2022 ECFS Conference

New Frontiers in Basic Science of Cystic Fibrosis

30 March – 02 April 2022, Albufeira, Portugal

Programme

Chairpersons:

Carlos Farinha (Lisbon, PT), Nicoletta Pedemonte (Genoa, IT) Jeffrey L. Brodsky (Pittsburgh, US)

Wednesday, 30 March 2022 (Day 1)	
13:30-17:00	**Pre-Conference Seminar (organised by the ECFS and the Patient organisations)**
	Antimicrobial resistance in cystic fibrosis
	Chairs: Pavel Drevinek (CZ) - Jane Davies (UK)
13:30-14:45	**Part 1: The phenomenon of antimicrobial resistance in cystic fibrosis**
	AMR, epidemiology, current strategy of antibiotic therapy in CF - Pavel Drevinek (CZ)
	How to determine and interpret antimicrobial susceptibility of CF pathogens - Rafael Canton (ES)
	AMR and *P. aeruginosa*: towards the MDR phenotype - Helle Krogh Johansen (DK)
	Does the CF resistome matter? Lucas Hoffman (US)
14:45-15:15	*Coffee break*
15:15-17:00	**Part 2: Novel antimicrobial approaches to fight CF infections**
	Does the CF resistome matter? - Clinical view - Lucas Hoffman (US)
	Preclinical development of new anti-biofilm therapies - Tom Coenye (BE)
	Phage therapy: The Belgian experience - Sarah Djebara (BE)
	What is in the clinical pipeline - Pierre-Régis Burgel (FR)
	General discussion and wrap-up - Jane Davies (UK)
17:30-18:00	**Official Opening of the Conference by the Conference Chairpersons**
18:00-19:00	**Opening Keynote Lecture**
	Mucins, CFTR and their Intimate connection - Camille Ehre (US)
19:00-19:45	**Welcome Reception**
19:45-21:30	*Dinner*

Thursday, 31 March 2022 (Day 2)	
07:30-08:45	*Breakfast*
08:45-10:30	**Symposium 1 – Gene expression and RNA processing**
	Chairs: Margarida Amaral (PT) - Uta Griesenbach (UK)
08:45-09:10	Splicing modulation as a therapeutic approach for CF patients carrying rare CFTR mutations – Batsheva Kerem (IL)
09:10-09:35	Modulation of CFTR exon 22/23 splicing and/or intron 22 alternative polyadenylation (ApA) usage may have therapeutic potential for the treatment of certain CFTR PTC variants - Normand Allaire (US)
09:35-10:00	MicroRNA-dependent regulation of CFTR and its therapeutic potential - Chiara De Santi (IE)
10:00-10:10	Abstract 01 - LncRNAs: emerging players in CFTR gene regulation - Jessica Varilh (FR)
10:10-10:20	Abstract 02 - Development of a new microRNA therapeutic approach for the treatment of all patients with Cystic Fibrosis - Christie Mitri (FR)
10:20-10:30	Abstract 03 - Transcriptomic and proteomic analysis identifies changes associated with several prototypical cystic fibrosis-causing mutations - Lucia Santos (PT)
10:30-11:00	*Coffee break* & **Poster viewing**
11:00-12:45	**Symposium 2 – Personalized medicine approaches**
	Chairs: Nicoletta Pedemonte (IT) - Agnieszka Swiatecka-Urban (US)
11:00-11:25	Rare mutations in Cystic Fibrosis: from molecular diagnosis to clinical applications - Felice Amato (IT)
11:25-11:50	Predicting pharmacological rescue of CFTR misfolding mutations in human translational models - Martina Gentzsch (US)
11:50-12:15	Rectal organoids as a tool for personalized medicine - Anabela Ramalho (BE)
12:15-12:25	Abstract 10 - Development of a High Throughput Functional Screen Allows Drug Repurposing and Reveals Novel Drug Candidates for rescuing CFTR function in Patient-Derived Organoids with Nonsense Mutations - Sacha Spelier (NL)
12:25-12:35	Abstract 12 - Identification of novel small molecule modulators for PTC mutations in CFTR - Luka Clarke (PT)
12:35-12:45	Abstract 14 - Proof of concept of ionocytes' CFTR content as a novel biomarker for cystic fibrosis diagnosis and follow up - Floriana Guida (IT)
12:45-14:30	*Lunch*

