2024/
Annual Report

Clinical Trials Network

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Contents

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2024 OUR YEAR IN NUMBERS

1009 PEOPLE with CF
newly enrolled into trials

Feasibility checks for **6** trials

x 9 protocols
from 8 sponsors
reviewed by people with CF, their families, doctors, research coordinators & statisticians

3 ★★★ EU
projects ongoing

CAR-CF
COVID-19 antibody response in CF
to measure **Covid-19** antibodies
enters follow-up phase

29 active trials supported

CFTR modulator (11)
No intervention (9)
Genetic therapies (3)
Others (6)

126 **Happy People**
at face to face training and meetings in June 2024

9 **scientific** **PUBLICATIONS & POSTERS** published

Message from the CTN Director

Lieven Dupont

We are delighted to present the 2024 Year Report of the European Cystic Fibrosis Society-Clinical Trials Network (ECFS-CTN). This report gives you an overview of our work in 2024 including an overview of the clinical trials that have been supported by our network, an overview of our organisational structure, a list of the specific activities within our network in the past year, as well as a description of the various European projects. We hope you will find it a useful resource but also an illustration of the high level of engagement and performance within the ECFS-CTN.

Since the network was founded in 2008, the range of activities has increased and the ECFS-CTN currently federates 68 sites across 20 countries in Europe, caring for 26.082 adult and paediatric CF patients. The ECFS-CTN remains committed to intensify clinical research in CF and to bring new medicines to people with CF as quickly as possible by addressing the initiatives listed in our 2024-2028 Strategic Plan.

2024 was yet another busy year: during 2024 we actively supported 29 trials, reviewed 9 protocols, and performed 6 feasibilities. The patient-centred project, PRO-CF (Patient reported outcomes in CF) continues to move forward under the guidance of Isabelle Sermet and Kate Hill from our Standardisation Committee. The 3 CTN Imaging subgroups have been working hard to develop recommendations and guidance for CT and MR imaging that could be adopted for research and clinical purposes and some of these recommendations have been published. The ECFS-CTN Training Committee led by Olaf Eickmeier brought together dedicated investigators and research coordinators on their annual training day.

On behalf of the CTN Executive Committee (EC), we want to especially acknowledge and thank Veerle Bulteel, Anne Verbrugge, Katia Reeber, Kate Hill, Fiona Dunlevy and Christine Dubois for their dedication and unwavering commitment to making the CTN such a highly-efficient and engaged team.
We are privileged to work with them and this report is the result of their intense collaboration with ECFS and the many enthusiastic collaborators and team members in our CTN sites.
We want to thank our partners for their sustained funding of our network, including the patient organisations from France, Germany, UK, Italy, Belgium, the Netherlands, Luxembourg, Switzerland, Ireland, Israel and Poland. We also thank CF Europe for coordinating the contributions from national patient organisations. We are also very thankful for the generous financial support of the Cystic Fibrosis Foundation (US) for supporting, additional research staff in our site.
We would like to gratefully acknowledge the current members of the CTN Executive Committee for their continued advice and energy that is instrumental in guiding the direction of the CTN: Nick Simmonds, Anna-Maria Dittrich, Hettie Janssens, Federico Cresta, Malena Cohen-Cymberknoh and Jutta Bend.
We also need to express our sincere gratitude to the leadership and members of the Standardisation Committee, the Protocol Review Teams and the Training Committee for all their efforts. And we also want to thank our CTN partners across the globe (CFF-TDN, CanACT, the PORG, CF Europe and the national CF trial networks throughout Europe) for their valued input. We also thank the ECFS office and Board for their continued support of our work, as well as our colleagues in the ECFSPR, with whom we continue to build closer links. We look forward to continue the CTN endeavour in the near future together with the drive and dedication of our investigators and research teams.

On behalf of the ECFS-CTN team and the Executive Committee,

Lieven Dupont
current ECFS-CTN director

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.

