fondazione per la ricerca
sulla fibrosi cistica - onlus
italian cystic fibrosis research foundation

14th CONVENTION OF FFC INVESTIGATORS IN CYSTIC FIBROSIS

24-26 November 2016
Hotel Poiano, Garda (Verona)

Final Program

Thursday, November 24th

9:00-10:50 Registration and poster display

10:50-11:00 *Greetings and opening remarks*

11:00-13:20

Plenary session 1

MICROBIOLOGY / INFECTION

Chairs: **Alessandra Bragonzi, Giovanni Taccetti**

1. **Bevivino A, Mengoni A, Taccetti G, Fiscarelli EV, De Alessandri A**
   Investigating the airway microbiome in cystic fibrosis patients with a severe decline in lung function: an opportunity for a personalized microbiome based therapy (FFC#14/2015, *Completed*)
2. **Bertoni G**
   Role of small RNA-based regulatory systems in cystic fibrosis airways infection by *Pseudomonas aeruginosa*: a new frontier in the identification of molecular targets for novel antibacterials (FFC#13/2015, *Completed*)
3. **Gemma S, Docquier JD**
   Development of metallo-enzyme inhibitors to overcome *Pseudomonas aeruginosa* antibiotic-resistance in cystic fibrosis patients (FFC#16/2015, *Completed)*
4. **Ghisotti DE**
   Phage Therapy against *Pseudomonas aeruginosa* Infections in cystic fibrosis patients (FFC#17/2015, *Completed*)
5. **Landini P**
   Antimetabolite drugs as inhibitors of *Pseudomonas aeruginosa* biofilm growth and virulence: potential chemotherapics and tools in target identification for new antimicrobials (FFC#18/2015, *Completed*)
6. **Mangoni ML**
   Development and preclinical testing of a novel antimicrobial peptide to treat *Pseudomonas aeruginosa*-induced lung infections (FFC#11/2014, *Completed*)
7. **Pacello F**
   Targeting extracellular protein disulphide isomerase to control *Burkholderia cenocepacia* lung infections (FFC#13/2014, *Completed*)

13:20 – 14:30 Lunch

14:30 – 16:30

Plenary session 2

CFTR RESCUE 1

Chair: **Oscar Moran, Carlo Castellani**

8. **Duga S, Seia M, Orrenius C**
   The plant cytokine kinetin and its analogues as potential therapeutic agents to correct CFTR splicing defects (FFC#5/2015, *In progress*)
9. **Pagani F**
   An RNA based approach based on ExSpeU1 for correction of CFTR splicing defects: analysis of efficacy in primary bronchial cells (FFC#5/2014, *Completed*)
10. **Millo E, Cichero E**
    Novel aminoarylthiazole derivatives as correctors of the chloride transport defect in cystic fibrosis: computer assisted drug design, synthesis and biological evaluation (FFC#7/2015, *Completed*)
11. **Lentini L, Pibiri I**
    Identification and validation of novel molecules obtained by integrated computational and experimental approaches for the read-through of PTCs in CF cells (FFC#1/2014, *Completed*)
12. **Moran O**
    The molecular structure and the folding of the whole Cystic Fibrosis Transmembrane Conductance Regulator (CFTR): corrector sites (FFC#4/2014, *Completed*)
13. **Luini A**
    A systems biology approach to the correction of Cystic Fibrosis: from building a network of proteostasis regulatory pathways to combinatorial targeting (FFC#2/2014, *Completed*)

16:30-17:00 Coffee break

17:00-19:00

Parallel poster session 1

Posters A – CFTR RESCUE

Chairs: **Alberto Luini, Giuseppe Magazzù**

14. **Chilin A**
    New generation trimethylangelicin (TMA) analogues for selective modulation of defective CFTR or inflammation (FFC#1/2016, *New*)
15. **Cozza G, Tosco A**
    Alternative strategies for F508delCFTR repair: novel targets for drug discovery approach in cystic fibrosis (FFC#2/2016, *New*)
16. **Ghigo A**
    Development of a PI3Kγ-derived peptide as a novel F508del-CFTR potentiator (FFC#4/2016, *New*)
17. **Leal T, Ceri S, Thao NK**
    Implementation of a new imaged-controlled sweat test for in vivo quantification of CFTR function: value for diagnosis and efficacy of new therapies (FFC#5/2016, *New*)
18. **Luini A**
    Understanding the mode of action of regulatory pathways controlling F508del- CFTR proteostasis and developing drugs that rescue F508del-CFTR by targeting these pathways synergistically (FFC#6/2016, *New*)
19. **Melotti P**
    Human intestinal organoids for detecting CFTR rescue molecules in human plasma (FFC#7/2016, *New*)
20. **Moran O**
    Identification of the binding sites of CFTR correctors (FFC#8/2016, *New)*

