**2019 ECFS Conference**

**New Frontiers in Basic Science of Cystic Fibrosis**

27-30 March 2019, Dubrovnik, Croatia

**Programme**

Chairpersons:

Isabelle Callebaut (Paris, FR), Carlos Farinha (Lisbon, PT), Martin Mense (Lexington, US)

| **Wednesday, 27 March 2019 (Day 1)** | |
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| **13:30-17:00** | **Pre-Conference Seminar - *CFTR:* new insights on structure and function, and implications for modulation**<br><br>**Chairs: Bertrand Kleizen (NL) / David Sheppard (UK)** |
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| *15:00 – 15:30* | *Coffee break* |
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| 17:30-18:00 | **Official Opening of the Meeting by the Conference Chairpersons** |
| 18:00-19:00 | **Opening Keynote Lecture**<br>The genetics and genomics of CF – Garry Cutting (US) |
| *19:00-19:45* | **Welcome Reception** |
| *19:45-21:30* | *Dinner* |
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| **Thursday, 28 March 2019 (Day 2)** | |
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| *07:30-08:45* | *Breakfast* |
| **08:45-10:30** | **Symposium 1 – Genetics, genomics, and transcriptomics**<br>**Chairs: Garry Cutting (US) – Alexandre Hinzpeter (FR)** |
| 08:45-09:10 | Enhancers, structural elements and 3D organization: key features in regulation of CFTR gene expression - Ann Harris (US) |
| 09:10-09:35 | Update on cystic fibrosis modifier genes- Harriet Corvol (FR) |
| 09:35-10:00 | Polyvariant mutant *CFTR* genes of mutations with varying consequences- Harry Cuppens (BE) |
| 10:00-10:10 | Poster 01 - Identification of *CFTR cis*-regulatory variants - Mégane Collobert (FR) |
| 10:10-10:20 | Poster 02 - Comparison of three genome editing techniques to correct the common W1282X mutation responsible for cystic fibrosis - Karen Mention (IE) |
| 10:20-10:30 | Poster 06 - Recruitment to cystic fibrosis airway fluid licenses transcription and subsequent acquisition of the pathological GRIM fate by human neutrophils - Rabindra Tirouvanziam (US) |
| *10:30-11:00* | *Coffee break* & **Poster viewing** |
| **11:00-12:45** | **Symposium 2 – Rare mutations and complex alleles**<br>**Chairs: Isabelle Sermet-Gaudelus (FR) – Nicoletta Pedemonte (IT)** |
| 11:00-11:25 | *CFTR* complex alleles - Alexandre Hinzpeter (FR) |
| 11:25-11:50 | Make sense out of nonsense with antisense - Shuling Guo (US) |
| 11:50-12:15 | A shortcut to bringing more patients with rare genotypes into the tent?- Phil Thomas (US) |
| 12:15-12:25 | Abstract 10 - Characterization of the rare S955P-CFTR mutation and its response to modulators in cellular models and patient-derived materials - Sofia Ramalho (PT) |
| 12:25-12:35 | Abstract 09 - The cystic fibrosis mutations L927P and I336K modulate CFTR pore dynamics during channel gating, but are rescued by ivacaftor and lumacaftor - Majid K. Al Salmani (UK) |
| 12:35-12:45 | Abstract 08 - Not all stop codons are created equal, neither do they always stop: morals from single-channel studies of E60X- and G542X-CFTR - Jiunn-Tyng Yeh (US) |
| *12:45-14:30* | *Lunch* |
| **14:30-15:30** | **Flash Poster Session (even numbers)**<br>**Chair: Bertrand Kleizen (NL)** |
| *15:30-16:00* | *Coffee break* & **Poster viewing** |
| **16:00-17:45** | **Symposium 3 – Epithelial channelome: (i) Folding and structure**<br>**Chairs: Isabelle Callebaut (FR) – Ineke Braakman (NL)** |
| 16:00-16:25 | Mechanism of ion conduction and gating in the calcium-activated chloride channel TMEM16A - Raimund Dutzler (CH) |
| 16:25-16:50 | A minimal helical hairpin motif provides molecular-level insights into misfolding and pharmacological rescue of *CFTR* - Michael Schlierf (DE) |
| 16:50-17:15 | NBD1 cotranslational folding intermediates as therapeutic targets - Bill Skach (US) |