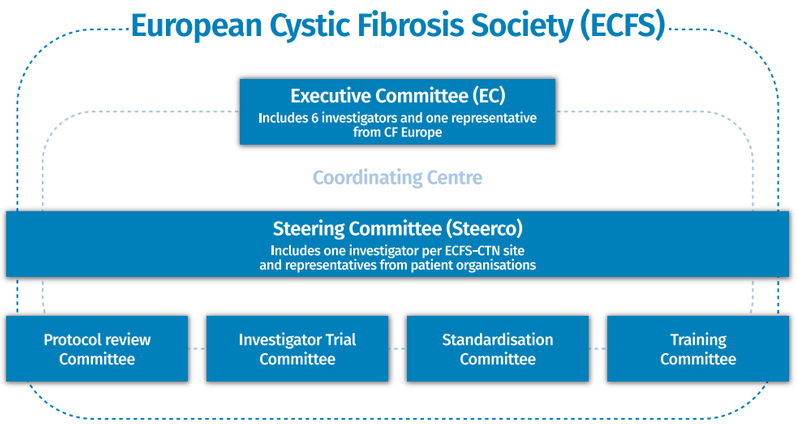
How we work

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.

In 2024, ECFS-CTN had 57 sites in 17 countries and a central coordinating centre in Leuven, Belgium.

At the end of 2024, ECFS-CTN renewed and expanded its membership. Click here for a list of current ECFS-CTN sites (in mid 2025).

https://www.ecfs.eu/ctn/list-ctn-centres



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

in 2024

At the end of 2024, ECFS-CTN renewed and expanded its membership.
Click here for a list of current ECFS-CTN sites (in mid 2025): www.ecfs.eu/ctn/list-ctn-centres

ECFS-CTN

Executive Committee

The executive team in 2024

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

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

Anna-Maria Dittrich
A doctor caring for children with CF in Hannover, Germany.

Malena Cohen-Cymberknoh
A doctor caring for children with CF in Jerusalem, Israel

Federico Cresta
A doctor caring for children & adults with CF in Genoa, Italy.

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

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

CTN activities

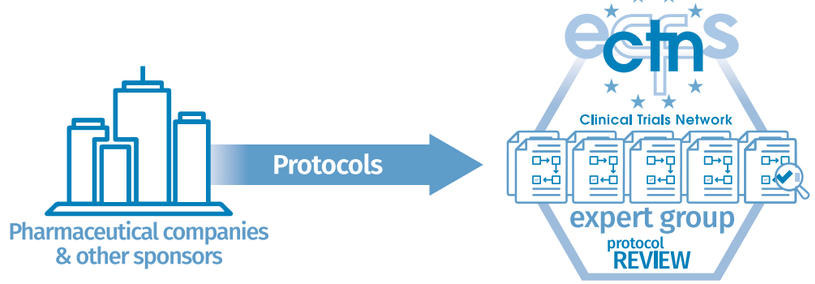
Supporting new trials

Protocol review

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 2024, we reviewed 8 commercial protocols from 7 different companies. We also reviewed 1 academic protocol.



The ECFS-CTN asked for clarifications or modifications for 4 protocols before approval. In total 8 protocols were approved. One was deferred, awaiting a second round. We also gave early advice for 1 protocol.

Feasibility

After a protocol has been approved to run in ECFS-CTN, we help clinical trial organisers to contact sites to see if they can participate in trials. We encourage companies to contact all eligible sites and to give all sites a chance to participate. In 2024, we coordinated feasibility checks for 6 protocols from 6 different sponsors.

CTN activities

Clinical trials in 2024

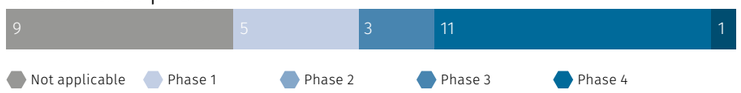
Trials in ECFS-CTN member sites

Across ECFS-CTN there were 29 studies active during 2024. As in previous years, the most common therapy investigated was CFTR modulators. 2024 also saw the setup or start of 3 genetic therapy trials.

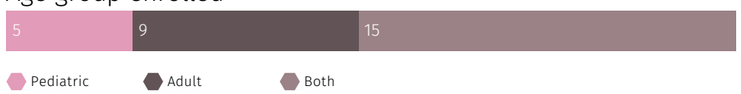
You can find a full list of the studies we supported in the appendix.

These graphs describe the 29 studies active in 2024, by clinical trial phase, by age group enrolled and by therapy investigated.

