

Newsletter HIT-CF Europe

August 2020



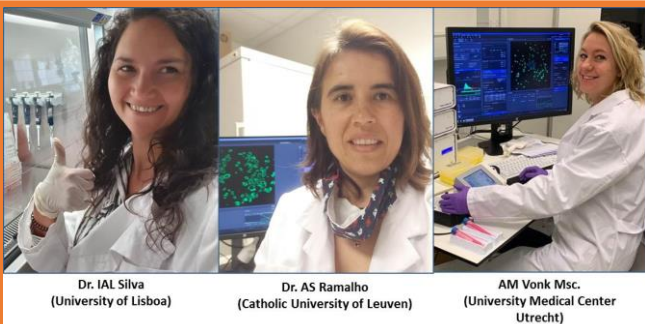
The HIT-CF Europe project aims to provide new treatment options to people with cystic fibrosis (CF) and ultra-rare genetic profiles. The project will evaluate the efficacy and safety of drug candidates provided by Eloxx Pharmaceuticals and Proteostasis Therapeutics, Inc. (PTI) in patients selected through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

Between September 2018 and March 2020 502 patients throughout Europe were recruited and their organoids were grown!



Due to the Covid-19 pandemic and the precautionary measures to stop the coronavirus from spreading, drug screening was put on hold between mid-March and the end of May

What is the current situation?



Since the end of May, drug screening gradually resumed. Based on the donor's genotype, PTI compounds, Eloxx compounds or both are being tested on the organoids whose response to the drug will be measured.

Screening of the PTI compounds should be finished by the end of 2020, upon which at least 50 participants covering a range of organoid responses will be selected for the PTI study. Thereafter Eloxx compounds will be tested.

Check out the participant [FAQ's](#) on the [HIT-CF website](#), or send an e-mail to HITCF@umcutrecht.nl

Will I receive more information about which study drugs were tested on my organoid in the lab and how it responded?

We will send you feedback on the organoid testing results. However, many of the drugs tested here are not yet available for clinical use. Therefore, depending on their development status, we will not always be able to share with you the details of your organoid response per drug. Please also bear in mind that you will receive this information only after the clinical trials have been completed.

At the end of 2020 at the earliest, the PTI clinical trial will be initiated:

double-blind, placebo-controlled, crossover protocol, approved by the CTN. The trial is anticipated to be executed in 14 countries (see map), but this will depend on where study participants will be located.

Eloxx: After summer, the study protocol of the Eloxx study will be submitted to the CTN.

