Newsletter May 2022
Issue 69

Letter from the President

Dear Friends and Colleagues,

I hope this letter finds you well.

End of March, we organised our first in-person conference after two years of disruption linked to the COVID-19 pandemic. More than 175 delegates were very happy to meet again at the 17th ECFS Basic Science Conference in Albufeira, Portugal. I take this opportunity to heartily thank Carlos Farinha, Nicoletta Pedemonte and Jeff Brodsky who chaired this conference. Their efforts and commitment during these past 2 hectic years were outstanding. Last year, they organised a series of webinars that helped keeping the CF basic science community updated and close together**.** This year, we resumed the in-person ECFS Basic Science Conference and they prepared a high-quality programme that prompted many lively discussions.

It is with great pleasure that we will welcome you in a few weeks to the 45th European Cystic Fibrosis Conference in Rotterdam, the Netherlands. The digital conferences we organised over the last two years were a great success. But after 2 years of digital events, we all long to meet again in person, and Rotterdam will be the perfect place for the CF community to gather together again.

The Scientific Committee has put together an outstanding programme and you will have access to more than 75 sessions including symposia, workshops, ePoster sessions amongst others. I would like to thank the Scientific Committee and the Steering Committee for all their hard work over the last year in preparing the programme, selecting the abstracts and organising the various sessions. I would also like to thank all of you who have submitted an abstract to the conference. We are very excited to learn about the new science on cystic fibrosis.

I will be delighted to present the ECFS award at the conference and we will also present the Gerd Döring award. As part of our commitment to support young researchers, three Young Investigators Awards will be presented to young investigators based on the merit of their abstract submissions.

Let me remind you of the ECFS elections, which are taking place this month. There are two vacancies at the Board this year. Trudy Havermans and Isabelle Sermet finish their terms and I wish to express my deepest thanks to them for their contribution to the Board over the past years. Your involvement in the ECFS Board Elections is critical and will contribute to shape the future direction of the Society. To provide leadership to our Society, it is essential that the Board has a strong support from the membership. So please cast your vote here without delay.

In other news, many thanks, as always, to Dr. Henry Ryley for compiling the current references in cystic fibrosis in this Newsletter.

Please contact us if you have news items you would like to have included in future Newsletters or published on our website.

We are very much looking forward to welcoming you in person to the Annual ECFS Conference in a few weeks from now.

Yours sincerely,

Isabelle Fajac, ECFS President.

Inside this issue

ECFS Board Elections

The European Cystic Fibrosis Society Board has two vacancies this year. Prof. Isabelle Sermet and Dr. Havermans finish their terms on the Board and we wish to express our deepest thanks to both of them for their contribution to the Board over the past years.
We have received nominations for four senior figures of the CF community.
The Board should represent the ECFS membership in all aspects of our activities and should be drawn from a number of different European countries and reflect important areas of our activity.
The Society needs you to vote in large numbers so that your support for those elected and appointed to lead the Society is clearly expressed.
The online secured voting poll can be found here. You will find all details on the candidates and also on the current composition of the Board. Please read their short summaries carefully, consider their potential contributions and vote accordingly.

POLLS CLOSE ON FRIDAY 03 JUNE 2022 (18:00 CEST)

Board Member's Job Description.

Ian Balfour-Lynn (UK)

Carlos M Farinha (PT)

Eddie Landau (IL)

Silke van Koningsbruggen Rietschel (DE)

ECFS Annual General Meeting

Save the date!

The ECFS Annual General Meeting will be held during the conference on Friday 10 June 2022 , from 18:30 to 19:45 in room Dock 10A. The Meeting will be organised in person only at the at the Rotterdam Ahoy Convention Centre. We need your active participation and look forward to a great discussion.

