



16th ECFS Basic Science Conference Pre-Conference Meeting

CFTR: new insights on structure and function and implications for modulation

Dubrovnik, Croatia, 27 March 2019 1.30 pm to 5 .00 pm

The pre-conference meeting is a collaboration by national CF Patient Associations (France, Germany, Italy, Belgium, Netherlands, UK), CFE and the ECFS

Moderators: Dr. Bertrand Kleizen (Utrecht University) and Professor David N. Sheppard (University of Bristol)

After almost 30 years of intensive biochemical and functional studies of CFTR, and recent advances in structural biology, we have achieved deep understanding of the relationship between CFTR structure and function. This knowledge provides the basis to understand how patient mutations cause disease, decipher mode of action of clinically-licensed drugs, and above all will determine CFTR's 'sweet-spots' for future drug development.

Current drug developments provide a brighter future for all individuals living with CF, especially newly diagnosed young children. Despite this, the research community, both academic and biotech, should not let down its guard. Several frequent CF patient mutations are not corrected by clinically-licensed drugs and, for sure, we do not know the potential adverse effects of life-long treatment with CFTR modulators.

This pre-conference will inform you about the latest insights into CFTR's structure-function relationship and its impact. It will provide a platform to discuss if and how we should shift our research efforts to make the future for all individuals living with CF even brighter.



Program

1.30-1.35 (5 min) Pre-conference introduction by **Bertrand Kleizen**:

General introduction

1.35-2.10 (25 min+ 10 min discussion): Professor **John F. Hunt** (Columbia University)

The allosteric effects of the F508del mutation in human CFTR elucidated by cryo-EM enabled by protein engineering

2.10-2.45 (25 min+ 10 min discussion): Professor **Isabelle Callebaut** (Sorbonne Université)

Conformational landscape of CFTR from experimental and simulation data

2.45-3.15 pm (30 min) **Coffee break (+ Discussion)**

3.15-3.50 (25 min+ 10 min discussion): Professor **Tzyh-Chang Hwang** (University of Missouri-Columbia)

On the molecular mechanism of action for CFTR potentiators

3.50-4.25 pm (25 min+ 10 min discussion): Professor **Isabelle Sermet-Gaudelus** (Institut Necker Enfants Malades/INSERM U1151)

CFTR therapeutics: current evidence, gaps in knowledge, and future directions

4.25-4.55 (30 min discussion)

Discussion

4.55-5.00 pm: **David N. Sheppard**

Conclusion