Metabolic effects of Growth Hormone

No registrations found.

Ethical review	Positive opinion
Status	Recruiting
Health condition type	-
Study type	Interventional

Summary

ID

NL-OMON20379

Source
NTR

Health condition

Small stature
Obesity
Cardiovasculair disease/Metabolic syndrome
Hyperlaxity

Kleine lengte
Obees
Cardiovasculaire ziekten/metabool syndroom
Hyperlaxiteit

Sponsors and support

Primary sponsor: Maastricht University Medical Centre (MUMC)
PO box 5800
6202 AZ Maastricht
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Source(s) of monetary or material Support: Pfizer bv
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Intervention

Outcome measures

Primary outcome

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?

Secondary outcome

-To assess the long-term (at start, after one and two years of treatment) term safety of

growth hormone therapy on metabolic risk parameters and body composition.

-Does rhGH treatment lead to a diminished degree of hypermobility and, because of that, to less (sub)luxations?

-Does the body composition changes during rhGH treatment?

Study description

Study objective

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.

Study design

After one year treatment and after two years of treatment.

Intervention

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.

Contacts

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Eligibility criteria

Inclusion criteria

•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.

Exclusion criteria

•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

Study design

Design

Study type:	Interventional
Intervention model:	Other
Allocation:	Non controlled trial
Masking:	Open (masking not used)
Control:	N/A , unknown

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	01-04-2013
Enrollment:	20
Type:	Anticipated

Ethics review

Positive opinion

Date:	07-08-2014
Application type:	First submission

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	NL4581
NTR-old	NTR4722
Other	NL39636.068.12 : METC

Study results