Metabolic effects of Growth Hormone

Gepubliceerd: 07-08-2014 Laatst bijgewerkt: 13-12-2022

The hypothesis is that rhGH treatment in children with KS results within 6 weeks in a change of metabolism recognizable as an increase of total energy expenditure (TEE). This change in metabolism can be used as a predictor of growth response in the...

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

Samenvatting

ID

NL-OMON20379

Bron
NTR

Aandoening

Small stature
Obesity
Cardiovasculair disease/Metabolic syndrome
Hyperlaxity

Kleine lengte
Obees
Cardiovasculaire ziekten/metabool syndroom
Hyperlaxiteit

Ondersteuning

Primaire sponsor: Maastricht University Medical Centre (MUMC)
PO box 5800
6202 AZ Maastricht
The Netherlands
Overige ondersteuning: Pfizer bv
Rivium Westlaan 142
2909 LD Capelle a/d IJssel
Telefoon: +31 (0)10 4064 200
Fax: +31 (0)10 4064 299

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

Objective 1:
Is there an increase in TEE during 6 weeks of treatment with rhGH in children with Kabuki Syndrome?
Objective 2:
What is the relation between the short-term (6 weeks) change in TEE as measured with the DLW technique and the long term change in height SDS during treatment with rhGH after one and two years?
Objective 3:
What is the effect of rhGH treatment on metabolic risk parameters typical for the metabolic syndrome in adults?
Objective 4:
What are the characteristics of hyermobility in the Dutch children and adults with Kabuki Syndrome:
-What is the prevalence of hypermobility
-Which limbs / joints are affected by hypermobility, with or without (sub)luxation’s
Are existing assessment tools for hypermobility (Beighton and Bulbena scores) usable in this population?
Objective 5:
What are the characteristics of body proportions in children with Kabuki Syndrome:
-How are the body proportions in Kabuki syndrome children?
-Are the body proportions in Kabuki syndrome children differently compared to the normal population?

Toelichting onderzoek

Doel van het onderzoek

The hypothesis is that rhGH treatment in children with KS results within 6 weeks in a change of metabolism recognizable as an increase of total energy expenditure (TEE). This change in metabolism can be used as a predictor of growth response in the first year of treatment and indicates a better body composition.

Secondary hypothesis is that hypermobility is present in all children with Kabuki Syndrome, mainly in the lower extremities, leading to, sometimes sever, (sub)luxations. Treatment with rhGH will lead to an increase in muscle strenth and improvement of composition of connective tissue, thus diminishing morbidity due to hypermobility in children with Kabuki Syndrome.

Onderzoeksopzet

After one year treatment and after two years of treatment.

Onderzoeksproduct en/of interventie

All subjects receive recombinant human (rh)GH in accordance with international guidelines for developmental syndromes.

The subjects will be included in a prospective study. Total body water (TBW), TEE, basal metabolic rate (BMR) and physical activity level (PAL) measurements are performed over a 6-wk period. Markers of metabolic risk factors will be determined during routine blood controls. During routine physical examinations assessment of hypermobility will be examined and photo’s will be made for calculating body proportions.

Contactpersonen

Publiek

Department of paediatrics
Maastricht University Medical Centre (MUMC)
PO box 5800
D.A. Schott
Maastricht 6202 AZ
The Netherlands
+31 (0)43 3875239

Wetenschappelijk

Department of paediatrics
Maastricht University Medical Centre (MUMC)
PO box 5800
D.A. Schott
Maastricht 6202 AZ
The Netherlands
+31 (0)43 3875239

Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

•Children with mutation in the KMT2D gene (also known as MLL2) or the KDM6A gene.

•Children who meet at least four out of five KS characteristics:

o Facial features: long palpebral fissures with eversion of outer third, arched eyebrows with sparse outer half, prominent and/or misshapen ears, and depressed nasal tip.

o Skeletal abnormalities.

o Intellectual disability (mild to moderate).

o Postnatal short stature.

o Abnormalities of dermal ridges.

•Informed consent.

•Age ≥ four years.

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

•Children with a chronological or bone age greater than 8 years for girls and 10 years for boys, because of the influence of puberty.

•Extremely low dietary intake (less than minimal required intake for age according to WHO criteria).

•Use of medication that might interfere with growth during GH therapy, such as corticosteroids and sex steroids.

•Previous or active malignancy

•Diabetes Mellitus

Onderzoeksopzet

Opzet

Type:	Interventie onderzoek
Onderzoeksmodel:	Anders
Toewijzing:	N.v.t. / één studie arm
Blindering:	Open / niet geblindeerd
Controle:	N.v.t. / onbekend

Deelname

Nederland

Status:	Werving gestart
(Verwachte) startdatum:	01-04-2013
Aantal proefpersonen:	20
Type:	Verwachte startdatum

Ethische beoordeling

Positief advies

Datum:	07-08-2014
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	NL4581

Register	ID
NTR-old	NTR4722
Ander register	NL39636.068.12 : METC

Resultaten