The effect of Ivacaftor in CF patients with a class III mutation

No registrations found.

Ethical review	Not applicable
Status	Recruitment stopped
Health condition type	-
Study type	Observational non invasive

Summary

ID

NL-OMON26309

Source
Nationaal Trial Register

Brief title
TICTAC-2

Health condition

Cystic Fibrosis (CF)

Sponsors and support

Primary sponsor: University Medical Center Utrecht (UMCU)
Source(s) of monetary or material Support: 1. ZON-MW, The Netherlands Organization for Health Research and Development
2. The NCFS, patient association of CF patients, The Netherlands.

Intervention

Outcome measures

Primary outcome

Sweat chloride concentration (SCC) before and after treatment with Ivacaftor.

Secondary outcome

• Pulmonary function (%FEV1) and airway resistance (Rint and bodybox).

• BMI (=weight (in Kg)/Length2 (in cm));

• Quality of life (measured with CFQ-questionnaire)

• Bile salt measurements in plasma and the feces.

• Elastase measurements in the feces

• Correlation between individual Ivacaftor induced CFTR function in vitro (organoid-based measurements) and the in vivo treatment effect;

• The CFTR stimulating ability of the concentration of Ivacaftor in the patient's blood samples, examined by in vitro testing (in the organoid model). We will also determine the plasma levels of Ivacaftor.

Study description

Background summary

In the Netherlands, the CFTR potentiator-drug Ivacaftor will be approved for the treatment of CF patients with a (class III) gating mutation, probably by the end of 2014. Primary objective is to objectively investigate the therapeutic potential of Ivacaftor in Dutch CF patients carrying the S1251N gating mutation. Children, adolescents and adults with Cystic Fibrosis who are indicated to start with Ivacaftor (6 years or older and have a compound/S1251N class III gating mutation) can participate in this multi center observational study. Main study parameter will be sweat chloride concentration before and after treatment with Ivacaftor.

Study objective

Treatment with Ivacaftor can lead to a therapeutic level of restoration of the CFTR protein channel activity in patients with a class III, S1251N gating mutation. This level of restoration is comparable to a combination of the natural food components curcumin and genistein. Measurements in vitro (in organoids) can predict the individual treatment efficacy of Ivacaftor.

Study design

Before and after treatment with Ivacaftor.

Intervention

This study will be an observational study. We will observate the intervention of starting with a

treatment with Ivacaftor.

Contacts

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Eligibility criteria

Inclusion criteria

• CFTR genotype compound/ S1251N

• Already had a rectal biopsy to produce an organoid

• Start a treatment with Ivacaftor

• Male and female patients, aged 6 years or older on the date of informed consent or;

• Signed informed consent form (IC).

Exclusion criteria

• Use of curcumin and or genistein at start or within two weeks prior to start of the study;

• Inability to follow instructions of the investigator.

Study design

Design

Study type:	Observational non invasive
Intervention model:	Factorial
Allocation:	Non controlled trial
Masking:	Open (masking not used)
Control:	N/A , unknown

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	01-12-2014
Enrollment:	10
Type:	Actual

Ethics review

Not applicable

Application type:	Not applicable

Study registrations

Followed up by the following (possibly more current) registration

ID: 41740
Bron: ToetsingOnline
Titel:

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
NTR-new	NL4728
NTR-old	NTR4873
CCMO	NL50276.041.14
OMON	NL-OMON41740

Study results