Methylphenidate for ADHD in Smith-Magenis syndrome

Gepubliceerd: 09-12-2020 Laatst bijgewerkt: 13-12-2022

We hypothesize that methylphenidate improves ADHD manifestations in children and adults with SMS.

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

Samenvatting

ID

NL-OMON20117

Bron
Nationaal Trial Register

Verkorte titel
Methylphenidate for ADHD in Smith-Magenis syndrome

Aandoening

Smith-Magenis syndrome; ADHD

Ondersteuning

Primaire sponsor: Amsterdam UMC
Overige ondersteuning: 's Heeren Loo

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

The primary outcome measure includes the Strengths and Difficulties Questionnaire (SDQ) (subscale hyperactivity/inattention).

Toelichting onderzoek

Achtergrond van het onderzoek

Smith-Magenis syndrome (SMS) is a rare genetic neurodevelopmental disorder characterized by intellectual disability and severe behavioural and sleep disturbances. Common behavioural manifestations in SMS include hyperactivity, attention deficits, impulsivity and emotion dysregulation. As a result, many patients are diagnosed with attention-deficit/hyperactivity disorder (ADHD), a condition often treated with methylphenidate. However, the effectiveness of methylphenidate for ADHD symptoms in SMS is unknown and may be different for SMS than observed in the general population, due to different aetiology and presentation of symptoms. The current N-of-1 study is a series of double-blind randomized and placebo-controlled multiple crossover trials within six participants who are diagnosed with SMS and ADHD. Each N-of-1 trial consists of a baseline period, a dose titration phase, three cycles of alternating two 7-days intervention periods each followed by a 7-days washout period, and a follow-up measurement. The goal of the current project is to investigate the efficacy of methylphenidate for ADHD symptoms in SMS. We aim to aggregate data from a series of N-of-1 trials to investigate the effectiveness of methylphenidate for ADHD in SMS.

Doel van het onderzoek

We hypothesize that methylphenidate improves ADHD manifestations in children and adults with SMS.

Onderzoeksopzet

The primary outcome measure (SDQ subscale hyperactivity/inattention) will be measured daily at the end of the day, except for the dose titration phase. The shortened version of the EDI as one of the secondary outcome measures will also be measured daily. Both questionnaires will be filled out digitally by patients and/or primary caregivers using the app m-Path, Castor Electronic Data Capture (EDC) or by using paper forms. At the end of each interventional period, patients and/or primary caregivers and supervisors of daily activities if present will be called to evaluate GAS goals, to go through the Personal Questionnaire, and to discuss possible side effects by using a standardized checklist of side effects of methylphenidate. Three months after terminating the third cycle of the N-of-1 trial, an optional contact moment will take place for a follow-up measurement in which the questionnaires will be filled out and the goals and items of GAS and PQ will be discussed again.

Onderzoeksproduct en/of interventie

Participants will receive twice daily methylphenidate or placebo.

Contactpersonen

Publiek

Amsterdam UMC / 's Heeren Loo
Agnies van Eeghen

+31 20 566 1415

Wetenschappelijk

Amsterdam UMC / 's Heeren Loo
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Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

- A diagnosis of SMS confirmed with standard genetic testing.
- Meet DSM-5 criteria for ADHD, and diagnosed with ADHD by a multidisciplinary team consisting of an intellectual disability physician, a psychologist, and a psychiatrist.
- Minimum age of six years old.
- Presence of a patient’s caregiver for proxy-reports.

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

- Presence of a contra-indication for treatment with methylphenidate.
- Planned general anaesthesia during the trial.
- Pregnancy.
- Breastfeeding females.
- Females of childbearing potential must be willing to use an effective method of contraception from the time consent is signed until 6 weeks after treatment discontinuation and inform if pregnancy occurs.
- During treatment with non-selective, irreversible monoamine oxidase (MAO) inhibitors, or within a minimum of 14 days of discontinuing those drugs.
- Current treatment with serotonergic drugs, acetazolamide, thiazide-diuretic and sodium

bicarbonate, sympathicomimetics, tricyclic antidepressants, or anti-psychotics.
- Current substance or alcohol abuse.
- Unable to swallow capsules.

Onderzoeksopzet

Opzet

Type:	Interventie onderzoek
Onderzoeksmodel:	Cross-over
Toewijzing:	N.v.t. / één studie arm
Blindering:	Dubbelblind
Controle:	Placebo

Deelname

Nederland

Status:	Werving nog niet gestart
(Verwachte) startdatum:	04-01-2021
Aantal proefpersonen:	6
Type:	Verwachte startdatum

Voornemen beschikbaar stellen Individuele Patiënten Data (IPD)

Wordt de data na het onderzoek gedeeld: Nee

Ethische beoordeling

Positief advies

Datum:	09-12-2020
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	NL9125
Ander register	METC AMC : METC2020_100

Resultaten