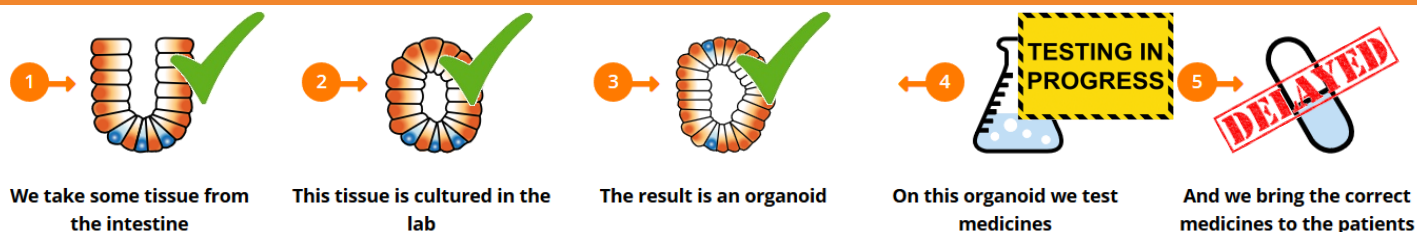


# Newsletter HIT-CF Europe

September 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 Eloxx Pharmaceuticals and Proteostasis Therapeutics, Inc. (PTI) in patients selected through preliminary tests in the laboratory on their mini-intestines – also called organoids.



## The entire HIT-CF team hopes that you have had a pleasant summer break!

We feel more than ready to move HIT-CF forward and are busy preparing the start of the two clinical trials, CHOICES and the Eloxx trial. Just before summer, the HIT-CF team, in collaboration with the clinical trials network, organised a webinar for the clinical trial sites that participated in the inclusion of participants and where the clinical trials might also take place. On the HIT-CF website, you can find several clips taken from that webinar containing interesting information. Click [here](#) to watch Kors van der Ent give a state of affairs.

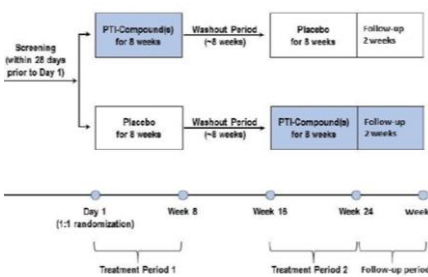


## Individual organoid responses will be shared after the clinical trials' inclusion period

The HIT-CF team regularly gets requests from study participants or doctors to share individual organoid test results, a process called 'deblinding'. In this [clip](#), Marlou Bierlaagh, the HIT-CF study coordinator, explains when screening results will be shared, and why this timing is so important. For patients with less than two stop mutations and an FEV<sub>1</sub> below 40%, Tezacaftor/Ivacaftor screening data can already be requested by their treating doctor at this moment. A request form that doctors can use to ask for individual results in these exceptional cases has been sent out to all sites. All other patients will be provided with screening results after the inclusion period of the clinical trials.

## Proteostasis merged with Yumanity, Eloxx acquires Zikani

As mentioned in previous newsletters, the highly dynamic world of the pharmaceutical industry, with its mergers and acquisitions, has already led to a significant delay of the HIT-CF project. After the acquisition of Proteostasis by Yumanity Therapeutics, the HIT-CF team started discussions with Yumanity to ensure continuation of the CHOICES Trial.



Click on the image to enlarge

If all goes well, selected study participants will be invited, through their doctors, to enrol in the clinical trials early next year. On the left, you can see the study protocol of CHOICES.

Simultaneously, Eloxx Pharmaceuticals acquired [Zikani](#), a company that focuses on ribosome modulating agents for rare diseases caused by nonsense mutations. You can find Eloxx's new logo below and the press release [here](#). Furthermore, Eloxx was awarded [funding](#) from the Cystic Fibrosis Foundation to further develop its ribosome modulating agents. The acquisition of the Zikani portfolio makes Eloxx an even more reliable HIT-CF partner.

## Ethical biobanking according to participants' preferences: a solution for all organoids

Watch this [clip](#) to hear Kors van der Ent explain the principle of ethical biobanking. Currently, the team is busy reaching out to the participating clinical sites to find a solution for the storage of organoids of participants that are not comfortable with commercial use. By the beginning of next year, it should be clear whether there is a solution for all participants who want to preserve their organoids, be it in the HUB or on a national/hospital level.

To learn more about the HIT-CF project, visit [www.hitcf.org](http://www.hitcf.org) or send an e-mail to [HITCF@umcutrecht.nl](mailto:HITCF@umcutrecht.nl).

