The effect of intranasal insulin on development and behaviour of children with Phelan-McDermid syndrome. Dutch: Het effect van insuline neusspray op ontwikkeling en gedrag van kinderen met het Phelan-McDermid syndroom.

Gepubliceerd: 18-12-2012 Laatst bijgewerkt: 18-08-2022

Intranasal insulin will improve development and behaviour in children with Phelan-McDermid syndrome.

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

Samenvatting

ID

NL-OMON24236

Bron
NTR

Verkorte titel
2013.2015

Aandoening

Phelan-McDermid syndrome, 22q13.3 deletion syndrome, insulin, intranasal, development, behaviour.
Dutch: Phelan-McDermid syndroom, 22q13.3 deletie syndroom, insuline, intransasaal, ontwikkeling, gedrag.

Ondersteuning

Primaire sponsor: University Medical Centre Groningen
Overige ondersteuning: ZonMw:project number 40-41500-98-11038, as part of the

program ‘Priority Medicines for Children’.

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

Developmental pace, calculated as the difference in developmental age equivalent between two assessments divided by the difference in calendar age in months at the time of these assessments (typically 6 months).

Toelichting onderzoek

Achtergrond van het onderzoek

Children with Phelan-McDermid syndrome have a severe general developmental delay and behavioural problems. The syndrome is caused by a deletion of 22q13.3 and the neurological problems are thought to result from haploinsufficiency of the SHANK3 gene. The SHANK3 protein is located in the postsynaptic density of neurons in conjunction with the insulin receptor. Insulin exerts effects on signal transduction and protein interactions in the postsynaptic density. Previous studies with intranasally administered insulin show a beneficial effect on cognitive function, declarative memory and behaviour. Moreover, a pilot study with six children demonstrated that intranasal insulin improves development and behaviour in children with the Phelan-McDermid syndrome.

The aim of this project is to validate the hypothesis that intranasal insulin improves development and behaviour in children with Phelan-McDermid syndrome.

Doel van het onderzoek

Intranasal insulin will improve development and behaviour in children with Phelan-McDermid syndrome.

Onderzoeksopzet

T = 0, 6, 12 and 18 months.

Onderzoeksproduct en/of interventie

Clinical trial with stepped wedge design. Participants will start with either intranasal insulin or placebo, once or twice a day, one puff in one or both nostrils (dependent on body weight). By steps, groups of participants will convert from placebo to intranasal insulin and remain on

insulin until the end of the study.

Contactpersonen

Publiek

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Wetenschappelijk

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Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

1. Age between 12 months and 18 years 0 months old at 1-1-2013;

2. Proven SHANK3 deletion by array-comparative genomic hybridization (array-CGH);

3. Parents need to speak and understand Dutch.

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

1. A contra-indication for the use of intranasal application (e.g. anatomical obstruction);

2. Severe perinatal brain damage (e.g. asphyxia, haemorrhage, infection);

3. A metabolic or muscle disease responsible for neurological symptoms, independent of the

22q13 deletion.

Onderzoeksopzet

Opzet

Type:	Interventie onderzoek
Onderzoeksmodel:	Parallel
Toewijzing:	Gerandomiseerd
Blindering:	Dubbelblind
Controle:	Placebo

Deelname

Nederland

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

Ethische beoordeling

Positief advies

Datum:	18-12-2012
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	NL3600
NTR-old	NTR3758
Ander register	METC / ABR : 2012/329 / NL-41213-042-12;
ISRCTN	ISRCTN wordt niet meer aangevraagd.

Resultaten

Samenvatting resultaten
On the moment of registration, the study has yet to begin. Therefore there currently are no publications concerning this study.