Methylphenidate for ADHD in Smith-Magenis syndrome

No registrations found.

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

Summary

ID

NL-OMON20117

Source
Nationaal Trial Register

Brief title
Methylphenidate for ADHD in Smith-Magenis syndrome

Health condition

Smith-Magenis syndrome; ADHD

Sponsors and support

Primary sponsor: Amsterdam UMC
Source(s) of monetary or material Support: 's Heeren Loo

Intervention

Outcome measures

Primary outcome

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

Secondary outcome

Secondary outcome measures are the shortened version of the Emotion Dysregulation Inventory (EDI) reactivity index, Goal Attainment Scaling (GAS), the personal questionnaire (PQ), and adverse effects.

Study description

Background summary

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.

Study objective

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

Study design

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.

Intervention

Participants will receive twice daily methylphenidate or placebo.

Contacts

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Eligibility criteria

Inclusion criteria

- 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.

Exclusion criteria

- 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.

Study design

Design

Study type:	Interventional
Intervention model:	Crossover
Allocation:	N/A: single arm study
Masking:	Double blinded (masking used)
Control:	Placebo

Recruitment

NL

Recruitment status:	Pending
Start date (anticipated):	04-01-2021
Enrollment:	6
Type:	Anticipated

IPD sharing statement

Plan to share IPD: No

Ethics review

Positive opinion

Date:	09-12-2020
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	NL9125
Other	METC AMC : METC2020_100

Study results