# Inspiratory Muscle Training in Myotonic Dystrophy type 1: a pilot study.

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We hypothesize that IMT training in DM1 patients will improve respiratory muscle function, which probably could prevent the development of atelectasis and pneumonia or even delay the need for HMV.

| | |
|---|---|
| **Ethische beoordeling** | Positief advies |
| **Status** | Anders |
| **Type aandoening** | - |
| **Onderzoekstype** | Interventie onderzoek |

## Samenvatting

### ID

NL-OMON27599

**Bron**
NTR

**Verkorte titel**
IMT study

**Aandoening**

Myotonic Dystrophy type 1

### Ondersteuning

**Primaire sponsor:** none
**Overige ondersteuning:** none

### Onderzoeksproduct en/of interventie

### Uitkomstmaten

**Primaire uitkomstmaten**

inspiratory muscle strenght and inspiratory endurance capacity after 12 weeks of training.

# Toelichting onderzoek

### Achtergrond van het onderzoek

Rationale: In case of hypercapnic respiratory failure in Myotonic Dystrophy type 1 (DM1) patients, the only optional treatment is home mechanical ventilation (HMV). However, HMV is frequently used insufficient and survival benefits are not clear. So, new treatments are needed. Inspiratory muscle training (IMT) has shown positive effects in other neuromuscular disorders. We hypothesize that an IMT program, using POWERbreathe devices, of 12 weeks at home will improve inspiratory muscle strength and endurance capacity in DM1 patients.
Objective: The primary objective is to analyse the effects of IMT on respiratory muscle strength and endurance capacity after a 12 week IMT training schedule at home.
Study design: A prospective intervention pilot study of 10 DM1 patients. Results of inspiratory muscle strength and endurance capacity after the 12 week training program will be compared with results at baseline.
Study population: Adult (≥ 18 year) DM1 patients (diagnosis DNA proven) with vital capacity and maximum inspiratory muscle strength < 80% of predicted, who are not using HMV or when HMV is not indicated yet.
Intervention: a 12 week IMT program at home consists of two training sessions per day (one session is 30 breaths) and at least five days per week, using a digital inspiratory muscle trainer (POWERbreathe KHP2). Intensity of the threshold will be programmed individually for every patient, based on individuals' maximum inspiratory muscle strength. Every two weeks a supervised training will be performed and threshold will be increased based on a predefined schedule in combination with training results.
Main study parameters/endpoints: Difference in respiratory muscle strength and endurance capacity after 12 weeks of IMT training at home compared to results at baseline.
Nature and extent of the burden and risks associated with participation, benefit and group relatedness: Patients have to perform at least ten sessions per week. One session exist of 30 breaths using the POWER breathe (which will take circa 3 min). Patients can experience some myalgia and dizziness due to the training. Patients could benefit of the training by strengthening their respiratory muscles and probably by secondary positive effects of decreasing pneumonia risks and in case of continuing therapy the development of respiratory failure can be delayed (and subsequently needs for HMV). In general, no adverse events of training in DM1 are described. To better understand the effects of training on the respiratory muscles in a neuromuscular disorder, additional tests with pulmonary function and diaphragm tests will be performed. Indirect benefits might be achieved as well, because at a group level we will learn more about training effects of respiratory muscles to develop novel treatment options for patients with DM1 and respiratory failure.

### Doel van het onderzoek

We hypothesize that IMT training in DM1 patients will improve respiratory muscle function, which probably could prevent the development of atelectasis and pneumonia or even delay the need for HMV.

**Onderzoeksopzet**

Study start (expected): 1-4-2020
Start inclusion: 1-5-2020
Study period: 1,5 years
End inclusion period: 01-09-2020

**Onderzoeksproduct en/of interventie**

12 weeks program of IMT.

# Contactpersonen

## Publiek

UMCG
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050 361 32 00

## Wetenschappelijk

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# Deelname eisen

## Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

In order to be eligible to participate in this study, a subject must meet all of the following criteria:
- Diagnosed with DM1 (based on DNA features).
- Age ≥ 18 years old.
- Reduced PImax and vital capacity (both <80% of predicted).
- Able to provide feedback.

## Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

A potential subject who meets any of the following criteria will be excluded from participation in this study:
- Need or indication for HMV (daytime hypercapnia, pCO2 ≥ 6.0 kPa)
- Inability to adequately use a POWERbreathe, for example air leak during maneuvers.
- Inability to communicate or answer questionnaires.
- Vital capacity <25% of predicted.
- Clinically unstable, for example pneumonia

# Onderzoeksopzet

## Opzet

| | |
|---|---|
| Type: | Interventie onderzoek |
| Onderzoeksmodel: | Anders |
| Toewijzing: | N.v.t. / één studie arm |
| Blindering: | Open / niet geblindeerd |
| Controle: | N.v.t. / onbekend |

## Deelname

Nederland

| | |
|---|---|
| Status: | Anders |
| (Verwachte) startdatum: | 01-04-2020 |
| Aantal proefpersonen: | 10 |
| Type: | Onbekend |

## Voornemen beschikbaar stellen Individuele Patiënten Data (IPD)

**Wordt de data na het onderzoek gedeeld:** Nog niet bepaald

**Toelichting**
The researchers can be contacted to discuss the details of sharing the data

# Ethische beoordeling

Positief advies

| | |
|---|---|
| Datum: | 08-01-2020 |
| Soort: | Eerste indiening |

# Registraties

## Opgevolgd door onderstaande (mogelijk meer actuele) registratie

Geen registraties gevonden.

## Andere (mogelijk minder actuele) registraties in dit register

Geen registraties gevonden.

## In overige registers

| Register | ID |
|---|---|
| NTR-new | NL8272 |
| Ander register | METC UMCG (following) : 2020/037 |

# Resultaten