2023/
Annual Report

Clinical Trials Network

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Contents

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2023 OUR YEAR IN NUMBERS

1399 PEOPLE with CF newly enrolled into trials

Feasibility checks for **11** trials

x12 protocols

from 11 sponsors

reviewed by people with CF, their families, doctors, research coordinators & statisticians.

3 EU projects ongoing

1906 enrolled into CAR-CF COVID-19 antibody response in CF

to measure **Covid-19** antibodies

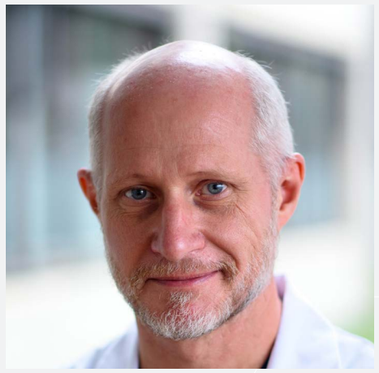
25 active trials supported

- CFTR modulator (17)
- Genetic therapy (mRNA) (1)
- Anti-infective (2)
- Others (5)

104 Happy People at face to face training and meetings in June 2023

7 scientific **PUBLICATIONS & POSTERS** published

Message from the CTN Director



Lieven Dupont

We are delighted to present the 2023 Year Report of the European Cystic Fibrosis Society-Clinical Trials Network (ECFS-CTN). This report gives you an overview of our work in 2023 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 57 sites across 17 countries in Europe. 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 that was presented at the recent Steerco Meeting in Brussels. Although the landscape of CF therapy and CF RCTs has shifted, there remain numerous opportunities for clinical studies (both commercial and investigator-led studies) that warrant an expansion of our current network.

2023 was yet another busy year: during 2023 we actively supported 25 trials, reviewed 12 protocols, and performed 11 feasibilities. Most active studies were assessing CFTR modulators and most studies were phase 3. Recruitment into these studies would not have been possible without people with CF taking part in clinical trials facilitated by all our research teams across Europe. ECFS-CTN is fortunate to be able to work with them and their commitment and support remains vital to ensure all people with CF have effective treatments in the near future.
The Investigator Trial Committee in the CTN continued to oversee the investigator-led CAR-CF study across our European sites. By the end of 2023, 42 sites in 13 countries were open to enrolment and 1906 people with CF were enrolled. This excellent result demonstrates the agility of the CTN to develop and execute similar investigator-led studies addressing important questions for the CF community. In 2023, we continued our collaboration with the ECFS Patient Registry (ECFSPR). Combining our strengths will help us to perform more research projects in the future.
We are indebted to the patient organisations and people with CF who provide us 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, 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 sub-groups have been working hard to develop recommendations and guidance for CT and MR imaging that could potentially be adopted for research and clinical purposes and we are looking forward to the publications of these recommendations. The ECFS-CTN Training Committee led by Olaf Eickmeier brought together 104 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 many of our sites, the CTN Core Centre in Leuven, and the CAR-CF study.
We would like to gratefully acknowledge the current and previous members of the CTN Executive Committee for their continued advice and energy that was and is instrumental in guiding the direction of the CTN: Damian Downey, Dario Prais, Philippe Reix, 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. 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.

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

Lieven Dupont
current ECFS-CTN director

ECFS-CTN

Organisation

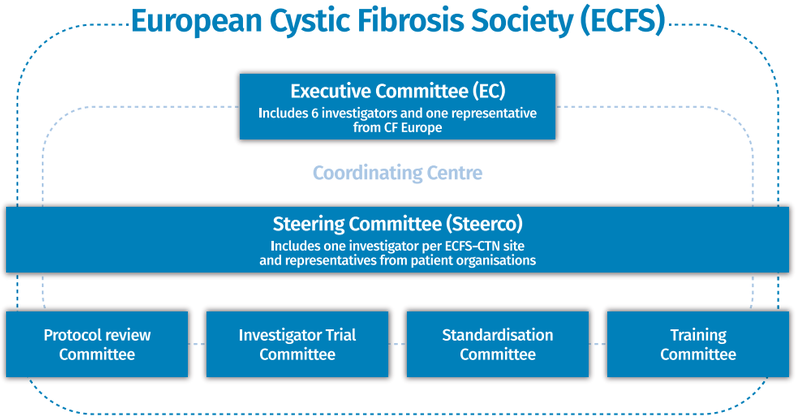
Our mission

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.

