Fondazione Ricerca Fibrosi Cistica - Onlus
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in collaborazione con

Azienda Ospedaliera Universitaria Integrata Verona

con il patrocinio di

CAMERA DI COMMERCIO INDUSTRIA ARTIGIANATO AGRICOLTURA VERONA

XVII CONVENTION OF FFC INVESTIGATORS IN CYSTIC FIBROSIS

14-16, November, 2019

Camera di Commercio Verona, Congress Center
Corso Porta Nuova, 96

Final Program

Thursday, November 14th

09:00 – 10:30 *Registration and poster display*

10:30 – 10:45 Welcome & introductory messages from: ***Matteo Marzotto****, FFC President;* ***Marco Cipolli****, SIFC President;* ***Gianna Puppo Fornaro****, LIFC President*

10:45 – 12:45 ***Plenary Session 1***

DISEASE MODELS & PREDICTIVE TESTING

Chairmen: **Lucarelli M., Cresta F.**

01. Netti P., Di Bernardo D.
A novel Full Thickness Cystic Fibrosis model on a microfluidic chip to study pathogenic mechanisms and evaluate therapeutic strategies (FFC#8/2017. Concluded – FFC#14/2019. Extension) (20')

02. 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. Ongoing) (8')

- *Discussion (10')*

03. Sorio C.
Testing intestinal organoids for the prediction of response to CFTR potentiators and correctors used in clinic (FFC#13/2018. Ongoing) (8')

04. Frulloni L., Lucidi V., de Jonge H.
Intestinal organoids for assessment and pharmacological correction of abnormalities in fluid transport and anion currents in patients affected by pancreatitis (FFC#6/2018. Ongoing) (8')

- *Discussion (8')*

05. Averna M., Marengo E.
Proteomic approach for the identification of new leukocytes biomarkers directly related to a restored CFTR activity following ex vivo treatment with VX-770 (FFC#12/2019. New, pilot) (8')

06. Laudanna C.
Monocyte integrin activation as a cystic fibrosis drug evaluation test (FFC#13/2019. New) (8')

- *Discussion (8')*

07. Lorè NI.
Phenotyping new genetically-diverse mouse models mirroring the complexity of the Cystic Fibrosis pathology (FFC#4/2017. Concluded) (15')
08. Bragonzi A.
Bridging airway mucus-microbiota-host genotype to define novel cystic fibrosis animal models (FFC#2/2019. New) (8')

- *Discussion (11')*

12:45 – 14:00 - Lunch bag & ***Poster view/discussion***

14:00 – 16:00 ***Plenary Session 2***

CLINICAL ISSUES

Chairmen: **Mangoni ML., Cimino G.**

09. 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. Ongoing) (8')
10. Battezzati A., Colombo C., Lucidi V., Lucanto MC., Mari A.
Early Derangements of Glucose Tolerance in Cystic Fibrosis: effect of CFTR Modulators (FFC#24/2019. New) (8')
11. Pasut G., Percudani R.
Therapeutic potential of a long-acting lung-specific DNase (DNase2b) for the treatment of CF (FFC#9/2018. Ongoing) (8')

- *Discussion 10'*

ALTERNATIVE ANTIMICROBIAL STRATEGIES

12. Antonelli G.
Ex vivo study on Type I and III interferon response and virus–bacteria interactions in cystic fibrosis patients: a new approach to try to develop alternative therapeutic strategy (FFC#14/2018. Ongoing) (8')
13. Leoni L.
Drug repurposing for antivirulence therapy against Pseudomonas aeruginosa (FFC#17/2018. Concluded) (15')

- *Discussion (12')*

14. Bevivino A., Mengoni A., Segata N.
A longitudinal metagenomic analysis to uncover microbial signatures of CF lung disease: unravelling host-microbial community interactions in humans and animal models (FFC#19/2017. Concluded) (15')
15. Visca P.
Gallium as an antibacterial agent in cystic fibrosis: animal studies for the delivery of inhalable formulations to the clinic (FFC#19/2019. New) (8')
16. Ascenzioni F.
Pharmacological inhibition of colistin resistance in gram-negative cystic fibrosis pathogens (FFC#15/2019. New) (8')

- *Discussion (13')*

16:00 – 16:30 Coffee break & ***Poster view/discussion***

16:30 – 18:30 ***Plenary Session 3***

LUNG TRANSPLANTATION

Chairmen: **Romano M., Messore B.**

17. Nosotti M.
Extracorporeal photopheresis as induction therapy to prevent acute rejection after lung transplantation in cystic fibrosis patients (FFC#24/2017. Concluded) (15')
18. Rea F., Paolo Schena F.
Identification of early molecular biomarkers of acute and chronic rejection in cystic fibrosis patients with lung transplant through the application of omics technologies (FFC#28/2018. Concluded) (15')
19. Scaravilli V.
Right ventricle dysfunction in cystic fibrosis patients undergoing lung transplantation (FFC#27/2019. New) (8')
20. 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. Ongoing) (8')

