Controlled growth hormone study in children with Prader Willi Syndrome.

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GH treatment improves height, weight, body composition, muscle strength, activity level, psychosocial development, psychomotor development in infants, metabolism and respiratory function versus no GH treatment in children with Prader Willi Syndrome.

Ethische beoordeling	Positief advies
Status	Werving gestopt
Type aandoening	-
Onderzoekstype	Interventie onderzoek

Samenvatting

ID

NL-OMON26880

Bron
Nationaal Trial Register

Verkorte titel
N/A

Aandoening

Prader Willi Syndrome

Ondersteuning

Primaire sponsor: The study is financially supported by Pfizer
Overige ondersteuning: Dutch Growth Foundation

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

To asses effects of GH-treatment vs. no GH-treatment in children with PWS on:
height, weight, body composition,

muscle mass, muscle strength and daily life activity.
Cognition, behaviour and social emotional development. Resting Energy Expenditure.
Psychomotor development in infants.

Toelichting onderzoek

Achtergrond van het onderzoek

Summary of the Dutch National Growth Hormone Study in Children with Prader Willi Syndrome.

Title:

Multicenter, randomized, controlled growth hormone study in children with Prader Willi Syndrome: effects on growth, body composition, activity level and psychosocial development" MEC 2001/230.

Short title:

Controlled growth hormone study in children with Prader Willi Syndrome.

Background:

Children with Prader Willi Syndrome have often short stature and have an abnormal body composition (Increased fatpercentage and decreased lean body mass). Physical activity level is therefore decreased in children with Prader Willi Syndrome. Children with Prader Willi syndrome often have mental retardation and behavioral problems.

Recent studies showed an improvement in height and body composition during growth hormone (GH) treatment in children with Prader Willi Syndrome. Preliminary data showed also an improvement in activity level, metabolism, respiratory function, behaviour and social-emotional development.

Aim:

To study the effects of GH-treatment versus no GH treatment in children with Prader Willi Syndrome on changes in height, weight, body composition, muscle strength, activity level, psychosocial development, metabolism and respiratory function.

Patients:

85 children with PWS, aged 6 mo. to 16 years at start of the study.

Intervention:

Treatment with GH: Genotropin ® 1mg/m2/d s.c.

Design:

Children will be randomly divided into 3 subgroups: infants, prepubertal children and adolescents. Infants and prepubertal children will be treated in a controlled design. (Start GH-treatment at start of the study or after 1 or two years resp.) Adolescents will be treated in a non-controlled design. All children over the age of 3 will receive dietary advice and an exercise program.

Anthropometric assessments will be performed every 3 months.

Yearly assessment of body composition (DEXA), metabolism (Indirect calorimetry), muscle strength (quadriceps dynamometry) and activity level (Activity monitoring) will be performed.

Cognition (subtests of WISC, WPPSI or BOS), behaviour (DBC), social emotional development (VABS) and quality of life (DUX 25) will be measured yearly.

In a subgroup of children pulmonary CO2 responsiveness will be measured.

Objectives:

Primary:

To asses effects of GH-treatment vs. no GH-treatment in children with PWS on
Height, weight, body composition
Muscle mass, muscle strength and daily life activity
Cognition, behaviour and social emotional development
Resting Energy Expenditure
CO2 responsiveness.

Secondary:

To study the effect of additional dietary advise and physical exercise on body composition in children with PWS treated with GH vs, not treated with GH.

Doel van het onderzoek

GH treatment improves height, weight, body composition, muscle strength, activity level, psychosocial development, psychomotor development in infants, metabolism and respiratory function versus no GH treatment in children with Prader Willi Syndrome.

Onderzoeksopzet

N/A

Onderzoeksproduct en/of interventie

Treatment with GH: Genotropin ® 1mg/m2/d s.c. vs. no GH-treatment.

Dietary and exercise advice.

Contactpersonen

Publiek

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Wetenschappelijk

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Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen

(Inclusiecriteria)

1. Genetically confirmed diagnosis of Prader Willi Syndrome;

2. Age between 6 months and 16 years at start of the study;

3. Bone age less than 16 years.

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

1. Extremely low dietary intake;

2. Severe scoliosis (consult spinal surgeon);

3. BMI SDS > +3SDS;

4. In children > 3 years, height SDS < 0 unless weight for height> +2SDS.

Onderzoeksopzet

Opzet

Type:	Interventie onderzoek
Onderzoeksmodel:	Parallel
Toewijzing:	Gerandomiseerd
Blindering:	Open / niet geblindeerd
Controle:	Actieve controle groep

Deelname

Nederland

Status:	Werving gestopt
(Verwachte) startdatum:	23-04-2002
Aantal proefpersonen:	85
Type:	Werkelijke startdatum

Ethische beoordeling

Positief advies

Datum:	15-03-2006
Soort:	Eerste indiening

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	NL572
NTR-old	NTR628
Ander register	: N/A
ISRCTN	ISRCTN49726762

Resultaten

Samenvatting resultaten
N/A