ECFS Annual General Meeting

ECFS Annual General Meeting 2022
10 June 2022, Rotterdam, The Netherlands

AGENDA

1. President's Report – Isabelle Fajac
2. Secretary's Report – Isabelle Sermet
3. Treasurer's Report – Trudy Havermans
4. ECFS Board Elections – Isabelle Fajac
5. Update on Journal of Cystic Fibrosis – Patrick Flume
6. ECFS CTN Report – Damian Downey
7. ECFS Standards of Care Report – Kevin Southern
8. ECFS Patient Registry Report – Andreas Jung
9. ECFS Education Report – Daniel Peckham
10. ECFS Working Group Reports:
    - Diagnostic Network Working Group
    - Exercise Working Group
    - Fungal Pathogens Working Group
    - Mental Health Working Group
    - Neonatal Screening Working Group
    - Pulmonary Exacerbations Working Group
11. ECFS Special Interest Groups
    - ECFS Nursing Special Interest Group
    - European CF Pharmacy Special Interest Group
    - European CF Nutrition Special Interest Group
    - European Psychosocial Special Interest Group
12. Presentation of the 2023 European CF Conference

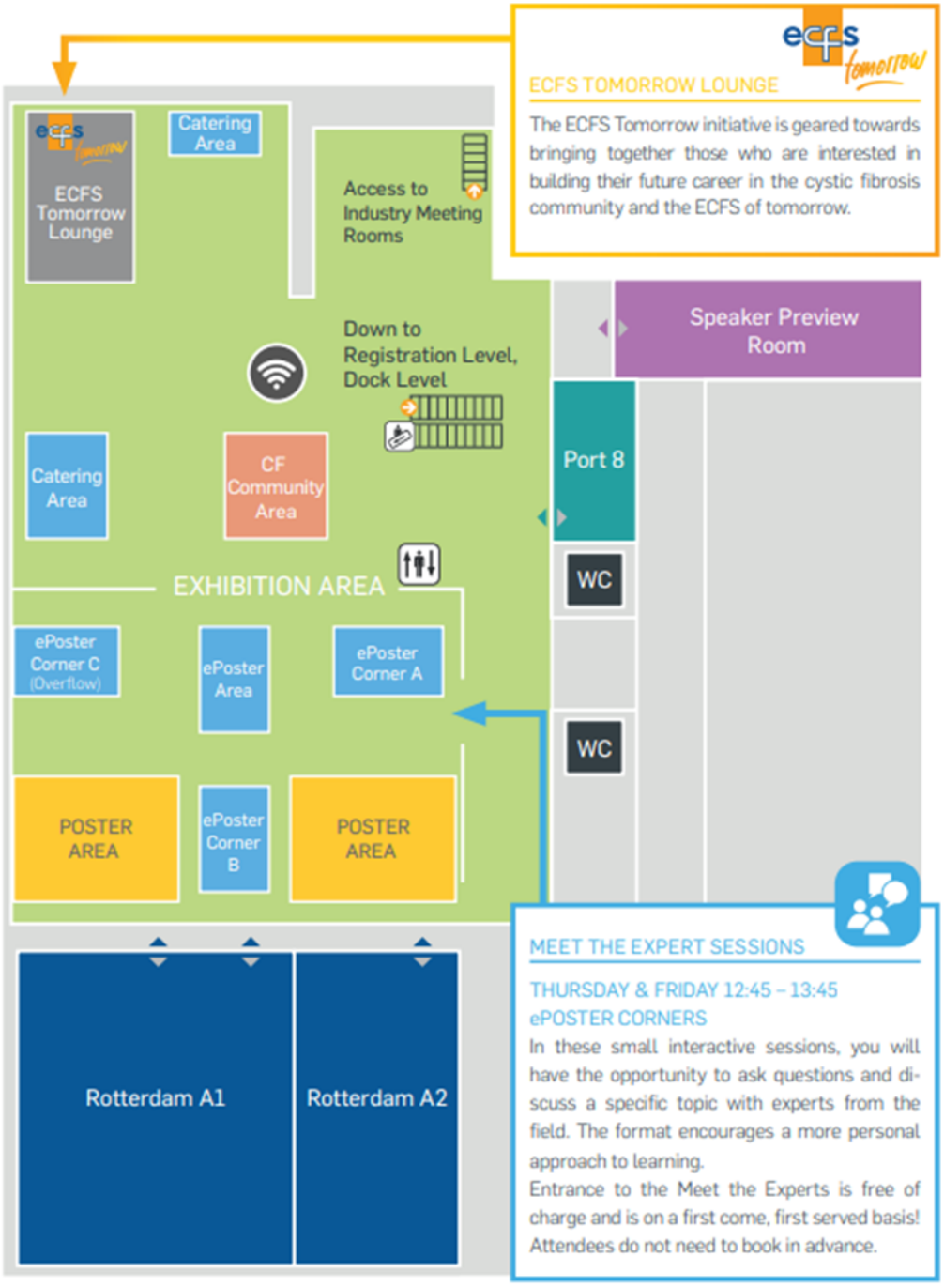
Upcoming Events

- **ECFS Board Summer Meeting**
  7 June 2022
  Rotterdam, the Netherlands
- **ECFS Annual General Meeting**
  10 June 2022
  Rotterdam, the Netherlands
- **ERS Congress** Hybrid format
  4-6 September 2022
  Barcelona, Spain
- **North American Cystic Fibrosis Conference**
  3-5 November 2022
  Philadelphia, PA, US
- **ECFS Winter Meetings**
  January 2023
  Brussels, Belgium
- **ECFS Diagnostic Network WG Meeting,**
  February 2023
  Montpellier, France
- **Basic Science Conference**,
  March 2023
- **46th European CF Conference**
  7-10 June 2023
  Vienna, Austria

Highlights of the Conference

The annual ECFS conference is taking place in Rotterdam, the Netherlands this year. You can find all the details about the scientific programme here.

Below are some highlights of the conference. Looking forward to seeing you in Rotterdam!



Conference App

