A B2-agonist as a CFTR activator in CF Part II

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Long term use (8 weeks) of oral B2-agonists increases CFTR function and improves disease parameters in patients with CF with a residual CFTR function.

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

Samenvatting

ID

NL-OMON24538

Bron
NTR

Verkorte titel
ABBA-2

Aandoening

Cystic Fibrosis

Ondersteuning

Primaire sponsor: University Medical Center Utrecht
Overige ondersteuning: ZonMw, NCFS

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

Pulmonary function (spirometry and airway resistance measured with the bodybox and Rint)

Toelichting onderzoek

Achtergrond van het onderzoek

Objective: Primary objective of this study is to evaluate the clinical effect of a long term treatment (8 weeks) with oral B2-agonists in CF patients with residual CFTR function, especially on lung function (spirometry and airway resistance).

Secondary objectives are to:

1. Evaluate the correlations between individual B2-agonist-induced CFTR function in vitro (organoid-based measurements) and the long term clinical treatment effect (eg. lung function and airway resistance).

2. Assess the effect of the salbutamol concentration in the blood on CFTR function in the background of patient specific parameters. We will do this by examining the CFTR-stimulating potential of the patients' blood in vitro (in the organoid model).

Study design: A multicentre open label intervention study.

Study population: Adults with Cystic Fibrosis with a compound/A455E or compound/ R117H mutation and proven residual CFTR function in vitro. We aim to include 20 patients.

Intervention: After baseline measurements all patients will receive oral Salbutamol during 8 weeks.

Main study parameters/endpoints: Pulmonary function (spirometry and airway resistance measured with the bodybox and Rint)

• Before and after treatment with salbutamol

Nature and extent of the burden and risks associated with participation, benefit and group relatedness: Patients participating in this study will be treated at home and will visit the hospital for two study visits. Salbutamol has been used in clinical practice for over decades in patients with asthma and no serious side effects have been reported. Therefore we do not expect serious problems or side effects during this study. We expect to see a clear clinical effect of long term treatment with oral salbutamol. When our hypothesis is confirmed, this is a major benefit for the patient. Not only during the study period but also for their further treatment. When this study confirms our hypothesis that organoids can predict clinical responders, this is a major benefit not only for the CF population but also for the individual patient. With the use of organoids we will then be able to generate optimal treatment strategies for individuals based on (combinations of) current and future drugs with only limited patient discomfort.

Doel van het onderzoek

Long term use (8 weeks) of oral B2-agonists increases CFTR function and improves disease parameters in patients with CF with a residual CFTR function.

Onderzoeksopzet

Before and after treatment with oral salbutamol

Onderzoeksproduct en/of interventie

4dd4mg salbutamol oral

Contactpersonen

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Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

- CFTR genotype compound/A455E or compound/R117H

- Already had a rectal biopsy to produce an organoid

- Males and females, aged 18 years or older on the date of informed consent

- Signed informed consent form (ICF)

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

- Severe acute exacerbation or pulmonary infection during last four weeks (needing intravenous treatment and/or systemic corticosteroids)

- Known cardiovascular medical history like cardiac failure, arrhythmias, ischemic cardiac disease, long QT interval syndrome and hypertension

- Known hyperthyroidism, thyrotoxicosis, galactose intolerance, lactase deficiency or glucose-galactose malabsorption

- Haemoglobin A1C (HBA1C) > 45 mmol/mol

- Use of oral B2-agonist one week prior to the start of the study (V1)

- Use of: heart glycoside, high dose sympathomimetics, theophylline, thiazide diuretics or non-selective beta-blockers

- Pregnancy or breastfeeding

- Participation in another drug-investigating clinical study at the start

- Inability to follow instructions of the investigator

Onderzoeksopzet

Opzet

Type:	Interventie onderzoek
Onderzoeksmodel:	Factorieel
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:	01-07-2015
Aantal proefpersonen:	20
Type:	Verwachte startdatum

Ethische beoordeling

Niet van toepassing

Soort:	Niet van toepassing

Registraties

Opgevolgd door onderstaande (mogelijk meer actuele) registratie

ID: 43663
Bron: ToetsingOnline
Titel:

Andere (mogelijk minder actuele) registraties in dit register

Geen registraties gevonden.

In overige registers

Register	ID
NTR-new	NL5108
NTR-old	NTR5240
CCMO	NL53059.041.15
OMON	NL-OMON43663

Resultaten

Samenvatting resultaten
Not applicable