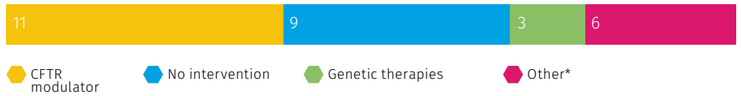
Clinical trial phase



Age group enrolled



Therapy investigated



*Other: anti-infectives (1 study), ENaC inhibitor (1 study), nutritional (1 study), anti-inflammatory (1 study), withdrawing inhaled therapies (1 study) and physical activity (1 study).

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

CTN activities

Clinical trials in 2024

a clinical TRIAL

In the words of people with CF who participated

Miriam,
Adult with CF, Italy

Miriam, an adult with CF from Italy, explains why she participated in a trial

“ When I started studying at Medical School, my parents were so worried about my health, they asked me how I could handle living far from home, doing CF treatments, living with CF symptoms and still being able to study and go to the hospital as a med student.

I remember I answered them “I feel like scientific research will find something for me too”.

Then, nine months later, my CF doctors told me that I was eligible for a clinical trial in which I would be given Trikafta to see how it would work on my rare mutation of CF. They explained to me that I would need to go to the hospital every two weeks then every month, to check out how my response to Trikafta was, by doing ECG, blood works, sweating test, PFTs, CT scans… it would truly be an intense period but I immediately accepted because not only it would have helped me, but my contribution would also help many other patients.

Now, two years later, my life has changed for the better.

I’m looking forward to give back to Medicine and Science what they gave me, and, as a patient who will become a doctor I want to treasure what I learned by being part of a clinical trial.

Now that I finally feel good, I just want to help people at their most vulnerable time. I’m going to live a life on both ends of the stethoscope :) ”

CTN activities

Our Covid-19 response

Covid-19 antibody response in CF

(CAR-CF)

CAR-CF is an investigator-initiated trial supported by ECFS-CTN. We are collecting 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 all conducting 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.

You can find more information on our website https://www.ecfs.eu/ctn/projects/CAR-CF.

CAR-CF received a research grant from the Cystic Fibrosis Foundation (CFF). French and Dutch sites received extra financial support from their national patient organisations.

A big thank you to the people with CF participating in the study, and to the patient organisations, investigators, and research coordinators.

Enrolment into CAR-CF closed at the end of 2023. In 2024, CAR-CF followed up patients during routine clinic visits, and took blood to test for Covid-19. The team were busy collecting and preparing data for analysis and reporting.

CAR-CF

Timeline

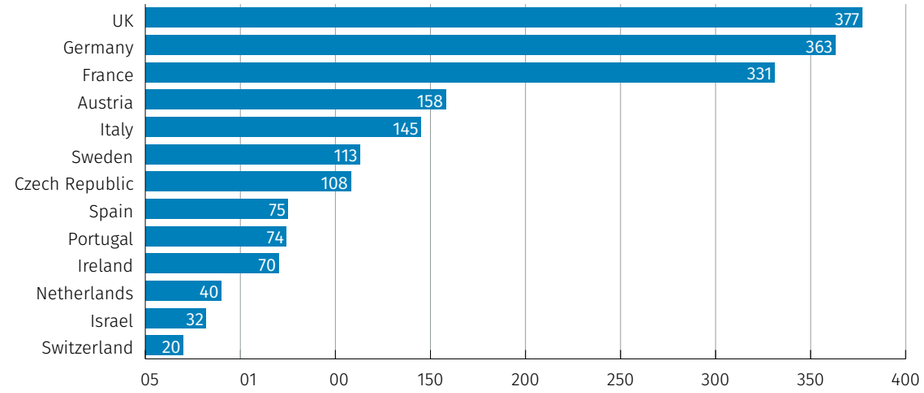
January 2020	July 2020	February 2021	May 2021	June 2021	December 2023	December 2025	2026
Plan to setup a CTN Investigator Trial Committee (ITC)	ITC decides to launch CAR-CF study	Lead investigators in each country selected	First site activated	First pwCF enrolled	Last pwCF enrolled	Last visit of the last pwCF planned	Data analysis, testing blood samples and results!

