do not receive their clinical care at one of the trial sites. It can also be difficult to understand the criteria used to select participants for research studies at different trial sites.

Clinicians on the other hand might receive several contacts from families who are interested in research studies, but without detailed clinical information, are unable to evaluate their eligibility. Collecting information can be difficult and time consuming especially without adequate support. Research studies have strict inclusion and exclusion criteria and an evaluation of tolerability and compliance which often require a medical evaluation.

The DMD Hub CRD has been created to collect information about people with DMD, who are interested in participating in clinical research studies. The objective of the DMD Hub CRD is to facilitate fairer and more equitable access to clinical research for people living with DMD, regardless of their geographical location, by supporting trial sites in identifying potentially eligible participants to take part in DMD clinical research studies in the UK.

If a healthcare professional at a trial site in the UK is looking to recruit patients for a specific study, they can ask the DMD Hub for assistance in identifying people who may be eligible to take part. The DMD Hub will review the information provided by those who have registered on the CRD, and if they match the criteria for the study, their information may be passed on to the recruiting site. As trial sites are only able to recruit a limited number of people for each study, participants who match the eligibility criteria will be selected at random to ensure a fair process.

The CRD is part of the DMD Hub, a network of clinical trial sites that deliver clinical research in DMD. The DMD Hub clinical trial sites support the CRD and have committed to consider enrolment of Participants through the CRD for all clinical research studies.

The DMD Hub CRD will also be able to provide information about how many people living with DMD are potentially eligible and willing to participate in specific clinical trials in the UK, to companies, clinicians or academics who are interested in bringing clinical trials and other clinical research studies in the UK. This will facilitate the set up and delivery of clinical research studies in the UK.

Through the CRD we are also aiming to answer some questions around clinical trial feasibility and patient preferences which can inform future clinical trials and research in the field. Finally, the CRD will be used to inform participants about other research projects which might be of interest for patients with DMD.

How do you register to participate?

Registration via the study website (www.dmdhubrecruits.org) is 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.

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 Database, 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.

How will my child benefit from registering?

Although one of the main objectives of this project is to facilitate fairer and more equitable access to clinical research studies for people living with DMD, regardless of their geographical location, Registration on the CRD will increase the chances but not guarantee that somebody will be recruited (or approached to be recruited) in a study. Recruitment to a clinical research study will depend on national recruitment targets, eligibility criteria and will remain a responsibility of the Principal Investigator for the study at each clinical trial site. As part of the wider remit of the DMD Hub, the objective is to implement a transparent and equitable national recruitment process across DMD Hub sites to ensure that each site aims to screen at least 1 patient using the DMD Hub CRD for every 2 patients screened from their local clinic list for every clinical research study.

Through the CRD you will also be informed about other research projects relevant for DMD which you and your child might be interested in.

How does the DMD Hub CRD work?

If a site is looking for participants to recruit to a specific research study, a Health Care Professional at the site can send an enquiry to the DMD Hub CRD project team. The DMD Hub CRD project team will identify a specified number of potentially eligible people based upon the information provided. Anyone registered on the DMD Hub CRD who matches the recruitment inclusion criteria for this particular research study, will then go through a randomisation process (using a computer program).

The DMD Hub project team will pass the contact details of the identified potential participant(s) to the Health Care Professional at the trial site for them to contact the individual's local neuromuscular specialist and the individual themselves to assess eligibility against the study specific inclusion and exclusion criteria, discuss the specific trial and potential recruitment.

The DMD Hub CRD will also collaborate with the North Star Network to ensure newly diagnosed patients are informed about the DMD Hub CRD. We will also inform participants in the DMD Hub CRD of other ad-hoc research opportunities (approved by the steering committee), that may fall outside of the specific scope of the CRD as described above.

A clinical trial site, as well as a pharmaceutical company, a clinician or academic interested in setting up a clinical trial or research study in the UK, can contact the CRD to ask about the number of people living with DMD and meeting certain criteria registered on the database. This is to facilitate clinical trials set up and enrolment.

You or your child will not receive any payment or any other financial benefit as a result of joining the CRD. The results of research arising from the database may have potential to generate income from third party organisations who are working/interested in the DMD field., but you will not receive any financial benefits from such developments by your participation in this study.

What information will I be asked to provide?

You will be asked questions about your child and how Duchenne Muscular Dystrophy affects them and to provide some details of the clinic you attend to receive care for DMD. There are also questions on their 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 CRD 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 research study. If I register, is this guaranteed?

