HIT-CF Organoid Study: Stratifying Cystic Fibrosis Patients Based on Intestinal Organoid Response To Different CFTR-modulators

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We can identify the predicted best clinical responders and predicted low-responders (based on amount of organoid swelling) to new CFTR-modulators out of 500 unique patient-specific intestinal organoids screened for in-vitro drug efficacy.

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

Samenvatting

ID

NL-OMON24027

Bron
NTR

Aandoening

Cystic Fibrosis CF

Ondersteuning

Primaire sponsor: University Medical Center Utrecht
Overige ondersteuning: European Union

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

Intestinal organoid response of 500 subjects to three drug products of

different pharmaceutical companies, ranked by best response per drug product.

Toelichting onderzoek

Achtergrond van het onderzoek

The organoid study is part of the HIT-CF project. The aim of the Hit-CF project is to develop ‘personalized treatments’ for patients with Cystic Fibrosis (CF) and uncommon genetic profiles throughout Europe. It consist of 2 studies which will run after one another. In the first part, the organoid study, we will identify subjects who could potentially benefit from specific drugs. We will do this by using mini-intestines and test drugs on those mini-intestines. The second part of the project is a clinical trial. In that trial we will investigate whether or not the subject really benefits from the drugs that are identified in the organoid study.

This Organoid Study is the first part of the HIT-CF project. The purpose of this study is to investigate if CF-patients with rare mutations can benefit from a treatment with a CFTR-modulating drug. We will assess this by taking a small tissue sample (rectal biopsy) from 500 patients across Europe. In the laboratory, we will generate mini-intestines (called intestinal organoids) from the tissue. Using the organoids we have made we can test which drugs can repair the disturbed salt transport caused by CF.

We will test drugs on organoids of 500 CF-patients across Europe. The 100 patients whose organoids show the best response to the drugs will be asked to participate in a new clinical trial testing that specific drug.

Doel van het onderzoek

We can identify the predicted best clinical responders and predicted low-responders (based on amount of organoid swelling) to new CFTR-modulators out of 500 unique patient-specific intestinal organoids screened for in-vitro drug efficacy.

Onderzoeksopzet

- Biopsy collection

Onderzoeksproduct en/of interventie

Intestinal (rectal) biopsy by forceps or rectal suction device

Contactpersonen

Publiek

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Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

–Male or female with confirmed diagnosis of CF

–Adult age on the date of informed consent

–An increased sweat chloride concentration (above 60 mmol/L) by pilocarpine iontophoresis (documented in patient records)

–Subject has signed and dated an ICF

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

–Subject has at least one of the following CFTR-mutations:

•F508del, G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, S549R, R117H, A455E, 3849+10kbC>T

–Subject has a combination of any two of the following mutations:

•G542X, R553X, W1282X, R1162X, E60X, Q493X, 1717-1G>A, 621+1G>T, 3120+1G>A, 1898+1G->A, CFTRdele2,3 and 2183AA->G

–History of any comorbidity that might pose an additional risk in administering study drug to subject

–History of Lung Transplantation

Onderzoeksopzet

Opzet

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

Deelname

Nederland

Status:	Werving gestart
(Verwachte) startdatum:	01-09-2018
Aantal proefpersonen:	500
Type:	Verwachte startdatum

Ethische beoordeling

Positief advies

Datum:	02-10-2018

Soort: Eerste indiening

Registraties

Opgevolgd door onderstaande (mogelijk meer actuele) registratie

ID: 48963
Bron: ToetsingOnline
Titel:

Andere (mogelijk minder actuele) registraties in dit register

Geen registraties gevonden.

In overige registers

Register	ID
NTR-new	NL7304
NTR-old	NTR7520
CCMO	NL65123.041.18
OMON	NL-OMON48963

Resultaten