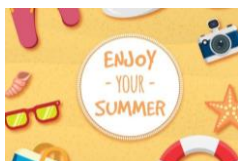


Newsletter HIT-CF Europe

June 2022



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 collaborating pharmaceutical companies in patients selected through preliminary tests in the laboratory on their mini-intestines – also called organoids.



The HIT-CF consortium wishes everyone a very nice summer. But before we send you off, we want to make sure you receive the latest updates from the project. During summer, keep an eye on CF Europe's [Facebook](#) and the [HIT-CF website](#). We will be dropping a new compilation video in which several people who play a major role in the further course of HIT-CF and CHOICES will introduce themselves and answer some questions. Stay tuned!



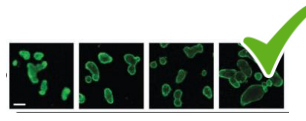
Ultra-rare mutations



Rectal biopsy



Intestinal organoids



Drug testing/ selecting responders



Clinical trial(s)

How far are we with the preparations of CHOICES? When will CHOICES start?

The first inclusions of pre-selected participants (see our [February newsletter](#)) are expected in **the last quarter of 2022**. Currently, the team is busy with the “fill and finish” process, meaning that the drugs' raw materials are processed into pills and packed in bottles. It is very important that this is done in a careful way, to ensure that the drug is safe for the study participants to take.

Another important step before CHOICES can finally kick-off, is obtaining permission from the national competent authorities to run the trial. As of 31 January 2022, the procedure of applying for permission is harmonized throughout Europe through the **Clinical Trials Information System** or **CTIS**. The big advantage of this is that one procedure leads to one decision applicable for all countries in which you want to run the trial, instead of having to submit a clinical trial application in every country separately. This increases efficiency and allows for a trial to start at the same time in different countries. You can find more information on CTIS [here](#). Although this centralized approach is welcomed by the HIT-CF team, it also causes some delays, as is always the case when a new system is implemented. Additional documents may be required, or in a different format, so please bear with us a little longer while our team is working as fast as they can to get permission to kick-off CHOICES.

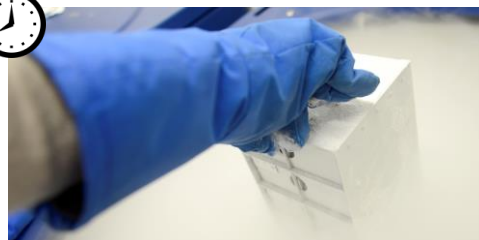
Progress in obtaining EMA's Qualification Opinion



Obtaining EMA's Qualification Opinion (QO) is a major step in the further development of the organoid assay as a predictor of clinical response. Getting EMA's “stamp of approval” would allow organoids to be used to guide personalized medicine, paving the way for people with CF caused by rare mutations to have access to innovative, possibly life-saving drugs. The HIT-CF team submitted the application to obtain the QO in March of this year. In May, we already received a first, very positive reaction from EMA, and they requested additional data which we will provide. Follow-up discussions are planned for September.

Biobanking in order to preserve the unique collection of rare mutation organoids

In the first phase of HIT-CF, no less than 502 organoids from people with CF with rare mutations were obtained. Strictly spoken, these would have to be destroyed after the end of the HIT-CF project. But as this collection has such potential to test future innovative therapies, the HIT-CF team is working hard and in collaboration with all stakeholders to preserve the organoids. For your organoid to be kept, you will have to sign a new informed consent. Your CF doctor will give you all necessary information in due course.



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