There is no guarantee that registering your child's details will mean you will be automatically approached to take part in a clinical 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. 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 child's health or details not recorded on the DMD Hub CRD mean that the study is not a suitable one for them.

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 CRD online platform about yourself and your child will be stored on a secure server located **in the UK**. Only members of the DMD Hub CRD 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.

All data will be subject to the regulations on data protection (national laws related to General Data Protection Regulation (GDPR UK) and all information received will be treated confidentially. We will retain data about you and your child for no longer than is necessary for the purpose of the project at Newcastle University OR for no longer than six years after the project has ended, in line with the University's Retention Schedule. All information will be encrypted and stored on a secure server located in the UK.

A key aspect of the DMD Hub CRD is that we are able to share participant information (including personal information about you and your child and information about a your child's condition) with health care professionals and other qualified personnel at clinical trial sites within the UK, and that they are able to feedback participant information to the CRD, in order to help with recruitment to DMD studies. To do this we will verify the trial sites requesting to utilise the DMD Hub CRD and then use a secure encrypted file sharing service with trial sites to share information. You will have the opportunity to give your permission for sharing data in this way on the participant consent form.

De-identied data may be shared with third parties (e.g. Pharmaceutical companies, academic institutions, charities) who have been approved by the DMD Hub CRD Steering Committee, but no identifiable data about you or your child will be shared with any organizations outside the one listed above. Duchenne UK, as the patient organisation supporting the CRD, may be granted access to participant contact information (name and contact information) for community outreach and communications purposes. You will be asked whether you are willing to share your contact details with Duchenne UK. This will not impact your enrolment in the CRD.

Data about you and your child will not be made available to employers, government organisations, insurance companies or educational institutions, nor to other members of your family.

Where can you find out more about how my/my childs information is used?

For more information about how we use your/your child's information consult the main study Patient Information Sheet, or the URL:

www.hra.nhs.uk/information-about-patients/

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

Who is running the DMD Hub CRD?

The CRD is partnership between the John Walton Muscular Dystrophy Research Centre (at Newcastle University) and Duchenne UK and is being coordinated by the DMD Hub team based at Newcastle University.

Participation in the DMD Hub CRD?

The DMD Hub CRD is designed to be a sustainable resource for the DMD community and therefore there is not a defined end date.

Once participants express their interest in being informed about clinical trials, they are potentially eligible for, they will remain in the database however participants are able to remove their data from the CRD at any time.

If the DMD Hub CRD will be discontinued for whatever reason, participants will be notified.

If a participant does to not update/verify their data in the CRD for 2 years, their data will not be used for any activity (including referrals to trial sites for potential inclusion in clinical trials).

Participants will be contacted directly by the CRD:

- to obtain their genetic report.
- every 6 months with a request to confirm data on the database is up to date or to update if necessary.
- at specific occasions when additional information is required to assess eligibility for a clinical trial, not already included in the dataset.

Otherwise, the CRD will not contact participants on a regular basis. If a participant is selected for a specific clinical research study, the clinical staff at the clinical trial site, rather than the CRD staff, will contact the participant to invite them for screening.

Potential Risks of Taking Part

There are no direct risks involved in participating in the DMD Hub CRD. However, participants should be aware that there is no guarantee that registering on the DMD Hub CRD will mean they will be approached to take part in any clinical research study. Participants are selected according to eligibility criteria set by the company running the study, and randomly selected from a pool of eligible patients.

Registration is always voluntary. Participants have the right to withdraw their consent to participate at any time, and any information provided will be deleted. To do so, contact: dmdhub@newcastle.ac.uk

Does my child have to join the DMD Hub CRD and can I withdraw them if I change my mind?

Joining the DMD Hub Central Recruitment Database 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 staff in charge. Contact details are provided below. 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.

To withdraw from the DMD Hub CRD please email: dmdhub@ncl.ac.uk

How will my child's details be updated?

You can amend your details at any time.

We will contact you every six months after you have registered and completed the CRD 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.

Who is funding the study?

The CRD is funded by Duchenne UK and is part of the DMD Hub. Launched in 2016, the DMD Hub is a network of clinical trial sites across the UK, with trained staff who are funded to carry out clinical research for DMD. The DMD Hub provides a central resource offering advice, guidance and training to sites to run DMD studies. Ultimately, the mission of the DMD Hub is to ensure all people living with DMD, both children and adults, have access to research.

Who has reviewed this project?

The Faculty of Medical Sciences at Newcastle University have reviewed and agreed to sponsor this project.

This project has also been reviewed and approved Derby Research Ethics Committee.

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:

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

Thank you for taking the time to read this information sheet