2020/
Annual Report

Clinical Trials Network

www.ecfs.eu/ctn
ecfs-ctn@uzleuven.be
Tel : +32-479 983839

Contents

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Message from the CTN Executive Director

Damian Downey
Director ECFS-CTN

We are excited to share with you, the 2020-year report of the ECFS-CTN (European Cystic Fibrosis Society - Clinical Trial Network). This report will give you an overview of our work in 2020, in particular, clinical trials that we have supported, a description of the European projects we are involved in, and specific activities including the impact of Covid-19.

During 2020 we supported 28 studies to bring new therapies closer to people with CF. Our work over the last number of years has supported the granting of a European license for “triple therapy” which has been a momentous achievement. This would not have been possible without people with CF taking part in clinical trials facilitated by all our research teams across Europe. Our journey does not stop here, as we continue to support new therapies in development to ensure all people with CF avail of effective treatments.

We have also witnessed the Covid-19 pandemic, which has been an incredibly challenging time for the CF community. We continue our fantastic collaboration with the ECFS-Patient Registry in order to publish updates of Covid-19 in CF. We have also published some advice to CF research teams to help them maintain important clinical trials during these trying times. The new Investigator Trial Committee in the CTN has overseen the initiation of the Covid-19 Antibody Response in CF (CAR-CF) study across our European sites. This is a new and exciting development for the CTN, and we are very thankful for all your support.

We are indebted to the patient organisations and people with CF who have provided their feedback and ideas. The various quotes in the report from people with CF, actively involved in the CTN, highlight the important work undertaken within our network. The patient-centred project on PROMS (Patient Reported Outcome Measures) continues to move forward under the guidance of Kate Hayes and Isabelle Sermet.

We gratefully acknowledge our partners for their sustained funding of our network, including the patient organisations from France, UK, Italy, Belgium, the Netherlands, Switzerland, Luxemburg and Germany. Thank you to CF Europe for coordinating this support. We are also very thankful for the financial support of the Cystic Fibrosis Foundation (USA) for supporting additional research staff in many of our sites as well as in the CTN Core Centre in Leuven, Belgium.

Our network could not have achieved the current level of success without a strong and engaged team. I would like to thank, Veerle Bulteel, Anne Verbrugge and Katia Reeber as well as our Executive Committee members for their unwavering support and engagement. The management of complex data and the development of this excellent report would not have been possible without Fiona Dunlevy, quality manager of the CTN.
Finally, we are so thankful for all your support. Please give us your feedback on this report and contact us if you have any comments or items you would like to have included in future reports. Feel free to share this report with your teams!

Yours sincerely,

Damian Downey
Director ECFS-CTN

ECFS CTN

Clinical Trials Network

2020 OUR YEAR IN NUMBERS

7 scientific **PUBLICATIONS** published

603 PEOPLE with CF newly enrolled into trials

Feasibility checks for **9** trials

3 Covid-19 PROJECTS

3 EU projects ongoing

15 protocols

from **6** companies and **3** academic partners reviewed by people with CF, their families, doctors, research coordinators and statisticians

28 active trials supported

- Restore CFTR function (24)
- Anti-infection (1)
- ENaC inhibitor (1)
- Mucociliary clearance & airway surface liquid (2)

ECFS-CTN

Organisation

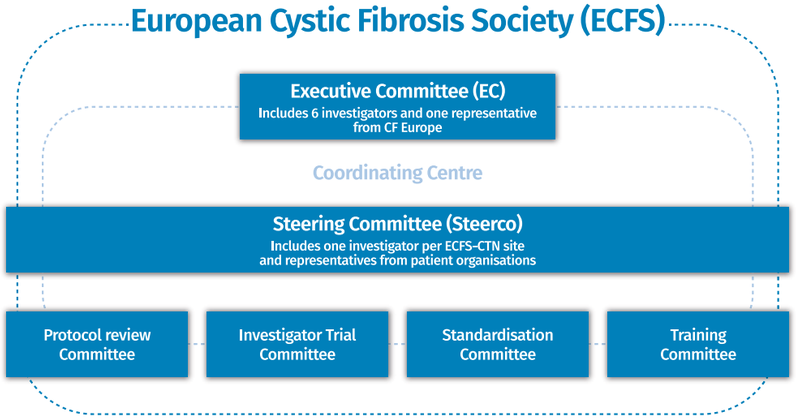
Our mission

Visit **www.ecfs.eu/ctn** to learn more about how ECFS-CTN speeds up clinical trials of new therapies for CF.

How we work

ECFS-CTN was founded in 2008 and aims to intensify clinical research in CF and to bring new medicines to people with CF as quickly as possible.

ECFS-CTN is made up of 58 sites in 17 countries and a central coordinating centre in Leuven, Belgium.