ECFS-CTN is made up of 57 sites in 17 countries and a central coordinating centre in Leuven, Belgium.

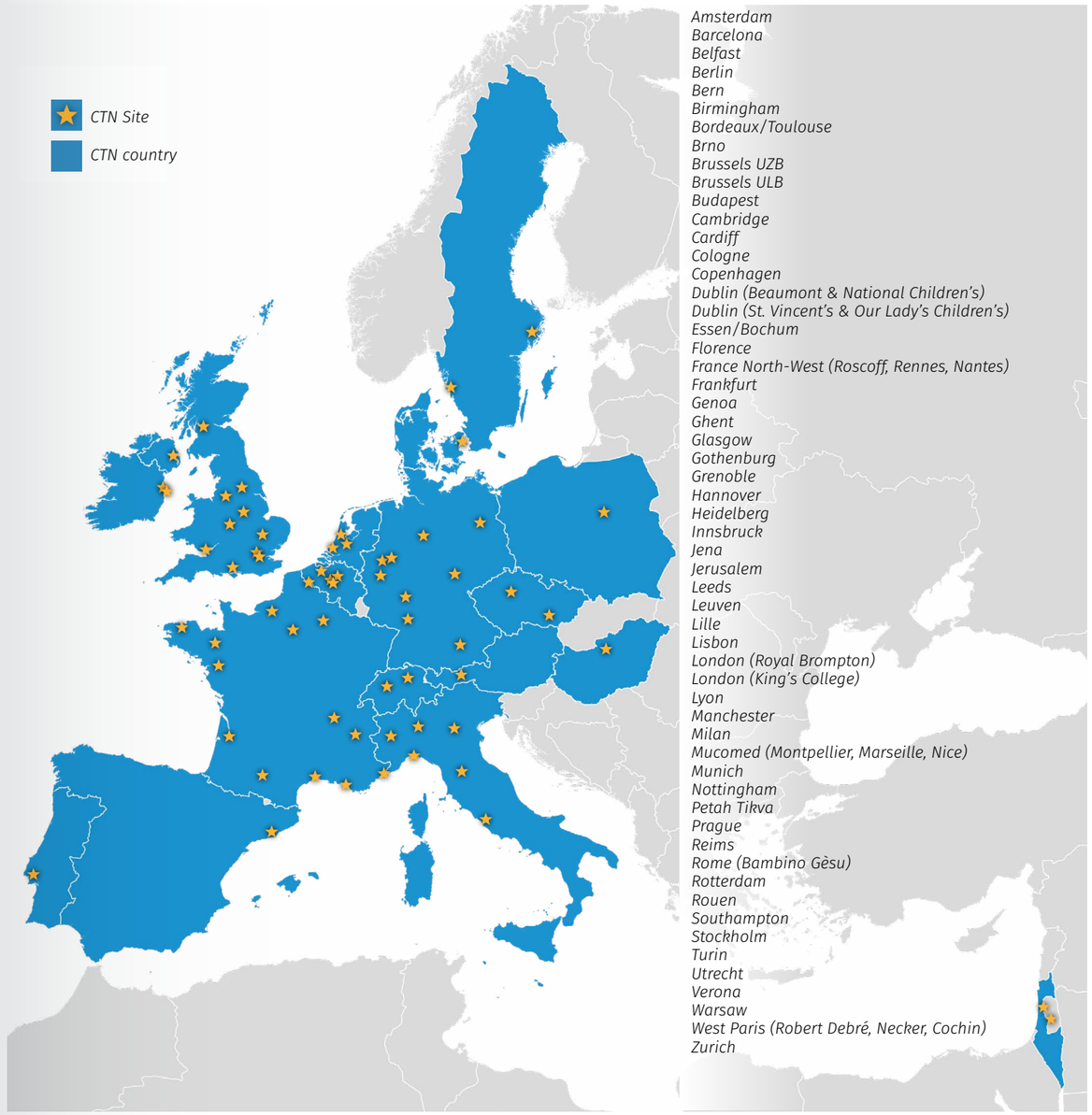


ECFS-CTN is run by:

- 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

The executive team in 2023

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.