21. **Salvi M**
    Modulation of protein kinases in the regulation of chaperone machinery leading F508delCFTR fate (FFC#10/2016, *New*)

Posters B – MICROBIOLOGY / INFECTION

Chairs: **Paolo Landini, Giovanni Taccetti**

22. **Lorè NI**
    Genetically diverse mice as innovative model for cystic fibrosis (FFC#11/2015, *In progress*)
23. **Tortoli E, Colombo C, Di Serio M**
    Establishment of single-cell and animal model to investigate pathogenesis of infection by *Mycobacterium abscessus* complex members in cystic fibrosis patients (FFC#13/2016, *New*)
24. **Bertoni G**
    Role of small RNA-based regulatory systems in cystic fibrosis airways infection by *Pseudomonas aeruginosa*: a new frontier in the identification of molecular targets for novel antibacterials (FFC#14/2016, *New*)
25. **Bragonzi A, Corvol H**
    Cystic fibrosis modifier genes related to *Pseudomonas aeruginosa* lung disease (FFC#15/2016, *New*)
26. **Ghisotti DE**
    Phage therapy against *Pseudomonas aeruginosa* infections in cystic fibrosis patients (FFC#16/2016, *New*)
27. **Pini A, d'Angelo I**
    Development of inhalable particles for optimal delivery of a potent antimicrobial molecule in *Pseudomonas aeruginosa* infected lungs (FFC#17/2016, *New*)
28. **Cirillo DM**
    Impact of anti-*Staphylococcus aureus* treatment on *Pseudomonas aeruginosa*-induced lung damage (FFC#15/2015, *In progress*)
29. **Riccardi G, Ungaro F**
    Inhalable formulations of new molecules effective against *Burkholderia cenocepacia*: from *in vitro* to *in vivo* applications (FFC#19/2015, *In progress*)
30. **Visca P, Peri F, Sorrentino R**
    Exploiting the potential of gallium for the treatment of *Pseudomonas aeruginosa* pulmonary infection (FFC#21/2015, *In progress*)

18:50-19:20 Meeting in front of the posters "Facilities" with the head of CFaCore Service

20:00-21:30 Dinner

Friday, November 25th

8:30-10:30 ***Plenary session 3***

CF INFLAMMATION

Chairs: **James Chmiel, Carla Colombo, Cristina Dechecchi**

31. **Hirsh E, Laudanna C**
    Targeting PI3Kγ scaffold function to activate airway CFTR, limit lung inflammation and promote bronchorelaxation in cystic fibrosis (FFC#23/2015, *Completed*)
32. **Cabrini G, Nassini R**
    TRPA1 channels as novel molecular targets for anti-inflammatory therapies in CF lung (FFC#17/2014, *Completed*)
33. **Romani L**
    Targeting pathogenic pathways leading to inflammatory Th17 responses in cystic fibrosis: from drug discovery to preclinical validation (FFC#22/2014, *Completed*)

34. **Romano M, Totani L, Marchisio M**
    Mechanisms and clinical implications of endothelial dysfunction in cystic fibrosis (FFC#23/2014, *Completed*)
35. **Pizzo E, Pedone EM**
    Identification and characterization of LPS-neutralizing human peptides: potential tools to control inflammation in cystic fibrosis lung disease (FFC#20/2014, *Completed*)
36. **Recchiuti A**
    Resolvin D1 for Targeting Chronic Lung Inflammation and Infection in Cystic Fibrosis (FFC#21/2014, *Completed*)

10:30-11:00 Coffee break

11:00-13:00

Plenary session 4

CFTR RESCUE 2

Chairs: **Christine Bear, Paolo Bernardi**

37. **Galietta LJV, Bandiera T**
    Task Force for Cystic Fibrosis (FFC/TFCF, *In progress*)
38. **Venerando A, Villella VR**
    A kinase-directed approach to rescue functionality of F508del CFTR (FFC#7/2014, *Completed*)
39. **Piacentini M, Maiuri L**
    Dissecting the role of TG2 in cystic fibrosis pathogenesis: identification of possible novel therapeutic targets (FFC#8/2015, *In progress*)
40. **Tamanini A, Aureli M**
    Identification of molecular targets to reduce the side effect of gating potentiators on the F508delCFTR plasma membrane stability (FFC#9/2015, *In progress*)
41. **Messina G**
    Evaluation of the biological and therapeutic properties of Mesoangioblasts -vessel associated progenitor cells- in the cell based therapy of the Cystic Fibrosis disease (FFC#6/2015, *Completed*)
42. **de Jonge H, Caldrer S**
    Assessment and pharmacological correction of abnormalities in bicarbonate (HCO3-) and mucus transport in intestinal biopsies and organoids of CF patients (FFC#3/2015, *Completed*)