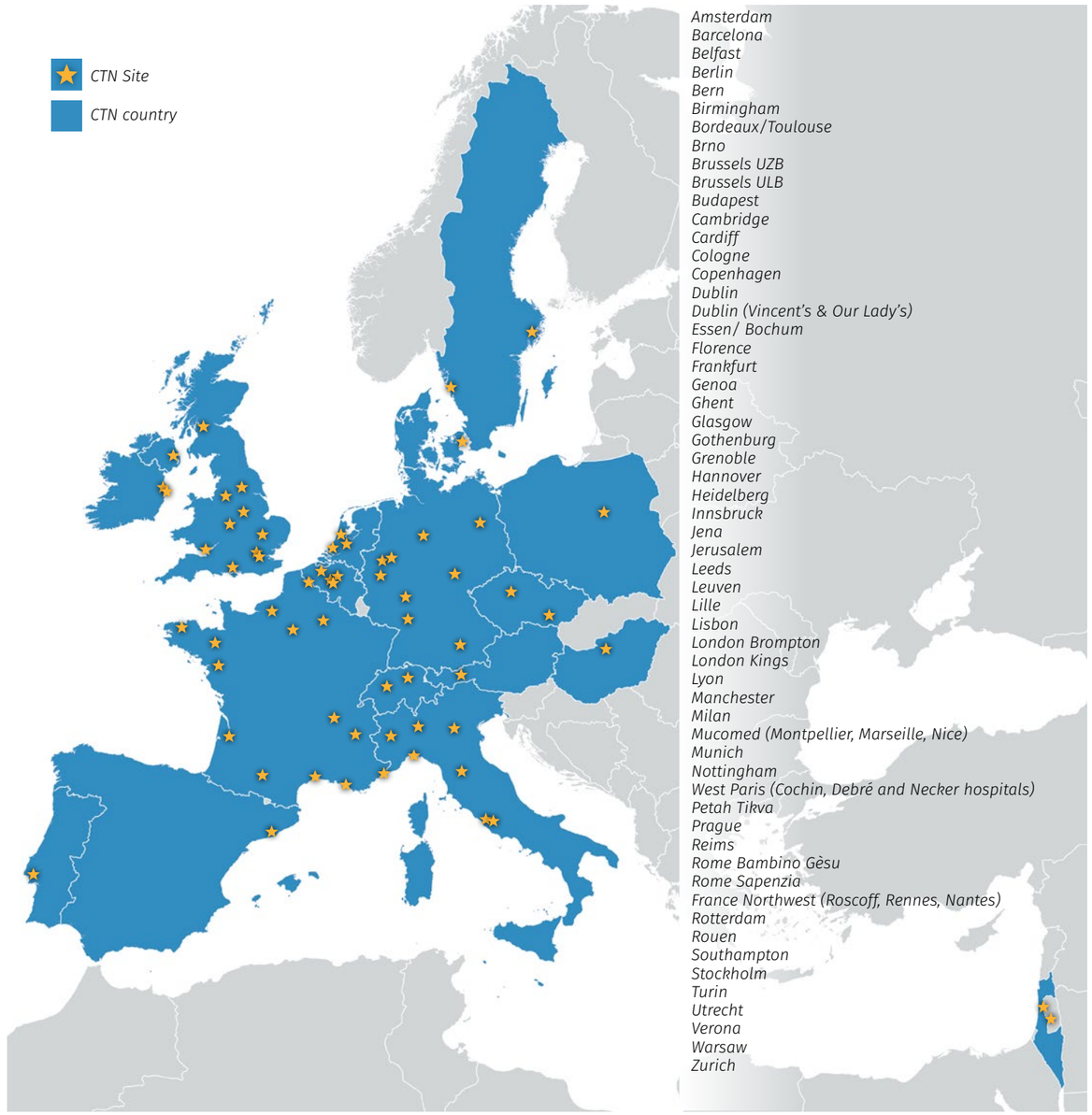


ECFS-CTN is run by:

- the Executive Committee, who meet by teleconference twice monthly. They develop network policies, steer actions to different committees and approve clinical trials to add to the CTN portfolio following protocol review.
- the Steering Committee (Steerco) is made up of 1 doctor from each member site, a representative from each of the funding patient organisations, executive committee members and CTN staff. Steerco members meet in person twice yearly to discuss CTN activities, strategies and common challenges.

The CTN Coordinating Centre has 5 staff members who organise the daily activities of CTN and support the various committees.

Our sites



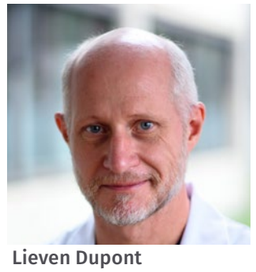
ECFS-CTN

Executive Committee transition

The executive team in 2020

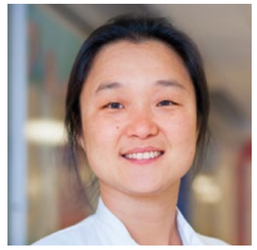
Silke
A doctor caring for children with CF in Cologne, Germany.

Damian Downey
A doctor caring for adults with CF in Belfast, Northern Ireland.



Lieven Dupont
A doctor caring for adults with CF in Leuven, Belgium.

Nicholas Simmonds
A doctor caring for adults with CF in London, England.



Hettie Janssens
A doctor caring for children with CF in Rotterdam, the Netherlands.

Nadine Dufeu
A doctor caring for adults with CF in Marseille, France.

Paola di Carli
Scientific Director of the French patient organization Vaincre la mucoviscidose.

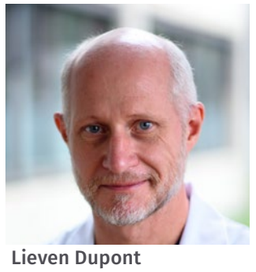
A big thank you to Silke, Nadine and Paola, who finished their terms on the Executive Committee at the end of 2020.

ECFS-CTN

Executive Committee transition

The executive team in 2021

Damian Downey
A doctor caring for adults with CF in Belfast, Northern Ireland.



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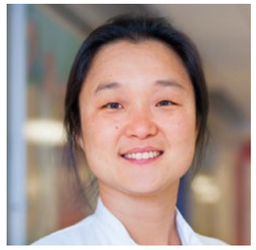


Hettie Janssens
A doctor caring for children with CF in Rotterdam, the Netherlands.

Dario Prais
A doctor caring for children with CF in Petah Tikva, Israel.

Philippe Reix
A doctor caring for children with CF in Lyon, France.

Jutta Bend
Coordinator of the German Clinical Trials Network and representing the patient voice in the ECFS-CTN.

Welcome to the new members Dario, Philippe and Jutta!