- *Discussion (13')*

CLINICAL MONITORING AND CARE

21. Bartoloni A., Viscoli C., Cariani L., Fiscarelli EV.
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. Ongoing) (8')
22. Romano M., Lanuti P.
Identification and validation of circulating microvesicles analysis as a new ex vivo assay to monitor cystic fibrosis disease (FFC#29/2018. Ongoing) (8')
23. Morana G.
Standardized Ventilation Inflammation Perfusion and Structure (VIPS) MRI platform for monitoring Cystic Fibrosis Lung Disease (FFC#26/2019. New) (8')

- *Discussion (13')*

24. Lleò M.
Investigating Achromobacter xylosoxidans pathogenicity and clinical role in CF lung infection (FFC#18/2019. New) (8')
25. Casciaro R., Graffigna G.
Patient Engagement in Cystic Fibrosis: a cross-sectional multi-stakeholder study (FFC#25/2019. New) (8')

- *Discussion (10')*

18:30 – 19:30 ***Small group meetings*** *(on individual initiative and self-managed, Poster hall)*

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Friday, November 15th

08:30 – 11:00 **_Plenary Session 4_**

POSSIBLE TARGETS AND MECHANISMS OF CFTR MODULATORS

Chairmen: **Bandiera T., Pisi G.**

26.Armirotti A.
Proteomic profiling of F508del-CFTR cells to identify new pharmacological targets for CF (FFC#1/2018. Concluded – FFC#1/2019. Extension) (20')
27. Baroni D.
Dissecting the rescue mechanisms mediated by CFTR correctors (FFC#3/2018. Ongoing) (8')
28. Galietta LJV.
Identification of deubiquitinases and ubiquitin ligases that affect mutant CFTR rescue (FFC#2/2017. Concluded – FFC#6/2019. Extension) (20')

- *Discussion (12')*

29. 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. Ongoing) (8')
30. Cozza G., Esposito S., Raia V.
Restoring defective proteostasis in Cystic Fibrosis: novel strategies for F508del-CFTR repair (FFC#4/2019. New) (8')
31. Piacentini M., Maiuri L., Delogu G.
Dissecting the mechanism of action of the TG2 inhibitor cysteamine on Cystic Fibrosis (FFC#10/2018. Concluded) (15')

- *Discussion (12')*

32. Duga S.
*Small molecules modulating splicing as novel CFTR amplifier drugs (*FFC#5/2019. *New) (8')*
33. Salvi M.
*Functional role of post-translational modifications in F508del-CFTR correction (*FFC#11/2019. *New, pilot) (8')*
34. Luini A., Tamanini A., Borgatti M.
*Targeting the signalling network controlling proteostasis and inflammation to rescue F508del-CFTR (*FFC#7/2019. New) (8')
35. Cigana C.
*Off-target effects of CFTR-modulators in preclinical infection models (*FFC#15/2018*, Ongoing) (8')*

- *Discussion (13')*

11:00 – 11:30 Coffee break and ***Poster view/discussion***

11:30 – 13:30 **_Plenary Session 5_**

SEARCH FOR CFTR MODULATORS AND ITS IMPLICATIONS FOR CARE

Chairmen: **Galietta LJV., Buzzetti R.**

36. Lee T.
State of the art and perspectives on CFTR modulators. (Lecture, 40')

- *Discussion (20')*

37. Garattini S.
The social cost of new drugs and its implications for research and care (Lecture, 40')
- *Discussion (20')*

13:30 – 14:30 Lunch bag and ***Poster view/discussion***

14:30 – 16:15 ***Plenary session 6***

POSSIBLE NEW MODULATORS OF MUTANT CFTR

Chairmen: **Cabrini G., Bresci S.**

38. Bandiera T., Pedemonte N., Galietta LJV.
Preclinical development of the ARN23765 corrector and search for its backup (FFC/TFCF extension 1, 2, 3. Ongoing) (15')
39. Pedemonte N., Cavalli A.
RNF5 inhibitors as potential drugs for Cystic Fibrosis basic defect (FFC#9/2017. Concluded) (15')
40. Barraja P., Scudieri P.
Towards the discovery of new correctors based on nitrogen heterocyclic systems (FFC#4/2018. Ongoing) (8')

- *Discussion (15')*

41. Hirsch E.
In depth-characterization of the molecular mechanisms underlying PI3Kγ-mediated regulation of CFTR (FFC#8/2018. Ongoing) (8')
42. Aureli M., Tamanini A.
Development of ganglioside GM1-based therapy to improve F508delCFTR rescue approaches (FFC#2/2018. Ongoing) (8')
43. 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#11/2018. Concluded – FFC#10/2019. Extension) (20')

