

20th ECFS Basic Science Conference

26 – 29 March 2025, Lido di Camaiore, Italy

Programme

Chairpersons:

Dr. Patrick Harrison - Cincinnati Children's Hospital, Cincinnati, OH, USA.

Prof. Batsheva Kerem - The Hebrew University, Jerusalem, Israel

Dr Dean Madden - Geisel School of Medicine at Dartmouth, Hanover, NH, USA

Wednesday, 26 March 2025 (Day 1)

17:30-18:00 **Official Opening of the Conference by the Conference Chairpersons**

20 years of ECFS Basic Science Conference – Margarida Amaral (PT)

18:00-19:00 **Opening Keynote Lecture:**
CFTR, from folding to function - Ineke Braakman (NL)

19:00-19:45 **Welcome Reception**

19:45-21:30 *Dinner*

Thursday, 27 March 2025 (Day 2)

07:30-08:45 *Breakfast*

08:45-10:30 Symposium 1 – Therapeutic approaches for nonsense and splicing mutations
Chairs: Batsheva Kerem (IL) / John Lueck (US)

08:45-09:10 Fighting the nonsense. From basic science to new drugs - Isabelle Sermet-Gaudelus (FR)

09:10-09:35 Premature stop-codon readthrough by R-ASO - Andrei A. Korostelev (US)

09:35-10:00 Development of a platform therapeutic for nonsense-associated cystic fibrosis - John Lueck (US)

10:00-10:10 Abs. 10 - Identification of novel pharmacological modulators of the NMD mechanism to recover CFTR function in patients with nonsense mutations - Arianna Venturini (IT)

10:10-10:20 Abs. 08 - Developing novel ribosome-directed small molecules and antisense oligonucleotides as therapeutic approaches for refractory CFTR variants - JaNise Jackson (US)

10:20-10:30 Abs. 06 - Nonsense suppression of CFTR PTC variants through attenuation of translation termination and mTOR inhibition - Feng Liang (US)

10:30-11:00 *Coffee break & Poster viewing*

11:00-12:45 Symposium 2 – Mucus and mucins in CF
Chairs: Camille Ehre (US) / Noel G McElvaney (IE)

11:00-11:25 Mucins under pressure: the key to understanding mucus stasis in CF- Camille Ehre (US)

11:25-11:50 Changing inflammatory profiles post Trikafta - Noel G McElvaney (IE)

11:50-12:15 Impact of inflammation on airway mucus properties and clearance - Marcus Mall (DE)

12:15-12:25 Abs. 19 - *Ex vivo* pig lung as a new cystic fibrosis model for the study of *Pseudomonas aeruginosa* biofilm infection and phage therapy application - Marco Cafora (IT)

12:25-12:35	Abs. 16 - Investigating the therapeutic effects of K ⁺ channel modulators on mucus and airway surface liquid in cystic fibrosis - Omar Hamed (UK)
12:35-12:45	Abs. 20 - Utilisation of human induced pluripotent stem cells for modelling cystic fibrosis lung disease in vitro - Mark-Christian Jaboreck (DE)
12:45-14:30	<i>Lunch</i>
14:30-15:30	Flash Poster Session (even numbers) Chair: Nicoletta Pedemonte (IT)
15:30-15:45	The Italian CF Research Foundation: funding strategies and outcomes - Ermanno Rizzi (IT)
15:45-16:15	<i>Coffee break & Poster viewing</i>
16:15-18:00	Symposium 3 – CFTR and cancer / ageing Chairs: Patrick Harrison (US) / Sacha Spelier (NL)
16:15-16:40	Cystic fibrosis associated colorectal cancer; a complex and urgent challenge to tackle - Sacha Spelier (NL)
16:40-17:05	Growing older with CF: new challenges for CF care and research – Isabelle Fajac (FR)
17:05-17:30	Cardiovascular disease and CF; Should we be concerned? – Damian Downey (UK)
17:30-17:40	Abs. 25 - Elexacaftor/tezacaftor/ivacaftor CFTR modulators mitigate senescence in cystic fibrosis - Anca Manuela Hristodor (IT)
17:40-17:50	Abs. 22 - Pancreatic cancer growth rate may be susceptible to variations in CFTR expression - Raquel Ibañez (ES)
17:50-18:00	Abs. 21 - Development of new models to study the impact of CFTR dysfunction on osteocytes in cystic fibrosis-related bone disease - Léa Thoraval (FR)
20:00-21:30	<i>Dinner</i>

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

Friday, 28 March 2025 (Day 3)

07:30-08:45	<i>Breakfast</i>
08:45-10:30	Symposium 4 – CFTR structure and function and therapeutic relevance Chairs: Dean Madden (US) / Margarida Amaral (PT)
08:45-09:10	Elexacaftor-tezacaftor-ivacaftor rescue of F508del-CFTR chloride channels - David Sheppard (UK)
09:10-09:35	Understanding and fostering CFTR potentiation - Isabelle Callebaut (FR)
09:35-10:00	Structure/function mechanisms of CFTR modulators - TC Hwang (US)
10:00-10:10	Abs. 54 - A novel active conformation of CFTR may drive functional recovery of F508del-mutant - Cédric Govaerts (BE)
10:10-10:20	Abs. 30 - <i>In silico</i> , <i>in vitro</i> and <i>ex vivo</i> characterization of CFTR pathogenic variants localized in the Fourth Intracellular Loop and their rescue by modulators - Emanuela Pesce (IT)
10:20-10:30	Abs. 34 - Novel class 3 CFTR correctors suitable for combinatorial treatments in cystic fibrosis - Marilia Barreca (IT)
10:30-11:00	<i>Coffee break & Poster viewing</i>