CTN activities

Supporting new trials

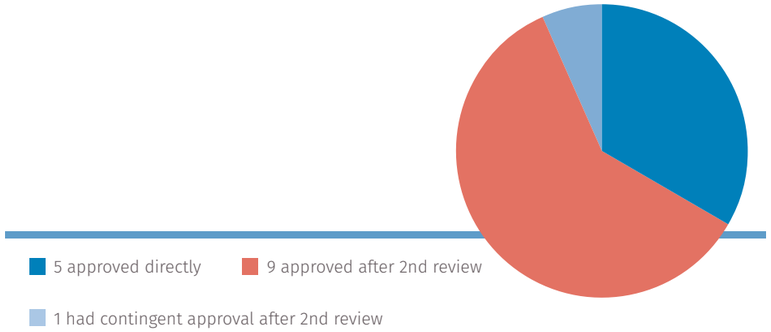
Protocol review & feasibility

Find out more on our website

www.ecfs.eu/ctn

Pharmaceutical companies who want to run clinical trials in ECFS-CTN sites must have their protocol reviewed by the ECFS-CTN protocol review team, including expert groups of CF doctors, research coordinators, academic researchers and people with CF and their families.

In 2020, we reviewed 15 protocols. These included 12 commercial protocols from 6 different companies and 3 protocols from investigator-initiated projects (HIT-CF-Europe, CAR-CF and CF-Storm).



The ECFS-CTN asked for clarifications or modifications for 5 protocols before approval. In total, 15 protocols were approved, one with a contingent approval. When a protocol is reviewed and approved, we tell all ECFS-CTN sites that the protocol had a successful review and whether we consider it high, medium or low priority.

After a protocol has been approved to run in ECFS-CTN, we help the pharma company identify appropriate sites to participate in the trial. In 2020, we coordinated feasibility checks for 9 trials (for 5 sponsors)

"I hope that my contribution to the review committee helps the experts and doctors to solve the puzzle of CF and that young patients can have the health that I have right now, preferably even better health."

Marc, Netherlands who lives with CF and is a protocol reviewer

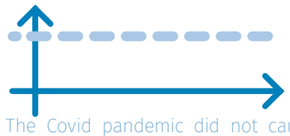
"The impact of a study on the existing burdensome treatment of a CF patient, often seems not to be the first concern of the scientific research professionals. Review by a family member adds some empathy..."

Bart, the parent of a person with CF and a protocol reviewer

CTN activities

Clinical trials in 2020

Trials in ECFS-CTN member sites

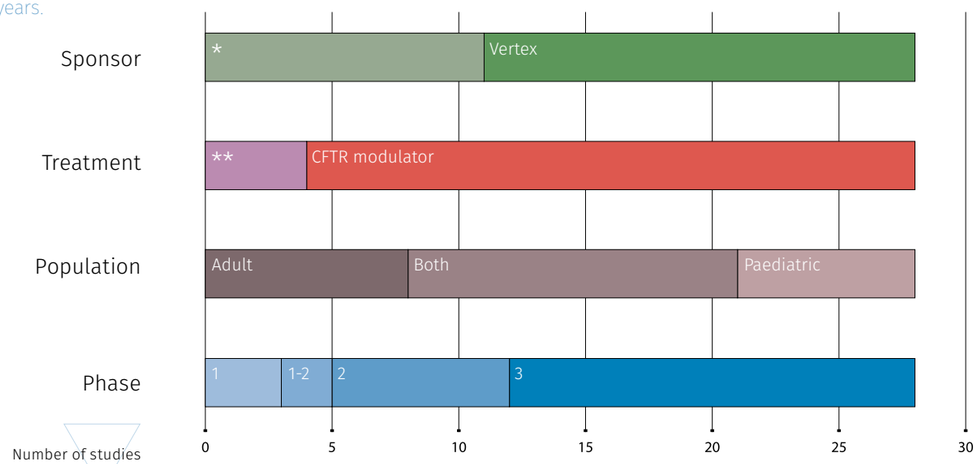


The Covid pandemic did not cause a drop in the numbers of people with CF enrolled in clinical trials in 2020, compared to previous years.

The 28 studies included in this year's analysis were mostly investigating CFTR modulators. Studies were mostly phase 2 and 3 with few phase 1 studies.

Between November 2019 and November 2020, ECFS-CTN sites enrolled 603 people with CF into clinical trials. Three quarters of the people enrolled were adults.

You can find a full list of the studies we supported in the appendix (p. 32-33)



* : Proteostasis sponsored 2 studies. The following sponsors had 1 study each: Abbvie, Alaxia, Boehringer Ingelheim, Corbus, Eloxx, Flatley, Ionis Pharmaceuticals, Synspira and UMC Erasmus.

** mucociliary clearance and airway surface liquid (2 studies), ENaC inhibitor (1 study), anti-infective (1 study).

Find details of all trials we support (and results) at:
www.ecfs.eu/ctn/clinical-trials

CTN activities

Clinical trials in 2020

a clinical TRIAL

In the words of people with CF who participated

Fanny, France is an adult with CF who participated in a trial for a CFTR modulator triple therapy that became available in 2020 for people with CF aged 12 years and older with certain CFTR mutations.

“ I participated in the trial because I wanted to be able to help with medical research as a volunteer and I had high hopes of improving my health by taking Kaftrio. Participation took time and personal investment from me because you have to follow a regular follow-up protocol at the Lyon hospital and be rigorous in taking the treatment. There is also an impact on professional and financial life because frequent visits to the hospital for clinical trial follow-up are on my days off, and it is not always easy to be free. When Kaftrio was approved in Europe in 2020, I felt immense relief because it gave me the results I hoped for, and I never wanted to be without it in the future. The French reimbursement deal reached in summer 2021 was excellent news, a promise of the bright future that awaits me. I can now benefit from this treatment without the constraints of the clinical trial protocol and I can’t wait to feel free and healthy! ”

CTN activities

Clinical trials in 2020

a clinical TRIAL

In the words of people with CF who participated

Johanna, Sweden is an adult with CF who participated in a trial for an anti-inflammatory drug. Unfortunately, the company stopped developing the drug for CF after disappointing trial results.