CTN activities

Our Covid-19 response

CAR-CF enrolment between May 2021 and December 2023

Thank you to the 1906 people with CF who enrolled in CAR-CF



CTN activities

Our work

Training

Each year at our summer conference, our ECFS-CTN Training Committee led by Dr Olaf Eickmeier (Frankfurt, Germany) organises a training day. In 2024, we brought together 126 investigators and research coordinators for a day of lectures, discussions and training.

The programme included training on trial design, research coordinator training and genetic therapies.

We also had session about how to discuss genetic therapy trials with people with CF. A big thank you to Marianne Martin, who lives with CF, who told us about her experience of genetic therapy trials and the importance of clear communication from investigators and research coordinators.

Expert advice to medicines regulator

The European Medicines Agency (EMA) regularly asks for expert advice on new or changing rules about how medicines are developed and tested in clinical trials. ECFS-CTN regularly contributes advice when the rule could impact the development of new treatments for CF.

ECFS-CTN is also a member of the European network of Paediatric Research at the European Medicines Agency (Enpr-EMA). Members of Enpr-EMA work together to improve pediatric research, and make sure that children have safe and effective medicines.

ecfs ctn

Clinical Trials Network

CTN activities

Standardisation

Standard operating procedures

ECFS-CTN writes and shares guidance known as Standard Operating Procedures (SOPs), which are detailed documents explaining the steps to follow for measuring clinical trial outcomes.

In 2024, we published several SOPs about sputum induction, epithelial cell models for theranostics, and body mass index. See the Publications section (p.22) for more details.

Central Reading Core Facilities for Lung Clearance Index and Lung Imaging

We make sure that all our member sites work in a similar way when it comes to some of the special techniques needed for the clinical trials. We train and certify sites using “central core” facilities. For example, for a measure of lung efficiency called Lung Clearance Index, and for CT scanning.

To find out more, visit:

https://www.lungclearanceindex.com/

https://lunganalysis.erasmusmc.nl/

For general enquiries for all our SOPs, core reading facilities and standardisation group, please contact Kate Hill at:

kate.hill@qub.ac.uk or the coordinating centre at ecfs-ctn@uzleuven.be

CTN activities

Standardisation

Standard Operating Procedures

SOPs

The sticky issue of sputum induction: SOPs to help!

Our network aims to intensify research in CF and bring new medicines to people living with CF as quickly as possible. One way we do this is by standardising research procedures and outcome measures and sharing of expertise amongst our dedicated CF researchers. This is more important than ever, with the expanding use of CFTR therapies.

One impact of CFTR modulators is that they reduce the stickiness of airway mucus and improve mucociliary clearance. This means that some people with CF on modulators produce less sputum. While this is great news for people with CF on modulators, it also presents a challenge. Sputum gives a window into the airway, and lets healthcare teams and researchers check for infections and signs of inflammation. People who don’t spontaneously cough up, or expectorate, sputum can be “induced” to produce a sputum sample. This involves inhaling some very salty water (called hypertonic saline), which makes you cough, bringing up bits of sputum from deep in the airways. Some people find the induced sputum procedure and the coughing uncomfortable, and sometimes there can be a feeling of chest tightness, or bronchoconstriction.

The induced sputum procedure has become an important way to access sputum samples, now that so many people on modulators don’t produce sputum spontaneously anymore. We wanted to make sure that the induced sputum procedure minimises discomfort as much as possible and maximise the chances of getting a successful sputum sample. To do this, we formed a team of experts from ECFS-CTN and from the ECFS Physiotherapy group to develop new SOPs for sputum induction. We made separate SOPs for children and for adults with CF, based on whether they spontaneously expectorate sputum or not.

While preparing the standardised procedure for children with CF, the team of experts had a lot of discussion about whether to recommend suctioning sputum from the airways of children with CF, who find it difficult to cough up samples. Some team members feared that children could find the suctioning traumatic, and did not want the SOP to recommend this technique. This sort of discussion and disagreement between experts highlights the challenges of working together to define a standardised approach. We compromised by explaining the techniques available without mandating suctioning, to help healthcare and research teams and people with CF to make informed decisions about sputum sampling.

We developed 4 SOPs in total (see the Publications list on p.22), and we extend our special thanks to Oriane Burgun, Marie Dautry, Jana Pleskova, Anna Chmelarova, Catherine Brown, Carwyn Bridges and Lauren Alexander.