The Conference App is always very popular as more than 75% of the delegates download and use it. Via the app, you will find all the information on the scientific programme, the exhibitors, the abstracts and ePosters. The ECFS 2022 Conference App is designed to enhance the experience of the participants before, during and after the Conference. Further information is available on the conference website and the App will soon be ready to download.

Meetings and Courses during the 2022 ECFS Conference

TUESDAY, JUNE 7		
08:00 – 17:00	Physiotherapy Short Course – The upper airway: Practical assessment and therapy – From research to daily work practice**	Dock 16
08:30 – 18:00	ECFS Board Meeting*	Dock 17
09:00 – 17:00	Discussing sex and intimacy with patients with CF is not 'opening a box of Pandora', but 're-opening a box of pleasure'! **	Dock 4
12:30 – 17:00	Clinical Nutrition Course – Practical interventions to optimise nutrition. A basic/revision course for 2022**	Dock 2
WEDNESDAY, JUNE 8		
08:00 – 12:00	Physiotherapy Short Course – The upper airway: Practical assessment and therapy – From research to daily work practice**	Dock 16
08:30 – 12:45	ECFS CTN Training and Development*	Dock 14
09:00 – 16:00	ECFS Nursing Special Interest Group Meeting**	Dock 15
09:00 – 17:00	European Psychosocial Special Interest Group (EPSIG) Meeting**	Dock 4
09:30 – 15:00	ECFS Neonatal Screening Working Group Annual Meeting	Dock 13
10:30 – 15:30	ECFS Cystic Fibrosis Nutrition Group Meeting**	Dock 2
11:00 – 12:30	ECFS Patient Registry Executive Committee Meeting*	Dock 8
11:00 – 17:00	ECFS Exercise Working Group Meeting*	Dock 6
12:30 – 14:00	International Physiotherapy Group for Cystic Fibrosis (IPG/CF) Annual Gen-	Dock 16
13:15 – 15:30	ECFS CTN Training Research Coordinators*	Dock 14
13:15 – 17:30	ECFS CTN Steering Committee Meeting*	Dock 5
13:30 – 17:30	European Cystic Fibrosis Pharmacy Group Meeting	Dock 3
14:30 – 17:00	Physiotherapy Case Presentations	Dock 16
15:15 – 16:45	ECFS Patient Registry Scientific Committee Meeting*	Dock 8
16:00 – 18:00	ECFS Fungal Pathogens Working Group Meeting	Dock 13
17:00 – 18:00	PhySIG Launch Event: Meet the new ECFS Physiotherapy Special Interest Group	Dock 16
17:00 – 18:30	Global CF*	Dock 2
17:00 – 18:30	International (Global) CF Registry Group*	Dock 15
17:00 – 18:30	IPG/CF Board Meeting*	Dock 8
17:30 – 18:15	ECFS CTN Executive Committee meeting*	Dock 9

