# Exercise testing in ambulatory pediatric patients with Duchenne Muscular Dystrophy and Becker Muscular Dystrophy: a pilot study

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The purpose of this study is to examine the feasibility of both the CPET and the 6MWT as outcome measures for exercise capacity in children with DMD and BMD. This study will focus on ambulatory children with DMD and BMD. These patients frequently…

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| **Ethical review** | Approved WMO |
| **Status** | Recruitment stopped |
| **Health condition type** | Heart failures |
| **Study type** | Observational non invasive |

## Summary

### ID

NL-OMON35527

**Source**
ToetsingOnline

**Brief title**
Exercise testing in Muscular Dystrophy

### Condition

- Heart failures
- Musculoskeletal and connective tissue disorders congenital
- Muscle disorders

**Synonym**
muscle disease, Muscular dystrophy

**Research involving**
Human

## Sponsors and support

**Primary sponsor:** Universitair Medisch Centrum Utrecht
**Source(s) of monetary or material Support:** SIA RAAK

## Intervention

**Keyword:** children, exercise testing, Muscular Dystrophy, pilot

## Outcome measures

### Primary outcome

Feasibility:

Feasibility outcome parameters include measurement completion, acceptability and adverse events:

Measurement completion:

Measurement completion rate is defined as the number of participants able to complete the exercise test. A premature determined exercise test can be caused by insufficient motivation and/or understanding of the test protocol. In this study, measurement completion for the 6MWT is defined as a test performance according ATS guidelines (27). Measurement completion of the CPET is defined as a test according Rowland*s criteria on physiological responses (table 1) or a minimal test durance of six minutes. Rowland*s criteria are subdivided in subjective and objective criteria, where every child has to meet the first and at least one of the latter.

A minimal accepted measurement completion rate for both tests is set at 90%.

Acceptability:

The willingness to perform the test again in the future based on experienced burden will be assessed with a *Visual Analoge Scale*after each exercise test. A higher score expresses less willingness to perform the test again in the future. During the 6MWT the fall frequency, duration from fall to fall recovery and number and duration of resting periods will be measured.

Adverse events:

Adverse events following exercise testing will be monitored:

Physical complaints will both be evaluated with a *Visual Analoge Scale* before and after the exercise test and every morning and evening in the seven consecutive days following the exercise test. Two day days after each exercise test a semi-structured interview will be conducted by telephone with each participant and his parents. Clinical features of muscle damage will be checked and reported (appendix 1) (28,29).

(Research protocol page 15-16)

**Secondary outcome**

Not applicable

# Study description

**Background summary**

Duchenne Muscular Dystrophy (DMD) and Becker Muscular Dystrophy (BMD) are caused by deficiency or reduced expression of the muscular protein dystrophin which leads to progressive deterioration of muscle strength and functional abilities during childhood and cardiomyopathy. There is no curative treatment,

although the use of corticosteroids prolongs the period in which children can walk. Several new therapeutic strategies, such as exon-skipping therapy and physical training programmes, aim at attenuating the disease course and optimising physical functioning and exercise capacity. To evaluate the effect of these interventions outcome measures are needed that are feasible and have good clinimetric properties in this specific patient group. In current practice, the six minute walk test (6MWT) is used to evaluate exercise capacity and has been used as an outcome measure in clinical trials. However, the feasibility of the 6MWT in dystrophy patients can be questioned because of reports of high fall frequency during the test and cognitive deficits in boys with DMD that interfere with the test performance (1). The cardio pulmonary exercise test (CPET) is considered the gold-standard to assess exercise capacity and is used extensively in other pediatric chronic diseases (2). The CPET has not been recently investigated in children with Duchenne and Becker Dystrophy, partially due to fears for exercise induced muscle damage to skeletal muscles or the heart. Importantly, several studies in adolescents and adults with DMD and BMD have shown no adverse effects of exercise (3,4). In order to develop reliable and valid outcome measures reflecting exercise capacity that can be used to test efficacy of experimental treatment, more insight in the feasibility of the CPET and the 6MWT in patients with Duchenne Muscular Dystrophy and Becker Dystrophy is required.

(Research protocol Summary; page 7)

**Study objective**

The purpose of this study is to examine the feasibility of both the CPET and the 6MWT as outcome measures for exercise capacity in children with DMD and BMD. This study will focus on ambulatory children with DMD and BMD. These patients frequently experience walking difficulties and fatigue performing their activities of mobility and self-care and are involved in large pharmaceutical and therapeutic intervention studies focused on the improvement of physical functioning. In order to develop reliable and valid outcome measures reflecting exercise capacity that can be used to test efficacy of experimental treatment, more insight in the feasibility of the CPET and the 6MWT in patients with Duchenne Muscular Dystrophy and Becker Dystrophy is required.

Questions to be answered are the following:
Feasibility outcome parameters include measurement completion, acceptability and adverse events and are further explained in the main outcome section (25).
1. Is the 6MWT a feasible outcome measure for exercise capacity in ambulatory children with DMD and BMD?
2. Is the CPET a feasible measure for exercise capacity in ambulatory children with DMD and BMD?

(Research protocol page 11-12)

**Study design**

pilot study

**Study burden and risks**

One study on exercise testing in children have reported a relatively high fall frequency in the 6MWT (1). Therefore a *safety chaser* (a person that walks behind the child) will be used to prevent fall incidents. There is no report of serious adverse events during or after exercise testing in this population (4,22,36) and extended precautions are taken in this study (cardiac evaluation, monitoring physiological responses and perceived burden) to reduce possible risks to a minimum.
In the short run, the benefits of this study for these children and their parents are that they will receive a comprehensive report on their level of exercise capacity and their cardiovascular and muscular responses to exercise. This will contribute to an optimal management of daily life functioning by both the patients and their parents and their medical caregivers. In the long run, it will contribute to the development of feasible and valid outcome measures for disease progression and therapeutic interventions.

(Research protocol page 23)

# Contacts

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# Trial sites

### Listed location countries

Netherlands

## Eligibility criteria

**Age**
Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Children (2-11 years)
Elderly (65 years and older)

### Inclusion criteria

Genetically confirmed diagnosis of DMD or BMD
Ability to follow instructions regarding testing
Parental and child informed consent
6-20 years of age
Ability to walk >= 20 meter without assistive devices

### Exclusion criteria

Concomitant medical problems that might intervene with the outcomes of the testing
Previous episodes of rhabdomyolysis
Reduced cardiac function that does not allow cardio pulmonary maximal exercise testing (according to pediatric cardiologist)

## Study design

### Design

**Study type:** Observational non invasive

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|---|---|
| Masking: | Open (masking not used) |
| Control: | Uncontrolled |
| Primary purpose: | Diagnostic |

### Recruitment

NL

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|---|---|
| Recruitment status: | Recruitment stopped |
| Start date (anticipated): | 01-02-2012 |
| Enrollment: | 10 |
| Type: | Actual |

## Ethics review

Approved WMO

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| Date: | 23-01-2012 |
| Application type: | First submission |
| Review commission: | METC Universitair Medisch Centrum Utrecht (Utrecht) |

## 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 | NL38598.041.11 |