“ I participated in the study for testing the new drug, with the belief that it would have a good effect and I would feel better in my illness. It was a bit tricky for me to come to the hospital for regular check-ups but otherwise it was not more issues and no problem with taking tablets every day.

I would like to participate in studies in the future. I would like to contribute to research in treatment for people living with CF. I feel a little disappointed that the medicine did not seem to have such a great effect to help patients. ”

CTN activities

Investigator-initiated trials

Introducing the new Investigator Trial Committee

Clinical trials are mostly carried out by pharmaceutical companies to test new medicines that they are developing. However, the CF community often needs to know other information such as “which medicines works best for a specific infection?” or “which medicines can I stop taking?” The clinical trials to answer these questions are called “investigator-initiated trials”, because the investigator (usually a CF doctor) organises the trial, instead of a pharmaceutical company.

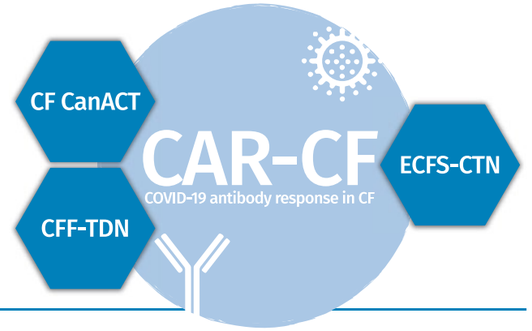
The CF community needs answers to many research questions. For example, a recent exercise in the UK identified research priorities from people with CF. Cystic Fibrosis Europe is launching a Europe-wide project to ask people with CF what research questions we should be focusing on.

ECFS-CTN wants to be ready to help answer these research questions. In 2020, we created a new committee, the “Investigator Trial Committee” or ITC for short. The ITC includes 4 CF doctors, 1 research coordinator and 1 representative from CF Europe to represent the patient voice.

The ITC meets once a month and is learning how to conduct multinational clinical trials. It is very complicated to organise clinical trials across multiple European countries, so we have plenty to learn!

CTN activities

Investigator-initiated trials



Our first trial: CAR-CF

Our first project is to collect blood samples from people with CF across Europe to detect whether the person had Covid-19 or not. We are working with patient organisations in Europe, Canada (CanAct) and the USA (CFF-TDN), who will do similar research in their countries. The project, called CAR-CF, will also look at how well people with CF develop immunity to Covid-19 after vaccination. It will run in 14 countries in Europe, plus in Canada and the USA, and we hope to collect blood samples from thousands of people with CF.

Jutta Bend

Jutta Bend is the coordinator of the German Clinical Trials Network which is run by the German patient organisation “Mukoviszidose eV”. She represents the patient voice on the Investigator Trial Committee (ITC).

“ It is a great opportunity and honour for me to represent the patient organisations as a member of the ITC, because this way patient-centered research can be realised. In CAR-CF – the first IIT project of the ITC – we experienced how important cooperation is, along with individual commitment. Performing such a complex study across Europe without the pharmaceutical industry as big sponsor is a huge effort. Not only because of cost issues, but also because of all the local paperwork, need for translations into local languages and various organisation issues (where to find storage room, how to send samples, who does what etc.). It is amazing to see people with CF and doctors working together to make this study happen. And I am looking forward to new projects that finally will improve the lives of people with CF. ”

CAR-CF

Timeline for a clinical trial

January 2020
Plan to setup a CTN Investigator Trial Committee (ITC)

July 2020
ITC first meeting – decided to launch CAR-CF study

Aug-Dec 2020
Protocol review and feasibility

February 2021
Country leads selected, regular teleconferences started

March 2021
First ethics submission

May 2021
First site activation

June 2021
First person with CF included

July 2021
Investigator meeting

2022
Planned end of inclusion

2024
Planned study end and publication of results

CTN activities

Our work

Training & meetings

The investigators from ECFS-CTN and patient organisation representatives met for two days in Brussels for the annual meeting at the end of January 2020. The ECFS patient registry meeting was organized in parallel, as well as various other CF working group meetings. As usual, we discussed clinical trials in CF, progress of the working groups and ideas for the future. Little did we know that this would be the last time we would see each other for a long time!

Our meetings moved online for the rest of the year:

- June 2020: steering committee meeting with 73 participants,
- June 2020: research coordinator training with 66 participants, focused on the impact of the Covid-19 pandemic on clinical trials, with testimonies from research coordinators,
- September 2020: research coordinator training with presentations about CFTR-targeting therapies, new CFTR modulators, induced sputum training and the influence of Covid-19 on CF clinical trials. We had 88 registrations, with some teams participating via one computer connection.

CTN activities

Our work

Standard operating procedures

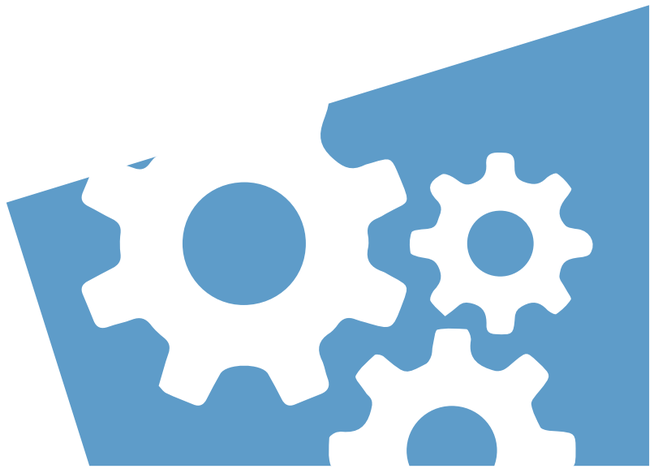
ECFS-CTN writes and shares guidance known as Standard Operating Procedures (SOPs), detailed documents explaining the steps to follow for measuring clinical trial outcomes. During 2020 we updated some of this guidance including the SOPs for:

- Spirometry for people over 6 years of age: to include Covid-19 precautions for aerosol generating procedures and to include the ATS/ERS Updated Guidelines.
- Sweat Test: to incorporate new technical specifications and instructions for use of the new Wescor Sweat inducer and elliptical collectors (if your site is using these). The older version of the SOP for the ‘older’ machinery is still valid!

