DMDhome

Decentralised study for validation of DMDhome, a platform for video capture and computer vision analysis of digital endpoints of upper/lower limb function relevant to the transfer phase in Duchenne muscular dystrophy (DMD).

Hub Summary

This decentralised study aims to evaluate whether new video-based electronic clinical outcome assessments (eCOAs) captured with the Atom5^{TM} platform DMDhome are able to detect disease progression from the late ambulatory to transfer stage and early non-ambulatory stage, compared with standard validated clinical assessments.

Study Number:

Description by Aparito

This decentralised study will be fully conducted at the patient's home. This study will enrol 28 ambulant and 16 non-ambulant DMD patients, and 15 age-/gender-matched healthy controls that can be siblings, friends or other family members. Participants must be 8 years of age and upwards.

Participants or/and their parents (if they are younger than 16) will self-onboard and provide electronic consent using the DMDhome web portal (htpps://dmdhome.atom5.co.uk). After the e-consent is signed, participants will be able to respond to screening questions and upload a proof of their DMD diagnosis, including genetic information if available. This proof could be a letter from their DMD specialist centre.

(PLEASE NOTE: The eligibility and e-consent process are meant to be accessed ideally from a computer, as it is designed as a web portal. The rest of the study procedures, ie. recording videos and answering PROs, can be done through the app).

For more information about this study, click **here** to listen to the webinar, and if you require any assistance during the sign-up process, you could book a 1:1 call with the study sponsors **here.**

Depending on your child's current health state you will also be asked to provide:

Ambulant patients:

- Most recent North Star Ambulatory Assessment (NSAA) score
- Most recent 10 Metre Walk Test (10MWRT) score
- Most recent Time to Stand from Supine (TSTANDS) score
- Most recent Performance of Upper Limb (PUL) 2.0 entry item score

Non-ambulant patients:

- Most recent Performance of Upper Limb (PUL) 2.0 entry item score

Following registration and screening, if a participant is deemed eligible, the study team will schedule a visit from a trained physiotherapist who will attend the participant's home to conduct the North Star Ambulatory Assessment, the Performance of the Upper Limb (PUL) 2.0 and the Egenklassification scale version 2.

The participant will also download the DMDhome study app and video record the following electronic clinical outcome assessments (eCOAs) using their smartphone:

Upper limb eCOAs (both ambulant and non-ambulant)

- Hands above head
- Hands to head
- Hands to mouth
- Hands to table from lap

Lower limb eCOAs (ambulant)

- Sit to stand
- Stand
- Stand to sit

Trial Status
Fully recruited

UK Locations
Oxford, Fully recruited

Trial Sponsor
Aparito

Length Of Participation
24 Months

Recruitment Target
44

Ambulatory
Ambulant and non-ambulant

Therapeutic Category
Non-interventional study

Age
8 and above

Mutation Specific
All treatment types

Muscle Biopsy
No Muscle Biopsy Required

MRI
No

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- Sit to stand and back to sit

Optional tasks that represent activities of daily living, e.g., drink from a glass, washing hands, feeding self, etc.

Participants will perform all assessments every 6 months and up to 24 months (5 time points).

Please note that this study is only open to people living in the UK.

Primary Outcome Measures

Change from baseline in DMDhome upper limb/lower limb eCOAs compared with physiotherapist assisted NSAA and PUL2.0 total scores at 6, 12, 18 and 24 months.

Secondary Outcome Measures

Evaluate test-retest reliability of new DMDhome eCOAs.

Evaluate the sensitivity to change of new DMDhome eCOAs over 24 months compared with PUL2.0 and NSAA.

Estimate the Minimal Clinically Important Difference of DMDhome scores vs NSAA and PUL 2.0

Correlate new eCOAs to patient-reported outcome measures such as the DMD Upper Limb PROM.

Evaluate the quality of life of patients/carers.

Other Outcome Measures

Identification of digital parameters during activities of daily living (ADLs) natural movement via video assessments and computer vision analysis. Participants will be able to choose tasks from four domains: food/nutrition, self-care, household/environment, leisure & communication.

Can I take part?

Inclusion Criteria

All DMD patients:

- ✔ Gender: male
- ✔ DMD diagnosis confirmed through genetic testing. Data on the exact genetic mutation will be collected as genetic modifiers may influence lower limb or upper limb function.
- ✔ Age 8 years and above.
- ✔ Patients currently treated with corticosteroids should be on stable oral corticosteroid treatment for the 6 months prior to screening (and anticipated constant dose throughout the study period, but which could be adjusted in line with weight gain following the standard of care)
- ✔ Patients not currently treated with corticosteroids, should plan start oral treatment within the next 3 months of study screening.
- ✔ Able to understand and to follow instructions to perform the tasks.
- ✔ Access to smartphone with iOS or Android system and Wi-Fi. If the participant or their family do not have access to a smartphone, one will be provided. Participant will need to return it after the end of the study. Access to Wi-Fi is required to be able to upload the videos.

Ambulant DMD patients, likely to have declining ambulatory function defined as:

- ✔ Time to stand from supine (TSTANDS) 7 sec, it will be collected at the screening visit.
- ✔ Time to walk/run 10 metres (10MWRT) 10 sec from last clinical visit, which cannot be older than 6 months.
- ✔ NSAA most recent score, not older than 6 months.

Non ambulant DMD patients

- ✔ PUL entry item score 2; most recent score, not older than 6 months
- ✔ Patient is not on a ventilator.

Healthy controls

- ✔ Healthy male participants aged 8 years and above.
- ✔ Gender-matched siblings, relatives, or friends of DMD patients can be included.
- ✔ Able to understand and perform the tasks.
- ✔ Access to smartphone with iOS or Android system and Wi-Fi.

Exclusion Criteria

- ✖ Other neuromuscular diseases
- ✖ Any condition, that could interfere with patient understanding and realisation of the tasks.

If you want more information about the study please contact dmdhome@aparito.com

If you want to join the study please visit: https://dmdhome.atom5.co.uk/

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

Duchenne UK

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