

## Letter from the President

Dear Friends,

There has been a lot of activity at the ECFS since the last edition of our newsletter and here is a brief review of the special projects, what's new with our society and a few highlights of the upcoming 47th European Cystic Fibrosis Conference taking place in a few days, this year in Glasgow, UK.

The ECFS Basic Science Conference was held in Malta from 20 March to 23 March. Once again, the conference was a resounding success with brilliant speakers and lots of questions and discussions from the audience. We would like to thank the organisers, Alexandre Hinzpeter (FR), Patrick Harrison (US) and Marie Egan (US) for putting together a high-quality programme. The [abstract book is available](#) on the ECFS website.

The ECFS Diagnostic Network Working Group met in Hannover from 8 to 10 February, with Burkhard Tümmler as host. All agreed this was a fantastic meeting scientifically and with excellent networking opportunities. The report of the meeting can be found [here](#).

We are rapidly approaching our annual conference in Glasgow and the final preparations are well underway. The Scientific Committee has put together an outstanding programme and we have some exciting new basic research and results from clinical trials to discuss. You can find the [final programme](#) on the ECFS website. 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 sorting the various sessions. Besides the conference App featuring the programme and other useful information, other initiatives to note include the Interactive Master Class in Clinical CF, some 'meet the experts' sessions organised in the early morning and open to all, and the ECFS Tomorrow lounge which will feature a series of interactive sessions. As part of our commitment to support young researchers, three Young Investigators Awards and twenty travel grants have been awarded to young investigators based on the merit of their abstract submissions. Congratulations to them for their prize! Please don't miss a visit to the "CF Community" booth area in the Exhibition Hall for information on the various ECFS initiatives and programmes.

The ECFS Annual General Meeting (AGM) will be held on Friday 07 June (18:30-19:45, M1) and is open to all ECFS members having a valid 2024 membership. We very much hope that many of you will attend it! The AGM is the moment where members can express their wishes and expectations for the Society. We value your involvement and hope to see more members contribute and input new ideas. More information on the AGM will appear further down in the newsletter.

Many thanks, as always, to Dr. Henry Ryley for compiling the current references in Cystic Fibrosis contained at the end of this Newsletter.

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

This will be my last newsletter as ECFS President and I would like to thank all of you for your commitment and collaboration during my Presidency. It was absolutely wonderful to work with you all during these exceptional times witnessing a revolution in cystic fibrosis care that is both fantastic and challenging. I am delighted to be handing over the reins to Prof. Jane Davies who will begin her Presidency in a few weeks. I look forward to seeing the successes of the Society in the future.

Yours sincerely,

Isabelle Fajac  
ECFS President



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## ECFS Board Elections

The European Cystic Fibrosis Society Board has three vacancies this year. Prof. Barry Plant and Dr. Pavel Drevinek finish their terms and we wish to express our deepest thanks to both for their contribution to the Board over the past 6 years. Prof. Isabelle Fajac will end her tenure as ECFS President this coming June and we are very grateful for her leadership, especially during the difficult years the Society lived during the pandemic. We have received nominations for three senior figures of the CF community. In our constitution, and in the interest of democracy, we do require to have a vote on the three candidates' applications. There is an opportunity if anyone wishes to express a NO vote to any of them in the secured webform. It is very important for us in the Society to clearly express our support for those elected and appointed to lead the Society and we would be very grateful if you would take the time to express your opinion on the three candidates' applications. More information on the candidates and elections can be found [here](#). The webform will remain open until 3 June, 23:59 CEST.

### Board Member Candidates

#### Jürg Barben



Jürg Barben is head of the Paediatric CF Centre in St. Gallen, responsible for the care of all children with CF from Eastern Switzerland. He is involved in daily care and clinical research. He has published over 40 papers on neonatal screening, CFSPID and sweat testing. As president of the Swiss Working Group for CF (2006-2014), he introduced CF neonatal screening in Switzerland. For two decades, he has been actively involved in ECFS activities in the Diagnostic Network (DNWG) and Neonatal Screening Working Group (NSWG). In 2019, he became leader of the ECFS NSWG, organising meetings and producing a book and six papers. From his work with the society and previously as CF Lead for the European Respiratory Society (ERS), he has a global view of CF care, independent of pharma support, and strongly advocates of equal access to drugs for all pwCF.

