MCID DMD

Minimal Clinically Important Difference (MCID) in Duchenne Muscular Dystrophy (DMD) tests

Hub Summary

This study is looking at whether we can quantify the smallest change in two outcome measures that is meaningful for families, patients and clinicians. Participation in this study includes just a one-off questionnaire which takes around 30-45 minutes to complete.

Study Number:

Description by Dubowitz Neuromuscular Centre, UCL Great Ormond Street Institute of Child Health

There have been an increasing number of studies in DMD. Although differences between the tests performed during the studies may be found, these differences may not be important for patients and their families. The Minimal Clinical Important Difference (MCID) is an idea developed to measure the smallest change that is considered important by patients, parents and clinicians. As more treatments are developed, there is a need to determine the MCID for other tests used in DMD studies. The North Star Ambulatory Assessment (NSAA) is a physiotherapy test for ambulant boys that involves exercises such as hopping, standing on one leg, getting up from the floor and running. The Performance of Upper Limb (PUL) is a test that measures upper limb function. Although NSAA and PUL are used in DMD studies, the patients' and their families' views on MCID have not been included.

This study aims to determine the MCID for these two tests using a questionnaire given to patients, families and clinicians. There will be some questions on Quality of Life questionnaires. The findings will help to understand the results of DMD studies and help to create studies that focus on patients' real needs and their point of view.

Primary Outcome Measures

This study is designed solely as a questionnaire for patients, parents and clinicians.

Can I take part?

Inclusion Criteria

For patients the inclusion criteria are:

- ✔ Confirmed diagnosis of Duchenne muscular dystrophy (DMD) either through genetic testing or muscle biopsy with less than 10% of revertant fibres.
- ✔ Male gender
- ✔ Older than 7 years for NSAA questionnaire and older than 10 years for PUL
- ✔ NSAA score available (for NSAA questionnaire)
- ✔ PUL version 1.2 or 2.0 score available (for PUL questionnaire)
- ✔ Willing and able to provide written informed assent/consent.

For parents the inclusion criteria are:

- ✔ Have a child that fulfils the inclusion criteria for patients participating in the study
- ✔ Willing and able to provide written informed consent.

Exclusion Criteria

- ✖ Diagnosis of Becker muscular dystrophy
- ✖ Participating in natural history studies and clinical trials involving investigational products is not an exclusion criteria
- ✖ Any other clinical condition that in the opinion of the investigators could interfere with participation in the current study

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

Trial Status
Trial complete

UK Locations
London - GOSH, Trial complete/terminated

Trial Sponsor
Dubowitz Neuromuscular Centre, UCL Great Ormond Street Institute of Child Health

Ambulatory
Ambulant and non-ambulant

Therapeutic Category
Non-therapeutic

Age
7 to 18 years

Mutation Specific
Non-mutation specific therapies

Muscle Biopsy
No Muscle Biopsy Required

MRI
No

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Duchenne UK

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