11:00-12:45	Symposium 5 – CFRD from different perspectives: How do pwCF, clinicians, researchers deal with diabetes in CF? a patient organisation-initiated symposium Chairs: Paula Sommer (UK) / Isabelle Sermet-Gaudelus (FR)
11:00-11:15	<i>Patient view video:</i> Perspectives of people with CF across Europe on the impact of CF diabetes on their lives
11:15-11:45	CFRD genetics: from pathophysiology to personalized medicine - Scott Blackman (US)
11:45-12:05	CFRD in adult care / case report demonstrating demands to improve care - Bibi Uhre Nielsen (DK)
12:05-12:25	Changing nutritional trends in people with CF – what is the impact on management of CF related diabetes? – Joanna Snowball (UK)
12:25-12:45	Organ on chip modelling of beta cell function/ pancreatic exocrine-endocrine interface/potential utility of cell therapies - Victoria Salem (UK)
12:45-14:00	<i>Lunch</i>
14:00-18:30	Free Afternoon
18:30-19:30	Flash Poster Session (odd numbers) Chair: Margarida Amaral (PT)
20:00 -21:30	<i>Dinner</i>
21:30-23:00	Evening Poster Session: Posters with odd numbers
Saturday, 29 March 2025 (Day 4)	
07:30-08:45	<i>Breakfast</i>
08:45-10:30	Symposium 6 – Lung regeneration Chairs: Amy L Ryan (US) / Marta Vilà González (ES)
08:45-09:10	iPSC-derived lung and pancreatic cells: unlocking regenerative therapies for cystic fibrosis - Amy L Ryan (US)
09:10-09:35	Transplantation of autologous epithelial cells to airways - Robert E. Hynds (UK)
09:35-10:00	hiPSC-derived airway epithelium: exploring CF physiopathology and regenerative strategies - Marta Vilà González (ES)
10:00-10:10	Abs. 61 - Mitochondrial metabolic rewiring drives wound healing in cystic fibrosis primary airway epithelium cultures treated with Elexacaftor/Tezacaftor/Ivacaftor - Mairead Aubert (FR)
10:10-10:20	Abs. 58 - Unveiling new insights of ionocytes' role in cystic fibrosis using single-cell RNA sequencing analysis and human induced pluripotent stem cell-derived airway epithelial cells - Míriam Salvà Barceló (ES)
10:20-10:30	Abs. 59 - GY971, a new anti-inflammatory agent for the treatment of Cystic Fibrosis lung disease - Ilaria Lampronti (IT)
10:30-11:00	<i>Coffee break & Poster viewing</i>

11:00-12:45	Symposium 7 – Gut microbiome, gut-lung axis Chairs: Damian Downey (UK) / Laurence Delhaes (FR)
11:00-11:25	A role for the gut-lung axis in regulating chronic infections in CF - Jennifer Bomberger (US)
11:25-11:50	Impacts of CF therapies on the gut microbiome - Chris van der Gast (UK)
11:50-12:15	Lessons from the gut-lung axis in the CFTR modulator era - Laurence Delhaes (FR)
12:15-12:25	Abs. 65 - The gut's role in pulmonary and extrapulmonary manifestation of Cystic Fibrosis - Alessandra Bragonzi (IT)
12:25-12:35	Abs. 64 - Gut microbiota-metabolome relationships across pwCF taking highly effective CFTR modulators: Preliminary findings from the GRAMPUS-CF study - Ryan Marsh (UK)
12:35-12:45	Abs. 63 - Modeling a Triple Culture Airway-on-a-Chip for Inflammation Studies in Cystic Fibrosis - Roberto Plebani (IT)
12:45-14:15	<i>Lunch</i>
14:15-16:00	Symposium 8 – Gene editing and delivery Chairs: Mitch Drumm (US) / - Anna Cereseto (IT)
14:15-14:40	Systemic in utero gene editing as a treatment for cystic fibrosis - Marie E. Egan (US)
14:40-15:05	Artificial intelligence-guided design of lipid nanoparticles for pulmonary gene therapy - Jacob Witten (US)
15:05-15:30	Development of mRNA and ribonucleoprotein delivery tools for lung targeted gene therapy in cystic fibrosis - Anna Cereseto (IT)
15:30-15:40	Abs. 74 - Journey to the Centre of the Airway: tackling cystic fibrosis with cell-penetrating VP22-ABEs - Joana Alves (IE)
15:40-15:50	Abs. 67 - Development of a novel Adenine Base Editor with temporospatial control of the editing - Immacolata Zollo (PT)
15:50-16:00	Abs 69 - Precise correction of challenging mutations causing cystic fibrosis through transient delivery of the prime editing machinery - Laudonia Lidia Dipalo (BE)
16:00-16:30	<i>Coffee Break</i>
16:30-17:30	Closing Keynote lecture Advancing therapies for cystic fibrosis in an evolving global landscape - Deepika Polineni (US)
20:00	<i>Dinner / Social Event</i>