#### Pierre-Régis Burgel



Pierre-Régis Burgel is Professor of Respiratory Medicine at Cochin Hospital/Université Paris Cité, France. He is President of the French CF Society, an associate editor of the Journal of Cystic Fibrosis, and member of the ECFS patient registry scientific committee. Over the past years he has participated to multiple ECFS task forces and to the ECFS standard of care. Current research interests include real-world evaluation of CFTR modulators in F508del and non-F508del people with CF and translational research programs in adults with CF.

#### Charles Haworth



Charles Haworth is a consultant at Royal Papworth Hospital, Cambridge, UK where he specialises in treating adults with CF, bronchiectasis and NTM infections. He was senior author of the ECFS Bone Health guidelines (2011) and the ECFS / CFF NTM guidelines (2015). He was a member of the EU funded "Inhaled Antibiotics in Bronchiectasis and Cystic Fibrosis" consortium and is a Principal Investigator within the ECFS Clinical Trials Network. He was an executive committee member of the UK CF Medical Association 2018-2021.

[Personal Statements, CVs and nominations can be found here.](#)

# ECFS Annual General Meeting

Save the date!

The Annual General Meeting will be held during the European conference in Glasgow on Friday 07 June 2024, from 18:30 to 19:45, in M1, Scottish Exhibition and Conference Centre. The Meeting will be organised in person. It is open to all ECFS members. We need your active participation and look forward to a great discussion.

## ECFS Annual General Meeting 2024 07 June 2024, Glasgow, United Kingdom

### Upcoming Events

- **ECFS Board Summer Meeting**  
04 June 2024  
Glasgow, United Kingdom
- **ECFS Annual General Meeting**  
07 June 2024  
Glasgow, United Kingdom
- **ERS Congress**  
07-11 September 2024  
Vienna, Austria
- **North American Cystic Fibrosis Conference**  
26-28 September  
Boston, MA, USA
- **ECFS Winter Meetings**  
January 2025  
Brussels, Belgium
- **ECFS Diagnostic Network WG Meeting**  
February 2025
- **Basic Science Conference**  
March 2025
- **48<sup>th</sup> European CF Conference**  
04-07 June 2025  
Milan, Italy

### Agenda

1. President's Report – Jane Davies
2. Secretary's Report – Silke van Koningsbruggen-Rietschel
3. Treasurer's Report – Barry Plant
4. ECFS Board Elections – Jane Davies
5. Update on Journal of Cystic Fibrosis – Patrick Flume
6. ECFS CTN Report – Lieven Dupont
7. ECFS Patient Registry Report – Egil Bakkeheim
8. ECFS Standards of Care Report – Kevin Southern
9. ECFS Education Report – Chris Smith
10. ECFS Working Group Reports:
  - [Diagnostic Network Working Group](#)
  - [Exercise Working Group](#)
  - [Fungal Pathogens Working Group](#)
  - [Mental Health in the Era of CFTR Modulators Working Group](#)
  - [Neonatal Screening Working Group](#)
  - [Pulmonary Exacerbations Working Group](#)
  - [Airway Epithelial Cell Models for Theranostics Working Group](#)
  - [Telehealth in CF Care 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](#)
  - [ECFS Physiotherapy Special Interest International Group](#)
12. Presentation of the 2024 European CF Conference

## 48th EUROPEAN CYSTIC FIBROSIS CONFERENCE

4 – 7 JUNE 2025 | MILAN, ITALY



### SAVE THE DATE

Abstract Submission open	December 2024
Abstract Submission Deadline	14 January 2025
Registration open	15 January 2025
Notification of Abstract Acceptance	Early March 2025
Deadline for Early Registration	20 March 2025

[WWW.ECFS.EU/MILAN2025](http://WWW.ECFS.EU/MILAN2025)

#ECFS2025

## Highlights of the Conference

The annual ECFS conference is taking place in Glasgow, UK 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 Glasgow!

### Pre-conference Courses and Workshop

Please note that full registration for the Annual ECFS-conference is not mandatory to participate in these pre-conference sessions.

