Differential Response to non-invasive ventilation in myotonic dystrophy

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Ethische beoordeling	Positief advies
Status	Werving gestart
Type aandoening	-
Onderzoekstype	Observationeel onderzoek, zonder invasieve metingen

Samenvatting

ID

NL-OMON23487

Bron
Nationaal Trial Register

Verkorte titel
REMeDY

Aandoening

Myotonic dystrophy type 1
Neuromuscular disorder

Ondersteuning

Primaire sponsor: MUMC
Overige ondersteuning: Prinses Beatrix Spierfonds

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

The primary objective is to investigate the effect of NIV on different factors including gas

exchange, lung function, daily functioning, fatigue and sleepiness, sleep quality, quality of life and compliance to the therapy.

Toelichting onderzoek

Achtergrond van het onderzoek

Patients with myotonic dystrophy (DM1) frequently develop sleep-disordered breathing (SDB) with or without alveolar hypoventilation, which may lead to fatigue, excessive daytime sleepiness, and impaired sleep quality, negatively influencing daily and cognitive functioning, and quality of life. Furthermore, SDB is associated with cardiac conduction disorders, and can lead to cardiac arrhythmias. The benefit of non-invasive ventilation (NIV) for patients with DM1 varies to a great extent, from a burdensome experience to a beneficial treatment. This also leads to difficulty motivating patients for NIV, and this seems a bigger problem in DM1 compared to other patient groups. Therefore, identification of patients with DM1 that do (or do not) respond well to NIV will become necessary to improve its cost-effectiveness and reduce the burden for patients and their loved ones. This requires consensus about the key performance measures of NIV. The study is a longitudinal observational study to profile a multidimensional response to NIV in patients with DM1. We will profile a multidimensional response to NIV in patients with DM1, enabling us to identify patients with DM1 that do (or do not) respond well to NIV.

In this prospective research, DM1 patients with SDB will be included when their treating physician at the local centre of home mechanical ventilation decides that there is an indication to start with NIV treatment. To determine the indication for NIV, physicians use the 'Veldnorm chronische beademing', a document which has been developed by and for physicians of the centres of home mechanical ventilation in the Netherlands. Data will be collected regarding: lung function test, blood gas, polysomnography or overnight measurement of O2 and CO2. Follow-up will take place up to 6 months after start with the NIV treatment. Follow-up data will be collected systematically and registered in an eCRF. We aim to include our patients within a timeframe of 2.5 years.

Doel van het onderzoek

DM1 patients that start with NIV will respond differently in terms of gas exchange, exercise capacity, daily functioning. fatigue, sleep quality, excessive daytime sleepiness and quality of life. This research will enable us to identify the different factors that influence the response to NIV.

Onderzoeksopzet

T0: baseline measurements
T1: start non-invasive ventilation
T2: follow-up after 2 weeks

T3: follow-up after 6 weeks
T4: follow-up after 6 months (and end of study)

Contactpersonen

Publiek

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Wetenschappelijk

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Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

- diagnosis myotonic dystrophy type 1
- age >18 years
- daytime pCO2 >6.0 kPa and complaints of alveolar hypoventilation OR daytime pCO2 >7.0 kPa without complaints of alveolar hypoventilation
- the general criteria regarding home mechanical ventilation ('dutch veldnorm 'Chronische beademing' versie 1.0, 2012) are met

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

- previous episode of non-invasive home mechanical ventilation or CPAP (continuous positive airway pressure) therapy in de last 5 years
- other medical condition leading to hypercapnia
- severe heart failure, NYHA IV

- instable angina pectoris

Onderzoeksopzet

Opzet

Type:	Observationeel onderzoek, zonder invasieve metingen
Onderzoeksmodel:	Anders
Toewijzing:	N.v.t. / één studie arm
Blindering:	Open / niet geblindeerd
Controle:	N.v.t. / onbekend

Deelname

Nederland

Status:	Werving gestart
(Verwachte) startdatum:	13-08-2019
Aantal proefpersonen:	80
Type:	Verwachte startdatum

Voornemen beschikbaar stellen Individuele Patiënten Data (IPD)

Wordt de data na het onderzoek gedeeld: Nee

Ethische beoordeling

Positief advies

Datum:	19-08-2019
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	NL7972
Ander register	METC azM/UM : METC 2018-0853

Resultaten