13:00-14:30 Lunch

14:30-16:30

Plenary session 5

THINKING ABOUT THE FUTURE OF CF RESEARCH

Chairs: **Giorgio Berton, Luis Galietta, Giulio Cabrini**

14:30-15:15

James Chmiel *CF Inflammation: achievements and perspectives. Why so much research and so negligible clinical application?*

15:15-15:35 Discussion

15:35-16:20

Christine Bear *CFTR function recovery: achievements and perspectives.* Openings and possible pitfalls of the new strategies

16:20-16:40 Discussion

16:40-17:10 Coffee break

17:10-19:00

Parallel Poster session 2

Posters C – TARGETING CFTR

Chair: **Claudio Sorio, Valeria Raia**

8bis. Duga S, Seia M, Orrenius C
The plant cytokine kinetin and its analogues as potential therapeutic agents to correct CFTR splicing defects (FFC#5/2015, *In progress*)

37bis. Galietta LJV, Bandiera T
Task Force for Cystic Fibrosis (FFC/TFCF, *In progress*)

39.bis Piacentini M, Maiuri L
Dissecting the role of TG2 in cystic fibrosis pathogenesis: identification of possible novel therapeutic targets (FFC#8/2015, *In progress*)

40bis. Tamanini A, Aureli M
Identification of molecular targets to reduce the side effect of gating potentiators on the F508delCFTR plasma membrane stability (FFC#9/2015, *In progress*)

43. **Atlante A**
Relationship between mitochondria and F508del-CFTR in Cystic Fibrosis *(*FFC#1/2015, *In progress)*

44. **Cavalli A, Pedemonte**
NRNF5/RMA1 ubiquitin ligase as a drug target for mutant CFTR rescue (FFC#2/2015, *In progress*)

45. **Romani L**
Anakinra in cystic fibrosis: from targeting pathogenic inflammation to correcting CFTR defect (FFC#9/2016, *New*)

46. **Signorelli P**
Myriocin potential as a phenotype-modifying therapeutical in cystic fibrosis (FFC#11/2016, *New*)

47. **Zegarra-Moran O**
Properties of airway mucus in cystic fibrosis: their modification by changes in the activity of CFTR and after application of bicarbonate (FFC#12/2016, *In progress*)

Posters D – INFLAMMATION

Chairs: **Mario Romano, Cesare Braggion**

48. **Gambari R, Corradini R**
MicroRNA therapeutics in CF: targeting CFTR and inflammation networks (MICRORNA-CF) (FFC#3/2016, *New*)

49. **Cigana C, Naggi A**
Interfering with glycosaminoglycans during *Pseudomonas aeruginosa* chronic lung infection: pre-clinical exploitation of a novel therapeutic strategy for cystic fibrosis (FFC#18/2016, *New*)

50. **Evangelista V**
Phosphodiesterases type-4 (PDE4) inhibitors and β 2-adrenergic agonists to reduce neutrophilic lung inflammation in cystic fibrosis. Preclinical studies and identification of biomarkers of efficacy (FFC#16/2013, *In progress*)

51. **Recchiuti A**
Resolvin D1 for targeting chronic lung inflammation, infection, and damage in cystic fibrosis (FFC#19/2016, *New*)

52. **Dechecchi MC, Aureli M**
A systematic investigation of miglustat-derivative iminosugar clusters as possible anti-inflammatory agents for cystic fibrosis lung disease (FFC#22/2015, *In progress*)

53. **Rimessi A**
Mitochondrial quality control machinery a role in the *Pseudomonas aeruginosa*-triggered inflammatory response in cystic fibrosis (FFC#20/2015, *In progress*)
54. **Strazzabosco M**
CFTR-defective biliary cells from human induced pluripotent-stem cells (iPSC) as a model to study the role of innate immunity in cystic fibrosis liver disease (FFC#24/2015, *In progress*)

Posters E – CLINICAL ISSUES

Chair: **Vincenzina Lucidi**

55. **Castellani C**
Outcomes of spontaneous application of carrier screening for cystic fibrosis: follow-up of its effects on birth prevalence, neonatal screening and reproductive behaviour of carrier couples (FFC#26/2015, *In progress*)
56. **Padoan R**
Cystic fibrosis and meconium ileus: a multicentric study on risk factors for adverse outcome in infancy (FFC#28/2015, *In progress*)
57. **Sorio C, Averna M**
Testing CFTR repair in cystic fibrosis patients carrying nonsense and channel gating mutations (FFC#29/2015, *In progress*)
58. **Battezzati A, Colombo C, Lucidi V, Magazzù G, Mari A**
Italian multicenter study of glucose tolerance defects in cystic fibrosis (FFC#20/2016, *New*)
59. **Bisogni S**
The use of Virtual Reality in the reduction of pain and anxiety during venipuncture in children with cystic fibrosis: a randomized controlled trial (FFC#21/2016, *New*)
60. **Signoretto C**
Environmental and human reservoirs of *Pseudomonas aeruginosa* and other bacterial species colonizing the lower airways of cystic fibrosis patients (FFC#22/2016, *New*)