[Registration to the courses](#)

#### Clinical Nutrition Course

##### **Optimising nutritional health for the 21st century - evidence and clinical practice**

Tuesday 4 June - Half day - 13:00 – 17:00

Registration 75 € (Low- and Middle-Income Countries 45 €)

This pre-conference course will provide health care providers with an opportunity to learn and interact with key experts in the field of nutrition. Focus will be placed on addressing some of the emerging situations faced by nutritional experts and provide practical solutions whilst highlighting key publications from the last year.

[Programme can be found here.](#)

#### Physiotherapy Short Course

##### **Musculoskeletal issues in cystic fibrosis - muscle dysfunction & strength - posture and pain**

Tuesday 4 and Wednesday 5 June – 1.5 Day

Tuesday: 08:30 – 16:30 - Wednesday: 08:30 – 12:30

Registration 270 € (Low- and Middle-Income Countries 150 €)

This course is organised by PhysIIG and the ECFS Exercise Working Group and will provide the theoretical and practical basis for muscle strengthening incorporation into clinical practice. The second day will include the assessment of posture and pain in people with cystic fibrosis (pWCF) and early pathways for prevention and treatment of MSK issues. [Programme can be found here.](#)

#### Psychosocial/Mental Health Course:

##### **Co-creating personalised treatment plan, how to live your best life**

Tuesday 4 June – Half-day - 13:00 - 17:00

Registration 75 € (Low- and Middle-Income Countries 45 €)

This course is organised by the European Psychosocial Special Interest Group (EPSIG) and the ECFS Mental Health Working Group and will explore how we as a team can support people with cystic fibrosis to live their best lives, particularly in the new era of modulators. The course will involve developing a deeper understanding of the theory and application of evidence-based treatments; this will include different kinds of approaches for decision-making. Such approaches include Motivational Interviewing, Strengths Based Approach, Acceptance and Commitment Therapy and more. After introducing the theory, we will share good practice examples using case studies and consider implementation through small discussions groups. The course will focus on people with CF discussing all ages from childhood to adulthood, including transition, developmental needs and life challenges.

[Programme can be found here.](#)

### Sweat test hands-on session 2024

Wednesday, 5 June, 16:00 - 18:00

Registration 55 € (Low- and Middle-Income Countries 25 €)

Deadline for registration to the Sweat test hands-on session: 28 May 2024, 23:59 CEST

To address variations in sweat testing practices across Europe, the ECFS-DNWG (with support of the ECFS Patient Registry) is offering hands-on training focused on specific sweat test equipment. This session will focus on the Macroduct® Advanced sweat induction and collection system, and the ChloroChek® Chloridometer® for analysis. Sessions will be led in English, German, Spanish, French and Dutch (separate tables according to language).

[Programme and registration](#)

### Meet the Experts Programme

Thursday, June 6, 07:45 - 08:45

- **Implementing a healthy lifestyle in cystic fibrosis - diet and physical activity** - ePoster Corner A - Room M4  
Daina Kalnins (Toronto, Canada) / Gemma Stanford (London, United Kingdom)
- **Maximising the value of registries: novel approaches to study design** - ePoster Corner B - Room M3  
Gwyneth Davies (London, United Kingdom) / Nicole Hamblett (Seattle, United States)
- **Sampling and diagnostics of airway infections in the future** - ePoster Corner C - Room M2  
Helle Krogh Johansen (Copenhagen, Denmark) / Michael Tunney (Belfast, United Kingdom)

Friday, June 7, 07:45 - 08:45

- **Physiotherapy data harmonisation** - ePoster Corner B – Room M3  
Lisa Morrison (Glasgow, United Kingdom) / Jenny Hauser (Hobart, Australia)
- **Pharmacology and psychological side effects of modulator therapy** – ePoster Corner C – Room M2  
Anna M. Georgiopoulos (Boston, United States) / Pierre-Régis Burgel (Paris, France)

### ECFS Tomorrow Lounge Programme

Thursday, June 6

12:45 – 13:45

- **Younger horizons: Mental health screening for children < 12 years in the era of modulators**  
Anna Georgiopoulos (Boston, United States) / Eddie Landau (Petah Tikva, Israel) / Alexandra Quittner (Hollywood, United States) / Marieke Verkleij (Amsterdam, Netherlands)

15:15 - 16:15

- **Modern day challenges for cystic fibrosis physiotherapy**  
Cecilia Rodriguez Hortal (Stockholm, Sweden) / Dan Beever (Nottingham, United Kingdom) / Gemma Stanford (London, United Kingdom)

