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.

Primary screen of Eloxx compound on organoids finished!

We are very happy to announce that our collaborators in the labs finalised the primary screening of the organoids with the Eloxx compound. Even at this early stage, it is clear that there are people with CF caused by rare stop mutations who respond to the Eloxx drug, which is great news. These results will now be validated through a secondary screen after which 26 people will be selected to participate in a clinical trial with the Eloxx drug. We anticipate to start this trial by the beginning of 2022.

CHOICES delayed until the end of this year

As we communicated in earlier newsletters, the merger of PTI with Yumanity Therapeutics is causing severe delays in the kick-off of CHOICES, the clinical trial in which PTI compounds will be given to 52 people with CF based on their organoid response. The HIT-CF team is currently looking into the best way to get the PTI drugs to the selected patients, but it is certain that this will not be before the end of this year. The HIT-CF team remains fully committed to carry out CHOICES and make HIT-CF a success for patients with rare mutations.

Briefing package to obtain EMA Qualification Opinion of organoid model ready

One of the major goals of HIT-CF is to have the organoid assay accepted as a valid measurement to support off-label use of approved CF drugs, so that people with CF caused by rare mutations can also benefit from innovative drugs based on their organoid response. An important step in the path towards that wide acceptance, is obtaining the European Medicine’s Agency (EMA) Qualification Opinion (QO) that the assay is a functional biomarker for CFTR function. The QO is a scientific advice and ‘quality mark’ that supports the validity of the organoid assay in predicting the efficacy of drugs in CF. The HIT-CF team has worked hard to prepare the concept briefing package accompanying our request and it will soon be submitted. We expect a decision by the EMA in the beginning of 2022.

Interview study on the perspectives of people with CF on organoid biobanking published

As mentioned in the previous newsletter, another goal of HIT-CF is to install a permanent biobank containing the organoids that were cultured for the project. To learn more about how people with CF feel about their tissue being stored and used for research and drug development by different parties, how they perceive ownership and what their wishes are concerning feedback, our ethics collaborator Mike Lensink performed 17 semi-structured interviews with people with CF. His findings will be used to inform the ethical framework and governance of the HIT-CF biobank. You can read the full article by clicking on the image on the left.