

**2017 ECFS Conference
New Frontiers in Basic Science of Cystic Fibrosis**

29 March - 01 April 2017, Albufeira, Portugal
Programme

Chairpersons: Ineke Braakman (Utrecht, NL),
Marcus Mall (Heidelberg, DE), Tzyh-Chang Hwang (Missouri-Columbia, US)

Wednesday, 29 March 2017 (Day 1)

13:30-17:00 **Pre-Conference Seminar "Gene editing & stem cells"**
Chairs: Patrick Harrison (IE) – Ulrich Martin (DE)

Coffee break – 15:05

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

18:00-19:00 **Opening Keynote Lecture**
Back to the Future - Ray Frizzell (US)

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

19:45-21:30 *Dinner*

Thursday, 30 March 2017 (Day 2)

07:30-08:45 *Breakfast*

08:45-10:30 Symposium 1 – Therapeutic approaches
Chairs: László Csanády (HU) - Martin Mense (US)

08:45-09:10 Discovery and Characterization of novel CFTR Modulators - Ashvani Singh (US)

09:10-09:35 Therapeutic potential of proteostasis modulation in cystic fibrosis - Nicoletta Pedemonte (IT)

09:35-10:00 The development of artificial anion transporters for cystic fibrosis - David Sheppard (UK)

10:00-10:10 Combined presentation Abstracts 83 and 20 - SPX-101 is a novel ENaC-targeted therapeutic for cystic fibrosis that restores mucus transport - David Scott (US) / ENaC internalization by SPX-101 is a novel CF therapy for all CFTR mutation - Juliana Sesma (US)

10:10-10:20 Abstract 112 - Amplifiers co-translationally increase CFTR levels at the ER membrane by improving membrane targeting of CFTR - John Miller (US)

10:20-10:30 Abstract 14 - A common mechanism for CFTR potentiators - Han-I Yeh (US)

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

11:00-12:45 Symposium 2 – Model systems

Chairs: Hugo De Jonge (NL) - Sinead Weldon (UK)

- 11:00-11:25 Mechanisms of Pulmonary Disease and Diabetes in Cystic Fibrosis Ferrets - John Engelhardt (US)
- 11:25-11:50 BAC Transgenic Mouse Models Expressing Human CFTR - Lara R. Gawenis (US)
- 11:50-12:15 Model systems to study CFTR dysfunction - Marc Chanson (CH)
- 12:15-12:25 Abstract 76 - Profiling of CFTR modulators GLPG1837 and GLPG2222 using intestinal organoids - Sara Musch (BE)
- 12:25-12:35 Abstract 118 - Human periodontal ligament stem cells: A novel model for cystic fibrosis research and personalized medicine - Mario Romano (IT)
- 12:35-12:45 Abstract 78 - Evaluation of the primary human nasal epithelial cell cultures as a biomarker in personalized Cystic Fibrosis treatment - Iwona Pranke (FR)

12:45-14:30 Lunch

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

Chair: Bertrand Kleizen (NL)

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

16:00-17:45 Symposium 3 – CFTR Structure

Chairs: TC Hwang (US) - Liz Miller (UK)

- 16:00-16:25 Calmodulin binding to CFTR R region and PKA-independent activation of CFTR - Julie Forman-Kay (CA)
- 16:25-16:50 3D structures of the full length CFTR protein: combining theoretical and experimental data - Isabelle Callebaut (FR)
- 16:50-17:15 The molecular structure of CFTR revealed by cryo-EM - Jue Chen (US)
- 17:15-17:25 Abstract 47 - The processing defect of $\Delta F508$ - and $\Delta Y512$ -CFTR are generated by similar mechanisms with different initial flaws - Jeng-Haur Chen (HK)
- 17:25-17:35 Abstract 52 - Molecular dynamics flexible fitting (MDFF) simulations identify new models of closed state CFTR - Hanoch Senderowitz (IL)
- 17:35-17:45 Abstract 51 - Biosynthetic and functional correction of CF-causing NBD2 defects - Patrick Thibodeau (US)

17:45-18:00 Break

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

Task Force on Personalised Medicine for CF

Special discussion groups:

1. Validation/ optimization of novel biomarkers
Discussion leader: Kris de Boeck (BE)
2. Assays for improved endpoints to evaluate novel drugs: intestinal and respiratory organoids
Discussion leader: Jeff Beekman (NL)
3. Novel therapeutic approaches to CF therapies: alternative channels and gene/cell therapies
Discussion leader: Marcus Mall (DE)

19:45-21:30 Dinner

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

Friday, 31 March 2017 (Day 3)

07:30-08:45 *Breakfast*

08:45-10:30 **Symposium 4 – Translational CF Research**

Chairs: John Engelhardt (US) - Kris De Boeck (BE)

08:45-09:10 Intestinal Organoids for personalised medicine - Jeff Beekman (NL)

09:10-09:35 Airway derived Organoids for personalised medicine - Martina Gentzsch (US)

09:35-10:00 Genetic and functional evidence for SLC26A9 as a CF modifier - Lisa Strug (CA)

10:00-10:10 Abstract 84 - Transduction of Rhesus macaque lung by AAV1 – Liudmila Cebotaru (US)

