Effects of exercise therapy in patients with Pompe's disease

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Continuing exercise therapy will prove beneficial effects, in Pompe patients in the long-term, on one or more measured outcomes: endurance, muscle strength, muscle function, core stability and quality of life.

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

Samenvatting

ID

NL-OMON22791

Bron
NTR

Verkorte titel
TEmPO study

Aandoening

Pompe's disease

Ondersteuning

Primaire sponsor: This study is funded by a grant from Prinses Beatrix Spierfonds.
Overige ondersteuning: Prinses Beatrix Spierfonds

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

Endurance

Toelichting onderzoek

Achtergrond van het onderzoek

Pompe's disease is a rare myopathy consisting of acid alpha-glucosidase enzyme deficiency, which leads to accumulation of glycogen in especially skeletal muscle cells, leading to progressive muscle weakness of mostly shoulder, hip and trunk muscles. Patients are supported with enzyme replacement therapy with beneficial effects. However these beneficial effects fade over time and patients still experience limitations due to their myopathy. The search for complementing non-medical therapies are therefore of great importance for these patients. In 2011 mildly affected patients followed a training program over 12 weeks. On the short term, training improved their endurance, muscle strength and function, core stability, fatigue and pain. With our current study we aim to evaluate the long term effects of the mentioned exercise therapy. We aim to determine whether patients continued the training program and reasons of quitting the therapy. We will also select a group of controls which didn't participate in the training program. A comparison will be made between these two groups on endurance, muscle strength and function, core stability and quality of life to evaluate whether trained patients perform better.

Doel van het onderzoek

Continuing exercise therapy will prove beneficial effects, in Pompe patients in the long-term, on one or more measured outcomes: endurance, muscle strength, muscle function, core stability and quality of life.

Onderzoeksopzet

Completion of measurements: within 6 months. A small delay exists due to the current corona crisis. We aim to start in september.

Onderzoeksproduct en/of interventie

N/A

Contactpersonen

Publiek

Erasmus MC
Gamida Ismailova

06-33342010

Wetenschappelijk

Erasmus MC
Gamida Ismailova

06-33342010

Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

- Participants of the training study in 2011
- Controls: didn't participate in the training study in 2011, but were eligible to participate: mildly affected in 2011
- Confirmed disease (DNA mutation analysis and enzyme activity in leukocytes)
- Receiving at least 8 years of enzyme replacement therapy

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

- Controls depending on walking aids in 2011
- Controls (partially) depending on ventilation in 2011

Onderzoeksopzet

Opzet

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

Deelname

Nederland

Status:	Werving gestart
(Verwachte) startdatum:	01-09-2020
Aantal proefpersonen:	46
Type:	Verwachte startdatum

Voornemen beschikbaar stellen Individuele Patiënten Data (IPD)

Wordt de data na het onderzoek gedeeld: Nog niet bepaald

Toelichting
N/A

Ethische beoordeling

Positief advies

Datum:	06-04-2020
Soort:	Eerste indiening

Registraties

Opgevolgd door onderstaande (mogelijk meer actuele) registratie

ID: 47930
Bron: ToetsingOnline
Titel:

Andere (mogelijk minder actuele) registraties in dit register

Geen registraties gevonden.

In overige registers

Register	ID
NTR-new	NL8508
CCMO	NL16769.078.07
OMON	NL-OMON47930

Resultaten

Samenvatting resultaten
N/A