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| 17:15-17:25 | Abstract 13 - The regulatory insertion enables novel conformations of NBD1 of CFTR - Daniel Scholl (BE) |
| 17:25-17:35 | Abstract 18 - CFTR conformational landscape explored by computational approaches - Ahmad Elbahnsi (FR) |
| 17:35-17:45 | Abstract 15 - Identifying the molecular targets for CFTR potentiators GLPG1837 and VX-770 - Han-I Yeh (US) |
| *17:45-18:00* | *Break* |
| **18:00-19:45** | **ECFS Basic Science Working Group session**<br>**Activating TMEM16A / Anoctamin 1 in CF: Friend or Foe?**<br>**Chairs: Luis Galietta (IT) – Nicoletta Pedemonte (IT)** |
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| *19:45-21:30* | *Dinner* |
| **21:30-23:00** | **Evening Poster Session:** Posters with Even numbers |

| **Friday, 29 March 2019 (Day 3)** | |
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| *07:30-08:45* | *Breakfast* |
| **08:45-10:30** | **Symposium 4 – Epithelial channelome: (ii) Cell physiology and ion transport**<br>**Chairs: Margarida Amaral (PT) – Phil Thomas (US)** |
| 08:45-09:10 | Exploiting species differences to investigate *CFTR* - David Sheppard (UK)) |
| 09:10-09:35 | Anionophores: Small-molecule CFTR surrogates as mutation agnostic CF drug candidates - Roberto Quesada (ES) |
| 09:35-10:00 | ATP12A – pig models - Mike Welsh (US) |
| 10:00-10:10 | Abstract 20 - Impact of CFTR function on airway mucus properties - Martial Delion (BE) |
| 10:10-10:20 | Abstract 21 - Early onset of airway mucus obstruction associated with increased mortality in neonatal *Slc26a9* deficient mice - Pamela Millar-Büchner (DE) |
| 10:20-10:30 | Abstract 24 - Dual role of pendrin as a bicarbonate secretion pathway and CFTR modulator in well-differentiated human nasal and bronchial epithelial cells - John W. Hanrahan (CA) |
| *10:30-11:00* | *Coffee break* & **Poster viewing** |
| **11:00-12:45** | **Symposium 5 - CFTR processing, trafficking and interactions**<br>**Chairs: Carlos Farinha (PT) - Luis Galietta (IT)** |
| 11:00-11:25 | A Proteomic Variant Approach (ProVarA) for personalized medicine of inherited and somatic disease – Darren Hutt (US) |
| 11:25-11:50 | Novel CFTR regulators identified by means of a functional genomics approach and their possible mechanisms of action - Nicoletta Pedemonte (IT) |
| 11:50-12:15 | Functional genomics of F508del-CFTR: Illuminating traffic mechanisms for innovative therapeutic strategies - Margarida Amaral (PT) |
| 12:15-12:25 | Abstract 47 - CFTR processing mutations cause distinct trafficking and functional |

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| | defects - Marjolein Ensinck (BE) |
| 12:25-12:35 | Abstract 49 - Role of the proteasome in the biosynthetic arrest of SLC26A9 by F508del-CFTR - Yukiko Sato (CA) |
| 12:35-12:45 | Abstract 48 - Monitoring the phospho-occupancy of CFTR in respiratory epithelia using mass spectrometry - Aiswarya Premchandar (CA) |
| *12:45-14:00* | *Lunch* |
| 14:00-18:30 | **Free Afternoon** |
| **18:30-19:30** | **Flash Poster Session (odd numbers)**<br>**Chair: Bertrand Kleizen (NL)** |
| *19:30 -21:30* | *Dinner* |
| **21:30-23:00** | **Evening Poster Session:** Posters with Odd numbers |