10:10-10:20 Abstract 107 - Nanoparticle-Mediated Delivery of Modified tRNAs as a Therapeutic Approach for CF caused by a Nonsense Mutation - Michael Torres (US)

10:20-10:30 Abstract 79 - Unravelling the mechanisms of airway epithelium repair in cystic fibrosis - Aderonke Sofoluwe (CH)

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

11:00-12:45 **Symposium 5 - Mucus and Mucins**

Chairs: David Thornton (UK) - Marcus Mall (DE)

11:00-11:25 Synergistic stimulation of gland secretion and mucus clearance - Jeff Wine (US)

11:25-11:50 Mucus Clearance in Normal Airways and Conversion into Attached Mucus in Chronic Lung Disease - Gunnar C. Hansson (SE)

11:50-12:15 Optical coherence microscopy (OCM) for assessment of mucus transport - Peter König (DE)

12:15-12:25 Abstract 81 - Relative roles of reduced mucus clearance and mucus hypersecretion in the pathogenesis of airway mucus plugging in mice - Sandra Christochowitz (DE)

12:25-12:35 Abstract 82 - CFTR and bicarbonate as determinants of airway mucus secretion and microrheology - Pierre Lesimple (FR)

12:35-12:45 Abstract 26 - Identification of proteins associated with goblet cell hyperplasia - Ilaria Musante (IT)

12:45-14:00 *Lunch*

14:00-18:30 **Free Afternoon**

18:30-19:30 **Flash Poster Session (odd numbers)**

Chair: Bertrand Kleizen (NL)

19:30 -21:30 *Dinner*

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

Saturday, 01 April 2017 (Day 4)

07:30-08:45 *Breakfast*

08:45-10:30 **Symposium 6 – Cell Physiology and Ion transport**

Chairs: David Sheppard (UK) - Luis Galiotta (IT)

08:45-09:10 Studying asymmetric movements in CFTR's two nucleotide binding sites - László Csanády (HU)

09:10-09:35 Functional Integrity of CFTR in the Cell Membrane: Role of ATP binding in Site 1 – TC Hwang (US)

09:35-10:00 Novel protease inhibitors to balance ENaC function in CF airways - Lorraine Martin (UK)

10:00-10:10 Abstract 05 - The contribution of the paracellular pathway to HCO₃⁻ secretion by human airway epithelial cells - Vinciane Saint-Criq (UK)

10:10-10:20 Abstract 27 - Role and structure-function relationship of the TMEM16A chloride channel - Paolo Scudieri (IT)

10:20-10:30 Abstract 29 - The calcium-activated potassium channel KCa3.1 Inhibits sodium absorption in airway epithelial cells - Génesis Vega (CL)

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

11:00-12:45 **Symposium 7 – CFTR Folding, Trafficking and Activity**

Chairs: Isabelle Callebaut (FR) - Margarida Amaral (PT)

11:00-11:25 Interactions and post-translational modifications: regulating CFTR trafficking - Carlos Farinha (PT)

11:25-11:50 A yeast model to understand biosynthesis, folding and traffic of ABC transporters - Liz Miller (UK)

11:50-12:15 Regulation of CFTR Channel Function by Lipids and Lipid-mediated Signaling - Nael McCarty (US)

12:15-12:25 Abstract 34 - Silent mutations affecting CFTRs translational landscape rescue folding mutations - Robert Rauscher (DE)

12:25-12:35 Abstract 102 - A high-content siRNA screen at near genomic scale reveals novel F508del-CFTR therapeutic targets - Hugo Botelho (PT)

12:35-12:45 Abstract 50 - Rescue Δ508-CFTR with nanobodies - Marie Overtus (BE)

12:45-14:30 *Lunch*

14:30-16:40 **Symposium 8 – Inflammation and Host Pathogen interaction**

Chairs: Jeff Wine (US) - Martina Gentzsch (US)

14:30-14:55 Bioactive lipids and CF lung inflammation, innocent markers or guilty agonists? - Bob Scholte (NL)

14:55-15:20 Cathepsin S in CF lung disease - Sinead Weldon (UK)

15:20-15:45 Novel genetically-diverse mouse models to unravel the complexity of the lung infection: Alessandra Bragonzi (IT)

15:45-16:10 The role of CFTR in the Neutrophil - Gerry McElvaney (IE)

16:10-16:20 Abstract 59 - Early alterations in airway mucins glycosylation and mucociliary clearance players in the development of cystic fibrosis lung disease - Ignacio Caballero (FR)

16:20-16:30 Abstract 63 - Altered Airway Macrophage Phenotype and Function in Mice with Mucociliary Clearance Dysfunction - Michelle Paulsen (DE)

16:30-16:40 Abstract 70 - SPLUNC1 is pH-dependent and reduces Burkholderia cepacia growth in airway surface liquid – Robert Tarran (US)

16:40-17:10 *Coffee Break*

17:10-18:10 **Flash Paper Session - CFTR structures**
Mike Gray (UK)

Or

How to write a successful paper
Emma Grainger (Elsevier)

18:10-18:15 *Break*

18:15-19:15 **Closing Keynote lecture**

CF Airway metagenome – Burkhard Tümmler (DE)

20:30 *Dinner / Social Event*