

The effect of simvastatin on the cognitive deficits of children with Neurofibromatosis I (NF1): a randomized, double-blind placebo-controlled study.

Gepubliceerd: 28-11-2005 Laatste bijgewerkt: 13-12-2022

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

Samenvatting

ID

NL-OMON21351

Bron

NTR

Verkorte titel

NF1 simvastatin trial

Aandoening

Neurofibromatosis type 1 is the most common single gene disease causing learning disabilities in humans. Children with NF1 commonly have cognitive dysfunctions like learning and attention deficits as well as impaired motor coordination. Half of the children seen at the multidisciplinary NF1 outpatient clinic of the Sophia Children's Hospital attends special education.

Ondersteuning

Primaire sponsor: Erasmus MC afd. Neurowetenschappen, Dr. Y. Elgersma

Overige ondersteuning: Sophia Kinderziekenhuis Fonds

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

1. Performance on neuropsychological tests on visuospatial memory and attention after 1 and 3 months (Rey Complex Figure test (recall), Bourdon Vos Test);

2. Performance on neurofysiological tests on adaptation of movements after 1 and 3 months (saccade-adaptation test, adaptation of eye-hand coordination);

3. Measurement of the size, number, localization and spectra of UBO's (unidentified bright objects, hyperintensities on T2 weighed MRI), on T2 MRI and 3D CSI 1H-MRS after 3 months.

Toelichting onderzoek

Achtergrond van het onderzoek

Recent research has shown that the cognitive phenotype of NF1 +/- mice can be reversed by the administration of statins. Because the majority of children with NF1 suffer from learning disabilities, statins could potentially make a large difference in the morbidity associated with NF1.

In a double-blind, randomized placebo-controlled trial, 60 children with NF1 are treated with simvastatin or placebo for three months. The effect of simvastatin treatment will be evaluated using neuropsychologic, neurophysiologic and radiologic parameters.

Doel van het onderzoek

Statin-treatment has been shown to normalize the learning- and attention deficits in NF1 +/- mice by decreasing Ras activity. The fact that statins are effective in NF1 mice, combined with their very good safety profile, makes them an ideal candidate drug to treat cognitive impairments associated with NF1 in human patients.

Onderzoeksopzet

N/A

Onderzoeksproduct en/of interventie

Simvastatin (10 mg/d for month 1, 20 mg/d month 2, 20 mg/d month 3 for children 8-12 years old or 40 mg/d month 3 for children 12-16 years old) or placebo once a day.

Contactpersonen

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Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

Children aged between 8 and 16 years, NF1 diagnosis according to the criteria of the National Institutes of Health, visiting the multidisciplinary NF1-outpatient clinic at the Erasmus MC – Sophia Children's Hospital; informed consent .

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

Pathology of the CNS (hydrocephalus, epilepsy, radiotherapy, neurosurgery, etc.), deafness and/or severely impaired vision, use of anti-epileptics and/or neuroleptics.

Additional exclusion-criteria (under METC review):

a. Insufficient cognitive abilities to obtain a reliable score on a verbal IQ test (WISC-RN);

- b. Contra-indications for simvastatin-treatment;
- c. Planned hospitalization within three months after planned date of inclusion.

Onderzoeksopzet

Opzet

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

Deelname

Nederland	
Status:	Werving gestopt
(Verwachte) startdatum:	20-01-2006
Aantal proefpersonen:	60
Type:	Werkelijke startdatum

Ethische beoordeling

Positief advies	
Datum:	28-11-2005
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	NL499
NTR-old	NTR542
Ander register	: N/A
ISRCTN	ISRCTN14965707

Resultaten

Samenvatting resultaten

<http://www.ncbi.nlm.nih.gov/pubmed?term=18632543>