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

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.

Thank you !

to Damian, Dario and Philippe

who ended their terms in December 2023

CTN activities

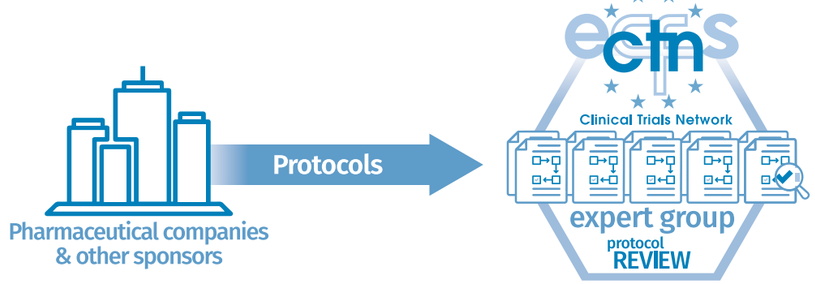
Supporting new trials

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 2023, we reviewed 8 commercial protocols from 7 different companies.
We also reviewed 4 academic protocols.



The ECFS-CTN asked for clarifications or modifications for 6 protocols before approval. In total 10 protocols were approved, 1 was deferred, awaiting a second round. 1 protocol was not approved by ECFS-CTN.

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 2023, we coordinated 11 feasibility checks (6 sponsors).

Clinical Trials Network

CTN activities

Clinical trials in 2023

Trials in ECFS-CTN member sites

Across ECFS-CTN there were 25 studies active during 2023. Most studies were assessing CFTR modulators and most studies were phase 3.

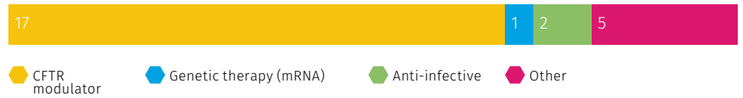
You can find a full list of the studies we supported in the appendix.

These graphs describe the 25 studies active in 2023, by clinical trial phase, by age group enrolled and by therapy investigated.

Clinical trial phase

Age group enrolled

Therapy investigated



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

CTN activities

Clinical trials in 2023

a clinical TRIAL

In the words of people with CF who participated

Jonas, 14, is an adolescent with CF from Switzerland who participated in a trial for a CFTR modulator triple therapy.

“ My older sister is also affected by CF and has already benefited greatly from Trikafta. Her health has improved a lot through this therapy. So it was obvious that I also wanted to get this medicine very quickly. It was a little extra work for me, and I always looked forward to the nice “study team”. I didn’t have to go to school on the days of the study visit.

At the beginning I found the questionnaire difficult to understand, but after a short time I almost knew the questions by heart. The most difficult thing for me was swallowing the “big” tablets, but I quickly learned that too. I have already taken part in many studies, my mother always said, even if I can’t benefit directly from them, someone can certainly benefit from it. And for this reason I will also take part in further studies.”

Alice, an adult with CF from Portugal, explains why she participates in clinical trials.

“ The reason is simple.

We come into this world because someone wanted us to, and luckily or unluckily we are given burdens that we didn’t ask for, but that we live with, and Cystic Fibrosis is a heavy one.

Choosing to live is a privilege, it’s taking control for the first time and “forever”, if that expression means anything.

There’s no measure of time that beats the time I’ve been given. Every day that I am able to walk from my home to my destination, every time I meet a new person, the smiles I collect and plant, are a victory.

It’s a gift to live at a time when the scientific community lives and works to let us live a little longer.

To be grateful for the life I have now is to continue participating and allowing others after me the possibility to choose to be here too.

For a life with time! Thank you.”

CTN activities

Clinical trials in 2023

a clinical TRIAL

In the words of people with CF who participated

Andreas, an adult living with CF from Belgium, participated in a trial for a CFTR modulator therapy.

Why did you participate in the trial?

I always try to participate in studies, because as a patient without a medical or pharmaceutical degree, I consider it my moral duty to put the only effort I can into the progress and advancement in the medical field. The Kaftrio study and its successor were extra motivating because of the promising results heralding from abroad.

Did it take a lot of time and effort to participate in the trial?