* closed meeting

** extra registration required

THURSDAY, JUNE 9		
12:00 – 13:00	CTN – CFF – TDN – CanAct Meeting*	Dock 17
12:00 – 14:00	UKCFMA Meeting*	Dock 2
12:30 – 13:30	ECFS Patient Registry Annual Report Working Group Meeting*	Dock 10B
12:30 – 13:30	ECFS Physiotherapy Special Interest Group (PhySIG) Meeting*	Dock 1
12:30 – 14:30	ECFS CTN Standardisation Committee meeting*	Dock 5
12:30 – 15:00	ECFS Standards of Care Group Meeting*	Dock 16
13:30 – 14:30	ECFS Patient Registry – Meeting for Interested Parties	Dock 10A
15:30 – 17:30	CFTR2 project*	Dock 5
16:30 – 17:30	ECFS Patient Registry / CF Europe Joint Project Meeting*	Dock 17
18:00 – 19:00	ECFS Patient Registry Software training – Walk in session	Dock 16
18:30 – 20:00	ECFS CTN Standardisation sub-group meeting*	Dock 5
18:30 – 20:00	ECFS Education Committee Meeting*	Dock 2
18:30 – 20:30	ECFS Pulmonary Exacerbation Working Group Meeting*	Dock 17
FRIDAY, JUNE 10		
07:30 – 08:30	ECFS Patient Registry Encounters*	Dock 17
12:00 – 14:00	ECFS CTN Lung Clearance Index Workshop	Dock 3
12:30 – 14:00	ECFS Mental Health Working Group Meeting*	Dock 17
12:30 – 14:30	ECFS Diagnostic Network Working Group Meeting	Dock 10A
12:30 – 14:30	ECFS Patient Registry Steering Group Meeting*	Dock 10B
14:30 – 15:30	Industry debrief & ECFS Meets the Industry*	Dock 17
15:00 – 16:00	ECFS Patient Registry Round Table for Companies*	Dock 2
16:00 – 17:00	ECFS Patient Registry Software training – New Variables and Data Quality	Dock 2
18:30 – 19:45	ECFS Annual General Meeting* – ECFS members only	Dock 10A
SATURDAY, JUNE 11		
13:30 – 15:00	ECFS Scientific Committee Meeting – Vienna 2023*	Dock 10A
14:00 – 15:30	National symposium (in English with simultaneous translation into Dutch): "How close is the future for all with CF"	Dock 1

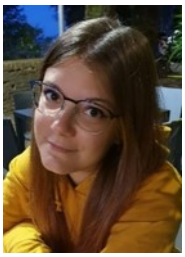
*closed meeting
** extra registration required

ECFS Award 2022

The ECFS Award is given annually to honour a person who has made an outstanding contribution to our basic understanding of cystic fibrosis or to the treatment or care of patients with cystic fibrosis.
This year, the ECFS wishes to acknowledge Prof. Kris De Boeck.
Kris De Boeck has been involved in cystic fibrosis paediatrics care for over 30 years. The development of appropriate diagnosis and care for people with cystic fibrosis has been her lifetime goal and her dedication to patients is boundless. Her leadership as Clinic Head of the Department of Paediatrics in the University Hospital Gasthuisberg in Leuven in Belgium has been outstanding.

Kris is also an international renowned scientist leading many international research programmes on cystic fibrosis and publishing relentlessly to improve knowledge on cystic fibrosis. She has contributed in standardising tests to assess CFTR function and improve cystic fibrosis diagnosis. She has contributed to many important consensus documents on paediatrics care.
Kris was also one of the leaders taking part in developing new CFTR modulators, assessing their use, advocating for early and widespread geographical access and studying them in rare genotypes.
Kris has been a strong advocate of the European Cystic Fibrosis Society and was instrumental in the set-up of the ECFS-Clinical Trials Network which has flourished under her leadership. She has also contributed enormously as an ECFS Board member and then as ECFS President, further developing the society to its leading international role in care, research and education on cystic fibrosis.
Her skills in communication and education are a model for us all and she is an inspiration for the next generation of healthcare professionals in cystic fibrosis.
We are delighted that Kris De Boeck was granted the ECFS Award 2022 and look forward to hearing her talk at the European CF Conference Opening Plenary on 8 June, 2022 in Rotterdam.

