Newsletter HIT-CF Europe

September 2019

The goal of HIT-CF Europe is to evaluate the efficacy and safety of three drug candidates in patients with CF and rare mutations, pre-selected by their mini-intestines in the laboratory. The first part of the HIT-CF project aims at enrolling 500 people with CF with two rare CFTR-mutations from across Europe. A biopsy from these people will be taken and organoids will be generated. Secondly, based on the reaction in the organoids, a smaller group of participants will be assigned to studies (clinical trials) with one of the drug candidates.











We take some tissue from the intestine

This tissue is cultured in the lab

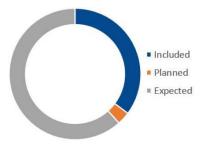
The result is an organoid

On this organoid we test medicines

And we bring the correct medicines to the patients

Stockholn

Already 176 patients enrolled in HIT-CF!



All participating CTN centres (marked with blue stars on the map to the right – 35 in total) are now fully operational and (ready to start) including patients. Up till now, 176 people with CF are enrolled in the HIT-CF project. Meanwhile, our lab technicians at Hubrecht Organoid Technology (HUB) are working hard to culture all biopsies

into organoids, and preparations have begun for the clinical trials which will kick-off in 2020. Stay tuned with the latest updates on the timelines of the clinical trials on our <u>website</u> and <u>Facebook</u> page.

Organoid technology in the media

Organoids show a high correlation between drug effects in the lab and the clinic, enabling us to identify responders to CFTR modulators. Read more about it in this exciting article published by the Utrecht group:

Cell Reports Rectal Organoids Enable Personalized Treatment of Cystic Fibrosis

We are hitting CF where it hurts!

An interesting storytelling article on HIT-CF was published in the <u>year report of the ECFS Clinical Trials Network</u>, including more background information on the project, and interviews with a scientist and doctor working on the project. You can download the article <u>here</u>.



Finally, we would like to use this opportunity to say a special thank you to the people with CF and their caregivers. Thank you for your interest in our project, your critical questions, your enthusiasm, your willingness to participate, your time and your trust! Also thank you to the patient organisations for your help with making people aware of this project and spreading information. Of course we also would like to thank the doctors and nurses in the participating centres for your efforts on recruiting and including patients and making this project a success!

Do you want to participate, or do you want more info about the HIT-CF project? Visit <u>the HIT-CF website</u> or send an e-mail to <u>HITCF@umcutrecht.nl</u>





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