Cystic Fibrosis Foundation Research Conference:
Pushing the Frontiers
June 18-22, 2017
Skamania Lodge
Stevenson, WA
AGENDA

SUNDAY, JUNE 18

Arrival from airport (shuttles provided from PDX throughout the day)
5 – 6 p.m. General registration
6 – 8 p.m. Welcome dinner and opening remarks

MONDAY, JUNE 19

7 – 8 a.m. Breakfast

SCIENTIFIC SESSION I: CFTR: What Do We Know and How Do We Apply It to Rare Mutations?
Moderator: Phil Thomas
8 – 8:15 a.m. Introduction
8:15 – 8:40 a.m. 1.1 How CFTR Modulators Work on Rare Mutations (Ineke Braakman)
8:40 – 9:05 a.m. 1.2 Understanding Complex Alleles (Garry Cutting)
9:05 – 9:30 a.m. 1.3 HTS for Nonsense Readthrough and NMD Agents (Bob Bostwick)
9:30 – 9:45 a.m. 1.4 Engineered tRNA Suppression of CFTR Nonsense Mutations (John Lueck)
9:45 – 10 a.m. 1.5 R334W CFTR Supports Bicarbonate Transport (Liudmilla Cebotaru)
10 – 10:30 a.m. Break

SCIENTIFIC SESSION II: Panel Discussion on In Vitro Models Predicting In Vivo Responses
Moderator: Marty Solomon
10:30 a.m.–12:30 p.m. 2.1 Panelists: AP Naren, Calvin Cotton, Martina Gentzch, and Theo Moraes

12:30 – 1:30 p.m. Lunch

SCIENTIFIC SESSION III: Therapeutic Approaches to Correct the MCC Defect in CF
Moderator: Mike Meyerburg
1:30 – 2 p.m. 3.1 ENaC as a Target to Restore Airway Hydration (Robert Tarran)
2 – 2:30 p.m.  **3.2 The Discovery of TMEM16A/ANO-1 Potentiators as Mucokinetics for CF** (Henry Danahay)

2 – 3 p.m.  **3.3 Development of Novel Mucolytics for CF** (William Thelin)

3 – 3:15 p.m.  **3.4 Angiotensin Receptor Blockers Reveal Anti-Inflammatory Properties to Improve Mucociliary Dysfunction in CF Cells In Vitro and in an Ovine Model In Vivo** (Matthias Salathe)

3:15 – 3:30 p.m.  **3.5 ATP12A Is the Major Source of Proton Secretion at Physiological pH Values in Normal and CF Human Cultures** (Ian Thornell)

3:30 – 5 p.m.  Free time

5 – 6 p.m.  Flash posters

6 – 7 p.m.  Dinner

**SCIENTIFIC SESSION IV: CFTR 3-D Structure**

**Moderator: Patrick Thibodeau**

7 – 7:18 p.m.  **4.1 CFTR Structural Studies** (Bob Ford)

7:18 – 7:36 p.m.  **4.2 What Are We Learning From Structural Models?** (Hanoch Senderowitz)

7:36 – 7:54 p.m.  **4.3 Toward High-Resolution Crystal Structures of the Cystic Fibrosis Transmembrane Conductance Regulator** (Martin Caffrey)

7:55 – 9 p.m.  **4.4 Panel Discussion: How Can CFTR Structural Information Be Exploited to Advance the Field?** Panelists: Bob Ford, Hanoch Senderowitz, Martin Caffrey, Jack Riordan and Phil Thomas

9 – 10:30 p.m.  Posters (and cocktails)

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### TUESDAY, JUNE 20

**7 – 8 a.m.**  Breakfast

**SCIENTIFIC SESSION V: CFTR Modulators and Host Responses**

**Moderator: Bruce Stanton**

8 – 8:30 a.m.  **5.1 Ivacaftor-Induced Changes in CF Monocytes Suggest Dysregulated Monocyte Responses May Contribute to CF Disease** (Katherine Hisert)

8:30 – 9 a.m.  **5.2 CFTR Modulator Impact on Macrophage Function and Inflammatory Networks** (Benjamin Kopp)

9 – 9:20 a.m.  **5.3 Pathways Influenced by Ivacaftor in Neutrophils** (Gerry McElvaney)

9:20 – 9:40 a.m.  **5.4 CFTR Modulation Alters Neutrophil Responses to NTM Infections** (Kerstin Pohl)

9:40 – 10 a.m.  **5.5 The Combined Effect of Ivacaftor With Intensive Antibiotic Treatment** (Pradeep Singh)

10 – 10:30 a.m.  Break
SCIENTIFIC SESSION VI: CF Inflammation
Moderator: Jay Kolls