No, the latest study is just about taking 2 pills every morning and a considerable amount of time I don’t have to put into other forms of therapy normally associated with my disease. It has saved me time on a daily basis, especially considering the extra efforts I can undertake on other and different aspects of life. The regular visits to the hospital are sometimes more of a nuisance, but I’m always glad to be able to check in with the study nurse.

Would you participate in another trial in the future?

For the first time in my life, I would doubt it. For the simple reason that it would probably mean pausing or maybe even stopping with my current treatment with Kaftrio and risking going back to my previous (much worse) health condition. If not, I would furthermore seriously consider the impact on my organs of all these heavy meds. Luckily I can trust and rely on the medical team of my hospital to inform me correctly.

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 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.

By the end of 2023, 42 sites in 13 countries were open to enrolment and 1906 people with CF were enrolled.

You can find the up-to-date number of recruited patients per site on https://www.ecfs.eu/ctn/projects/CAR-CF. The first results are expected in 2024.

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.

CAR-CF

Timeline for a clinical trial

January 2020	July 2020	Aug-Dec 2020	February 2021	March 2021	May 2021	June 2021	July 2021	2022 & 2023	2024
Plan to setup a CTN Investigator Trial Committee (ITC)	ITC first meeting – decided to launch CAR-CF study	Protocol review and feasibility	Country leads selected, regular teleconferences started	First ethics submission	First site activation	First person with CF included	Investigator meeting	Recruitment ongoing	Planned study end and publication of results

CTN activities

Our Covid-19 response

Eimear Conroy

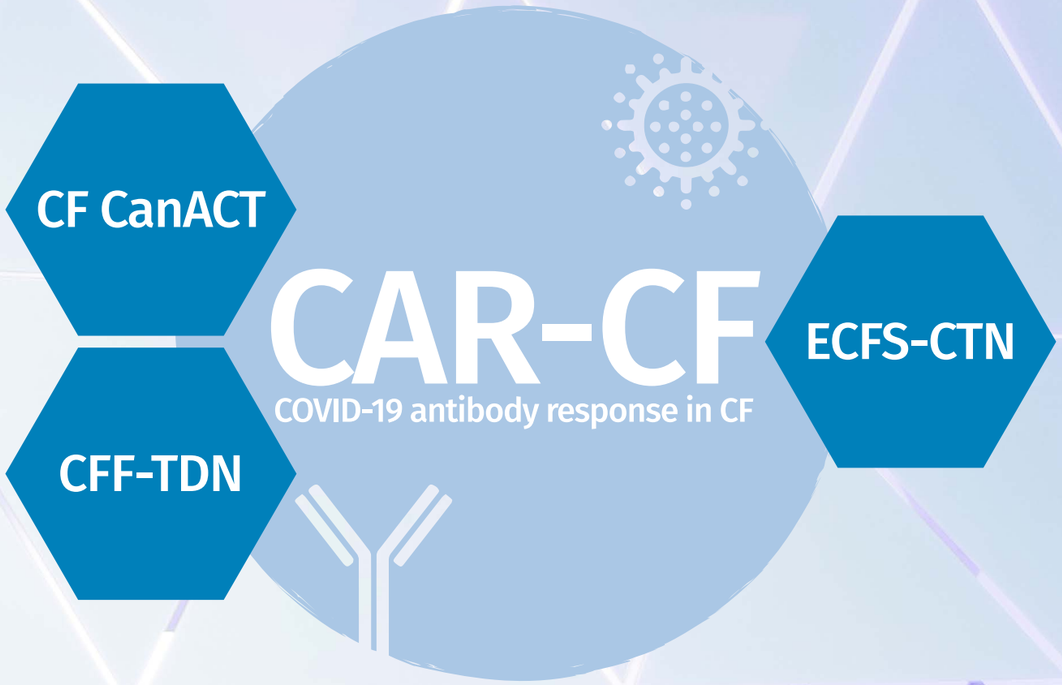
Eimear Conroy, a researcher in Belfast, explains how CF samples are tested in the lab.