Friday, June 7

11:00 - 12:00

- **Technology for managing diabetes in people with cystic fibrosis**  
Dee Shimmin (Leeds, United Kingdom) / Ben Yusuf (Leeds, United Kingdom)

13:00 - 13:30

- **Certify & Celebrate: A Relaxed Recognition Gathering for our Travel Grant Winners**  
Gordon MacGregor (Glasgow, United Kingdom) / Robert Gray (Glasgow, United Kingdom) / Lisa Morrison (Glasgow, United Kingdom)

15:15 - 16:15

- **CFSPID - what is the role of psychosocial professionals and how to organise support and care**  
Lynne Carty (London, United Kingdom) / Faye Johnson (Manchester, United Kingdom)

## Speaker Corner

New for this year! The Speaker corner allows speakers to engage with participants in interactive discussions during the coffee breaks, fostering connections and exchanging ideas. Join us at the Tomorrow Lounge with the following speakers:

Thursday 06 June 10:30 – 11:00	Friday 07 June 10:30 – 11:00
Ann Raman (Ghent, Belgium)	Stina Järholm (Gothenburg, Sweden)
Mirjam Stahl (Berlin, Germany)	Patrick Harrison (Cincinnati, United States)
Kevin Southern (Liverpool, United Kingdom)	Silvia Buroni (Pavia, Italy)
Julia Hentschel (Leipzig, Germany)	Marcus Mall (Berlin, Germany)
Patrick Flume (Charlestown, United States)	Daniel Peckham (Leeds, United Kingdom)
Thursday 06 June 16:30 – 17:00	Friday 07 June 16:30 – 17:00
Andres Floto (Cambridge, United Kingdom)	Edward McKone (Dublin, Ireland)
Isabelle Scheers (Brussels, Belgium)	Christopher Goss (Seattle, United States)
Nicola Ivan Lorè (Milan, Italy)	Karen Raraigh (Baltimore, United States)
	Laurence Kessler (Strasbourg, France)
	Robert Gray (Glasgow, United Kingdom)

New!

New!

## ECFS Conference App

The Conference App is always very popular among the delegates.

Via the App, you will find all the information on the scientific programme, the exhibitors, the abstracts and ePosters. The ECFS 2024 Conference App is designed to enhance the experience of the participants before, during and after the Conference and also contribute to reducing the printings and paper waste. Further information is available on the conference website and the App will soon be ready to download.

**DOWNLOAD THE ECFS 2024**

# CONFERENCE APP

Access the detailed Conference Programme via the App:

- Interactive Conference Programme
- Abstracts and ePosters
- Speaker Profiles
- Information A-Z





Search for "ECFS 2024" in Apple App Store or Google Play Store

The ECFS 2024 App is supported by  4DMT



## Meetings and Courses during the 2024 ECFS Conference

### TUESDAY, JUNE 4

08:30 – 16:30	Musculoskeletal issues in Cystic Fibrosis - Muscle Dysfunction & Strength - Posture and Pain** Programme here
08:30 – 17:00	PhySIIG Airway Clearance Instructors Course**
08:30 – 18:00	ECFS Board Meeting*
13:00 – 17:00	Co-creating personalised treatment plan, how to live your best life** Programme here
13:00 – 17:00	Optimising nutritional health for the 21st century - evidence and clinical practice Programme here

### WEDNESDAY, JUNE 5

08:30 – 12:30	PhySIIG Airway Clearance Instructors Course**
08:30 – 12:30	Musculoskeletal issues in Cystic Fibrosis - Muscle Dysfunction & Strength - Posture and Pain**
08:30 – 12:45	ECFS CTN Training and Development*
09:00 – 16:00	ECFS Nursing Special Interest Group Meeting**. Programme here
09:00 – 16:00	European Psychosocial Special Interest Group (EPSIG) Meeting** Programme here
09:30 – 15:00	ECFS Neonatal Screening Working Group Annual Meeting** Programme here
10:30 – 15:45	ECFS Nutrition Group Meeting** Programme here
12:45 – 17:00	ECFS Exercise Working Group Meeting
13:30 – 18:15	ECFS CTN Steering Committee Meeting*
13:30 – 16:30	ECFS CTN Training Research Coordinators*
13:30 – 17:30	European Cystic Fibrosis Pharmacy Group Meeting
13:30 – 17:30	ECFS CTN Steering Committee Meeting*
14:00 – 16:00	Physiotherapy Case Presentations
16:00 – 17:30	Global CF*
16:00 – 18:00	ECFS Physiotherapy Special Interest International Group (PhySIIG) Annual General Meeting and network-
16:00 – 18:00	ECFS-DNWG Sweat Test Hands-on Session** Programme here
17:00 – 18:30	International (Global) CF Registry Group*
17:30 – 18:25	ECFS CTN Executive Committee meeting*

