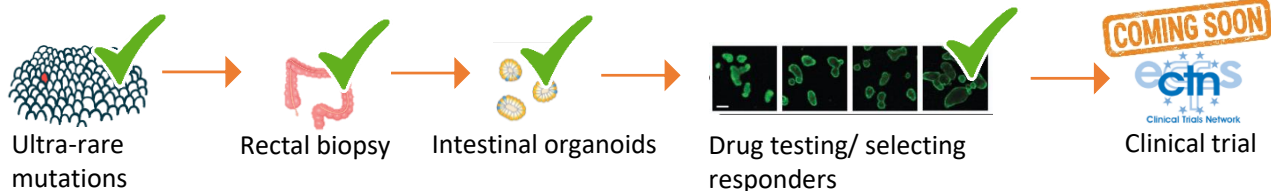


# Newsletter HIT-CF Europe

February 2024



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.



## CHOICES trial is awaiting final approval

CHOICES is the clinical trial in which 52 HIT-CF participants will be treated with a new modulator combination. These people are selected based on their organoid response. As explained earlier, each clinical trial that is performed in the European Union, must undergo evaluation through a centralised procedure. This process is currently ongoing, and we expect to receive approval (the “green light”) by the end of April. Meanwhile, CHOICES has already been approved in the UK. This makes us hopeful of a smooth approval in the EU, too.

Currently, the contracts with all clinical trial sites (the hospitals where CHOICES will be run) are being prepared, and investigator meetings are being organized in March, so all doctors and clinical trial nurses know exactly what to expect when running CHOICES.

## New treatment opportunities for those not selected for CHOICES!

We are happy to confirm that new treatment opportunities have come up for HIT-CF participants who are not selected for the CHOICES trial. ReCode Therapeutics has developed an mRNA therapy for people with CF with rare mutations, including nonsense/stop mutations. mRNA stands for messenger ribonucleic acid and contains instructions to tell our cells how to make a protein, such as the CFTR channel. mRNA is not able to alter or modify a person's genetic makeup (DNA). In people with CF, the CFTR protein is not functioning well or isn't there at all. By delivering correct copies of CFTR mRNA to the lung cells (through inhalation), this problem can be bypassed, and the cells can once again start making functional CFTR channels.

The innovative mRNA therapy developed by ReCode Therapeutics will be tested now for the first time in people with CF. The trial is planned to start before this summer in centres in The Netherlands, the UK and in France. If you live in one of these countries, or if you live another country but willing to travel to a participating centre, please discuss the possibilities with your treating physician. You can also directly contact the HIT-CF team ([HITCF@umcutrecht.nl](mailto:HITCF@umcutrecht.nl)) to explore your possibilities. It is expected that trials will be expanded to other countries in the coming years. We will make sure to inform you further as soon as we receive more details. Do you want to learn more about genetic therapies and genetic therapy trials? Make sure to check out the [website of the CF Trust!](#)

## Attend the HIT-CF dissemination event during CF Europe's AGM!



On Wednesday 5 June in Glasgow, UK, just ahead of the annual ECFS conference, CF Europe is organising its annual general meeting (AGM). The AGM will be followed by a panel discussion and interactive workshops, including one dedicated to HIT-CF, CHOICES and the ReCode trials, in the afternoon. This event is reserved for CF Europe member organisations, and you should have already received a first communication on this. More information will follow soon!

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)