European research projects

ECFS-CTN is a partner in several EU projects

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. We helped revise and tailor some general clinical trials training to paediatric clinical trials.

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 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 is a research project which aims to provide better treatment and better lives for people with CF and rare mutations. To do this, a novel CFTR modulator combination was tested in the laboratory on mini-intestines, also called organoids, from over 500 people with CF from all over Europe. Secondly, based on the reaction in the organoids, 52 participants whose organoids show a variety of responses were selected to participate in CHOICES, a clinical trial with the novel modulator combination. All participating sites in CHOICES are part of the ECFS-CTN.

Over the course of the project, the HIT-CF community has had to deal with the characteristic volatility of the biotech and pharmaceutical industries. Nevertheless, the consortium has been able to consolidate collaboration with new partners, such as Fair Therapeutics, and is confident that these partners will fully support the efforts of the community to bring new drugs to people with CF with ultra-rare mutations.

2024 marked the initiation and completion of participant enrollment into CHOICES. 40 people were successfully randomized into the clinical trial and are now being treated with diponecaftor. The first results of CHOICES are expected mid-2025.

https://www.hitcf.org

European research projects

News from HIT-CF

Elise Lammertyn,
Head of research at CF Europe

Elise Lammertyn is head of research at CF Europe, the European umbrella organisation of national patient organisations. She gives an update on HIT-CF.

“ In 2024, the safety and efficacy of diponecaftor was evaluated in CHOICES, a clinical trial with 40 participants whose organoids show a variety of responses to the new triple therapy. All participating sites in CHOICES are part of the ECFS-CTN. CHOICES finally got clearance to start enrolling participants in spring 2024. In early June, the moment had finally arrived: the first patient was enrolled at UMC Utrecht (the Netherlands), something she had been looking forward to for a long time. Over the course of the third and fourth quarter of 2024, 15 CTN centers in 9 countries included a total of 40 adults with CF in CHOICES. All participants were pre-selected using the innovative organoid test, which uses laboratory-grown organoids from patients own cells to predict clinical response to the therapy in the lab. A special thanks goes out the clinical research teams at the participating sites for their patience, flexibility and collaboration throughout this process! The whole CF community is now looking forward to the results of this ground-breaking trial, which are expected mid-2025.

In order to maximize the potential of the large patient pool that was established for HIT-CF, and to offer an alternative to participants who were not selected for CHOICES, additional partnerships were established. ReCode Therapeutics developed an investigational inhaled therapy based on mRNA for people with CF with rare mutations, including nonsense/stop mutations. Their phase I clinical trial started recruitment in the second half of 2024, running in 10 sites in 3 countries. HIT-CF participants not selected for CHOICES were actively informed about the possibility of participating in this mRNA trial.

Finally, the consortium started the endeavor of reconsenting the HIT-CF participants so that their organoids remain available for future studies by storing them in a new European Cystic Fibrosis Stem Cell Biobank. The HIT-CF project in its current form officially comes to a close in the course of 2025. This means that all biological materials that were collected during the project, such as organoids, need to be destroyed, unless a new consent is given by whoever the organoids originate from. As it would be a pity for these unique materials to go to waste, the consortium is trying to reach as many participants as possible to obtain a new consent.

HIT-CF is a truly historic achievement as it creates new avenues of drug development for patients with ultra-rare diseases. The close collaboration of the patient community, caregivers, academia and regulators to develop drugs in an affordable way is unprecedented. We look forward to sharing the results of the CHOICES trial as soon as they become available. Be sure to keep an eye on the HIT-CF website and newsletters for the latest updates. ”

