

Brain INVOLVEMENT in Dystrophinopathies (BIND): Deep Functional Phenotyping of Duchenne Muscular Dystrophy and Becker Muscular Dystrophy Patients (WP5) Part 1: a Multicentre Online Phenotyping and Neurobehavioural Data Collection Study

Hub Summary

This study is looking at the connection between the behavioural aspects of DMD and a patient's DMD gene mutation. Participants will be asked to complete an online questionnaire, which will take approximately 70mins and can be completed at multiple sittings. This is open to male DMD patients between 5 and 17yrs old.

Study Number: NCT04583917

Description by University College London

The objective of this study is to collect data from a large cohort of individuals with DMD and BMD focusing on the neurobehavioural aspects of these conditions and their correlation to the location of the DMD gene mutation.

Depending on the timeline of Covid-19 restrictions being lifted, the next step of this study is to invite a subgroup of these participants to attend a clinic appointment where further cognitive assessments will be performed.

The study is funded by the European Commission and will involve 6 countries (Denmark, The Netherlands, France, Spain, Italy and the UK).

Recruitment in the UK will take place in two sites; UCL Great Ormond Street Institute of Child Health and Newcastle University. The UCL site will be recruiting only children with DMD and BMD, while the Newcastle site will be recruiting both children with DMD and BMD, as well as adults with BMD. Participants will have a choice regarding which site is most suitable for them.

Please contact:

[Andria Papageorgiou](#) or [Isabella Vainieri](#) for GOSH

[Monika Malinova](#) for Newcastle University

Primary Outcome Measures

The primary outcome measure is the DAWBA questionnaire, which will be completed online and will approximately take 70 minutes that can be completed in multiple sittings. When analysed the DAWBA provides a probability of full range of diagnosis of psychological problems as defined by DSM-5.

Secondary Outcome Measures

The secondary outcome measures include;

- PARS-III (by proxy-report) or PARS-A (self-report)
- Kempenhaeghe Learning Questionnaire
- Kempenhaeghe History taking Questionnaire

Trial Status

Recruiting

UK Locations
London - GOSH,
Recruiting, Newcastle,
Not yet recruiting

Trial Sponsor
University College
London

Age
5-17

Mutation Specific
Non-mutation specific
therapies

Muscle Biopsy
No Muscle Biopsy
Required

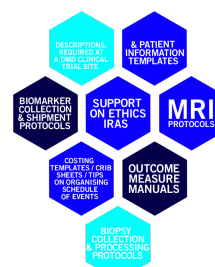
MRI
No

Phase
Observational

Length Of Participation
Until the online
questionnaires are
completed or they
withdraw from the
study.

Recruitment Target
800

Ambulatory
Ambulant and non-
ambulant



dmdhub.org

Can I take part?

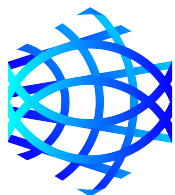
Inclusion Criteria

- Male
- Age 5-17 years
- Genetically-proven diagnosis of DMD
- Genetic mutation that affects the initial part of the gene; the middle part of the gene; or the end of the gene.

Exclusion Criteria

- Lack of a molecular diagnosis of DMD or BMD
- Mutation falls outside the regions of interest
- A severe co-morbidity or planned surgical intervention within 6 months from the study which could interfere with the well-being of the participant

For contact details and to find out more, please refer to dmdhub.org.



Duchenne
UK