

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.

No registrations found.

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

Summary

ID

NL-OMON24236

Source

NTR

Brief title

2013.2015

Health condition

Phelan-McDermid syndrome, 22q13.3 deletion syndrome, insulin, intranasal, development, behaviour.

Dutch: Phelan-McDermid syndroom, 22q13.3 deletie syndroom, insuline, intransasaal, ontwikkeling, gedrag.

Sponsors and support

Primary sponsor: University Medical Centre Groningen

Source(s) of monetary or material Support: ZonMw:project number 40-41500-98-11038, as part of the program 'Priority Medicines for Children'.

Intervention

Outcome measures

Primary outcome

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).

Secondary outcome

Behaviour, improvement of behaviour represented by the increase in test scores between assessment (typically 6 months).

Study description

Background summary

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.

Study objective

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

Study design

T = 0, 6, 12 and 18 months.

Intervention

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.

Contacts

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Eligibility criteria

Inclusion criteria

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.

Exclusion criteria

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.

Study design

Design

Study type:	Interventional
Intervention model:	Parallel
Allocation:	Randomized controlled trial
Masking:	Double blinded (masking used)
Control:	Placebo

Recruitment

NL	
Recruitment status:	Pending
Start date (anticipated):	01-01-2013
Enrollment:	20
Type:	Anticipated

Ethics review

Positive opinion	
Date:	18-12-2012
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	NL3600
NTR-old	NTR3758
Other	METC / ABR : 2012/329 / NL-41213-042-12;
ISRCTN	ISRCTN wordt niet meer aangevraagd.

Study results

Summary results

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