

XVIII Convention of Investigators in Cystic Fibrosis

(Webinar, November 19-20, 2020) Preliminary program for oral presentations

Thursday, 19 November 2020

08.25 – 08.30 Introduction & Greetings

Session 1

BASIC MECHANISMS FOR RECOVERY OF MUTATED CFTR

Chairman: Gambari R. Co-chairman: Borgo G.

08.30 - 08.45 **Baroni D.**

Dissecting the rescue mechanisms mediated by CFTR correctors (FFC #3/2018, concluded)

08.46 - 09.00 Hirsch E.

In depth-characterization of the molecular mechanisms underlying PI₃Kγ-mediated regulation of CFTR (FFC#8/2018, concluded)

09.01 - 09.15 Gambari R., Corradini R.

Revealing the microRNAs-transcription factors network in cystic fibrosis: from microRNA therapeutics to precision medicine (CF-miRNA-THER) (FFC#7/2018, concluded)

09.16 – 09.30 Discussion (Replies to chat interventions)

09.31 - 09.40 Technical break

09.41 - 09.55 Aureli M., Tamanini A.

Development of ganglioside GM1-based therapy to improve F508delCFTR rescue approaches (FFC#2/2018, concluded)

09.56 - 10.10 Luini A., Tamanini A., Borgatti M.

Targeting the signalling network controlling proteostasis and inflammation to rescue F508del-CFTR (FFC#7/2019, concluded)

10.11 - 10.25 **Salvi M.**

Functional role of post-translational modifications in F508del correction (FC#11/2019, concluded)

10.26 – 10.40 Discussion (Replies to chat interventions)

10.41 - 11.00 Coffee and technical break

Session 2

THERAPY ORIENTED IN VITRO/EX VIVO PREDICTIVE MODELS (Theratyping)

Chairman: Galietta L. Co-Chairman: Braggion C.

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11.01 - 11.15 Eramo A., Lucarelli M.

Establishment of Conditionally Reprogrammed Airway Epithelial Stem Cell cultures from nasal epithelia of Cystic Fibrosis patients: exploring response to CFTR-modulating drugs for correlation with genetic profile (theratyping) and restoring CFTR function through gene editing approaches (FFC#12/2018, concluded)

11.16 - 11.30 **Sorio C.**

Testing intestinal organoids for the prediction of response to CFTR potentiators and correctors used in clinic (FFC#13/2018, concluded)

11.31 – 11.45 Frulloni L., de Jonge H., Lucidi V.

Intestinal organoids for assessment and pharmacological correction of abnormalities in fluid transport and anion currents in patients affected by pancreatitis (FFC#6/2018, concluded)

11.46 – 12.00 Discussion (Replies to chat interventions)

12.01 – 12.10 Technical break

12.11 - 12.25 Averna M., Marengo E.

Multiomic approach for the identification of new leukocytes biomarkers directly related to a restored CFTR activity following ex vivo treatment with VX770 12/19 (FFC#12/2019, concluded)

12.26 - 12.40 Laudanna C.

Monocyte integrin activation as a cystic fibrosis drug evaluation test (FFC#13/2019, concluded)

12.41 - 12.55 **Pedemonte N., Cavalli A.**

Theratyping orphan mutations in Italian cystic fibrosis patients: efficacy of CFTR modulators and RNF5 inhibitors (FFC#9/2019, in progress)

12.56 - 13.10 Netti P., Di Bernardo P.

Investigating epithelial-stromal crosstalk in full thickness cystic fibrosis model on chip for evaluating novel therapeutic strategies (FFC#14/2019, in progress)

13.11 – 13.30 Discussion (Replies to chat interventions)

13.31 – 14.00 Lunch and technical break

Session 3

CLINICAL ISSUES

Chairman: Cipolli M. Co-chairman: Gangemi M.

14.01 - 14.15 Pasut G., Percudani R.

Therapeutic potential of a long-acting lung-specific DNase (DNase2b) for the treatment of CF (FFC#9/2018, concluded)

14.16 - 14.30 Romano M., Lanuti P.

Identification and validation of circulating microvesicle analysis as a new ex vivo assay to monitor cystic fibrosis disease (FFC#29/2018, concluded)

14.31 – 14.45 Bartoloni A., Viscoli C., Cariani L., Fiscarelli E.

Aspergillus pulmonary disease in cystic fibrosis (CF) patients: multicentre perspective observational study based on new diagnostic tests to evaluate the prognostic value on the CF disease (FFC#26/2018, concluded)



14.46 – 15.00 Discussion (Replies to chat interventions) 15.01 – 15.10 Technical break

15.11 - 15.25 Palleschi A., Aliverti A.