- *Discussion (14')*

16:15 – 16:45 Coffee break and ***Poster view/discussion***

16:45 – 18:10 ***Plenary Session 7***

GENE AND RNA EDITING

Chairmen: **Sorio C., Majo F.**

44. Di Leonardo A.
Investigating CRISPR-CAS13b as a tool for the RNA editing of CFTR mRNA with premature stop codon (FFC#5/2018. Concluded) (15')
45. Cereseto A., Arosio D., Debyser Z.
SpliceFix: fixing splicing defects in the CFTR gene through CRISPR/Cas9 technology (FFC#1/2017. Concluded) (15')
46. Cereseto A., Arosio D.
Harnessing CRISPR/Cas9 technology to revert F508del-CFTR defect (FFC#3/2019. New) (8')

- *Discussion (14')*

TARGENTING NON F508del-CFTR MUTATIONS

47. Lentini L., Pibiri I.
Optimization of a new lead promoting the readthrough of nonsense mutations for the CFTR rescue in human CF cells (FFC#3/2017. Concluded) (15')
48. Mangoni ML.
Antimicrobial peptides from amphibian skin for treatment of lung pathology in cystic fibrosis: advanced in vitro and in vivo functional characterization (FFC#8/2019. New) (8')
49. Pedemonte N., Cavalli A.
Theratyping orphan mutations in Italian cystic fibrosis patients: efficacy of CFTR modulators and RNF5 inhibitors (FFC#9/2019. New) (8')

- *Discussion (14')*

18:10 – 19:00 ***Small group meetings*** *(on individual initiative and self-managed, Poster hall)*

20:00 – 23:00 Welcome dinner and entertainment

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Saturday, November 16th

09:00 – 10:40 ***Plenary Session 8***

NON TUBERCOLOUS MYCOBACTERIA

Chairmen: **Visca P., Delfino E.**

50. Fraziano M.
Preclinical study of a combined host- and pathogen directed approach based on bioactive liposomes and bacteriophages against Mycobacterium abscessus infection (FFC#21/2019, New) (8')
51. Cirillo DM.
Preclinical evaluation of liposomes carrying bioactive lipids as an immune therapeutic tool against in vivo infection with Mycobacterium abscessus (FFC#16/2018. Concluded – FFC#17/2019. Extension) (20')
52. Pasca MR.
New weapons against Mycobacterium abscessus and other nontuberculous mycobacteria (FFC#19/2018, Ongoing) (8')

- *Discussion (12')*

ADVANCES IN CF MICROBIOLOGY

53. Notomista E., Pizzo E. *In vitro and in vivo efficacy of an antimicrobial and antibiofilm designed peptidomimetic against CF lung pathogens* (FFC#18/2018. Ongoing) (8')
54. 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. Ongoing) (8')

55. Biavasco F.
- Induction of viable but non-culturable forms, possibly responsible for treatment failure, in "in vitro" biofilms of Pseudomonas aeruginosa. Role of antibiotics and antibiotic concentrations (FFC#13/2017. Concluded) (15')
- Fighting Pseudomonas aeruginosa persisters in cystic fibrosis pulmonary infections: improved detection and therapeutic strategies (FFC#16/2019. Extension) (5')

- *Discussion (12')*

10:40 – 11:10 *Coffee Break and* ***Poster view/discussion***

11:10 – 13:25 ***Plenary Session 9***

CF INFLAMMATION: THERAPEUTICAL APPROACHES?

Chairmen: **Bruni P., Cigana C., Lucanto MC.**

56. Bellet MM.
Thymosin alpha 1 in cystic fibrosis: from the lung to the gut (FFC#21/2018. Concluded) (15')
57. Bianchi ME.
Preclinical testing in cystic fibrosis of a repurposed molecule targeting HMGB1 (FFC#22/2018. Concluded) (15')

- Discussion (10')

58. Dechecchi MC., Guaragna A.
- Evaluation of anti-inflammatory treatments for CF lung disease in murine models of lung infection in vivo (FFC#23/2018. Concluded) (15')
- 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. Extension) (5')
59. Romani L.
Pharmacology and therapeutics of inhaled indoles, as aryl hydrocarbon receptor ligands, in cystic fibrosis (FFC#24/2018. Ongoing) (8')
60. Lampronti I., Chilin A.
Multi-task evaluation of TMA analogues as anti-inflammatory treatments for CF lung disease (FFC#*22/2019. New) (8')*

- *Discussion (10')*

61. Pistocchi AS.
*Potential action of phages as immunomodulators in cystic fibrosis (*FFC#23/2019. *New) (8')*
62. Ungaro F., Merkel OM.
Enabling pulmonary delivery of siRNA in cystic fibrosis lung inflammation: therapeutic potential of hybrid lipid/polymer nanoparticles (FFC#25/2018. Concluded) (15')
63. Ferrera L.
Properties of airway mucus in cystic fibrosis: their modification by changes in the activity of CFTR and after application of bicarbonate (FFC#12/2016. Concluded) (15').
64. Boschi F.
Testing the anti-inflammatory effects of matrix metalloprotease inhibitors in P. aeruginosa-infected CFTR-knockout mice by in vivo imaging techniques (FFC#21/2017. Concluded Dec. 31, 2018. Presentation only with poster no. 63)

- *Discussion (10')*

13:25 – 13:30 Closing remarks

13:30 – 14:00 Poster detachment