10:30 – 11 a.m.  6.1 A Phase 2 Study of the Safety, Pharmacokinetics, and Efficacy of Anabasum (IBT-101) in CF (James Chmiel)
11 – 11:30 a.m.  6.2 Neutrophil Elastase: Balancing the Cutting Edge (Stuart Elborn)
11:30 a.m. – 12 p.m.  6.3 Early Life Inflammation in CF: Results from the AREST CF Study (Stephen Stick)
12 – 12:15 p.m.  6.4 Altered Immune Function and Defects in Gut Microbiome Maturation in Infants With CF (Barbara Kazmierczak)
12:15 – 12:30 p.m.  6.5 Probing the Origin of Airway Inflammation in CF Infants: High Content BAL Phenotyping Outlines Opportunities for Disease Monitoring and Targeted Immunotherapy (Rabindra Tirouvanziam)

12:30 – 1:30 p.m.  Lunch
1:30 – 6 p.m.  Free time/organized activity (Bonneville Lock and Dam or Multnomah Falls Hike)
6 – 7 p.m.  Dinner

SCIENTIFIC SESSION VII: Difficult to Treat Infections in CF
Moderator: John LiPuma

7 – 7:30 p.m.  7.1 NTM Transmission (Andres Flotto)
7:30 – 8 p.m.  7.2 Tackling Mycobacterium Abscessus (Diane Ordway)
8 – 8:30 p.m.  7.3 S. aureus Small Colony Variants in CF (Lucas Hoffman)
8:30 – 8:45 p.m.  7.4 Selective Forces Driving Pathogen Diversification During Chronic Infections of the CF Airway (Vaughn Cooper)
8:45 – 9 p.m.  7.5 Respiratory Virus Infection Dysregulates Nutritional Immunity to Stimulate Pseudomonas aeruginosa Biofilm Biogenesis (Jennifer Bomberger)

9 – 10:30 p.m.  Posters (and cocktails)

WEDNESDAY, JUNE 21

7 – 8 a.m.  Breakfast

SCIENTIFIC SESSION VIII: Advances in Genetic Editing
Moderator: Marie Egan

8 – 8:30 a.m.  8.1 Homologous Recombination Driven Genome Editing With Adeno-Associated Viruses Derived From Human Hematopoietic Stem Cells (Jeff Ellsworth)
8:30 – 9 a.m.  8.2 CRISPR-Mediated Homology-Independent Targeted Integration (HITI) of CFTR Super Exons to Correct Multiple CF-Causing Mutations With a Single Cas9/gRNA (Patrick Harrison)
9 – 9:20 a.m.  8.3 Editing CFTR Mutations in iPSCs (Brian Davis)
9:20 – 9:40 a.m.  8.4 Lessons Learned from Gene Editing Clinical Trials: What Can We Use to Advance Gene Editing in CF? (Anthony Conway)
9:40 – 10 a.m.  8.5 A CRISPR/Cas9 Based Gene Editing Platform to Create Cell Lines Expressing CFTR Variants From the Native Gene Locus and Compatible With Functional Assay (Hillary St. John)

10 – 10:30 a.m.  Break

**SCIENTIFIC SESSION IX: Delivery to the Lung**
**Moderator: James Dahlman**

10:30 – 11 a.m.  9.1 UK Lentivirus Trial (Stephen Hyde)

11 – 11:30 a.m.  9.2 Developing Antisense Therapeutics for CF (Lulu Huang)

11:30 – 11:50 a.m.  9.3 Development of Novel Lipid Nanoparticles for In Vivo Delivery of mRNA and siRNA in Lung Tissue (Xavier de Mollerat du Jeu)

11:50 a.m. – 12:10 p.m.  9.4 Mucus Penetrating Non-Viral Vectors (Justin Hanes)

12:10 – 12:30 p.m.  9.5 Boosting Endosomal Escape of Lipid Nanoparticle-Delivered Messenger RNA (Gaurav Sahay)

12:30 – 1:30 p.m.  Lunch

1:30 – 3:30 p.m.  **Workshop: Improving Access and Utilization of CF Animal Models (Outcomes From the Animal Models Survey and 2016 Workshop)**

Lane Clarke, John Engelhardt, and Katherine Tuggle

3:30 – 6 p.m.  Free time

6 – 7 p.m.  Dinner

**SCIENTIFIC SESSION X: Stem Cells and Tissue Regeneration**
**Moderator: Finn Hawkins**

7 – 7:30 p.m.  10.1 Transplantation of Airway Epithelial Stem Cells (Susan Reynolds)

7:30 – 8 p.m.  10.2 Understanding the Airway Stem Cell Niche (Jason Rock)

8 – 8:30 p.m.  10.3 Generating iPSCs from Large Animal Models (Amy Firth)

8:30 – 8:45 p.m.  10.4 From Human Pluripotent Stem Cells to Committed Airway Stem Cells (Hongmei Mou)

8:45 – 9 p.m.  10.5 iPS Cells: Derived Anterior Foregut Progenitor Cells Enable Novel CFTR Nonsense Mutation Drug Screen (Jed Mahoney)

9 – 9:15 p.m.  Closing remarks (Bill Skach)

9:15 – 10:30 p.m.  Posters (and cocktails)

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**THURSDAY, JUNE 22**

6 – 8 a.m.  Breakfast available

Depart for airport (shuttles provided to PDX throughout the day)