14:30-15:30	**Flash Poster Session (even numbers)**
	Chair: Alexandre Hinzpeter (FR)
15:30-16:00	*Coffee break* & **Poster viewing**
16:00-17:45	**Symposium 3 – Rare mutations: molecular defects and protein correction**
	Chairs: Carlos Farinha (PT) – Ineke Braakman (NL)
16:00-16:25	CFTR modulation: insight from 3D structures - Isabelle Callebaut (FR)
16:25-16:50	Perspectives on precision therapeutics for rare CF genotypes - Eric Sorscher (US)
16:50-17:15	Rescue of mutant CFTR chloride channels by a mimetic peptide targeting the AKAP function of PI3Kgamma – Alessandra Ghigo (IT)
17:15-17:25	Abstract 26 - Structural plasticity of the Nucleotide Binding Domain 1 (NBD1) of CFTR is linked to pathogenesis of cystic fibrosis. - Rafael Colomer Martinez (BE)
17:25-17:35	Abstract 30 - Characterization of the [1898+3A>G;186-13C>G] complex allele by means of patient-derived nasal epithelial cells: molecular and functional analysis of CFTR mRNA and protein - Cristina Pastorino (IT)
17:35-17:45	Abstract 29 - Rescue of rare CFTR trafficking mutants highlights a structural location-dependent pattern for correction - Sónia Zacarias (PT)
19:45-21:30	*Dinner*
21:30-23:00	**Evening Poster Session:** Posters with Even numbers

Friday, 01 April 2022 (Day 3)

07:30-08:45	*Breakfast*
08:45-10:30	**Symposium 4 – CFTR folding and trafficking**
	Chairs: Jeff Brodsky (US) – Marianne Carlon (BE)
08:45-09:10	Requirements for CFTR folding and transport - Ineke Braakman (NL)
09:10-09:35	CFTR's Site 1: Degenerate but not disabled - T.C. Hwang (US)
09:35-10:00	Cif: a therapeutic target in persistent airway infections – Dean R. Madden (US)
10:00-10:10	Abstract 36 - Characterization of corrector ARN23765 mechanism of action via Photo-Affinity Labeling (PAL) approach - Fabio Bertozzi (IT)
10:10-10:20	Abstract 37 - New kinase therapeutic targets for cystic fibrosis from a global functional genomics screen - Hugo Botelho (PT)
10:20-10:30	Abstract 38 - Rescue F508del-CFTR with nanobodies - Marie Overtus (BE)
10:30-11:00	*Coffee break* & **Poster viewing**
11:00-12:45	**Symposium 5 – CFTR: beyond the airway**
	Chairs: Mike Gray (UK) - Pascale Fanen (FR)
11:00-11:25	CFTR: a new horizon in pancreatitis - Peter Hegyi (HU)
11:25-11:50	The "CF gut", its abnormalities directly and indirectly related to CFTR dysfunction, and strategies to improve gut fluidity and alkalinity in the CF gut beyond CFTR rescue - Ursula Seidler (DE) [Pre-recorded talk]
11:50-12:15	Physiology and pathology of CFTR in the kidney - Karl Kunzelmann (DE)
12:15-12:25	Abstract 54 - CFTR activity is determined by the store-independent activation of SPCA2/STIM1/ORAI1 complex in secretory epithelial cells - Arpad Varga (HU)
12:25-12:35	Abstract 56 - Cystic Fibrosis-related Bone Disease: CFTR class II mutations deregulate osteoclast formation and favor RANK+MCSFR+ circulating pre-osteoclasts - Johan Sergheraert (FR)
12:35-12:45	Abstract 60 - Integrative analysis of vascular impairment in models of cystic fibrosis - Lucas Treps (FR)
12:45-14:00	*Lunch*
14:00-18:30	**Free Afternoon**
18:30-19:30	**Flash Poster Session (odd numbers)**
	Chair: Felice Amato (IT)
19:45 -21:30	*Dinner*
21:30-23:00	**Evening Poster Session:** Posters with Odd numbers

