Arterial Stiffness in patients with Duchenne muscular dystrophy

Gepubliceerd: 04-03-2020 Laatst bijgewerkt: 19-03-2025

Aortic stiffness is increased in DMD patients due to the absence of dystrophin. Aortic stiffness is reduced by ACEi therapy, which reduces the progression of LV dysfunction.

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

Samenvatting

ID

NL-OMON23048

Bron
NTR

Verkorte titel
DMDAS

Aandoening

Duchenne muscular dystrophy

Ondersteuning

Primaire sponsor: None
Overige ondersteuning: None

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

The primary end-point of this study is the difference in PWV between DMD patients and published paediatric reference values.
The primary end point of the follow up part of the study is the difference influence of ACEi

treatment on arterial stiffness 3 months and 6 months after the start of treatment.

Toelichting onderzoek

Achtergrond van het onderzoek

Rationale: Cardiomyopathy in Duchenne muscular dystrophy (DMD) is frequent and characterized by progressive left ventricular (LV) fibrosis leading to dysfunction and dilatation. Factors increasing LV afterload, such as aortic stiffness, may promote LV wall stress and lead to worsening of LV function. DMD patients are currently treated with Angiotensin converting enzyme inhibitors (ACEi) to delay onset and progression of LV dysfunction but the working mechanism of this medication remains largely unknown.

Objective: We aim to investigate if aortic stiffness, measured by pulse wave velocity (PWV), is present in patients with DMD. Next, we aim to investigate if treatment with ACEi reduces aortic stiffness over time and the influence on cardiac function.

Study design: This prospective observational study has a cross sectional and longitudinal part.
-Cross sectional part: PWV measurement will be performed using the Arteriograph. Echocardiography will be performed to investigate the cardiac function.
-Longitudinal part: will exist of patients that have an indication for ACEi therapy from regular care. PWV will be measured 3, 6 and 9 months after the start of ACEi. Echocardiography will be performed to investigate the cardiac function.

Study population: DMD patients between 4-15 years that do not use ACEi or any other cardiac medication.

Main study parameters/endpoints: The primary end-point of this study is the difference in PWV between DMD patients and published paediatric reference values.
The primary end point of the follow up part of the study is the difference between PWV in DMD patients before and after the start of ACEi.

Nature and extent of the burden and risks associated with participation, benefit and group relatedness: The PWV measurement by the Arteriograph has no known risks due to its non-invasive nature. Patients will not directly benefit from participating in the study. A better understanding of the course of cardiovascular changes and the influence of ACEi treatment could lead to refinement and a more individualist ACEi treatment strategy for DMD patients in the future.

Doel van het onderzoek

Aortic stiffness is increased in DMD patients due to the absence of dystrophin. Aortic stiffness is reduced by ACEi therapy, which reduces the progression of LV dysfunction.

Onderzoeksopzet

Cross sectional part: Baseline visit: assessment of PWV and cardiac function

Longitudinal part: assessment of PWV 3, 6, 9 months after start ACEi. Assessment of cardiac function 9 months after start ACEi.

Contactpersonen

Publiek

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Wetenschappelijk

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Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

- Age between 4-15 years
- Confirmation of the diagnosis of Duchenne Muscular Dystrophy by DNA mutation or muscle biopsy.
- Who visit the DMD-out patient clinical of the LUMC for their annual visit

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

- Previous exposure to cardiac medication, including ACEi (history or current use)
- Unable to lie in supine position for echocardiography

- Impossibility to measure jugulum-symphysis distance

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:	04-03-2020
Aantal proefpersonen:	34
Type:	Verwachte startdatum

Voornemen beschikbaar stellen Individuele Patiënten Data (IPD)

Wordt de data na het onderzoek gedeeld: Nog niet bepaald

Ethische beoordeling

Positief advies

Datum:	04-03-2020
Soort:	Eerste indiening

Registraties

Opgevolgd door onderstaande (mogelijk meer actuele) registratie

ID: 54937
Bron: ToetsingOnline

Titel:

Andere (mogelijk minder actuele) registraties in dit register

Geen registraties gevonden.

In overige registers

Register	ID
NTR-new	NL8429
CCMO	NL71572.058.19
OMON	NL-OMON54937

Resultaten