Arterial Stiffness in patients with Duchenne muscular dystrophy

No registrations found.

Ethical review	Positive opinion
Status	Recruiting
Health condition type	-
Study type	Observational non invasive

Summary

ID

NL-OMON23048

Source
Nationaal Trial Register

Brief title
DMDAS

Health condition

Duchenne muscular dystrophy

Sponsors and support

Primary sponsor: None
Source(s) of monetary or material Support: None

Intervention

Outcome measures

Primary outcome

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.

Secondary outcome

Relation between arterial stiffness and cardiac function at baseline and 12 months of the study
Accuracy of PWV measured by Arteriograph versus PWV measured by cardiac MRI

Study description

Background summary

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.

Study objective

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.

Study design

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.

Contacts

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Eligibility criteria

Inclusion criteria

- 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

Exclusion criteria

- 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

Study design

Design

Study type:	Observational non invasive
Intervention model:	Other
Allocation:	Non controlled trial
Masking:	Open (masking not used)
Control:	N/A , unknown

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	04-03-2020
Enrollment:	34
Type:	Anticipated

IPD sharing statement

Plan to share IPD: Undecided

Ethics review

Positive opinion

Date:	04-03-2020
Application type:	First submission

Study registrations

Followed up by the following (possibly more current) registration

ID: 54937
Bron: ToetsingOnline
Titel:

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

No registrations found.

In other registers

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

Study results