

2018 ECFS Conference
New Frontiers in Basic Science of Cystic Fibrosis
21-24 March 2018, Loutraki, Greece

Programme

Chairpersons:

Marcus Mall (Berlin, DE), Isabelle Callebaut (Paris, FR), John Engelhardt (Iowa City, US)

Wednesday, 21 March 2018 (Day 1)

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| 13:30-17:00 | Pre-Conference Seminar “Lung and gut microbiome: what has to be considered for CF” Chairs: Geneviève Héry-Arnaud (FR) / Stuart Elborn (UK) |
| 13:30-13:40 | General introduction - Geneviève Héry-Arnaud (FR) |
| 13:45-14:00 | Area of technology in microbial diagnostics - Sebastien Boutin (DE) |
| 14:05-14:20 | Recent data on lung and gut microbiome - Michael Tunney (UK) |
| 14:25-14:40 | Pathophysiological factors of CF dysbiosis - Leah Cuthbertson (UK) |
| 14:45-15:00 | The effect of CFTR modulation on CF lung microbiota: What do we know and is it important? - Pradeep Singh (USA) |
| 15:00-15:30 | <i>Coffee break</i> |
| 15:30-15:45 | Modelling the microbiome - Julien Tap (FR) |
| 15:50-16:40 | Forum discussion in interaction with the audience |
| 16:45-17:00 | Wrap-up and next steps to move forward - Stuart Elborn (UK) |
| 17:30-18:15 | Official Opening of the Meeting by the Conference Chairpersons Followed by: New research opportunities for CF scientists: EU-OPENSSCREEN and CORBEL Bahne Stechmann (DE) |
| 18:15-19:15 | Opening Keynote Lecture Pathogenesis and therapy of the muco-obstructive component of CF lung disease Richard Boucher (US) |
| 19:15-20:00 | Welcome Reception |
| 20:00-21:30 | <i>Dinner</i> |

Thursday, 22 March 2018 (Day 2)

07:30-08:45 *Breakfast*

08:45-10:30 **Symposium 1 – CFTR Expression, Folding, Trafficking and Activity**

Chairs: Isabelle Callebaut (FR) – Christine Bear (CA)

08:45-09:10 CFTR structures, insights and horizons - Bob Ford (UK)

09:10-09:35 Interplay between NBD1 subdomain dynamics and CFTR coupled domain folding in cystic fibrosis - Gergely Lukacs (CA)

09:35-10:00 Folding and misfolding of CFTR variants - Ineke Braakman (NL)

10:00-10:10 Abstract 01 - What can we learn from structural models of CFTR? - Hanoch Senderowitz (IL)

10:10-10:20 Abstract 04 - Nanobodies as tools to stabilize different conformational states of CFTR - Maud Sigoillot (BE)

10:20-10:30 Abstract 09 - Escape from the ER: Mechanisms for Correction of CFTR Misfoldin - Douglas Cyr (US)

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

11:00-12:45 **Symposium 2 – Cell Physiology and Ion Transport**

Chairs: David Sheppard (UK) - Nicoletta Pedemonte (IT)

11:00-11:25 SPX-101 is a novel peptide therapeutic for the treatment of CF lung disease - Robert Tarran (US)

11:25-11:50 Role of CFTR and non-CFTR channels and transporters in airways surface liquid pH regulation - Michael Gray (UK)

11:50-12:15 TMEM16 proteins control mucus production and exocytosis - Karl Kunzelmann (DE)

12:15-12:25 Abstract 32 - Role of pendrin (SLC26A4) in secretion by primary bronchial epithelial cells - Dusik Kim (CA)

12:25-12:35 Abstract 33 - Early neonatal mortality of *Slc26a9* deficient mice is triggered by airway mucus obstruction - Pamela Millar-Büchner (DE)

12:35-12:45 Abstract 30 - ATP12A as an alternative therapeutic target in cystic fibrosis lung disease - Paolo Scudieri (IT)

12:45-14:30 *Lunch*

14:30-15:30 **Flash Poster Session (even numbers)**

Chair: Nicoletta Pedemonte (IT)

15:30-16:00 *Coffee break & Poster viewing*

16:00-17:45 **Symposium 3 – Model systems**

Chairs: John Engelhardt (US) - Margarida Amaral (PT)

