Young adult Prader-Willi (YAP) Syndrome Study: Effects of growth hormone after final height: A clinical care to the optimal dosage of growth hormone in young adults with PWS.

Published: 12-01-2012
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To evaluate the effects of GH, after final height is reached, on weight, body composition, psychosocial functioning, carbohydrate metabolism and serum lipids. To find an optimal dose growth hormone for adults with PWS. We aimed to stabilize the body...

Ethical review	Approved WMO
Status	Completed
Health condition type	Endocrine disorders congenital
Study type	Interventional

Summary

ID

NL-OMON50380

Source
ToetsingOnline

Brief title
YAP study

Condition

- Endocrine disorders congenital
- Hypothalamus and pituitary gland disorders
- Appetite and general nutritional disorders

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.

Intervention

Keyword: Growth hormone, Prader-Willi syndrome, Young adults

Outcome measures

Primary outcome

A manualized treatment for an optimal dosage GH in young adults with Prader

Willi Syndrome

To assess the long term effects of GH-treatment on:

- body composition

- carbohydrate metabolism

- serum lipids

- blood pressure

- psychosocial functioning

- psychiatric functioning

Secondary outcome

- To study the effects of long term GH-treatment on thyroid hormone levels,

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

- To study the compliance to the diet.

- To study the influence of GH treatment on start, first symptoms and

development of psychiatric disorders.

- To study the effect of disturbances in amino acids profile, neopterin and

monoaminergic neurotransmission, which are measured with peripheral markers, on

the pathophysiology of the PWS psychiatric syndrome.

-To study fenotype-genotype correlations in young adults with PWS

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, preliminary results show that in patients with Prader Willi syndrome discontinuation of GH results in a decrease of lean body mass, an increase of body fat percentage and a deterioration of psychosocial behaviour. We expect the Transitionstudy (dubble blind, placebo controlled study) to show that young adults with PWS might also benefit from GH-treatment, with regard to body composition, and psychosocial wellbeing.

Psychiatric disorders are reported in adults with Prader-Willi syndrome. No longitudinal studies have described the prevalence, natural course and prevention of psychiatric disorders in adults with PWS. Psychiatric symptoms and psychiatric disorders occur frequently in persons with PWS, therefore it is of utmost importance to study this prevalence and course into adulthood in a longitudinal design. The obtained information of this amendment unites perfectly with the earlier and future obtained data on behaviour and cognition in children with PWS, who are part of a unique cohort of 140 Dutch children with PWS (MEC 205.253/2001/230 amendment (number unknown) approved at June 30th, 2010 and MEC 2007-189 amendment 1, approved September 9th, 2010).

Study objective

To evaluate the effects of GH, after final height is reached, on weight, body composition, psychosocial functioning, carbohydrate metabolism and serum lipids.

To find an optimal dose growth hormone for adults with PWS. We aimed to stabilize the body composition and psychosocial functioning and psychiatric functioning.

Study design

Before (re)starting GH, patient visits the outpatient clinic for the following assessments
- Oral glucose tolerance test
- Laboratory test
- weight, Waist-hip ratio
- Blood pressure
- DXA scan

Additional tests during the following years:
- DXA for body composition and bone density [at start, at 6 months, at 12 months and yearly thereafter]
- Nutritional intake during 7 days (via daily intake book) [at 12 mo intervals]
- Laboratory assessments [at start, at 6 months, at 12 months and yearly thereafter]
- Oral glucose tolerance test (every 5 years)

Intervention

Treatment with Norditropin 0,33mg/m2/dag. The GH dose will be titrated to serum IGF-1 levels between +1 SDS and +2 SDS, based on IGF-1 measured after 6 months GH treatment and a DXA scan.

Study burden and risks

Burden:
- Filling out questionnaires
- Oral glucose tolerance test (once every 5 years)

Benefit:
The positive effects of GH on bodycomposition and psychosocial behaviour.
The positive effects of GH on psychiatric functioning.

Group relatedness:
applicable for other adolescents and adults with Prader Willi syndrome

Contacts

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Scientific

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

1) Genetically confirmed Prader-Willi syndrome
2) Attained adult height
3) Treated with GH during childhood or received GH treatment in the last few years

Exclusion criteria

- non cooperative behaviour
- medication to reduce weight (fat)
- severe sleep related breathing disorders (according to physician)
- never received GH

Study design

Design

Study phase:	3
Study type:	Interventional
Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Completed
Start date (anticipated):	24-10-2012
Enrollment:	75
Type:	Actual

Medical products/devices used

Product type:	Medicine
Brand name:	Norditropin
Generic name:	Somatropin
Registration:	Yes - NL outside intended use

Ethics review

Approved WMO

Date:	12-01-2012
Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)

Approved WMO

Date:	17-10-2012
Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)

Approved WMO

Date:	02-08-2013
Application type:	Amendment

Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	21-12-2015
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	08-01-2016
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	15-01-2016
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	17-08-2018
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	27-08-2018
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	02-02-2021
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	03-03-2021
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	EUCTR2011-001313-14-NL
CCMO	NL36206.078.11