Saturday, 02 April 2022 (Day 4)	
07:30-08:45	*Breakfast*
08:45-10:30	**Symposium 6 – Restoring epithelial homeostasis**
	Chairs: Marcus Mall (DE) - Luis Galietta (IT)
08:45-09:10	Novel mechanisms of TGF-beta signaling in CF - Agnieszka Swiatecka-Urban (US)
09:10-09:35	Investigating the therapeutic potential of phages as antibacterials and immunomodulators- Anna Pistocchi (IT)
09:35-10:00	Apical hydration protects the CF airway epithelium from *P. aeruginosa* by restoring junctional networks - Marc Chanson (CH)
10:00-10:10	Abstract 63 - Identification of drugs activating CFTR-independent fluid secretion in nasal organoids based on a high-content screening assay - Lisa Rodenburg (NL)
10:10-10:20	Abstract 64 - ATP12A upregulation in airway epithelial cells by inflammatory stimuli - Daniela Guidone (IT)
10:20-10:30	Abstract 65 - SLC26A4 but not TMEM16A directly regulates ASL pH under inflamed conditions in nasal epithelia derived from donors with rare class I mutations - Livia Delpiano (UK)
10:30-11:00	*Coffee break* & **Poster viewing**
11:00-12:45	**Symposium 7 – Mucus and mucins**
	Chairs: Camille Ehre (US) - Karl Kunzelmann (DE)
11:00-11:25	Role of submucosal glands and mucous strands in airway host defense - Lynda S. Ostedgaard (US) [Pre-recorded talk]
11:25-11:50	What is wrong with the CF lung mucus and how can we fix it? - Gunnar Hansson (SE)
11:50-12:15	The *Xenopus tropicalis* tadpole as a model system to define mechanisms of mucus function - David Thornton (UK)
12:15-12:25	Abstract Abstract 84 - Extracellular vesicle lncRNA MALAT1 drives HDAC11-dependent chronic inflammation in cystic fibrosis airway neutrophils - Brian Dobosh (US)
12:25-12:35	Abstract 85 - HiPSC-derived AECs as a novel platform to study the role of ionocytes in mucociliary clearance - Marta Vila Gonzalez (UK)
12:35-12:45	Abstract 83 - Hydrogel-encapsulated niclosamide for topical treatment of inflammatory airway diseases - Raquel Centeio (DE)
12:45-14:15	*Lunch*

14:15-16:00	**Symposium 8 – Gene-based therapeutic approaches**
	Chairs: Jeff Beekman (NL) - Martina Gentzsch (US)
14:15-14:40	CFTR gene editing – recent highlights, future goals and therapeutic opportunities - Patrick Harrison (IE)
14:40-15:05	Development of suppressor tRNA gene therapies targeting nonsense associated CF- John Lueck (US)
15:05-15:30	Progression towards a first-in-man lentiviral vector trial - Uta Griesenbach (UK)
15:30-15:40	Abstract 89 - Development of *in vitro* transcribed mRNA therapeutics for cystic fibrosis - Ruhina Maeshima (UK)
15:40-15:50	Abstract 91 - CFTR super exon splice site and polyA signal affect CFTR expression and function - Hillary Valley (US)
15:50-16:00	Abstract 94 - Correction of the CFTR 1717-1G>A splicing mutation through CRISPR based technology - Alessandro Umbach (IT)
16:00-16:30	*Coffee Break*
16:30-17:30	**Closing Keynote lecture**
	How to tackle what is still missing: striking CF by mechanistic approaches – Margarida Amaral (PT)
20:00	*Dinner / Social Event*