\* closed meeting

\*\* extra registration required

#### THURSDAY, JUNE 6

07:00 – 09:30	Journal of Cystic Fibrosis (JCF) Editorial Board Meeting*
07:30 – 08:30	ECFS CTN Standardisation Committee Meeting*
12:00 – 13:00	CTN – CFF – TDN – CanAct Meeting*
12:30 – 13:45	ECFS Education Committee Meeting*
12:30 – 15:00	ECFS Standards of Care Group Meeting*
13:30 – 14:30	PhysSIIG (ECFS Physiotherapy Special Interest International Group) committee meeting*
15:00 – 16:00	CFTR2 project*
15:00 – 17:00	ECFS Telehealth for CF (THCF) Working Group Meeting*
17:00 – 18:00	ECFS Patient Registry Software training & ECFS Tracker new features
18:30 – 20:00	ECFS CTN Standardisation sub-group meeting*
18:30 – 20:30	ECFS Pulmonary Exacerbation Working Group*

#### FRIDAY, JUNE 7

07:30 – 08:30	CF STORM Breakfast Meeting*
12:00 – 14:00	ECFS CTN Lung Clearance Index workshop
12:30 – 14:30	ECFS Diagnostic Network Working Group Meeting** Programme here
12:30 – 14:00	ECFS Mental Health Working Group Meeting
12:30 – 14:30	ECFS Patient Registry Steering Group Meeting
16:00 – 17:00	ECFS Patient Registry Software training / walk in session
16:00 – 18:00	UKCFMA Meeting
18:30 – 19:45	ECFS Annual General Meeting*

#### SATURDAY, JUNE 8

13:00 – 15:00	Scientific Committee Meeting Milan 2025*
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All programmes of the meeting and courses are available [here](#). Please note registration to the courses will not be possible on site in Glasgow.

\*closed meeting

\*\*extra registration required

## ECFS Award 2024

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. Scott Bell**.

### Scott Bell



Scott Bell is a pulmonologist who is a leading international clinician and scientist in respiratory medicine and in cystic fibrosis. He was trained in Sydney in Australia and also spent a year in Wales in UK. He was involved very early on in the care of people with cystic fibrosis and held various positions as Medical Director of the CF centre, the Lung Transplant Program and Thoracic Medicine at the Prince Charles Hospital in Brisbane. He became a Professor of Respiratory Medicine at the University of Queensland and also held senior executive leadership positions for many years in Australia's largest health service. He is currently Chief Executive Officer of the Translational Research Institute in

Brisbane.

Prof Bell has contributed to consensus statements nationally and internationally in the fields of CF and bronchiectasis. He has participated in many European and international groups that framed policy of adult CF Care. He made significant contributions in the field of CF bacterial infection by studying *P. aeruginosa* infection and the mechanisms of infection acquisition and transmission. He also led a Lancet Commission at the time of huge change in the outcomes of people with CF, "The Future of Cystic Fibrosis Care" which is a blueprint for the models of CF care and is a very highly cited paper. He is one of Australia's leading adult physicians in supporting the pipeline of clinical trials in CF and provides expert advice on approaches to trials in the CFTR modulator era.

Prof Bell is deeply committed to student training in scientific approach, critical and creative thinking, communication, project management and networking. He inspired and supervised trainees in thoracic medicine undertaking research projects. He also guided them for career development.

Prof Bell has worked to enhance community awareness of better lung health and research translation into practice through partnerships between patients, advocacy groups, clinicians, researchers and policy-makers. Besides his contribution to many groups of the ECFS, he held the key position of Editor-in-Chief of the Journal of Cystic Fibrosis for 7 years. Under his leadership the Journal flourished with a steadily rising numbers of submitted manuscripts and similar rising numbers of downloads of articles which reached 1 million per year at the end of his tenure. He also pioneered a lay edition of the journal that requires authors to provide a summary of their work that is accessible to people with CF and lay persons, and is a key vehicle for public, patient involvement and engagement.