Please contact Kate Hayes at: k.hayes@qub.ac.uk or the coordinating centre at ecfs-ctn@uzleuven.be for details of these SOPs and all the others too!

Plain language glossary

ECFS-CTN is working with CF Europe and its member patient organisations to create a plain language glossary of scientific terms that are often used in clinical trials. In 2020 we created a pilot glossary of 11 terms. We also got ready to survey people with CF and people from the general public to ask for their feedback and advice on improving the glossary. We plan to expand and finalise our glossary in time for the ECFS conference in June 2022.



CTN activities

Patient Reported Outcome Measures

PROMS

2020 update

Over 2 years ago we began a special patient-centred project, working with CF Europe, the federation of patient organisation across Europe.
This project, about Patient Reported Outcomes in CF, has 3 interlinked parts:

1. We formed the Patient Advisory Group, comprising patients, family members and representatives from both ECFS-CTN and CF Europe. The group met monthly via teleconferences over the course of 2 years, reviewing existing patient reported outcome measures used in CF, to develop a new patient-created questionnaire: the PRO-CF (Patient Reported Outcomes in CF).

2. The Patient Advisory Group linked their findings with the results of another PROMs project, an interview-based study of 125 people with CF and their families to see what topics they would like to be included in a new questionnaire too.

3. A survey of clinical CF sites across the CTN and ECFS members to see what tools and questionnaires they most commonly used in clinic visits.

The end result is the PRO-CF questionnaire, which aims to more accurately assess people with CF's quality of life and allow them to report their symptoms, including areas not previously captured in existing tools.

During 2020, we tested the questionnaire by wider survey to check that the content was accurate and easy to understand. Following expert patient feedback, we refined the tool and published a research paper in the Journal of Cystic Fibrosis, outlining the importance and the process of the patient-created questionnaire (see Journal of CF paper and the lay summary).

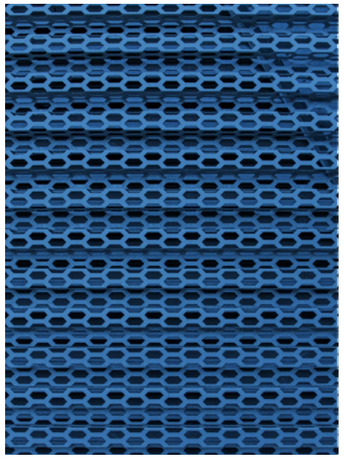
We presented summary posters and abstracts of these projects at the European and North American congresses in 2020, to help spread the important work of the group.

This project will continue into 2021 with applications for funding to test it in research studies for people with CF and those awaiting transplant.

CTN activities

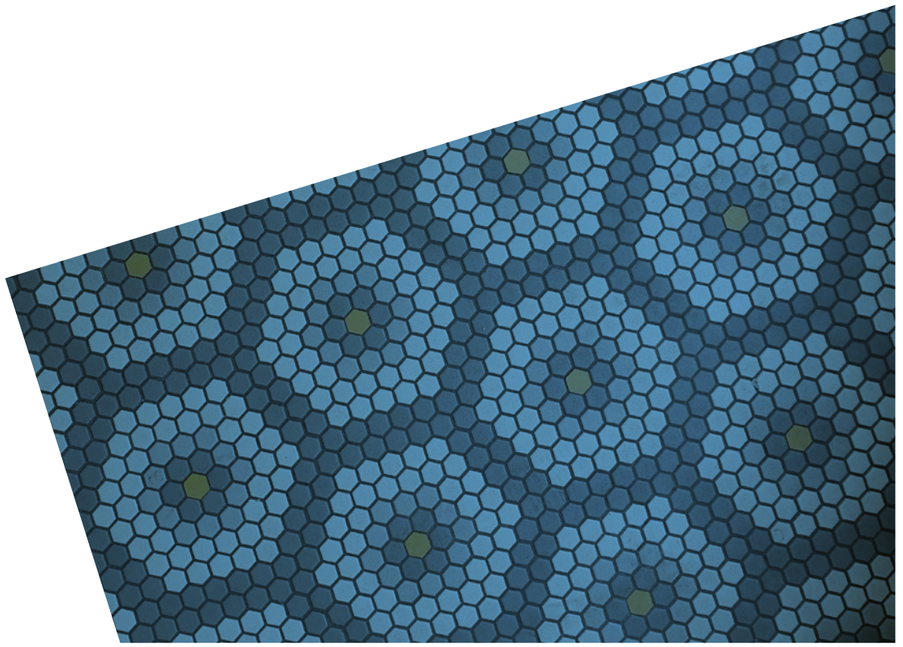
Patient involvement is key

PROMS



Audrey, a person living with CF in France, participates in the PROMS project and was an author on the 2020 scientific paper. She tells us why people with CF must be partners in research projects such as this.

" Participating in this project is so rewarding, there is a real need for a more adapted tool for PROMS in CF. And who could talk better about quality of life than the patients themselves ? I really hope that this will allow us to capture more about the impact of CF on the daily life. It is a great honour to sign this scientific paper as a second author, alongside my paediatrician and my current CF doctor, and it is a pleasure to work together as a group to improve the life of the whole CF community. "



CTN activities

Increasing & maintaining research capacity

Financial support to sites

Additional Research Capacity (ARC) award (2017-2020)

The CFF awarded $3,049,953 to fund additional research personnel in ECFS-CTN sites meeting certain criteria, as well as some structural support between 2017 and 2020. This funding allowed sites to take on more trials, and in some cases more complicated trials. The funding also allowed sites to dedicate more staff time to existing trials.