“ I am a research technician in the CAR-CF central laboratory team in Queen’s University Belfast (QUB). I work closely with Dr Vanessa Brown, who manages all aspects of laboratory operations for CAR-CF at QUB. I have been involved in sending kits for taking and storing blood samples to sites, the transportation and management of samples and the maintenance of CAR-CF laboratory documentation. Towards the end of 2023, I started testing samples for signs of SARS-CoV-2 infection (serology testing for antibodies). I previously worked on the research and development of SARS-CoV-2 serology assays throughout the pandemic, so it is brilliant to see these platforms utilised in clinical trial research.

Throughout the entire study, we will receive and process over 10,000 samples collected from 42 sites across Europe. Once shipments are organised and samples are received into the central laboratory, they are logged into the sample management software and stored securely at the central laboratory to await testing.

Initially, I performed a pilot study in which we evaluated different methods to test for SARS-CoV-2 antibodies. From the data generated, we chose a method called a “multiplex platform” which can detect antibodies against SARS-CoV-2 Nucleocapsid, S1 RBD and Spike proteins in a single serum or plasma sample. This will tell us whether the antibodies in a blood sample are from vaccination or from natural infection with the virus.

I look forward to seeing how the CAR-CF data will contribute to CAR-CF research and help people living with CF. ”



Damien,

an adult with CF from France is participating in CAR-CF for several reasons:

“it advances our knowledge”

“it doesn’t cost anything in terms of time, clinic visits and tests”

“other patients are also participating”

“I’m happy to participate and am motivated for future trials”

Closer links with the ECFS Patient Registry

Combining our strengths

Stronger together

In 2023, we continued our collaboration with the ECFS Patient Registry (ECFSPR). As special projects of ECFS, our two groups have complementary expertise. Combining our strengths will help us perform powerful research projects.

In 2023, we:

- launched our new aligned visual identities to show that we are sister groups within ECFS, who work closely together.
- had two in-person scientific meetings to advance joint projects and understand how we can combine our strengths.
- had a joint staff training day on scientific writing skills.

Closer links with the ECFS Patient Registry

Combining our strengths

Back row (left to right)
Veerle Bulteel, Fiona Dunlevy, Lieven Dupont, Anne Verbrugge, Damian Downey, Andreas Jung, Egil Bakkeheim, Uros Krivec

Front row (left to right)
Laura Kirwan, Domenique Zomer, Kate Hill, Katia Reeber, Jacqui Van Rens, Annalisa Orenti, Lutz Naehrlich

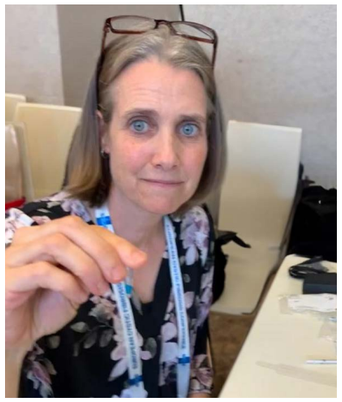
CTN activities

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. This year, we brought together 104 investigators and research coordinators for a day of lectures, discussions and training.

The programme included training on sweat test, genetic therapies and early phase trials. We also had an important panel discussion about the participant experience of taking part in clinical trials. Our special thanks go to Valerie Storms, who lives with CF and shared her valuable experience of trial participation.



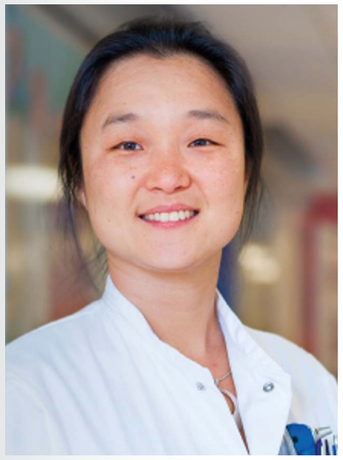
Expert colleagues from our partner clinical trials network in the USA gave a hands-on workshop on sweat-testing. Thanks to Edith Zemanick (pictured) and Mary Cross from the USA CF Foundation's National Resource Center for Sweat Testing in Colorado, USA for joining us. Thank you also to Bryce McEuen and his team from ELITech for supplying equipment and their expertise.

CTN activities

Our work

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. We recently provided expert advice on consultations about computerised systems in clinical trials, a new EMA project about clinical trials called ACT EU, and about trials with only one treatment group (single arm trials). We are committed to checking that new European rules about medicines development are adapted to rare diseases such as CF.



