

Newsletter HIT-CF Europe

March 2020



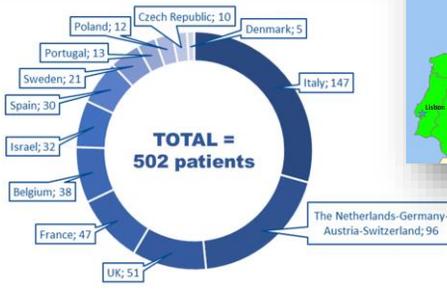
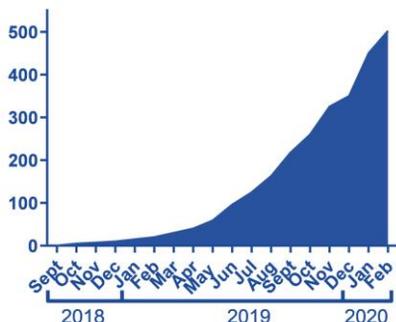
The HIT-CF Europe project aims to provide new treatment options to people with cystic fibrosis (CF) and ultra-rare genetic profiles. The project will evaluate the efficacy and safety of drug candidates provided by Eloxx Pharmaceuticals and Proteostasis Therapeutics, Inc. (PTI) in patients selected through preliminary tests in the laboratory on their mini-intestines – also called organoids.

Phases 1 & 2 completed with the recruitment of 502 patients throughout Europe!

47 participating CF centres from 16 countries in collaboration with the ECFS-CTN contributed to this great success!



Step 1:
Collection of
biopsies



Step 2:
Growing the
organoids

Rectal biopsies were collected from 502 CF patients and sent to Hubrecht Organoid Technology (HUB, The Netherlands) to be processed into organoids. Based on the donor's genotype, PTI compounds, Eloxx compounds or both will be tested on the organoids whose response to the drug will be measured.

What are the next steps?

Step 3:
Drug
screening on
organoids

Organoids are tested in 4 labs: the HUB, Leuven (Belgium), Utrecht (The Netherlands) and Lisbon (Portugal) Universities. PTI compounds will be tested until June; screening of Eloxx compounds is due to start in the winter 2020-2021.

Step 4:
Selection for
clinical trials

Selection of at least 50 participants for the PTI study covering a range of organoid responses.

Step 5:
Clinical
Trials

Double-blind, placebo-controlled, crossover protocol, currently under review by CTN. The PTI study is planned to start 2nd half of 2020.

Neither patients nor doctors will know who is a high, intermediate or low responder and which compound each person takes, until the end of the trial.

Timeline may have to be adjusted depending on the evolution and consequences of the Covid-19 crisis.

January
2020

March
2020

On-
going

Autumn
2020

Winter
2020



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To learn more about the HIT-CF project, visit www.hitcf.org or send an e-mail to HITCF@umcutrecht.nl