

18th ECFS Basic Science Conference
29 March – 01 April 2023, Dubrovnik, Croatia

Programme

Nicoletta Pedemonte (Genoa, IT), Alexandre Hinzpeter (Paris, FR),
Camille Ehre (Chapel Hill, US)

Wednesday, 29 March 2023 (Day 1)

19:00-19:30 **Official Opening of the Conference by the Conference Chairpersons**

19:30-20:15 **Welcome Reception**

20:15-21:30 *Dinner*

Thursday, 30 March 2023 (Day 2)

07:30-08:45 *Breakfast*

08:45-09:45 Opening Keynote Lecture

Single-channel studies of CFTR - David Sheppard (UK)

09:45 -10:15 *Coffee break & Poster viewing*

10:15-12:00 Symposium 1 – CFTR structure and function

Chairs: Alexander Hinzpeter (FR) - Cédric Govaerts (BE)

10:15-10:40 Regulation of the CFTR chloride channel through its interaction with protein kinase A - László Csanády (HU)

10:40-11:05 Regulation of CFTR function by post-translational modifications - Mauro Salvi (IT)

11:05-11:30 F508del-CFTR rescue by second-site mutations in its transmembrane domains - Paola Vergani (UK)

11:30-11:40 Abstract 04 - A synonymous polymorphism associated with rare CFTR variants confers alterations to protein biogenesis, pharmacologic response, and clinical phenotype - Kathryn Oliver (US)

11:40-11:50 Abstract 01 - Extending the success of Trikafta to rare mutations - insights from CFTR structure and modulator binding - Noemie Stanleigh (IL)

11:50-12:00 Abstract 11 - Exploring the potentiating effects of VX-445 on CFTR: a functional perspective - Maria-Cristina Ardelean (UK)

12:00-13:00 Flash Poster Session (even numbers)

Chair: Olivier Tabary (FR)

13:00-14:15 *Lunch*

- 14:30-16:15 Symposium 2 – CFTR interactome**
Chairs: Mauro Salvi (IT) - Carla Ribeiro (US)
- 14:30-14:55 An overview on CFTR interactions: old partners and new players - Carlos M. Farinha (PT)
- 14:55-15:20 CFTR dysfunction and its impact on exocrine/endocrine signaling in the CF ferret pancreas – John Engelhardt (US)
- 15:20-15:45 Pharmacological chaperones, which rescue CFTR-F508del, target a gatekeeper controlling cargo access to ER exit sites - Stefano Marullo (FR)
- 15:45-15:55 Abstract 15 - A PI3K γ mimetic peptide promotes F508del-CFTR localization at plasma membrane through a PKD1-dependent mechanism - Marco Mergioti (IT)
- 15:55-16:05 Abstract 17 - Effect of the lipid environment on the F508del CFTR rescue and stability at the plasma membrane - Nicoletta Loberto (IT)
- 16:05-16:15 Abstract 18 - Non-canonical inhibitors target CAL binding: new approaches to stabilize functional F508del-CFTR - Dean Madden (US)

16:15-16:45 *Coffee break & Poster viewing*

16:45-18:30 Symposium 3 – Care for rare: precision medicine to bring innovative therapies to people with rare mutations (a patient organisation-initiated symposium)

Chairs: Nicoletta Pedemonte (IT) – Elise Lammertyn (BE)

- 16:45-16:53 Patient view
- 16:53-17:22 Learning from rarity: lessons from studies on disease-relevant models, in the path from basic defects to personalized therapies – Nicoletta Pedemonte (IT)
- 17:22-17:51 *Ex vivo* models / translational models - Isabelle Sermet Gaudelus (FR)
- 17:51-18:20 Regulatory aspects, hurdles and needs on the way to realise personalised medicine in cystic fibrosis - Marco Cipolli (IT)
- 18:20-18:30 Abstract 57 - Pan-ethnic characterization of CFTR variants - Justin Ideozu (US)

20:00-21:30 *Dinner*

21:30-23:00 Evening Poster Session: Posters with Even numbers

Friday, 31 March 2023 (Day 3)

07:30-08:45 *Breakfast*

08:45-10:30 Symposium 4 – Mucus and mucins

Chairs: Camille Ehre (US) - Giulio Cabrini (IT)

- 08:45-09:10 Mucin in high resolution - Deborah Fass (IL)
- 09:10-09:35 Progressive development of abnormal mucus precedes chronic *P.Aeruginosa* infection in the cystic fibrosis rat - Susan Birket (US)
- 09:35-10:00 The distinct components of airway mucus – Anna Ermund (SE)
- 10:00-10:10 Abstract 71 - Low molecular weight alginate oligosaccharides enhance the diffusion of lipid-nanoparticles through cystic fibrosis mucus - Ruhina Maeshima (UK)
- 10:10-10:20 Abstract 21 - Structure of domains in polymerizing mucins and their importance for the formation of normal and CF mucus - Gunnar C Hansson (SE)
- 10:20-10:30 Abstract 23 - Sputum viscoelasticity is driven by concentration in organic components - Lydia Esteban Enjuto (FR)