We are delighted that Scott Bell was granted the ECFS Award 2024 and look forward to hearing his talk at the European CF Conference Opening Plenary on 05 June, 2024 in Glasgow.

## Gerd Döring Award 2024

To celebrate the enormous contribution of Professor Döring, the ECFS has initiated a Gerd Döring Award that is given annually to honour an exceptional early career young European scientist. The award is primarily judged on a paper published in the previous 3 calendar years, which has made a significant impact on the understanding or treatment of cystic fibrosis. This year, the Gerd Döring Award has been granted to **Livia Delpiano**, in acknowledgement of her recent remarkable contribution to cystic fibrosis. The Award, which includes a monetary grant of € 5,000 to support research, will be presented during the Opening Plenary of

### Livia Delpiano



Livia Delpiano has long been fascinated by the respiratory system since her Master's research on silicosis at the University of Genoa. Later, her interest deepened while studying lung pathology and the properties of airway mucus in CF at Giannina Gaslini Hospital. Her PhD at Newcastle University furthered her understanding of CF airway pathophysiology, focusing on exploring alternative ion channels to restore salt/fluid secretion for CF patients who are ineligible for current therapies. Now, as a postdoc at the University of Cambridge, Livia continues exploring this field.

### Research

In her paper, “Dynamic regulation of airway surface liquid pH by TMEM16A and SLC26A4 in cystic fibrosis nasal epithelia with rare mutations”, Livia explored strategies to address pH imbalances in CF airways through alternative channels and transporters. She studied the roles of TMEM16A and SLC26A4 in ion transport and airway surface liquid (ASL) pH regulation, particularly in the absence of functional CFTR, under normal and inflammatory conditions. She identified conditions where these proteins were most effective, offering potential substitutes for CFTR dysfunction and restoring ASL pH to physiological levels. Additionally, Livia identified two FDA-approved drugs that activate one of these proteins, effectively restoring ASL pH. This research offers promising insights into ASL pH-directed therapy, irrespective of CF mutation, highlighting its potential to enhance mucociliary clearance. Moreover, it emphasises the importance of personalised medicine in identifying optimal treatments for rare CF mutations.

## ECFS 2024 – 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 under the age of 35 having applied for the award.

The Young Investigator Award includes a monetary grant of €750, a free registration to the Conference, and a 2024 ECFS membership subscription. We wish to extend our congratulations to the following Young Investigators:

## Ranmal Avinash Bandara



My name is Ranmal Avinash Bandara. I attended the University of Toronto for my undergraduate studies where I specialized in immunology. I then started my master's degree in laboratory medicine and pathobiology at the University of Toronto in Professor Jim Hu's lab where I then reclassified to the PhD programme in my second year. I am currently a fourth year PhD student working at the Hospital for Sick Children in Toronto supervised by professors Jim Hu and Amy Wong.

**About the research presented at the 47th European CF Conference, abstract number WS05.06: Integration of the LacZ and CFTR transgene using Find and cut-and-transfer (FiCAT)**

The research I am currently focusing on is the Find and cut-and-transfer (FiCAT) system. The system uses the Cas9 protein to create a double stranded break followed by transposition of a transposon by the piggybac transposase linked to cas9. I have created a 22 kilobase pair plasmid containing all the components of FiCAT including the CFTR or LacZ genes inserted into the transposon. This allows us to integrate the LacZ and CFTR gene at CRISPR/Cas9 cut sites. I have shown using this system that both the lacZ or CFTR transgenes can be integrated at the AAVS1 or GGTA1 loci. Furthermore, I have shown an increase in LacZ expression due to successful integration when targeting the AAVS1 locus detected by chemiluminescence and confirmed by X-gal staining.

## Johan Sergheraert



I am a dental surgeon in Reims University Hospital and more particularly specialized in periodontology. After 5 years as a dental student in Nice University, I moved to Reims to complete an internship. At the same time, I completed a PhD thesis in Biomatériaux et Inflammation en site osseux (BIOS) Laboratory (Reims) and joined the cystic fibrosis-related bone disease (CFBD) research program since 2019. After 4 years of work and a pandemic, I defended my PhD thesis in December 2023.

