Global Growth hormone study in Adults with Prader-Willi Syndrome

No registrations found.

Ethical review	Not applicable
Status	Pending
Health condition type	-
Study type	Interventional

Summary

ID

NL-OMON27149

Source
NTR

Brief title
GGAP Study

Health condition

Prader-Willi Syndrome

Sponsors and support

Primary sponsor: Erasmus Medical Center
Source(s) of monetary or material Support: Foundation for Prader-Willi Research
Prader-Willi Fonds
Pfizer (sponsoring of growth hormone and placebo)

Intervention

Outcome measures

Primary outcome

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

Secondary outcome

Secondary endpoints are total fat mass, bone density, physical health, endurance, psychosocial functioning and quality of life.

Study description

Background summary

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).

Study objective

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.

Study design

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

Intervention

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.

Contacts

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Eligibility criteria

Inclusion criteria

- 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

Exclusion criteria

- 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

Study design

Design

Study type:	Interventional
Intervention model:	Crossover
Allocation:	Randomized controlled trial
Masking:	Double blinded (masking used)
Control:	Placebo

Recruitment

NL

Recruitment status:	Pending
Start date (anticipated):	01-09-2020
Enrollment:	72
Type:	Anticipated

IPD sharing statement

Plan to share IPD: Undecided

Ethics review

Not applicable

Application type:	Not applicable

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

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

No registrations found.

In other registers

Register	ID
NTR-new	NL8274
Other	METC EMC : ABR71549

Study results