Gerd Döring Award 2022



The Gerd Döring Award is an initiative of the European Cystic Fibrosis Society and is given annually to honour an exceptional young European scientist in their early career. The Award includes a monetary donation of 5,000 euro to support research. This year, the Award is given to Alessandra Murabito (IT) in acknowledgement of her work.

ALESSANDRA MURABITO

Alessandra Murabito is a Molecular Biotechnologist who obtained in 2022 her PhD in Biomedical Sciences and Oncology with a specialisation in Functional Genomics applied to Translational Research from the University of Torino, Italy. Since her bachelor's degree, her work has mostly been focused on the development of new therapeutic strategies to target respiratory diseases.

About the research presented at the 45th European CF Conference, Abstract WS18.02

In her paper, **A PI3Kγ mimetic peptide triggers CFTR gating, bronchodilation, and reduced inflammation in obstructive airway diseases** (doi: 10.1126/scitranslmed.abl6328), she demonstrated that a cell-permeable peptide targeting PI3Kγ (PI3Kγ MP) is a promising medicinal product targeting several CF pathological features. The PI3Kγ MP, by inducing a localised and compartmentalised increase in cAMP, acts as a bronchodilator and anti-inflammatory drug, potentially replacing in clinical practice β2 adrenergic receptor agonists which are well known to have severe side effects. Moreover, the study reports that this peptide doubles the therapeutic efficacy of Kaftrio in rescuing the function of F508del-CFTR, indicating that the peptide might represent an avenue for reinstating F508del-CFTR activity close to 100% of that of the wild-type channel, a condition potentially matching that of healthy carriers of CF mutations, as well as to improve CFTR function in patients carrying rare mutations that will become eligible for Kaftrio in the future.

ECFS 2022 – Young Investigators Award

Following the recommendations of the Steering Committee during the abstracts review process, the ECFS wishes to commend the quality of the work presented in the abstracts of some young investigators. The Young Investigator Award (given to a selection of applicants under the age of 35) includes a monetary grant of € 750, free registration to the Conference, and a 2022 ECFS membership subscription. We wish to extend our congratulations to the following Young investigators:

SIMONE AMISTADI

Simone Amistadi is a graduate student working at the CIBIO Department, University of Trento, Italy, in the Laboratory of Molecular Virology headed by Anna Cereseto. He obtained his Master of Science in Cellular and Molecular Biotechnology at the University of Trento in 2020, with a thesis entitled "Precise CRISPR-based genome editing to correct cystic fibrosis mutations". He is continuing his research with the aim of developing novel gene-editing approaches, including the most recent prime- and base-editors, for the treatment of cystic fibrosis.

About the research presented at the 45th European CF Conference, abstract number WS16.02:

Base editing strategy to repair the CFTR 2789+5G>A splicing mutation

The 2789+5G>A mutation is among the fifteen most frequent defects causing cystic fibrosis (CF), identified in almost 1% of CF patients (CFTR2 database). This mutation causes an aberrant splicing which determines the production of a non-functional CFTR protein. In this work, they applied CRISPR adenine base editors to precisely correct the 2789+5G>A mutation.

The most effective base editing strategy was initially set up using a minigene model mimicking the splicing defect generated by the mutation. The sgRNA showing the highest editing efficiency and splicing correction was then validated in CF clinically-relevant models, such as intestinal organoids and primary bronchial epithelial cells. In these models, derived from compound heterozygous patients for the 2789+5G>A mutation, the research team obtained up to 15% A-to-G base correction. The editing correlated with the restoration of correct splicing and functional recovery of the CFTR channel measured as organoids area and short-circuit current experiments.

