TODDLER Study

Transforming Outcomes in Duchenne muscular dystrophy using DigitaL Endpoints Remotely

Hub Summary

This is an observational study to assess the feasibility of using the Syde® device to remotely assess motor function in children <4 years old. 30 boys with DMD (and their parent/caregiver) and 30 boys without DMD aged 1-3 years old will be enrolled in the study. Participants will receive a watch-like device to wear on their ankles (the Syde device®) for three blocks of 28-days over six months during their normal daily activities. We will find out 1) if young boys are happy to wear the device, 2) how it compares to other tests, and 3) if it can detect changes in walking ability.

Study Number: PID: 19385

Description by University of Oxford

Our aim is to find the best way to measure movement in young boys with DMD. This is important because without good measures we cannot test DMD medicines in younger boys in clinical trials. Giving medicines earlier to younger boys, before muscle damage has happened, could help improve their outcomes and their lives. There are no drugs being tested in this study, but the findings of the study could help us to test drugs for DMD in the future.

Unfortunately, we can't simply use movement tests designed for older boys because toddlers can struggle with listening, understanding, following instructions or doing their best in a test. We now have a little watch-like wearable device (Syde® device) which boys can wear on their ankles. It records how they move as they go about their normal daily lives. This device is now used in clinical trials in children over four. In this study, our team is aiming to test whether the device can be used in children under four and how it compares to other movement tests.

The Toddler study is being run by the STRONG team at the University of Oxford. This is a remote study which means that there are no hospital visits. All appointments over ~7 months will take place by phone, online or in your home. Taking part is completely voluntary.
If your son were to take part in the study, he would be given a watch-like device to wear on his ankles as he goes about his normal daily life.

Our study physiotherapist would also travel to your home twice to test your son's movements and development at the beginning and end of the study. With your help, they would also test your son's movements during two online visits.

We would also ask you to share information about your son's medical history and provide hospital appointment letters or results, and to complete questionnaires about your son's abilities and your experience of the wearable device.

To find out more about this study and to register your interest in taking part, please contact the study team: toddlerstudy@paediatrics.ox.ac.uk

Primary Outcome Measures

To assess the feasibility of using the Syde device to remotely assess motor function in boys with DMD under the age of four, including within-patient variability in SV95C, test-retest reliability, compliance and acceptability.

Secondary Outcome Measures

To investigate whether SV95C distinguishes boys with DMD from controls.
To assess correlation between SV95C and other motor assessment and parent-reported outcomes.
To assess impact of cognitive, behavioural, and language impairment on SV95C and other motor assessments.
To determine the sensitivity of SV95C to change.
To assess the validity of remote online functional motor assessment using NSAA.

Can I take part?

Inclusion Criteria

- Male
- Aged 1-3 years old
- Ambulant (able to walk 10m independently)
- Genetically confirmed diagnosis of DMD
- Parent(s)/legal guardian(s) able and willing to provide written informed consent for the child to participate in the study
- Parent(s)/legal guardian(s) able and willing to participate in the stud

Trial Status
Recruiting

UK Locations
Oxford, Recruiting

Trial Sponsor
University of Oxford

Phase
N/A

Length Of Participation
Approximately 7 months

Recruitment Target
30 (DMD), 30 (controls)

Ambulatory
Ambulant

Therapeutic Category
Observational

Age
1-3 years

Mutation Specific
All treatment types

Muscle Biopsy
No Muscle Biopsy Required

MRI
No

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Exclusion Criteria

Participant with DMD:
- Limb surgery/trauma (within 6 months)
- Significant comorbid chronic or acute conditions affecting motor function (within 3 weeks)
- Prematurity (born <37 weeks' gestation)
- Oral corticosteroids to treat DMD (before enrolment)
- Enrolment in therapeutic clinical trials
- Any comorbidity which could limit their ability to complete the study assessments (according to the investigator's clinical judgement)

Healthy control participant:
- Limb surgery/trauma (within 6 months)
- Significant comorbid chronic condition affecting motor function
- Significant acute condition affecting motor function (within 3weeks of enrolment)
- Prematurity (born <37 weeks' gestation)
- Neurodevelopmental concerns or delay in acquisition of WHO developmental milestones
- Any comorbidity which could limit their ability to complete the study assessments (according to the investigator's clinical judgement)

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

Duchenne UK

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