**About the research presented at the 47th European CF Conference, abstract number WS14.01: Elexacaftor/Tezacaftor/Ivacaftor treatment partially normalizes osteoclasts' bone resorption activity in cystic fibrosis-related bone disease**

I have worked on cystic fibrosis-related bone disease for many years in the CFBD research programme of Bios Laboratory. The presented work focuses on the impact of CFTR Class II mutations on osteoclasts phenotype and the effects of CFTR modulators. We have shown a defect in osteoclast differentiation process associated with an increased S1P secretion and a defect in their resorption activity. Interestingly, we demonstrated that Elexacaftor/Tezacaftor/Ivacaftor treatment partially restored the osteoclastic phenotype by normalizing the levels of S1P secretion and increasing osteoclasts' resorption activity. However, Elexacaftor/Tezacaftor/Ivacaftor treatment did not allow the number of multinucleated cells or the resorption capacity to be back to control levels.

## Irene Tomarelli



I am a twenty-seven-year-old Italian resident in Pulmonology at Università Degli Studi in Milan, Italy. During the first three years of residency, I have been working with patients with both acute and chronic respiratory infections, including people with cystic fibrosis and bronchiectasis.

**About the research presented at the 47th European CF Conference, abstract number WS02.03: Disease burden in people with cystic fibrosis according to CFTR genotype and eligibility to CFTR modulator therapy: an ECFS Patient Registry analysis**

We performed a transversal-observational study that aims to assess the clinical burden of people with cystic fibrosis not eligible to Elexacaftor/tezacaftor/ivacaftor (ETI) therapy. The analysis was based on 2019 ECFS-Patient Registry; 48,732 patients were included and divided in five groups according to their genotype: F508del ( $\geq 1$  F508del variant), FDA-approved (no F508del,  $\geq 1$  FDA-approved variant), Compassionate group (no F508del or FDA-approved variants,  $\geq 1$  variant responding to ETI), non-protein group (no CFTR protein) and other variants. Our analysis highlighted heterogeneity among CF patients not eligible for ETI: FDA-approved variants showed a lower clinical burden than Compassionate group, which on the other side presented a disease burden comparable to traditional genotypes. This study suggests that any potential expansion of ETI indications should consider not only the presence of a demonstrated in-vitro response, but also the disease burden associated with genotypes and possible future clinical implications. The Compassionate variants merit careful consideration in this regard.

## 2024 ECFS Basic Science Conference – Valletta, Malta

The ECFS Basic Science Conference was held in Valletta, Malta from 20 – 23 March 2024. We would like to thank the organisers Alexandre Hinzpeter (FR), Patrick Harrison (US) and Marie Egan (US) for putting together a high-quality programme. We welcomed 158 delegates and all were really happy to meet. The [abstract book](#) is available on the ECFS website.



## ECFS Pharmacy App

ECFS Education are proud to announce that the free **ECFS Pharma** app is now available to download on iOS devices! It provides a quick way to assess practical drug information for the treatment of CF. It will soon be available for Android devices too.

The app has been in development over the past few years. It is an extension to the ECFS Standards of Care and ECFS Pharmacy Group jointly published book in 2020, titled 'ECFS Standards of Care book: Optimising Pharmaceutical Care in Cystic Fibrosis'.

**Free to download for both ECFS and non-ECFS members, it contains the latest information on CF-related medications;** type of medication, indications, routes of administration, doses, side effects information, possible drug-drug interactions, with clear adult and paediatric information. A simple search function and the ability to favourite medications allows them to be viewed quickly.

The bonus for ECFS members includes access to publications, the ECFS Education Platform, resources, and ECFS related information, via this app.



## Current References in CF

Many thanks to Dr. Henry Ryley for compiling the current references in Cystic Fibrosis that you can download through this [link](#). We would like to remind our members that the Journal of Cystic Fibrosis is no longer available as a paper copy or being sent in the post. The JCF is available online and can be accessed [here](#) or by [logging into My ECFS](#) and navigating to the 'Journal of cystic fibrosis' tab.

Have news items you would like to include, or want to write a short piece on your topic of interest for future Newsletters?

Contact us!

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