Publications & conference presentations

Arising from ECFS-CTN projects

Name	Description	Link
Bridging the Gap: Challenging Lung Infections and Clinical Trial Development in CF	Short article in the Journal of Cystic Fibrosis from the ECFS-CTN Investigator Trial Committee.	Read the article here
“Other bacteria” book chapter	In the ECFS book “Inflammation and Infection”.	Read the article here
A pan-European perspective: guidance for the use of chest CT in children and adults with cystic fibrosis	Conference abstract and poster at NACFC Boston September 2024, from the Imaging Standardisation group.	Read the article here
E-Learning within the ECFS - a multidisciplinary cross-sectional survey	Conference abstract and poster at the 2024 ECFS conference, from the ECFS Education committee (of which ECFS-CTN is a member).	Read the article here
Chest computed tomography imaging practices of people with cystic fibrosis: insights from radiologists, radiographers, and pulmonologists	Conference abstract and poster at the 2024 ECFS conference, from the Imaging Standardisation group.	Read the article here
Scanning the landscape: ECFS Clinical Trial Network: CT imaging monitoring strategies	Workshop Session Presentation at the 2024 ECFS conference in the session: Diagnosing and Screening for Complications.	Not publicly available
‘Giving me the reins’: assessing quality of life from the patients’ point of view	Conference abstract and poster at the 2024 ECFS conference, from the Standardisation PROMs group.	Read the article here
My view counts too! A perspective centred on children aged between 2 and 5 years of quality of life with cystic fibrosis	Conference abstract and poster at the 2024 ECFS conference, from the Standardisation PROMs group.	Read the article here
Novel patient-reported outcome questionnaire to assess quality of life of adolescents and adults with cystic fibrosis	Conference abstract and poster at NACFC Boston September 2024, from the Standardisation PROMs group.	Read the article here
New patient-reported outcomes for children with CF aged 2 to 6 and their parents	Conference abstract and poster at NACFC Boston September 2024, from the Standardisation PROMs group.	Read the article here
ECFS-CTN SOP for certification for transepthelial electrical measurements with the Ussing Chamber	A standard process for measuring the quality of a cell culture technique.	Read the article here
ECFS-CTN SOPs for sputum induction for expectorating and non-expectorating young children, older children and adults	Four documents describing standard processes for obtaining induced sputum in different groups of people with CF.	Read the article here
ECFS-CTN SOP for isolation, cultivation and application of primary respiratory epithelial cells obtained by nasal brushing	A standard process for growing cell cultures from cells obtained by nasal brushing.	Read the article here

Financial support to sites

Increasing & maintaining research capacity

A new grant in 2024

Continued Research Capacity (CRC) 2 award

In 2024, the CFF kindly awarded a continuation of funding to ECFS-CTN, worth $3.3 million over 3 years. The new award, called CRC2, will help maintain capacity at sites and the CTN coordinating centre, and will support training and software.

We are all extremely grateful to the CFF for providing this support!

The CFF previously supported ECFS-CTN with the following grants:

- Additional Research Capacity (ARC) award ($3 million, 2017-2020)
- Continued Research Capacity (CRC) award ($3.5 million, 2021-2023)

Financial report 2024

Income, funding & outgoings

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 2024: CFF and European patient organisations (from France, Germany, UK, Italy, Belgium, the Netherlands, Luxembourg, Switzerland, Ireland and Poland). We also thank CF Europe for coordinating the contributions from national patient organisations.

Financial report 2024

Income, funding & outgoings

Reflects the period 1 Jan – 31 Dec 2024:

Income & funding	Euro €
ECFS Support	100,000
National CF associations	118,037
Services to companies	124,905
Core Facilities	45,559
TMS license	3,500
CFF CRC grant used in 2024	663,589
Total Income & funding	***1,055,590***

Outgoings	
Human resources [1]	447,570
RC training (including travel) [1]	19,675
Travel / Meetings	44,635
IT and office equipment	1,383
Corporate design	4,765
Server	15,735
Legal advice	1,815
Other	1,246
Software Development [2]	33,375
Site RC support [2]	418,123
IIT study monitor [2]	80,410
Total Expenditures	***1,068,732***
Year result	**-13,142**

Abbreviations: CFF = Cystic Fibrosis Foundation, CRC = continued research capacity, RC = research coordinator, TMS = trial management system software

1 Partially paid from the Cystic Fibrosis Foundation (CFF) CRC grant

2 Paid in full from CRC grant

Appendix

Studies supported by ECFS-CTN in 2024

GENETIC THERAPY

New — A phase 1 study evaluating safety and tolerability of RCT2100 inhaled mRNA in people with and without CF (Recode Therapeutics RCT2100-101)

New — A phase 1 study to test if BI 3720931 gene therapy is tolerated and if it improves lung function in people with CF (Boehringer Ingelheim 1504-0001; Lenticlair™ 1)