Continued Research Capacity (CRC) award (2021-2023)

As a continuation of the ARC award, the CFF kindly awarded 3 more years of funding totalling $3,063,615 for 2021-2023 to maintain the capacity at sites and the CTN coordinating centre. We are all extremely grateful to the CFF for providing this support!

Clinical Trials Network

European research projects

ECFS-CTN is a partner in several ongoing EU projects

This project has received funding from the European Union's Horizon 2020 research and innovation programme "Health, demographic change and well-being" under grant agreement no 755234.

The European Commission (via H2020) is funding a clinical trial of the orphan drug OligoG CF-5/20 in CF

In the first half of 2018, CTN reviewed the clinical trial protocol and performed feasibility to help find CF centres to participate in the trial.

The team has received regulatory approvals from Austria, UK, Germany and Ireland, and ethics approvals from Austria and UK. Ethics approvals from Germany and Ireland are expected to follow in 2020 in order to proceed with the clinical study in 2020.

This project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under grant agreement no 777389. The Joint Undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA.

Collaborative network for European clinical trials for children (c4c)

c4c is facilitating new and safer medicines for children by building a European network for paediatric clinical trials (in all diseases, not just CF).

Our role in this vast project is in the education work package. In 2020, we helped tailor some general clinical trials training paediatric clinical trials. We also created training for the cASPerCF trial to investigate aspergillus treatment in children with CF. The c4c consortium is financing and running this trial and the first patients will enrol in 2021.

https://www.imi.europa.eu/

https://conect4children.org/

European Reference Network-LUNG

ECFS-CTN is a core network within ERN-LUNG and provides advice to groups who are setting up new clinical trial networks for other lung diseases.

https://www.ern-lung.eu/

European research projects

ECFS-CTN is a partner in several ongoing EU projects

This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement no 755021.

The HIT-CF-Europe project

HIT-CF Europe aims to provide better treatment for people with CF and rare mutations. Drug candidates of several companies are tested in the lab on patient-derived mini-intestines (called organoids). Then, based on results in the organoids, a smaller group of patients will be assigned to clinical trials with the drug candidates. All participating sites are ECFS-CTN members.

In 2020, despite Covid-19, organoids were screened with the CFTR modulating drug candidate from Proteostasis Therapeutics, Inc. (PTI). Plans for the clinical trial (called "CHOICES") progressed and 52 participants whose organoids show a variety of responses will be invited to take part.

The HIT-CF community has had to deal with the characteristic high volatility of the biotech and pharmaceutical industries. In December 2020, PTI merged with Yumanity Therapeutics, which is now the formal owner of the CF portfolio and Eloxx merged with Zikani. The HIT-CF consortium is confident that these new partners will fully support the efforts of the community to bring new drugs to people with CF with ultrarare mutations.

https://www.hitcf.org

Elise Lammertyn,
Head of research at CF Europe

Elise Lammertyn gives an update on HIT-CF from the CF Europe perspective.

" In 2020, HIT-CF Europe had to deal with quite a few unforeseen circumstances causing delays: the Covid-19-pandemic and related lockdowns temporarily prevented our colleagues in the labs from performing the organoid screening, and the dynamics of partnering pharmaceutical companies with takeovers and mergers created insecurity about the planning of the clinical trials. The most important mission of CF Europe, as responsible partner for communication and dissemination, was to provide study participants with correct and transparent information about all these developments. We also tried to keep the interest in CF caused by rare mutations alive. With the EMA granting market authorization for Kaftrio and rapid reimbursement in a number of countries, there are still people with CF without access to innovative therapies, especially people with CF with rare mutations currently not eligible for modulator treatment. The HIT-CF project paves the way – that in the future each individual patient can benefit from new CF therapies. Meanwhile, the consortium worked hard on creating a governance framework for the exploitation of a HIT-CF organoid biobank, taking into account patient preferences for future organoid use beyond HIT-CF. "

Covid-19

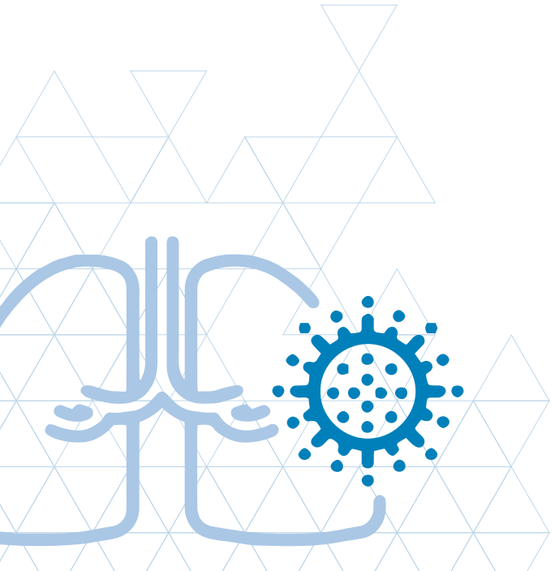
The impact on people with CF and on CF clinical trials

How we tracked the impact

We worked with the ECFS Patient Registry to collect information about the number of people with CF who had Covid-19. We published regular updates on the ECFS website and two scientific papers describing how people with CF experience Covid-19 and the risk factors for poor outcomes.

Covid-19 has impacted our activities in ECFS-CTN. When the pandemic was declared in spring 2020, we started sending regular surveys to our sites to understand how the pandemic impacted clinical trials of new medicines for CF.

We discovered that CF clinical trials were disrupted in spring 2020 but disruption mostly finished by summer 2020. We collected ideas from all the sites on how to handle the challenges of performing clinical trials during a pandemic. We put the tips together and shared them with our sites so that they could learn from each other's experiences, and published a paper in a journal called Trials.