10:30-11:00	Coffee break & Poster viewing
11:00-12:45	Symposium 5 – Inflammation and modulators Chairs: Deborah Fass (IL) - Luis Galiotta (IT)
11:00-11:25	The CF airway inflammatory milieu enhances the efficacy of CFTR modulators – Carla Ribeiro (US)
11:25-11:50	Molecular targets for anti-Inflammatory therapies - Giulio Cabrini (IT)
11:50-12:15	Inflammation, resolution and repair in the era of highly effective modulator therapy for CF - Robert Gray (UK)
12:15-12:25	Abstract 36 - Pulmonary ketogenesis promotes tolerance to <i>P. Aeruginosa</i> infection in the CF lung - Sebastian Riquelme (US)
12:25-12:35	Abstract 16 - Differential neutrophilic responses to <i>Pseudomonas aeruginosa</i> reveal novel protein-based treatments relevant to Cystic Fibrosis - Tia Rizakos (CA)
12:35-12:45	Abstract 38 - Fertility deficiency in female cystic fibrosis patients: determining the role of the uterus' endometrium using cutting-edge organoid models - Ellen De Pauw (BE)
12:45-14:00	Lunch
14:00-18:30	Free Afternoon
18:30-19:30	Flash Poster Session (odd numbers) Chair: Susan Birket (US)
20:00-21:30	Dinner
21:30-23:00	Evening Poster Session: Posters with Odd numbers

Saturday, 01 April 2023 (Day 4)	
07:30-08:45	Breakfast
08:45-10:30	Symposium 6 – ABC proteins and their response to modulators Chairs: David Sheppard (UK) - Paola Vergani (UK)
08:45-09:10	Development of targeted pharmacotherapy to correct ABCB4 deficiency - Tounsia Aït-Slimane (FR)
09:10-09:35	Understanding conformational transitions favoring CFTR NBD1 unfolding to design conformation-specific therapeutics – Cédric Govaerts (BE)
09:35-10:00	Design of novel potentiators based on protein-protein interactions - Norbert Odolczyk (PL)
10:00-10:10	Abstract 46 - A novel CFTR corrector displaying a unique mode of action - Peter van der Sluijs (NL)
10:10-10:20	Abstract 50 - Use of computer simulations to investigate ivacaftor-induced conformational changes in CFTR - Diana-Florentina Veselu (UK)
10:20-10:30	Abstract 54 - Elexacaftor/Tezacaftor/Ivacaftor triple therapy corrects function of CFTR rare variants - Isabelle Sermet Gaudelus (FR)
10:30-11:00	Coffee break & Poster viewing

11:00-12:45 Symposium 7 – Gene-based molecular approaches

Chairs: Marie Egan (US) - Elise Lammertyn (BE)

11:00-11:25 New approach to treat all CF patients with an antisense oligonucleotide - Olivier Tabary (FR)

11:25-11:50 RNA-based therapies for cystic fibrosis - Stephen Hart (UK)

11:50-12:15 Mutation-tailored base and prime editing approaches allow re-writing CFTR to treat cystic fibrosis. – Marianne Carlon (BE)

12:15-12:25 Abstract 58 - A precise adenine base editor corrects W1282X-CFTR without bystander effects - Lúcia Santos (PT)

12:25-12:35 Abstract 59 - Targeted repair of cystic fibrosis mutations across an entire exon using Cas9 and a single guide RNA and donor template - Emma Collins (IE)

12:35-12:45 Abstract 75 - CFTR functional rescue after delivery of nebulized LNP/mRNA to primary human bronchial epithelial cells derived from patients with different CFTR genotypes - Daniella Ishimaru (US)

12:45-14:00 *Lunch*

14:15-16:00 Symposium 8 – Alternative targets for cystic fibrosis

Chairs: Carlos M. Farinha (PT) - Isabelle Sermet Gaudelus (FR)

14:15-14:40 Pharmacological therapies for PTC mutations - Luis Galiotta (IT)

14:40-15:05 The chloride versus bicarbonate dilemma in airway muco-obstruction. Lessons from the basolateral side - Carlos Flores (CL)

15:05-15:30 Development of an hiPSC-based CF lung disease model to study TMEM16A as an alternative therapeutic target in cystic fibrosis – Sylvia Merkert (DE)

15:30-15:40 Abstract 76 - Identification of pharmacological modulators of the calcium-signaling cascade - Michele Genovese (IT)

15:40-15:50 Abstract 82 - TAVT-135, a novel chloride ion transporter for the pan-genotypic treatment of cystic fibrosis: electrophysiological and mucus-hydration properties - Roger Ilagan (US) – Presenter Andreas Maetzel (US)

15:50-16:00 Abstract 78 - BK modulation as a therapeutic target for Cystic Fibrosis - Arthur Mitchell (UK)

16:00-16:30 *Coffee Break*

16:30-17:30 Closing Keynote lecture

In vivo gene editing - Marie Egan (US)

20:00 *Dinner / Social Event*