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

Dr. Hettie Janssens, is a pediatrician from Rotterdam in the Netherlands. She represents ECFS-CTN at the European network of Paediatric Research at the European Medicines Agency (Enpr-EMA).

" Enpr-EMA is a group of research networks, investigators and centers who are performing clinical studies in children, enabling networking and collaboration within and outside the EU. It acts as a platform for sharing good practices for high quality ethical research in children.

The ECFS-CTN is member of the coordinating group as a category 1 member, which means that it has research experience and ability, network organisation and processes, scientific competences and capacity to provide expert advice, quality management, training and educational capacity to build competences and public involvement. Enpr-EMA has many different activities that aim to promote research in medicines for children.

In 2023, I represented ECFS-CTN at the in-person Enpr-EMA two day meeting. All the different working groups presented their work. For instance one group works on ways to address the cross-border challenges of multinational paediatric clinical trials. Other working groups work on improving the role of the paediatric research nurse across Europe, or on defining criteria for good quality paediatric clinical trial sites. Another working group is developing guidance to facilitate inclusion of children in cross-border clinical trials.

Members of the Enpr-EMA can also give input on the regulatory activities and pharmaceutical legislation of EMA to speed up the development and introduction of medicines for children. If members of ECFS-CTN are interested to be involved in these activities, do not hesitate to contact us. "

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.

We published several SOPs and papers in 2023, see the Publications section (p. 30) for more details.

Central Reading Core Facilities for Lung Clearance Index and Lung Imaging

We make sure that all our trial network 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 some “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

Patient-reported outcomes

The Standardisation Committee & a specialist team continued to work on the analysis of the new patient-led CF-specific quality of life questionnaire: the PRO-CF. The researchers checked the structure and content of the tool. It is a long complicated process to make sure that the questionnaire can be used in clinical trials. This phase of the testing was made possible with the help of the team led by Lieven Dupont, Trudy Havermans and the KU Leuven team in Belgium. Thanks also to CF Europe and especially to the 143 people with CF who made this phase of testing possible!

To refine the questionnaire, more testing was then done by a specialist team: Simona Caldani, Emmanuel Devouche, and Emilie Cappe from the Université de Paris, France. The tool is now in version 3 and we are testing it against other PROMs (another part of the process to make it ready).

Alongside this testing, several linked studies are adapting the tool for children aged 6 to 12 years (completed initial testing with 140 children) and aged 2 to 5 years (completed testing with 91 children) and a parent-report tool to complement the infant/toddler one (completed testing with 127 parents). A huge thank you to all the children and their parents who made this possible. Testing of all these tools will continue into 2024 to make them ready for testing in wider studies.

CTN Imaging in CF Special Interest Group

2023 saw lots of change in this group as the lead Prof Harm Tiddens stepped down after over 30 years of CF research and clinical practice in Rotterdam, Netherlands. A huge thank you to Harm for all his years of dedication and expertise. He continues to oversee the work as Isabelle & Kate continue to coordinate the 3 imaging sub-groups: developing recommendations/guidance for CT and MR imaging that could potentially be adopted for research and clinical purposes. Each group is led by a specialist in the field. Our special thanks to each of the following groups:

- CT monitoring strategies: led by Prof Michael Fayon (Bordeaux, France) working in partnership with the ERS and the European Cancer Organisation
- MRI protocols: led by Prof Pierluigi Ciet (Rotterdam, Netherlands)
- Low dose guidance in CT imaging: led by Prof Michael Maher (Cork, Ireland)

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 worth $3 million for 2021-2023 to maintain the capacity at sites and the CTN coordinating centre and to support training and software. We are all extremely grateful to the CFF for providing this support!

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 have been 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.

