The effect of long-term Simvastatin treatment on cognitive function and daily life in children with Neurofibromatosis 1: a one year randomized controlled trial.

Gepubliceerd: 30-12-2009 Laatst bijgewerkt: 18-08-2022

Simvastatin can have a positive effect on cognitive function and daily life functioning in children with Neurofibromatosis 1.

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

Samenvatting

ID

NL-OMON25915

Bron
Nationaal Trial Register

Verkorte titel
NF1-SIMCODA

Aandoening

Neurofibromatosis 1; Neurofibromatose type 1; NF1

Ondersteuning

Primaire sponsor: Erasmus MC – Sophia’s Children Hospital
Overige ondersteuning: ZonMW – Translationeel Onderzoek

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

1. Cognitive Function: WISC III-R;
2. Behavioral Problems on the Child Behavioral Checklist (CBCL, parents): internalizing behavioral problems, attention problems.

Toelichting onderzoek

Achtergrond van het onderzoek

Children with Neurofibromatosis type 1 (NF1, prevalence 1:3000) commonly display cognitive deficits that have a large impact on behaviour, school performance and quality of life. Mouse model studies showed that the learning deficits are due to increased RAS signalling. This increased signalling, as well as the synaptic plasticity deficits and learning deficits of these mice can be rescued by statin-mediated inhibition of HMG-CoA reductase. Statins are the most commonly prescribed drugs worldwide, and have a very favourable safety profile. Hence, this provides us with a unique opportunity to assess the effect of a targeted treatment on cognitive performance and behaviour in patients. We have previously conducted a 12-week pilot study in children with NF1 (ISRCTN14965707). Although we did not find a significant improvement compared to placebo in the primary outcome measures, we did obtain preliminary data that statins improved some measures. We consider it certainly possible that there is indeed a significant therapeutic benefit, but that the overall treatment was too short to detect this in the pilot trial. In addition, the clinical outcome measures may have been unable to reflect a potential benefit in daily life functioning. Furthermore, we found indications that ADHD-medication may mask the beneficial effect of Simvastatin on attention, thus patients that are treated with ADHD-medication will be excluded from this trial. This trial will aim to clarify the effect of long-term Simvastatin treatment on cognition and daily life functioning in children with NF1.

Doel van het onderzoek

Simvastatin can have a positive effect on cognitive function and daily life functioning in children with Neurofibromatosis 1.

Onderzoeksopzet

Baseline (T=0 months) and end of treatment (T=12 months).

Onderzoeksproduct en/of interventie

Participants are treated for 12 months with Simvastatin (10 mg/d in month 1, 20 mg/d in month 2, and then 20 mg/d for participants 8-12 years old or 40 mg/d for participants 13-16 years old) or equivalent placebo once a day in the morning).

Contactpersonen

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Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

1. Age 8 to 16 years;

2. NF1 diagnosis with genetic confirmation;

3. Oral and written informed consent from parents and participants aged older than 12 years.

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

1. Segmental NF1;

2. Pathology of the CNS (other than asymptomatic gliomas);

3. Deafness;

4. Severely impaired vision;

5. Use of ADHD-medication;

6. Use of anti-epileptic drugs;

7. Use of anti-psychotic medication;

8. Use of Simvastatin;

9. Insufficient comprehension or production of the Dutch language;

10. An IQ below 48.

Onderzoeksopzet

Opzet

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

Deelname

Nederland

Status:	Werving gestopt
(Verwachte) startdatum:	01-03-2010
Aantal proefpersonen:	84
Type:	Werkelijke startdatum

Voornemen beschikbaar stellen Individuele Patiënten Data (IPD)

Wordt de data na het onderzoek gedeeld: Nog niet bepaald

Ethische beoordeling

Positief advies

Datum:	30-12-2009
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	NL2033
NTR-old	NTR2150
CCMO	NL27196.000.09
ISRCTN	ISRCTN wordt niet meer aangevraagd.

Resultaten

Samenvatting resultaten
N/A