Global Growth hormone study in Adults with Prader-Willi Syndrome

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Our hypothesis is that growth hormone threatment in adults with Prader-Willi Syndrome will improve their metabolic and cardiovascuar health, thereby resulting in a reduced mortality.

Ethische beoordeling	Niet van toepassing
Status	Werving nog niet gestart
Type aandoening	-
Onderzoekstype	Interventie onderzoek

Samenvatting

ID

NL-OMON27149

Bron
NTR

Verkorte titel
GGAP Study

Aandoening

Prader-Willi Syndrome

Ondersteuning

Primaire sponsor: Erasmus Medical Center
Overige ondersteuning: Foundation for Prader-Willi Research
Prader-Willi Fonds
Pfizer (sponsoring of growth hormone and placebo)

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

The primary endpoint is change in lean body mass (LBM (kg)) as assessed by Dual Energy X-

ray Absorptiometry (DEXA) scan

Toelichting onderzoek

Achtergrond van het onderzoek

Rationale:
Prader-Willi Syndrome (PWS) is a rare, complex disorder. The mortality in PWS is 3% per year, even in young patients. Besides insatiable appetite and intellectual disability, patients with PWS show symptoms of growth hormone (GH) deficiency, like small hands and feet, a low muscle mass and increased body fat. Treatment with GH in patients with PWS improves the body composition and can therefore prevent obesity and the complications of obesity, like diabetes mellitus and cardiovascular diseases. Currently, GH treatment (GHt) in adults with PWS is only reimbursed if they have proven GH deficiency. However, due to the unsuitability of 'regular' GH tests for patients with PWS, (functional) GH deficiency cannot be proven in these patients. GHt in children with PWS has resulted in a tremendous improvement of the physical and mental health. We hypothesize that GHt in adults with PWS will improve their metabolic and cardiovascular health, thereby resulting in a reduced mortality.

Objective:
To measure the effect of GHt on metabolic and cardiovascular health in adults with PWS.

Study design:
Randomized, double-blinded, placebo controlled crossover trial for two years with a washout period of 3 months.

Study population:
72 adults with PWS of 30 years or older who have not been treated with GH during the past three years from the participating countries Australia and the Netherlands.

Intervention:
Participants will be randomized to placebo or GHt for one year. After a washout period of 3 months, the patients who received placebo during the first year will be switched to GHt during the second year and vice versa.

Main study parameters/endpoints:
The primary endpoint is change in lean body mass (LBM (kg)) as assessed by Dual Energy X-ray Absorptiometry (DEXA) scan. Secondary endpoints are total fat mass, bone density, physical health, endurance, psychosocial functioning and quality of life (QoL).

Doel van het onderzoek

Our hypothesis is that growth hormone threatment in adults with Prader-Willi Syndrome will improve their metabolic and cardiovascuar health, thereby resulting in a reduced mortality.

Onderzoeksopzet

t=0, t=3, t=6, t=9, t=12, t=15, t=18, t=21, t=24, t=27

Onderzoeksproduct en/of interventie

Participants will be randomized to placebo or growth hormone treatment for one year. After a washout period of 3 months, the patients who received placebo during the first year will be switched to growth hormone treatment during the second year and vice versa.

Contactpersonen

Publiek

Erasmus University Medical Center, Rotterdam
Anna Rosenberg

010-7040704

Wetenschappelijk

Erasmus University Medical Center, Rotterdam
Anna Rosenberg

010-7040704

Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

- The patient is diagnosed with PWS
- The patient is 30 years or older
- GH therapy was suspended at least three years before starting the study

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

- Non cooperative behaviour

- Known malignancies
- Poorly controlled diabetes (HbA1c > 64 mmol/mol (8%))
- Untreated obstructive sleep apnea (apnea-hypopnea index > 5)
- BMI above 40 kg/m2
- Osteosynthesis material
- Testosterone suppletion is not stable for three months

Onderzoeksopzet

Opzet

Type:	Interventie onderzoek
Onderzoeksmodel:	Cross-over
Toewijzing:	Gerandomiseerd
Blindering:	Dubbelblind
Controle:	Placebo

Deelname

Nederland

Status:	Werving nog niet gestart
(Verwachte) startdatum:	01-09-2020
Aantal proefpersonen:	72
Type:	Verwachte startdatum

Voornemen beschikbaar stellen Individuele Patiënten Data (IPD)

Wordt de data na het onderzoek gedeeld: Nog niet bepaald

Ethische beoordeling

Niet van toepassing

Soort:	Niet van toepassing

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	NL8274
Ander register	METC EMC : ABR71549

Resultaten