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

No registrations found.

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

Summary

ID

NL-OMON24027

Source
NTR

Health condition

Cystic Fibrosis CF

Sponsors and support

Primary sponsor: University Medical Center Utrecht
Source(s) of monetary or material Support: European Union

Intervention

Outcome measures

Primary outcome

Intestinal organoid response of 500 subjects to three drug products of different pharmaceutical companies, ranked by best response per drug product.

Secondary outcome

Not applicable

Study description

Background summary

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.

Study objective

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.

Study design

- Biopsy collection

Intervention

Intestinal (rectal) biopsy by forceps or rectal suction device

Contacts

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Eligibility criteria

Inclusion criteria

–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

Exclusion criteria

–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

Study design

Design

Study type:	Interventional
Intervention model:	Other
Allocation:	Non controlled trial
Masking:	Open (masking not used)
Control:	N/A , unknown

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	01-09-2018
Enrollment:	500
Type:	Anticipated

Ethics review

Positive opinion

Date:	02-10-2018
Application type:	First submission

Study registrations

Followed up by the following (possibly more current) registration

ID: 48963
Bron: ToetsingOnline
Titel:

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

No registrations found.

In other registers

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

Study results