16:00-16:25 Early airway disease in the CF pig model - David Stoltz (US)

16:25-16:50 Airway organoids - Jeffrey Beekman (NL)

16:50-17:15 Patient derived nasal cells, a new tool in translational research for Cystic Fibrosis? - Isabelle Sermet-Gaudelus (FR)

17:15-17:25 Abstract 45 - BMI-1 transduced basal vells as a renewable resource for air liquid interface cell culture models of cystic fibrosis - Stephen Hart (UK)

17:25-17:35 Abstract 51 - Development of a robust protocol for gene-editing human pluripotent stem cells - Sara Cuevas Ocaña (UK)

17:35-17:45 Abstract 49 - Nasal epithelial cells expansion and reprogramming with the anti-SMAD protocol to evaluate CFTR rescue for precision medicine - Ilaria Musante (IT)

17:45-18:00 *Break*

18:00-19:45 ECFS Basic Science Working Group session:

Cystic Fibrosis: beyond the airways

Chairs: Margarida Amaral (PT) – Jeffrey Beekman (NL)

Intestine: modelling *in vitro* intestinal organs - Sylvia Boj (NL)

Pancreas: CF-related diabetes - James Shaw (UK)

Kidney: Disturbed kidney acid/base physiology in patients with CF - Jens Leipziger (DK)

19:45-21:30 *Dinner*

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

Friday, 23 March 20187 (Day 3)

07:30-08:45 *Breakfast*

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

Chairs: David Stoltz (US) - Ric Boucher (US)

08:45-09:10 Unravelling the molecular mechanisms controlling respiratory tract mucus gel formation - David Thornton (UK)

09:10-09:35 Reporter Muc5b-GFP mouse to study Muc5b functions - Jean-Luc Desseyn (FR)

09:35-10:00 The biophysics of airway mucus in health and disease - Brian Button (US)

10:00-10:10 Abstract 56 - Intramolecular interactions of the airway mucin MUC5B and their role in packaging within secretory granules - Caroline Ridley (UK)

10:10-10:20 Abstract 71 - Lack of CFTR results in impaired innate immunity in small airways - Joseph Zabner (US)

10:20-10:30 Abstract 59 - Identifying pathways regulating goblet cell metaplasia: phenotypic screening with bronchospheres - Henry Danahay (UK)

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

11:00-12:45 Symposium 5 - Inflammation

Chairs: Bettina Schock (UK) - Rob Tarran (US)

11:00-11:25 Mechanisms of neutrophil activation in early cystic fibrosis airway disease - Rabindra M. Tirouvanziam (US)

11:25-11:50 Bacteria-driven peribronchial lymphoid neogenesis in cystic fibrosis and bronchiectasis - Pierre-Regis Burgel (FR)

11:50-12:15 Role of IL-1 receptor signaling in the pathogenesis and as potential therapeutic target in CF airway inflammation - Marcus Mall (DE)

12:15-12:25 Abstract 66 - L iminosugars: new anti-inflammatory drugs for CF lung disease?- Maria Cristina Dechechchi (IT)

12:25-12:35 Abstract 61 - ENaC has a pivotal role in modulating autoinflammation and glycolysis in cystic fibrosis - Thomas Scambler (UK)

12:35-12:45 Abstract 125 - Complex network involving miRNA and RNA-BP in CF - Alexandra Pommier (FR)

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| 12:45-14:00 | Lunch |
| 14:00-18:30 | Free Afternoon |
| 18:30-19:30 | Flash Poster Session (odd numbers) Chair: Alexandre Hinzpeter (FR) |
| 19:30 -21:30 | Dinner |
| 21:30-23:00 | Evening Poster Session: Posters with Odd numbers |

Saturday, 24 March 2018 (Day 4)