In 2023, the team worked hard to prepare the (re)submission into CTIS (Clinical Trials Information System), a procedure which is necessary to obtain permission to run a trial in Europe, while making all practical arrangements. The first participants are expected to be included in CHOICES in 2024.

https://www.hitcf.org

European research projects

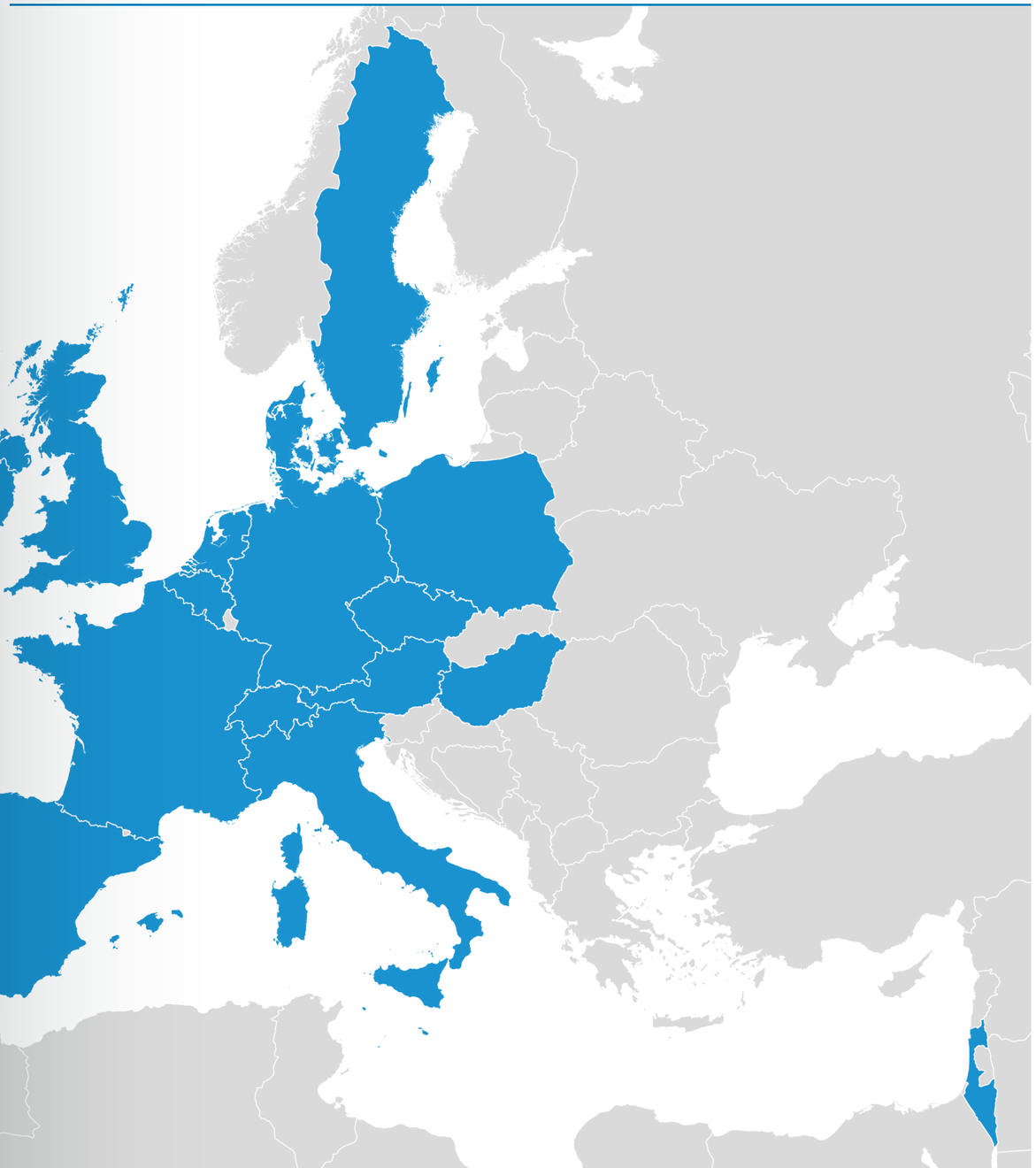
News from HIT-CF

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 2023, the HIT-CF project underwent a restart. Due to several setbacks (Covid-19, pharmaceutical partners dropping out, and rejection of the protocol for the CHOICES trial by the Clinical Trials Information System or CTIS), the project experienced significant delays, and December 2023 was actually the official end date for HIT-CF. So, the team had to pull out all the stops on various fronts. They engaged with the European Commission, the initial funder of HIT-CF, to obtain an extension of the project. Meanwhile, it was crucial to secure additional funding to guarantee the start-up and continuation of CHOICES, and we are very pleased to say that several generous investors were willing to support Fair Therapeutics. Moreover, the team worked hard to prepare the resubmission into CTIS, a procedure which is necessary to obtain permission to run a trial in Europe. While we are still waiting for approval by CTIS, which is expected in 2024, the CHOICES protocol has already been approved by the MHRA (Medicines and Healthcare products Regulatory Agency) in the UK, as these are separate procedures since Brexit. This was of course very good news which was welcomed with great enthusiasm by the consortium. Nevertheless, out of practical and ethical considerations, the trial won’t start in the UK until the trial is also approved in the EU.

