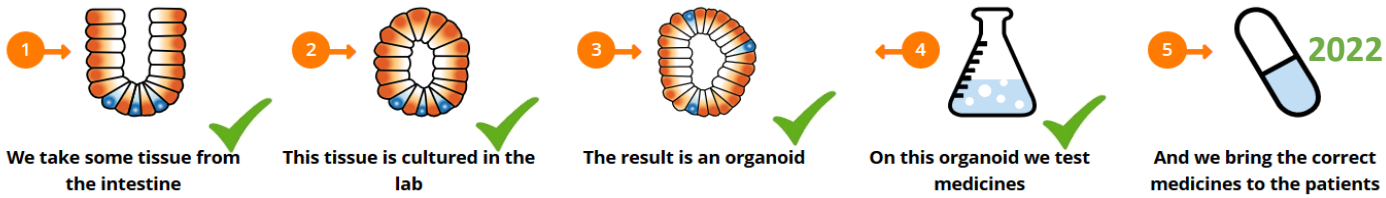
Newsletter HIT-CF Europe

November 2021

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.



After two years of virtual meetings, the HIT-CF consortium was able to meet physically on 14 and 15 October in Brussels. With this newsletter, we would like to inform you of the current status of the HIT-CF project. Despite COVID-19 and industry-related delays we can report **important steps forward**.

FAIR Therapeutics has taken over the role of Proteostasis in the CHOICES trial

Last year, the CHOICES trial was at risk because HIT-CF industry partner Proteostasis merged with Yumanity, which has no focus on CF. We are extremely happy that the Dutch start-up company **FAIR Therapeutics was able to obtain an exclusive licence on the CF drug portfolio** from Yumanity. FAIR Therapeutics has made it its mission to bring drugs to people with rare forms of CF at affordable prices. The consortium is currently discussing with **the European Commission to accept FAIR therapeutics as partner in the** consortium. In the meantime, FAIR Therapeutics is preparing shipment of the drug's raw materials from the US and to process them into pill format. We expect that once the necessary quality controls have been successfully completed, we will receive approval from the regulatory authorities to start the CHOICES clinical trial and **recruit the first participants in the first half of 2022.** FAIR Therapeutics is looking forward to add new opportunities to broaden the treatment portfolio for people with CF and other rare lung diseases

Eloxx Pharmaceuticals remains committed to recruiting HIT-CF participants into their clinical trials

Also Eloxx merged with another company (Zikani). The new management of Eloxx Pharmaceuticals has decided to speed up the marketing of their drug ELX-002 through a procedure called '**Fast Track Designation**'. Eloxx will adapt their clinical strategy accordingly and will recruit people with CF both from the HIT-CF study as well as from the US. Eloxx first has to complete a number of studies in patients with G542X alleles, which were importantly delayed by the COVID-19 pandemic. Therefore, HIT-CF participants will be invited to the study about a year later than expected.

The consortium is actively working on solutions for HIT-CF participants who do not respond to any of the medicines tested so far

The organoids of more than half of all HIT-CF participants do not react to any of the drugs tested (CFTR modulators & stop codon readthrough drugs). The HIT-CF consortium is working hard to give these "non-responders" an **advantageous position** to participate in drug research that can improve their lung function and quality of life. Alternative therapies, such as anti-inflammatory therapy, mRNA and gene therapy are being explored. The HIT-CF consortium will do maximal efforts for non-responding participants to have the first benefit of these new types of therapies. In this context, the consortium is glad to announce that, pending a formal decision of the European Commission, **Santhera will be a new partner within HIT-CF**. Santhera is preparing a clinical study with lonodelestat, which is a potent suppressor of inflammatory responses in the lungs. We expect that in the first half of 2022 about 60-80 people who participate in HIT-CF will be invited to participate in this trial. More information will follow.

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

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