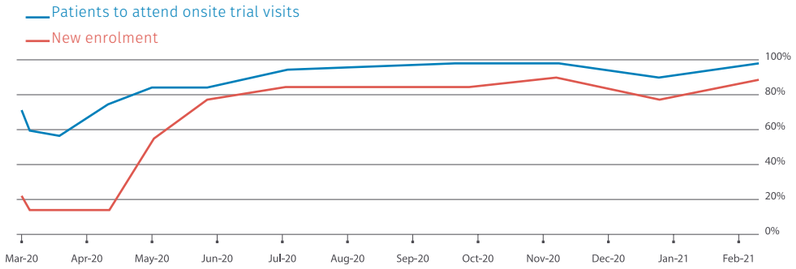
Covid-19

The impact on people with CF and on CF clinical trials

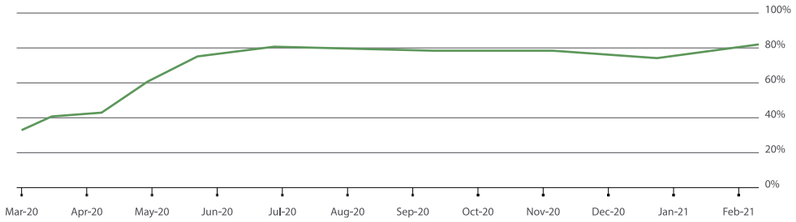
The data from our surveys

Our surveys showed that Covid-19 disrupted clinical trials in spring 2020. This was mostly resolved by summer 2020.

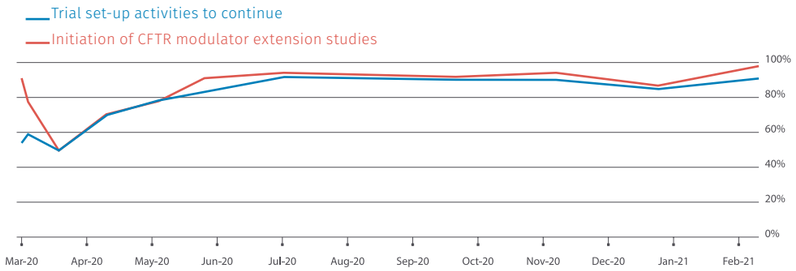
Ongoing trials, % of sites allowing



% of sites where people with CF are willing to attend onsite trial visits



New trials, % of sites allowing



Covid-19

The impact at a site

In the words of a research coordinator

Arianna Bisogno is a research coordinator in Milan, Italy. She explains how her site kept clinical trials running throughout the pandemic.

“ More than a year has passed since the start of the world’s largest health emergency in the last hundred years. The health emergency due to Covid-19 has had a dramatic impact in many areas, including on the dynamics of clinical trials, but fortunately not entirely negative.

Although, in Italy, Lombardy was the area most affected by the epidemic, there have never been any interruptions to the ongoing clinical trials. However, we have seen some difficulties with starting new projects and stopping enrolling new patients into the studies.

The main problem was the fact that in some cases the enrolled patients had difficulty in going to the center for the visit but fortunately this never meant the interruption of the therapy. This pandemic has accelerated the shift from on-site trial clinics to remote and virtual clinics, which was already partially underway in the world of clinical research. The pandemic gave further impetus to this trend, as even study participants authorised to move to the test site were unwilling to do so.

For reasons of health precaution, the monitoring visits to the clinical center were canceled but a “digital” solution was immediately sought in order not to lose control over the management of the study and above all over the quality and accuracy of the data. In clinical research, digital solutions have never been widespread and those used are sometimes fragmented and partial.

A further advantage obtained in this emergency phase was undoubtedly the streamlining of the bureaucratic procedures linked to each study. In fact, the procedures implemented (remote visits, drug shipment, monitoring) were authorised in a very short time by the ethics committee, and above all the rapid planning and launch of clinical studies designed for research on Covid-19 has shown that some aspects and clinical trial practices could be improved, simplified or modernized in ways that would benefit patients, professionals and all research. In the future, it will be important that the conduct of research incorporates these lessons to ensure the highest quality of research. ”

Covid-19

The impact at a site

In the words of a person with CF

Serena is a person with CF in Milan, Italy. She recounts her experience of taking part in a clinical trial for a CF therapy during the pandemic.

“ Being part of a clinical trial is already in itself a challenge full of emotions and dotted with ups and downs, but certainly with the addition of a pandemic this experience has become even more “peculiar”.

Suddenly finding yourself physically disconnected from your referral clinic and having the possibility to speak with your doctors only virtually via phone calls immediately felt surreal: telemedicine was a concept that seemed absurd to me and I started missing waking up at dawn to go to the hospital and carry out all the medical examinations that the trial required like never before.

Suddenly all these things that I found kinda annoying before the advent of the pandemic appeared to me as an oasis in the desert: I would have given anything to get all this back.

Instead I was left alone with sterile phone calls that, although scrupulous and still very useful, were not at all comparable to what I was used to.

All this, however, made me realise how important the development and improvement of telemedicine is as a resource that can be used on any occasion.

After a while, it was time to resume with the visits to the hospital and it was very pleasant for me: for the opportunity of seeing doctors and nurses, but above all because the check-ups and the medical examinations carried out in first person instilled in me a very strong sense of security, something that in the first moments of the pandemic had been totally replaced by uncertainty.

Luckily, I did not find any insurmountable problems in being part of a clinical trial during the pandemic since everything was always managed at its best even if, as I explained above, the difference was felt particularly on a psychological level more than on a practical level. ”

Financial report 2020

Income & expenses

ECFS-CTN is funded by grants and by charging fees for scientific services to pharma companies.