70bis. Taccetti G
Pseudomonas aeruginosa eradication in patients with cystic fibrosis: a randomised multicentre study comparing classic treatment protocols with classic treatment together with antibiotic treatment of upper airways (FFC#30/2015, *In progress*)

18:50-19:20 Meeting in front of the posters "Facilities" with the head of "Primary Cultures Service"

20:15-23:00 Evening Party

Saturday, November 26th

8:30-10:30

Plenary session 6

INFECTION / INFLAMMATION

Chairs: **Gian Maria Rossolini, Natalia Cirilli**

61. **Bragonzi A, Iraqi F**
Cystic fibrosis modifier genes related to *Pseudomonas aeruginosa* lung disease (FFC#9/2014, *Completed*)
62. **Maria M. Lleò**
A CF, IL-8 transgenic mouse model for the in vivo, long-term monitoring of the anti-inflammatory role of metallo-protease inhibitors and antibiotics with mechanisms of action similar to that of azithromycin (FFC#10/2015, *Completed*)

63. **Berlutti F**
    Anti-inflammatory and anti-bacterial activity of bovine lactoferrin administered by aerosol in airway infections of pre-clinical wt and CF mouse models (FFC#12/2015, *Completed*)
64. **Pinton P**
    Mitochondrial Ca2+-dependent inflammasome activation exacerbates the P. aeruginosa-driven inflammatory response (FFC#19/2014, *Completed*)
65. **Sonnino S**
    The role of Glucocerebrosidase GBA2 in cystic fibrosis lung inflammation: from molecular mechanism to therapeutic strategies (FFC#24/2014, *Completed*)
66. **Pilette C, De Rose V**
    Impaired secretory IgA and mucosal immunity in cystic fibrosis: contribution to lung pathology and impaired defence against bacterial infection, and role of CFTR-related epithelial changes in the regulation of the receptor-mediated IgA transcytosis (FFC#26/2014, *Completed*)

10:30-11:00 Coffee break

11:00-13:00

Plenary session 7

CLINICAL AND AROUND ISSUES

Chairs: **Roberto Buzzetti, Marco Cipolli**

67. **Corti A**
    GSH inhalation therapies in CF: how useful, how safe? Set-up of a CF murine model for monitoring of inflammation in vivo and assessment of convenient alternatives (FFC#18/2014, *Completed*)
68. **Zaza G, Chilosi M**
    In vitro study of potential pro-fibrotic effect of Everolimus in different human airway cell lines. Searching for new biomarkers to optimize MTOR-inhibitor immunosuppressive treatment of cystic fibrosis patients undergoing lung transplantation (FFC#28/2014, *Completed*)
69. **Braggion C**
    CF Clinical guidelines (FFC#25/2015, *Completed*)
70. **Taccetti G**
    Pseudomonas aeruginosa eradication in patients with cystic fibrosis: a randomised multicentre study comparing classic treatment protocols with classic treatment together with antibiotic treatment of upper airways (FFC#30/2015, *In progress*)
71. **Cirilli N, Raia V**
    Intra-individual biological variation in sweat chloride concentrations (FFC#27/2015, *Completed*)
72. **Tortoli E, Cariani L, Di Serio C, Niemann S**
    Transmissibility and clinical significance of *Mycobacterium abscessus* in patients with cystic fibrosis (FFC#27/2014, *Completed*)

13:00-13:10 Closing remarks: **Carlo Castellani** *A clinician's point of view on the FFC-network's research*

13:10-13:30 Poster detachment

NOTES.
- The official language will be Italian, except plenary sessions 3, 4, 5 of November 25 and projects presented by foreign speakers, which will be in English
- The project presentations in the plenary sessions will have a maximum duration of 14 minutes, followed by five-six minutes of discussion
- The presentations in the poster sessions will have a maximum duration of 9 minutes, followed by six minutes of discussion
- The slides for the project presentations in the plenary sessions should not exceed the number to 15. They will be written in English, will have a simple setting and be legible from a distance (please, prove in advance the distance effect)
- The posters (printed on one sheet, with simple setup and clearly legible up to 2 meters) will have width of 90 cm and a height of 140 cm
- All new or ongoing projects will be presented as posters even if some of them will have an oral presentation in the plenary sessions
- In this program the projects are marked with the names of the principal investigators and partners. The corresponding abstracts in the book of abstracts may also have the names of research collaborators
- The presence of a clinical chairman in almost all sessions intends to contribute in the discussion to identify the binding of basic study with possible clinical perspectives.