Phase 1 safety testing of VX-522 inhaled mRNA in adults with CF and a CFTR genotype not responsive to CFTR modulator therapy (VX21-522-001)

RESTORE CFTR FUNCTION

New — Phase 2 efficacy and safety testing of dirocaftor/posenacaftor/nesolicaftor in adults with CF (UMC Utrecht CHOICES)

Phase 3 open label testing of the triple combination VX-121 (vanzacaftor)/tezacaftor/deutivacaftor in children with CF aged 1-11 years (VX21-121-105)

Phase 3 testing of long term ivacaftor treatment in children with CF aged less than 24 months and who have a CFTR variant known to respond to ivacaftor (VX15-770-126)

Phase 3 efficacy and safety testing of VX-121 combination therapy in people with CF who have one F508del mutation and one minimal function mutation (VX20-121-102)

Phase 3 efficacy and safety testing of VX-121 combination therapy in people with CF who have one of the following combinations of mutations 1) two F508del mutations, 2) one F508del mutation and one gating or residual function mutation, 3) no F508del mutation and at least one mutation responsive to triple combination therapy (VX20-121-103)

Phase 3 open-label extension observation of long-term combination therapy with VX-121/tezacaftor/deutivacaftor in people with CF (VX20-121-104, parent studies: VX20-121-102 and VX20-121-103)

Phase 3 open-label long-term efficacy and safety testing of Kaftrio in people with CF aged 2 years and older (VX20-445-112)

Phase 3 efficacy and safety testing of Kaftrio in people with CF aged 6 years and older with a non-F508del mutation that is responsive to Kaftrio (VX21-445-124)

Phase 3 open label testing of long term treatment with Kaftrio with people with CF with non-F508del CFTR variants (VX21-445-125)

Phase 3 open label testing of long term treatment with the triple combination vanzacaftor/tezacaftor/deutivacaftor in people with CF aged 1 year and older (VX22-121-106)

Phase 3 open-label testing of Kaftrio in children with CF aged 12 to less than 24 months (VX22-445-122)

ANTI-INFECTIVE

Early phase 1b/2a safety and efficacy testing of nebulised phage treatment of chronic lung infection with *Pseudomonas aeruginosa* in people with CF (BiomX BMX-04-001)

Finding the optimal regimen for *Mycobacterium abscessus* treatment (University of Queensland, FORMaT)

ANTI-INFLAMMATORY

New — Phase 2 safety and efficacy testing of BI 1291583 in adults with CF bronchiectasis (Boehringer Ingelheim 1397-0013; Clairafly)

ENaC INHIBITOR

New — Phase 2 testing of effiacy, safety and how the body acts on the drug ETD001 in people with CF (Enterprise Therapeutics ET-ENAC-03)

PHYSICAL ACTIVITY

New — Yoga outcomes get assessed in CF (Royal Brompton Hospital, YOGA-CF)

NUTRITIONAL /GASTROINTESTINAL

New — Acceptability of a new CREON formulation containing pancreas powder gastro-resistant pellets (Meda Pharma PANC-GRP-3001)

Appendix

Studies supported by ECFS-CTN in 2024

OTHER

New	Understanding gut symptoms in people with CF (University of Nottingham, GRAMPUS-CF)
New	A natural history study of exocrine pancreatic function in infants with CF less than 12 months of age (VX24-445-130)
New	Establishing natural history in an advanced New CF care era (RCSI, ENHANCE)
	A longitudinal study on the impact of treatment with Kaftrio in the real world on people with CF in Europe (VX-CFD-005)
	Artificial intelligence to control acute pulmonary exacerbations in cystic fibrosis (Royal Papworth Hospital, ACE-CF)
	Quality of life in people with CF treated with Orkambi or Symkevi and their primary caregivers in the UK (VX20-CFD-004)
	A trial to see if people with cystic fibrosis taking Kaftrio have changed respiratory function after reducing nebulised mucoactive therapies (Alder Hey Childrens Hospital, CF STORM)
	Covid-19 antibody responses in CF: a study to measure antibodies to SARS-CoV-2 in blood samples from people with CF (CAR-CF)
	Real world clinical outcomes with novel modulator therapy combinations in people with CF (RCSI, RECOVER)

Clinical Trials Network

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