Bumetanide to Ameliorate Tuberous Sclerosis Complex Hyperexcitable Behaviors

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We propose to conduct a pilot study to confirm the efficacy of bumetanide as add-on treatment to reduce irritability and other behavioral symptoms typical in children and adolescents with TSC, with and without mental retardation.

Ethische beoordeling	Niet van toepassing
Status	Werving nog niet gestart
Type aandoening	-
Onderzoekstype	Interventie onderzoek

Samenvatting

ID

NL-OMON26926

Bron
Nationaal Trial Register

Verkorte titel
BATSCH

Aandoening

Tuberous Sclerosis Complex (TSC)

Ondersteuning

Primaire sponsor: University Medical Center Utrecht
Overige ondersteuning: Michelle Foundation

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

Aberrant Behavior Checklist (ABC) irritability subscale

Toelichting onderzoek

Achtergrond van het onderzoek

Pilot open label study confirming the applicability and efficacy of bumetanide as add-on treatment for behavioural problems and seizures in 2x10 patients with TSC and a history of behavioural problems between 8 and 15 years of age. After the baseline screening, included subjects receive bumetanide for 91 days, followed by a 28 days discontinuation phase, after which endpoint monitoring will be repeated to evaluate the persistence of treatment effect. Test battery assessment will take place at baseline, at the end of bumetanide treatment (Day 91) and after the discontinuation phase (Day 119). The primary endpoint of the study is the ABC-I subscale at Day 91. In addition, a number of cognitive and neurophysiological markers as well as measures of seizure control will be tested.

Doel van het onderzoek

We propose to conduct a pilot study to confirm the efficacy of bumetanide as add-on treatment to reduce irritability and other behavioral symptoms typical in children and adolescents with TSC, with and without mental retardation.

Onderzoeksopzet

• Pre-treatment and screening

o Screening for eligibility

o Baseline measurements

• Treatment (D0 to D91)

o Blood analysis at D4, D7, D14, D28, D56

o End of treatment outcome measurements D91

• Washout (D91 to D119)

o End of washout outcome measurements D119

Onderzoeksproduct en/of interventie

The investigational product will consist of bumetanide, which will be provided for 91 days as an add-on treatment, supplementary to the regular use of AEDs or other (allowed) co-

medications. Dose reductions to manage side effects will be allowed at any time. Based upon the expected chance of frequent mild to moderate hypokalemia, all subjects will receive standard potassium supplementation during the 91 days of treatment. The treatment period will be followed by a wash-out period to evaluate
return of symptomatology and reversibility of treatment effect.

Contactpersonen

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Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

General inclusion criteria:

1. Males and females aged 8-15 years

2. Definite diagnosis of TSC: either meeting criteria for clinical definite TSC, or a mutation identified in the TSC1 or TSC2 gene

3. History of behavioral problems

4. Written informed consent

Specific inclusion criteria Group 1:

- No intellectual disability (TIQ>70)

Specific inclusion criteria Group 2:

- Intellectual disability (TIQ≤70)

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

1. Inability to comply with the protocol-specified procedures

2. Presence of a severe medical or genetic disorder other than related to TSC or epilepsy

3. Serious, unstable illnesses

4. Renal insufficiency, congenital or acquired renal disease with decreased concentration capacity and liver insufficiency interfering with excretion or metabolism of bumetanide

5. Behavioral treatment

6. Treatment with psychoactive medications, including antipsychotics, antidepressants, anxiolytic drugs, psychostimulant drugs, except melatonin. If clinically feasible, then it is allowed to stop psychoactive medication to allow enrolment in the study after a 4 week washout
period of their psychoactive medication. Notably, an exception is treatment with AEDs, which are allowed albeit on a stable regime in terms of types of AEDs and dosage from 2 months prior to the study to the end of the study

7. Treatment with NSAIDS, aminoglycosides, digitals, antihypertensive agents, indomethacin, probenecid, acetazolamide, Lithium, other diuretics, drugs known to have a nephrotoxic potential

8. Documented history of hypersensitivity reaction to sulfonamide derivatives

9. Body weight < 30 kg

Onderzoeksopzet

Opzet

Type:	Interventie onderzoek
Onderzoeksmodel:	Parallel
Toewijzing:	N.v.t. / één studie arm
Blindering:	Open / niet geblindeerd
Controle:	N.v.t. / onbekend

Deelname

Nederland

Status:	Werving nog niet gestart
(Verwachte) startdatum:	01-03-2017
Aantal proefpersonen:	20
Type:	Verwachte startdatum

Ethische beoordeling

Niet van toepassing

Soort:	Niet van toepassing

Registraties

Opgevolgd door onderstaande (mogelijk meer actuele) registratie

ID: 45855
Bron: ToetsingOnline
Titel:

Andere (mogelijk minder actuele) registraties in dit register

Geen registraties gevonden.

In overige registers

Register	ID
NTR-new	NL5962
NTR-old	NTR6328
CCMO	NL58183.041.16
OMON	NL-OMON45855

Resultaten