Effects of growth hormone treatment after final height in Prader-Willi Syndrome: A double-blind multicenter, cross-over study on the effects of growth hormone versus placebo on body composition and psychosocial behaviour in transition

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to evaluate the effects of GH versus withdrawal of GH, after final height is reached, on weight, body composition, psychosocial functioning, carbohydrate metabolism, circulating lipids, and respiratory function during transition period until the age…

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Neurological disorders congenital
Study type	Interventional

Summary

ID

NL-OMON39086

Source
ToetsingOnline

Brief title
Effects of GH after final height in PWS

Condition

- Neurological disorders congenital

Synonym
Prader-Willi Syndrome

Research involving
Human

Sponsors and support

Primary sponsor: Erasmus MC, Universitair Medisch Centrum Rotterdam
Source(s) of monetary or material Support: Stichting Kind en Groei. Daarnaast wordt het groeihormoon en placebo kosteloos ter beschikking gesteld door de firma Pfizer

Intervention

Keyword: Growth hormone, Prader-Willi syndrome, transition

Outcome measures

Primary outcome

To assess effects of GH-treatment versus placebo on

- body composition

- carbohydrate metabolism

- psychosocial functioning

- sleep-related breathing disorders

- circulating lipids

- blood pressure

- development of psychiatric disorders

Secondary outcome

- To study the effects of GH-treatment versus placebo on thyroid hormone

levels, IGF-I and IGF binding proteins, adiponectin, ghrelin.

- To study compliance to the diet.

- To study if certain bloodmarks are related to the development of psychiatric

disorders

Study description

Background summary

GH improves height velocity, and body composition in PWS children. Preliminary data also suggest improvement of psychosocial functioning during GH. When epiphysial fusion is complete and final height is reached, GH-treatment has to be discontinued. However, discontinuation of GH results in a decrease of lean body mass, an increase of body fat percentage and a deterioration of psychosocial behaviour. A preliminary study showed that also young adults with PWS might benefit from GH-treatment, with regard to body composition, and psychosocial wellbeing. Adults and adolescents with PWS often develop psychiatric disorders, how these psychiatric disorders develop is unknown.

Study objective

to evaluate the effects of GH versus withdrawal of GH, after final height is reached, on weight, body composition, psychosocial functioning, carbohydrate metabolism, circulating lipids, and respiratory function during transition period until the age of 24. Describe the development of psychiatric disorders in adolescents with PWS.

Study design

After stratification for BMI, gender, originally followed in the GH study vs. otherwise GH-treated patients, subjects will be randomized to either placebo or GH-treatment group, according to a double blind, placebo-controlled cross-over design during the first 2 years. After 2 years, all patients receive GH treatment after ATT-GHRH test has been performed. Anthropometric assessments and blood pressure will be performed every 3 months. Six-monthly, assessment of body composition (DXA), carbohydrate metabolism and circulating lipids and other laboratory parameters will be performed. Yearly, evaluation of sleep-related breathing (polysomnography), cognition and behaviour (GIT, TVZ) will be performed and a psychiatric investigation will be done.

Intervention

Treatment with GH: Genotropin 0.67 mg/m2/day s.c. or placebo.
Psychiatric investigation.

Study burden and risks

Invasive: Patients will be treated with GH and placebo by daily subcutaneous injecties. It has been shown that these injections are usually very well tolerated. Venapuncture will be performed every 6 months, which is necessary

for monitoring during GH treatment

Non-invasive: Diet and exercise are part of the regular management of PWS. Three-monthly, a general physical examination will be performed, which is not painfull and will take approximately 15 minutes. Two times a year a DEXA measurement will be performed. Subjects are lying down as the measurement is performed. They are accompanied by one parent. Radiation dose is very low. Once a year, GIT will be performed, which requires the patient to concentrate. Behaviour is assessed by parent questionnaires, and therefore will not be a burden to the patients.
Every year, a polysomnography will be performed in the sleep center. Patients will be accompanied by one parent. Several electrodes will be placed on the face, limbs and thorax. Polysomnography is recommended for all subjects with PWS, irrespective of GH treatment. Yearly psychiatric evaluation that wil take 3 hours

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age

Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

genetically confirmed diagnosis of Prader-Willi syndrome
treated with GH for at least 2 years
final height is reached or epiphysial fusion is complete
aged 18-24 years

Exclusion criteria

non-cooperative behaviour
extremely low dietary intake of less than minimal required intake according to WHO
medication to reduce weight (fat)

Study design

Design

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

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	16-06-2008
Enrollment:	26
Type:	Actual

Medical products/devices used

Product type:	Medicine

Brand name:	Genotropin
Generic name:	Somatropin
Registration:	Yes - NL outside intended use

Ethics review

Approved WMO Date:	27-02-2008
Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	05-03-2008
Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	09-09-2010
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	11-07-2013
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	30-07-2013
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)

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
EudraCT	EUCTR2007-004716-31-NL
CCMO	NL19376.078.07