The best muscletest for Duchenne muscular dystrophy; Outcome measures for Duchenne muscular dystrophy trials

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1. Assessment of sensitivity of several outcome measures in muscle force and function in Duchenne muscular dystrophy2. Assessment of reproducability of different outcome measures3. Assessment of clinical relevance of measured change over time in...

Ethical review Approved WMO

Status Recruitment stopped **Health condition type** Muscle disorders

Study type Observational non invasive

Summary

ID

NL-OMON32991

Source

ToetsingOnline

Brief title

Outcome measures for Duchenne

Condition

· Muscle disorders

Synonym

Duchenne, muscular dystrophy

Research involving

Human

Sponsors and support

Primary sponsor: Leids Universitair Medisch Centrum

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Source(s) of monetary or material Support: Prinses Beatrix Fonds; ZonNW, Prosensa

Intervention

Keyword: Duchenne muscular dystrophy, outcome measures, strength, trials

Outcome measures

Primary outcome

- 1. Average change of testresults over time (6-12 months)
- 2. intraclass correlation coefficient
- 3. smallest detectable difference

of the following tests:

- Manual Muscle Testing
- Quantitative Muscle Testing
- Timed Function Tests
- Six Minute Walk Test
- North Star Ambulatory Assessment
- Lungfunction
- QoL schale (DISABKIDS)

Secondary outcome

minimal clinical important difference of QoL schale and North Star Ambulatory

Assessment

Study description

Background summary

Duchenne muscular dystrophy is an X-linked genetic disorder, caused by a mutation in the dystrophin gene. This protein has an important function in maintaining the stability of the muscle membrane. In Duchenne muscular dystrophy this dystrophin is absent, leading to contraction-induced muscle damage. Clinically, this leads to progressive muscle weakness, causing wheelchair dependance from approximately the age of ten and death due to respiratory failure or a cardiomyopathy around the age of 20 in untreated patients. At this moment there are many research developments into a possible treatment for this untreatable muscle disease. The use of reliable and sensitive outcome measures in future trials is of great importance. Unfortunately, there is no consensus about which outcome measure to use in Duchenne muscular dystrophy. The present study aims to investigate which tests are usable as outcome measures for development of therapies in Duchenne muscular dystrophy.

Study objective

- 1. Assessment of sensitivity of several outcome measures in muscle force and function in Duchenne muscular dystrophy
- 2. Assessment of reproducability of different outcome measures
- 3. Assessment of clinical relevance of measured change over time in different outcome measures.

Study design

Prospective, observational study. Inclusion of patients is achieved through the dystrophinopathy database in the LUMC and through the ALADIN (All Against Duchenne In the Netherlands) consortium. The expected duration of the study is 2 years.

Study burden and risks

Participants are being asked to visit the LUMC three times, with intervals of six months. During these visits several tests are being undertaken assessing muscle strength, muscle function and lungfunction: Manual Muscle Testing, Quantitative Muscle Testing, Six Minute Walk Test, Timed Function Tests, North Star Ambulatory Assessment and Lungfunction Tests (FVC, MIP, MEP). Before each testday, participants are also asked to complete a questionaire about quality of life and to indicate change in muscle strength, muscle function, walking and lungfunction since the previous testing day.

The riscs during participation consists of the change to vall during one of the tests. The consequences of a possible fall are being minimized by using a soft surface if possible and by escorting the participant during the Six Minute Walk

Test to support/catch him in case of a fall.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age

Adolescents (12-15 years) Adolescents (16-17 years) Children (2-11 years)

Inclusion criteria

Duchenne muscular dystrophy age over 5 years

Exclusion criteria

wheelchair dependance

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Study design

Design

Study type: Observational non invasive

Masking: Open (masking not used)

Control: Uncontrolled

Primary purpose: Other

Recruitment

NL

Recruitment status: Recruitment stopped

Start date (anticipated): 01-04-2010

Enrollment: 60

Type: Actual

Ethics review

Approved WMO

Date: 14-09-2009

Application type: First submission

Review commission: METC Leids Universitair Medisch Centrum (Leiden)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register ID

CCMO NL26469.058.09