Use of multivolume MRI instead of ionizing imaging techniques for surveillance in young patients after lung transplantation for cystic fibrosis (FFC#27/2018, concluded)

15.26 - 15.40 Terlizzi V., Padoan R., Tosco A., Claut LE.

Cystic Fibrosis screen positive inconclusive diagnosis (CFSPID): an italian multicenter survey evaluating prevalence, clinical data, management and outcome (FFC#30/2018, concluded)

15.41-15.55 Casciaro R., Graffigna G.

Patient Engagement in Cystic Fibrosis: a cross sectional multi-stakeholder study (FFC#25/2019, concluded)

15.46 – 16.00 Discussion (Replies to chat interventions)

Friday, 20 November 2020

Session 4

NEW PATHS TO RESCUING MUTATED CFTR

Chairman: Pedemonte N. Co-chairman: Dechecchi C.

08.31 – 08.45 **Barraja P., Scudieri P.**

Towards the discovery of new correctors based on nitrogen heterocyclic systems (FFC#4/2018)

08.46 - 09.00 Armirotti A.

Proteomic profiling of F508del-CFTR cells to identify new pharmacological targets for CF (FFC#1/2019)

09.01 – 09.10 Discussion (Replies to chat interventions)

09.11 - 09.25 Duga S., Melfi R.

Small molecules modulating splicing as novel CFTR amplifier drugs (FFC#5/2019, concluded)

09.26 - 09.40 Rusnati M., Fossa P., Orro A.

Rescuing defective CFTR applying a drug repositioning strategy based on computational studies, surface plasmon resonance and cell-based assays (FFC#10/2019, concluded)

09.41 – 09.50 Discussion (Replies to chat interventions)

09.51 – 10.00 Technical break

Session 5

NEW APPROACHES TO ANTIMICROBIAL TREATMENTS

Chairman: Cirilli N. Co-chairman: Lorè N.



10.01 - 10.15 Pasca MR.

New weapons against *Mycobacterium abscessus* and other nontuberculous mycobacteria (FFC#19/2018, concluded)

10.16 – 10.30 **Cirillo DM.**

Preclinical evaluation of liposomes carrying bioactive lipids as an immune therapeutic tool against in vitro and vivo infection with *Mycobacterium abscessus* (FFC#17/2019, concluded)

10.31 – 10.40 Discussion (Replies to chat interventions)

10.41 – 10.55 **Notomista E., Pizzo E.**

In vitro and in vivo efficacy of an antimicrobial and antibiofilm designed peptidomimetic against CF lung pathogens (FFC#18/2018, concluded)

10.56 – 11.10 Sanguinetti M., Vitali A., Iafisco M., Catalucci D.

Biocompatible and inhalable antimicrobial-loaded nanoparticles for the counteraction of biofilm formation and antibiotic resistance: towards a potential new therapy for CF related infections (FFC#20/2018, concluded)

11.11 – 11.30 Discussion (Replies to chat interventions)

11.31 – 11.50 Coffee and technical break

Session 6

INFLAMMATION IN CYSTIC FIBROSIS: AN OBSTACLE COURSE

Chairman: Cabrini G. Co-chairman: Aureli M.

11.51 – 12.05 **Romani L.**

Pharmacology and therapeutics of inhaled indoles, as aryl hydrocarbon receptor ligands, in cystic fibrosis (FFC#24/2018, concluded)

12.06 - 12.20 Dechecchi MC., Guaragna A.

Evaluation of anti-inflammatory treatments for CF lung disease in murine models of lung infection in vivo: insights on the anti-inflammatory effect of β -sitosterol and anti-inflammatory/anti-infective activity of L-Miglustat (FFC#20/2019, concluded)

12.21 – 12.35 Lampronti I., Chilin. A

Multi-task evaluation of TMA analogues as antiinflammatory treatments for CF lung disease (FFC#22/2019, concluded)

12.36 – 12.50 Discussion (Replies to chat interventions)

12.51 – 13.20 Lunch and technical break

13.21 - 13.35 **Antonelli G.**

Ex vivo study on Type I and III interferon response and virus—bacteria interactions in fibrosis cystic patients: a new approach to try to develop alternative therapeutic strategy (FFC#14/2018, concluded)

13.36 – 13.50 **Cigana C.**

Off-target effects of CFTR-modulators in preclinical infection models (FFC#15/2018, concluded)



13.50 – 14.04 **Pistocchi AS.** Potential action of phages as immunomodulators in cystic fibrosis (FFC#23/2019, concluded)

14.04 – 14.19 Discussion (Replies to chat interventions)

14.19 – 14.24 *Closing Remarks*

Note. Chat interventions are only possible in written form directly on the screen and are reserved for registered participants