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| 07:30-08:45 | Breakfast |
| 08:45-10:30 | Symposium 6 – Host-pathogen interactions Chairs: Stuart Elborn (UK) - Pierre-Régis Burgel (FR) |
| 08:45-09:10 | Anaerobic bacteria in Cystic Fibrosis pulmonary infection - Michael Tunney (UK) |
| 09:10-09:35 | CF airways metagenome – Burkhard Tümmler (DE) |
| 09:35-10:00 | Invasive sampling of the lungs of children with CF suggests a revised model of CF infection pathogenesis - Pradeep Singh (US) |
| 10:00-10:10 | Abstract 77 - Intracellular persistence of <i>Pseudomonas aeruginosa</i> in airway epithelial cells and CF lungs - Dao Nguyen (CA) |
| 10:10-10:20 | Abstract 70 - Airway surface liquid acidification initiates host defense abnormalities in Cystic Fibrosis- Juliette Simonin (FR) |
| 10:20-10:30 | Abstract 58 - Using <i>Xenopus tropicalis</i> to develop a live <i>in vivo</i> model of mucociliary clearance, Eamon Dubaissi (UK) |
| 10:30-11:00 | Coffee break & Poster viewing |
| 11:00-12:45 | Symposium 7 – Therapeutic Approaches Chairs: Jane Davies (UK) - Pradeep Singh (US) |
| 11:00-11:25 | CFF therapeutics discovery beyond F508del - Martin Mense (US) |
| 11:25-11:50 | Recent advances in gene and base editing - Patrick Harrison (IE) |
| 11:50-12:15 | Development of a PI3Kγ-derived peptide as a standalone therapy to activate F508del-CFTR, limit lung inflammation and promote bronchorelaxation in Cystic Fibrosis - Emilio Hirsch (IT) |
| 12:15-12:25 | Abstract 90 - New correctors rescue F508del-CFTR activity at low nanomolar concentrations - Tiziano Bandiera (IT) |
| 12:25-12:35 | Abstract 87 - <i>In Vitro</i> Characterization of Clinical Stage Novel Corrector PTI-801 and Potentiator PTI-808 in Primary Airway Cell Models - John Preston Miller (US) |
| 12:35-12:45 | Abstract 86 - Restoration of CFTR function by Antisense Oligonucleotide splicing modulation - Batsheva Kerem (IL) |
| 12:45-14:30 | Lunch |
| 14:30-16:15 | Symposium 8 – Translational CF Research Chairs: Kris De Boeck (BE) - Isabelle Sermet-Gaudelus (FR) |
| 14:30-14:55 | Modeling patient specific responses to CFTR modulator therapies- Challenges and opportunities - Christine Bear (CA) |
| 14:55-15:20 | Translating molecular and cellular knowledge in treatments for Cystic Fibrosis - Margarida Amaral (PT) |

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| 15:20-15:45 | Personalized medicine, Task Force update - Jane Davies (UK) |
| 15:45-15:55 | Abstract 116 - Drug repurposing for cystic fibrosis subjects with rare CFTR mutations - Eyleen de Poel (NL) |
| 15:55-16:05 | Abstract 119 - Effects of Lumacaftor-Ivacaftor Therapy on CFTR Function in Phe508del Homozygous Patients with Cystic Fibrosis - Simon Y. Graeber (DE) |
| 16:05-16:15 | Abstract 34 - SLC6A14 overexpression modifies Δ F508-CFTR function in human bronchial epithelial cells - Yu-Sheng Wu (CA) |
| 16:15-16:45 | <i>Coffee Break</i> |
| 16:45-17:45 | Flash Paper Session Chair: Mike Gray (UK) |
| 16:45-17:00 | IL-17A impairs host tolerance during airway chronic infection by <i>Pseudomonas aeruginosa</i> - Cristina Cigana (IT) |
| 17:00-17:15 | Quorum Sensing Down-Regulation Counteracts the Negative Impact of <i>Pseudomonas aeruginosa</i> on CFTR Channel Expression, Function and Rescue in Human Airway Epithelial Cells - Emmanuelle Brochiero (CA) |
| 17:15-17:30 | Analysis of long-range interactions in primary human cells identifies cooperative CFTR regulatory elements - Stéphanie Moisan (FR) |
| 17:30-17:45 | Anti-cystic fibrosis properties of the CB subunit of crotoxin: correction of Δ F508-CFTR chloride channel dysfunction - Grazyna Faure (FR) |
| 17:45-18:00 | <i>Break</i> |
| 18:00-19:00 | Closing Keynote lecture Pharmacological modulation of ion transport in CF: CFTR and beyond - Luis Galiotta (IT) |
| 20:30 | <i>Dinner / Social Event</i> |