ECFS-CTN helps pharma companies improve the design of clinical trials. It is important that we are not financially dependent on pharma companies so that we have no conflict of interest when giving scientific advice on clinical trials. Therefore, we limit our earnings from services to pharma, and rely on the generous support of other stakeholders to make up the shortfall. ECFS-CTN is grateful to the following organisations for funding our work in 2020: CFF, European patient organisations (from France, Germany, UK, Italy, Belgium, the Netherlands, Luxembourg, and Switzerland). We also thank CF Europe for coordinating the contributions from national patient organisations.

Financial report 2020

Income & expenses

Reflects book-keeping year 1 Jan – 31 Dec 2020:

CTN - Income & expenses 2020	Euro €
ECFS Support	100,000
National CF associations	113,750
Services to companies	164,542
LCI Core Centre	50,213
Other income	3,500
Total Income	***432,005***
Human resources	290,643
Travel / meetings / courses	30,300
Software Development / Maintenance	28,922
Dedicated Server	11,910
Office equipment	1,554
Designer year report	1,008
Subscriptions online tools	919
Total Expenditures	***360,256***
CTN result 2020	
Year result	***71,749***

Appendix

Studies supported by ECFS-CTN in 2020

RESTORE CFTR FUNCTION

New Phase 2 testing of ABBV-3067 alone or in combination with ABBV-2222 in people with CF aged 18 and older with 2 F508del mutations. (Abbvie M19-530)

New Phase 2 safety and drug behaviour testing of ELX-02 in people with CF aged 16 years and older, with 1 or 2 G542X mutations. (Eloxx EL-004)

New Phase 1/2 testing of how the drugs FDL169 and FDL176 interact in people without CF and in people with CF and 2 F508del mutations. (Flatley FDL169-2018-10)

Phase 1 safety and drug behaviour testing of PTI-801 in healthy volunteers and in adults with CF (Proteostasis PTI-801-01)

Phase 1 safety and drug behaviour testing of PTI 808 in adults with and without CF (Proteostasis PTI-808-01)

New Phase 3 safety and drug behaviour testing of Kaftrio in people with CF aged 12 years and over. (Vertex VX18-445-109)

New Phase 2 safety and efficacy testing of VX-121 combination triple therapy in adults with CF. (Vertex VX18-121-101)

Phase 3 testing of ivacaftor in children with CF aged under 2 years with a gating mutation (Vertex VX15-770-126)

Phase 2 open-label long-term observation of Orkambi's effect on CF progression in children aged 2-5 years with 2 F508del mutations (Vertex VX16-809-121)

Phase 3 testing of ivacaftor in children with CF aged under 2 years with a gating mutation (Vertex VX15-770-124)

Phase 3 open-label extension testing of Kaftrio in people aged 12 years and older with 1 or 2 F508del mutations (Vertex VX17-445-105; parent studies: VX17-445-102 and VX17-445-103)

Phase 3 open-label testing of VX-659 in triple combination with ivacaftor and tezacaftor in people aged 12 years and older with 1 or 2 F508del mutations (Vertex VX17-659-105)

Long term rollover testing of VX-661 in combination with ivacaftor in people aged 12 years and older with 1 or 2 F508del mutations (Vertex VX14-661-110)

Phase 3 open-label extension observation of long-term treatment with Symkevi in children aged 6 years and older with 1 or 2 F508del mutations (Vertex VX16-661-116; parent studies: VX16-661-113 and VX16-661-115)

New Phase 3 safety and drug behaviour testing of Kaftrio in children with CF aged 6-11 years. (Vertex VX18-445-106)

New Phase 3 open-label extension observation of long-term treatment with Kaftrio in people with CF with 1 F508del mutation and 1 gating or residual function mutation (Vertex VX18-445-110; parent study: VX18-445-104)

New Phase 3 open-label extension observation of long-term treatment with Kaftrio in people with CF aged 6 years and older (Vertex VX19-445-107; parent study: VX18-445-106 Part B)

New Phase 3 open-label extension observation of long-term treatment with Kaftrio in people with CF aged 6 years and older (Vertex VX19-445-107; parent study: VX18-445-109)

Long term rollover testing of Orkambi in people aged 6 years and older with 2 F508del mutations (Vertex VX15-809-110)

New Phase 3 safety and efficacy testing of Kaftrio in children with CF aged 6-11 year with 1 F508del mutation and 1 minimal function mutation. (Vertex VX19-445-116)

New Phase 3 safety and efficacy testing of VX-445 combination triple therapy in people with CF aged 12 years and older with 1 F508del mutation and 1 residual function mutation. (Vertex VX18-445-104)

New Long term safety testing of VX-445 combination therapy in unblinded phase 3 testing of in people with CF aged 12 years and older. (Vertex VX18-445-113)

ANTI-INFLAMMATORY

ANTI-INFECTIVE

Phase 2 testing of lenabasum of in people aged 12 years and older with recent antibiotic treatment for pulmonary exacerbation (JBT101-CF-002)

New Phase 2 testing of safety and efficacy of inhaled SNSP113 in adults with CF. (Synspira SNSP113-19-201)

New Phase 1 study to evaluate safety and tolerability and to find the right dose of hypothiocyanite (OSCN-), bovine lactoferrin (bLF) and their combination (ALX-009) in males without CF and then in adults with CF and non-CF bronchiectasis. (ALX-009-CL-038)

MUCOCILIARY CLEARANCE

Inhaled hypertonic saline in preschoolers (UMC Erasmus SHIP-002)

Phase 2 safety and efficacy testing of inhaled BI 1265162 when added to standard care in people with CF aged 12 years and older. (BI 1399-0003)

OTHER

New Phase 1/2 testing of safety and drug behaviour of the ENaC inhibitor ION-827359 in adults with and without CF. (Ionis ION-827359-CS1)

Kaftrio = Elexacaftor, tezacaftor and ivacaftor Orkambi = lumacaftor and ivacaftor Symkevi = tezacaftor and ivacaftor

Clinical Trials Network

www.ecfs.eu/ctn
ecfs-ctn@uzleuven.be
Tel : +32-479 983839