MATTIJS BULCAEN

Mattijs Bulcaen is a 24-year-old Biochemist trained at KU Leuven, Belgium. During his master´s programme, he combined an R&D company internship with leading a student competition team (Sensus) and graduated in 2020 summa cum laude with congratulations from the exam committee, for which he received the Best Master Student award. His journey as a CF researcher started around the same time as his master thesis in the group of Professor Carlon on gene editing strategies for drug refractory CFTR mutations. Drawn by the biochemical nature of this research and propelled forward by the possible implications for CF patients, he is very eager to continue this exciting research, now as PhD student.

About the research presented at the 45th European CF Conference, abstract number WS16.01:

Correction of the drug-refractory CFTR mutation L227R by prime editing

Gene therapy for CF has been pursued since the early 90s and focused on a gene addition approach, which adds CFTR cDNA to restore CFTR function. Gene correction, on the other hand, allows the restoration of mutations in patient's chromosomes, thereby preserving endogenous gene expression and regulation and possibly providing a permanent cure.

They hypothesised that prime editing (PE) could permanently correct drug-refractory CF-causing mutations, such as L227R (c.680 T>G). Using several PE guide RNA designs, they were able to achieve 19 ± 2,6 % (mean ± SEM) genetic correction in HEK293T overexpressing the L227R CFTR mutant cDNA. DNA correction was next verified at protein level via detection of restored CFTR glycosylation by Western blot, plasma membrane localisation by flow cytometry and ion channel function by halide sensitive YFP quenching. To validate our approach in a more translational model, they delivered PE, together with optimised guides into patient-derived intestinal organoids via lentiviral vectors and measured functional recovery via forskolin-induced swelling, indicating PE-mediated correction of the endogenous CFTR gene.

LISA RODENBURG

Lisa Rodenburg first pursued a degree in biomedical sciences, then later chose to continue her studies at medical school, where she eventually acquired her medical degree. During her studies, she developed an interest for translational research, and her aim is to translate science from the laboratory to patients. She started her PhD studies in 2019 in the group of Jeffrey Beekman and Kors van der Ent, where they use patient-derived organoids to study cystic fibrosis.

About the research presented at the 45th European CF Conference, abstract number WS16.05:

Identification of drugs activating CFTR-independent fluid secretion in nasal organoids based on a high-content screening assay

Her research focuses on the use of patient-derived nasal cells to study respiratory diseases *in vitro*. They have collected nasal cells of approximately 150 people with cystic fibrosis (CF) in their biobank, and they developed a nasal organoid swelling assay to study epithelial fluid secretion. For this project, their aim was to identify compounds that activate alternative ion channels or transporters. These compounds might restore the epithelial fluid balance in people with CF, independent of their CFTR mutation type. They set up a high-content screening assay in nasal organoids and screened an FDA-approved drug library to identify drugs activating non-CFTR chloride channels. This resulted in 13 hit compounds which they selected for further mode of action studies. To investigate via which mechanism these hit compounds induce organoid swelling, they created knockouts of specific ion channels or transporters in nasal cells using CRISPR-Cas9. Future experiments with these knockout cells will reveal the role of these targets in nasal organoid swelling.

2022 ECFS Basic Science Conference – Albufeira, Portugal

The 17th ECFS Basic Science Conference was held in Albufeira, Portugal from 30 March to 02 April 2022. We would like to thank the organisers, Carlos Farinha (PT), Nicoletta Pedemonte (IT) and Jeff Brodsky (US) for putting together a high-quality programme. We welcomed 175 delegates and all were really happy to meet again in person. The abstract book is available on the ECFS website.

Meetings

The 21st Scientific Meeting hosted by the scientific board of the Mukoviszidose e.V. (FGM) will take place on 29 - 30 September 2022 in Schloss Montabaur (Germany).
This year the focus will be placed on innovative therapies in CF especially on CFTR modulation, alternative channels, gene therapy and other anti-infective therapies.
Language: English
Free registration
Abstract submission deadline: 15 June 2022
Registration deadline 15 August 2022

Preliminary programme

Please contact us if you have news items you would like to have included in future Newsletters or published on our website!

Email:
david.debisschop@ecfs.eu

Current References in Cystic Fibrosis

Many thanks to Dr. Henry Ryley for compiling the current references in Cystic Fibrosis that you can download through this link.

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