



BMD Hub Central Recruitment Database
Principal Investigator/Data Controller: Prof Michela Guglieri,
Translational and Clinical Research Institute, Newcastle University

Information for Participants

You are being invited to take part in The BMD Hub Central Recruitment Database. 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 BMD Hub Central Recruitment Database Co-ordinator - email: bmdhub@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 the BMD Hub Central Recruitment Database because you have Becker Muscular Dystrophy (BMD).

What is the BMD Hub Central Recruitment Database?

The BMD Hub Central Recruitment Database is a national contact list of patients with BMD who are interested in participating in research studies. Over the past few years, there has been an increased interest in BMD with upcoming natural history and interventional clinical trials. However, access to these research studies is often limited to patients followed up in expert neuromuscular centres, more likely to be selected to conduct research studies, due to the complexity of the disease and the infrastructure required to set up clinical trials. This creates inequality of access and inevitably some challenges to people with BMD and their families who might feel excluded from participation in clinical trials unless they are regularly seen at one of the clinical trial sites. Also, as Becker muscular dystrophy is a rare disease, clinicians might struggle to identify patients to participate in clinical trials.

The objective of the BMD Hub Central Recruitment Database is to understand if maintaining a centrally coordinated national recruitment contact list of patients with BMD who are interested in participating in research studies in the UK facilitate a fairer access to clinical trials and therefore it is beneficial to patients with BMD and trial sites.

How do you register to participate?

Registration via the study website will be voluntary and initiated by a person with Becker 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 the BMD Hub Central Recruitment Database and to contact their main neuromuscular clinic to confirm some of the provided information if required. Participants will also be asked to provide consent to allow the clinical

trial site to feedback to the BMD Hub CRD regarding the outcome of any referral made on their behalf.

It is anticipated that the BMD Hub Central Recruitment Database will be active for 24 months initially, but if useful we will explore the possibility to continue maintaining the BMD Central recruitment database long term and/or integrate it into existing initiatives such as the DMD Hub.

How will you benefit from registering?

Although one of the main objectives of the BMD Hub Central Recruitment Database is to facilitate recruitment in research studies for patients with BMD, regardless of their geographical location, participation will not guarantee that somebody will be recruited (or approached to be recruited) in a clinical trial or natural history study. Recruitment in a research study will depend on national and local recruitment targets, eligibility criteria and will remain a responsibility of the Principal Investigator for the study at each clinical trial site.

How does the BMD Hub Central Recruitment database work?

If a site is looking for participants to recruit to a specific research study, a Health Care Professional at the site will be able to send an enquiry the BMD Hub Central Recruitment Database to identify a specified number of potentially eligible people based upon the information provided to the BMD Hub Central Recruitment Database. Anyone registered on the BMD Hub Central Recruitment Database who matches the recruitment inclusion criteria for this particular research study, will then be selected at random (using a computer program) and the required number of participants passed by the BMD Hub Central Recruitment Database coordinator for referral to the recruiting trial site. The coordinator will pass the contact detailed of the identified potential participant to the Health Care Professional at the trial site for them to contact the individual and/or their local neuromuscular specialist to assess eligibility against the study specific inclusion and exclusion criteria, discuss the specific trial and potential recruitment.

This study is looking to assess whether a centrally coordinated national recruitment contact list for patients with BMD is an effective tool in supporting recruitment to research studies for Becker Muscular Dystrophy in the UK. A survey will be conducted around 12 months after the launch of the BMD Hub Central Recruitment Database 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 data generated from your participation in the BMD Hub Central Recruitment Database may have commercial 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 Becker Muscular Dystrophy affects you and to provide some details of the clinic you attend to receive care for Becker 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 BMD Hub Central Recruitment Database 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 BMD Hub Central Recruitment Database online platform about yourself will be stored on a secure server located **in the UK**. Only members of the BMD Hub Central Recruitment Database 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 the BMD Hub Central Recruitment Database this will not identify you by name.

A key aspect of the BMD Hub Central Recruitment Database is that we are able to share your information with clinical trial sites within the UK, in order to help with recruitment to BMD 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 BMD Hub Central Recruitment Database and then use a secure encrypted file sharing service with trial sites. You will have the opportunity to give your permission for sharing your data in this way on the participant consent form.

Anonymized data maybe shared with organisations such as patient organisations and industry partners who have been approved by the BMD Hub Central Recruitment Database Steering Committee but no identifiable data will be shared with any organizations outside the one listed above.

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 BMD Hub Central Recruitment Database?

The BMD Hub Central Recruitment Database is coordinated by the John Walton Muscular Dystrophy Research Centre (Newcastle University), funded by Edgewise Therapeutics, and collaborating with UK patient organisations focused on BMD.

Do you have to join the BMD Hub Central Recruitment Database, and can you withdraw if I change my mind?

Joining the BMD Hub Central Recruitment Database 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 also contact you every 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.

Who is funding the BMD Hub Central Recruitment Database?

The BMD Hub Central Recruitment Database is funded by a grant from Edgewise Therapeutics to Newcastle University. Edgewise Therapeutics is not involved in the design or conduct and have no rights to the data collected as part of the project or outputs of the project.

Who has reviewed this project?

This project 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:

Prof 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 BMD Hub Central Recruitment Database.
- I give consent for the sharing of my personal information with BMD Hub clinical trial sites regarding recruitment to Becker 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 BMD Hub Central Recruitment Database 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 Becker Muscular Dystrophy via the BMD Hub Central Recruitment Database.
- I am happy to consent to be included in the BMD Hub Central Recruitment Database.
- I am happy for the clinicians at site to update the BMD Hub Coordinator on the outcome of screening I was selected for via the CRD.
- I am aware that anonymised aggregated data from the CRD may be provided to DMD Hub and BMD Hub sites as well as companies interested in running trials in the UK.

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.