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. If we can continue at this pace, CHOICES can start by the end of the spring of 2024. 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
ECFS-CTN Measurement of Height SOP	A standard process to measure a person's height.	Download here
ECFS-CTN Measurement of Weight SOP	A standard process to measure a person's weight.	Download here
ECFS syllabus for the multidisciplinary team and wider field: a guide for comprehensive education	As part of a wider ECFS Education project, the ECFS-CTN training committee created a training syllabus for those involved in clinical trials.	Read abstract here See poster from the European Cystic Fibrosis Society conference, 2023
Giving a voice to children: what does QoL mean to children with CF?	A conference presentation at the 2023 European Young Investigator Meeting about the PROMs project.	not available
Hear my voice: research by and with children with cystic fibrosis	A scientific publication about the paediatric PROMs project.	Read the article here
Standardising European Imaging Practice: ECFS-CTN and ERS	Conference presentation and poster at the 2023 North American CF conference from the Imaging Standardisation group.	See the conference poster here
Inequal access to CFTR modulators across ECFS-CTN countries	Conference poster at the 2023 European Cystic Fibrosis Society conference.	See the conference poster here

Clinical Trials Network

Financial report 2023

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 2023: CFF and European 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.

Financial report 2023

Income & expenses

Reflects book-keeping year 1 Jan – 31 Dec 2023:

CTN - Income & expenses 2023	Euro €
ECFS Support	100,000.00
National CF associations	118,037.00
Services to companies	186,380.00
LCI Core Centre	50,129.00
EU projects	15,209.00
Trial Management System license	3,493.00
Total Income	***473,248.00***
Travel / Meetings	37,450.00
Human resources	285,240.00
Computer & Software / Office equipment	3.019.59
Publication	5,835.72
Training - Research Coordinators Support	21,329.00
Software Development / Maintenance	3,750.00
Dedicated server	14,884.00
Total Expenditures	***371,508.31***
CTN result 2023	
Year result	**101,739.69**

Appendix

Studies supported by ECFS-CTN in 2023

GENETIC THERAPY

New Early 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 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)

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

New 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 2 study of galicaftor/navocaftor/ABBV-119 combination therapy in people with CF with 1 or 2 F508del mutations (Abbvie M19-771)

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 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 extension observation of long-term treatment with tezacaftor in combination with ivacaftor in people aged 6 years and older with 1 or 2 F508del mutations (VX17-661-116)

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)

Phase 3b open-label testing extension testing of Kaftrio in people with CF (Vertex, VX19-445-115; parent study =VX18-445-109)

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

Phase 3b open-label safety and efficacy testing of the effects of long-term treatment with Kaftrio in people with CF aged 6 years and older with 1 F508 del mutation and 1 minimal function mutation (Vertex VX20-445-119)

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 (Vertex VX21-445-124)

A long term evaluation of how Kaftrio impacts people with CF in Europe (VX20-CFD-005)

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

Phase 3 open-label long-term safety evaluation of Kaftrio in people with CF (VX20-445-121)

ANTI-INFECTIVE

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

New Finding the optimal regimen for Mycobacterium Abscessus treatment (FORMaT)

OTHER

New Artificial intelligence to control acute pulmonary exacerbations in cystic fibrosis (ACE-CF)

New Quality of life in people with CF treated with Orkambi or Symkevi and their primary caregivers in the UK (Vertex VX20-CFD-004)

A trial to see if people with cystic fibrosis taking Kaftrio have changed respiratory function after reducing nebulised mucoactive therapies (the CF STORM trial)

Covid-19 Antibody Responses in Cystic Fibrosis: 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 (RECOVER)

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

Clinical Trials Network

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