| **Saturday, 30 March 2019 (Day 4)** | |
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| *07:30-08:45* | *Breakfast* |
| **08:45-10:30** | **Symposium 6 – Inflammation and host-pathogen interactions**<br>**Chairs: Marcus Mall (DE) – Gunnar Hanson (SE)** |
| 08:45-09:10 | Implication of host antimicrobial peptides in the modulation of bacterial-bacterial interactions in CF airways - Lhousseine Touqui (FR) |
| 09:10-09:35 | Viral-bacterial co-infections in cystic fibrosis – Jennifer Bomberger (US) |
| 09:35-10:00 | Biofilm formation in cystic fibrosis - Tom Coenye (BE) |
| 10:00-10:10 | Abstract 67 - Effects of short-term Lumacaftor-Ivacaftor therapy on lung microbiome in Phe508del homozygous patients with cystic fibrosis - Sébastien Boutin (DE) |
| 10:10-10:20 | Abstract 56 - Metabolic reprogramming of cystic fibrosis macrophages by the IRE1a-XBP1 pathway leads to an exaggerated inflammatory response - Samuel Lara-Reyna (UK) |
| 10:20-10:30 | Abstract 55 - SPLUNC1 peptidomimetics inhibit Orai1 to reduce pulmonary inflammation - Saira Ahmad (US) |
| *10:30-11:00* | *Coffee break* & **Poster viewing** |
| **11:00-12:45** | **Symposium 7 – Model systems**<br>**Chairs: Martin Mense (US) – Jeff Beekman (NL)** |
| 11:00-11:25 | A single cell atlas of the airway epithelium reveals the CFTR-rich pulmonary ionocyte - Aron Jaffe (US) |
| 11:25-11:50 | Mechanisms of airway epithelium repair in cystic fibrosis - Marc Chanson (CH) |
| 11:50-12:15 | Understanding altered airway mucins sialyation and decreased mucociliary transport in cystic fibrosis pig airways using single cell gene expression – airway cells - Pascal Barbry (FR) |

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| 12:15-12:25 | Abstract 82 - Development of a steady-state lumen area screening assay to measure CFTR function in organoids of cystic fibrosis patients - Marne Hagemeijer (NL) |
| 12:25-12:35 | Abstract 76 - Human induced pluripotent stem cell derived p63 expressing epithelial cells provide a personalized model for CF lung epithelium - Bob J Scholte (NL) |
| 12:35-12:45 | Abstract 75 - Primary human nasal epithelial cell air/liquid interface cultures: an in vitro model system for assessing CFTR function - Calvin Cotton (US) |
| *12:45-14:15* | *Lunch* |
| **14:15-16:10** | **Symposium 8 – Therapeutical approaches**<br>**Chairs: Martina Gentzsch (US) – David Sheppard (UK)** |
| 14:15-14:40 | Novel developments in genetic therapies for cystic fibrosis - Stephen Hart (UK) |
| 14:40-15:05 | Update on CF Gene Therapy – Chris Boyd (UK) |
| 15:05-15:30 | Co-potentiators as a novel therapeutic paradigm for CFTR mutations that are not responsive to available modulators - Peter Haggie (US) |
| 15:30-15:40 | Abstract 87 - Genetic repair of CFTR function in cystic fibrosis organoids using CRISPR/Cas9 adenine base editing - Eyleen de Poel (NL) |
| 15:40-15:50 | Abstract 102 - The pharmacology of novel TMEM16A potentiator compounds - Martin Gosling (UK) |
| 15:50-16:00 | Abstract 33 - Identification of pharmacological modulators of the TMEM16A chloride channel by high-throughput screening - Michele Genovese (IT) |
| 16:00-16:10 | Abstract 90 - CFTR Super Exon Partially Corrects W1282X-CFTR - Hillary Valley (US) |
| *16:10-16:45* | *Coffee Break* |
| **16:45-17:45** | **Workshop: How to write a successful paper (title TBC)** **Breakout Room**<br>**Chair: Cliff Taggart (UK)** |
| **16:45-17:45** | **Flash Paper Session**<br>**Chair: Marc Chanson (CH)** |
| 16:45 – 16:58 | Esomeprazole increases airway surface liquid pH in primary cystic fibrosis epithelial cells - Vinciane Saint-Criq (UK) |
| 16:58 – 17:11 | *Staphylococcus aureus* impacts pseudomonas aeruginosa chronic respiratory disease in murine models - Cristina Cigana (IT) |
| 17:11 – 17:25 | Targeting of the Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) Protein with a Technetium-99m Imaging Probe - Filipa Mendes (PT) |
| 17:25 – 17:35 | Abstractr 85 - Lack of cystic fibrosis transmembrane conductance regulator causes low cortical bone thickness and high cortical porosity in newborn pigs - Frédéric Velard (FR) |
| 17:35 – 17:45 | Abstract 86 - Pancreatic ductal fluid and bicarbonate secretion of the ferret and pig models of cystic fibrosis (CF) - Emese Tóth( HU) |
| *17:45-18:00* | *Break* |
| **18:00-19:00** | **Closing Keynote lecture**<br>Organoids for cystic fibrosis research - Jeffrey Beekman (NL) |
| *20:30* | *Dinner / Social Event* |