Fondazione Ricerca Fibrosi Cistica - Onlus
DA VENT'ANNI, PIÙ RICERCA, PIÙ VITA.

Con il patrocinio e l'ospitalità di
Camera di Commercio Verona

15th CONVENTION OF FFC INVESTIGATORS IN CYSTIC FIBROSIS

26-28 October 2017

Camera di Commercio Verona, Congress Center

Corso Porta Nuova, 96

Final Program

Thursday, October 26th

10;00 – 11;30 *Registration and poster display*

11;30 – 13;30

Plenary session 1

ENTERING THE CLINICAL WORLD

Chairmen: **Roberto Buzzetti**, **Claudio Sorio**
Buzzetti R *An introduction: why is production of independent clinical research in Italy so difficult?* (20 min. + discussion 20 min.)

1. **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, completed)
2. **Sorio C, Averna M**
   Testing CFTR repair in cystic fibrosis patients carrying nonsense and channel gating mutations (FFC#29/2015, completed)
3. **Padoan R**
   Cystic fibrosis and meconium ileus: a multicentric study on risk factors for adverse outcome in infancy (FFC#28/2015, completed)
4. **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*)

13;30 – 14;30 *Lunch bag and Poster view*

14;30 – 16;30

Plenary Session 2

NEW PROPOSALS FOR RECOVERING CFTR FUNCTION

Chairmen: **Alberto Luini**, **Valeria Raia**

5/5bis. **Cozza G, Tosco A, Ferrari E**
Alternative strategies for F508delCFTR repair: novel targets for drug discovery approach in Cystic Fibrosis (FFC#2/2016 *Pilot completed*, FFC#10/2017 *Extension*)

6/6bis. **Ghigo A**
Development of a PI3Kγ-derived peptide as a novel F508del-CFTR potentiator (FFC#4/2016 *Pilot completed,* FFC#11/2017 *Extension)*

7/7bis. **Salvi M**
Modulation of protein kinases in the regulation of chaperone machinery leading F508delCFTR fate (FFC#10/2016 *Pilot completed,* FFC#12/2017 *Extension*)

8. **Cavalli A, Pedemonte N**
RNF5/RMA1 ubiquitin ligase as a drug target for mutant CFTR rescue (FFC#2/2015, Completed*)*

9/9bis. **Galietta LJV, Bandiera T**
FFC Strategic Project: Task Force for Cystic Fibrosis (TFCF, completed) and TFCF preclinical study (New), (35 min. including discussion)

- Raia V *Final clinical commentary (5 min.)*

16;30 - 17;00 *Coffee break and Poster View*

17;00 – 19;00

Plenary Session 3

NEW INSIGHTS ON CF MICROBIOLOGY/INFECTION

Chairmen**: Annamaria Bevivino**, **Giovanni Taccetti**

10/10bis. **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 *Pilot completed,* FFC#20/2017 *Extension; presented by* **Cirillo DM***)*

11. **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, Completed)

12. **Ghisotti DE**
Phage therapy against Pseudomonas aeruginosa Infections in Cystic Fibrosis Patients (FFC#16/2016, Completed)

13. **Riccardi G, Ungaro F**
Inhalable formulations of new molecules effective against Burkholderia cenocepacia: from in vitro to in vivo applications (FFC#19/2015, *Completed; presented by* **Buroni S,**)

14/14bis. **Visca P, Peri F, Sorrentino R**
Exploiting the potential of gallium for the treatment of Pseudomonas aeruginosa pulmonary infection (FFC#21/2015, *Completed*; FFC#18/2017, *Extension*)

15/15bis. **Lorè NI**
Genetically diverse mice as innovative model for cystic fibrosis (FFC#11/2015, *Completed)*
*Phenotyping new genetically-diverse mouse models mirroring the complexity of the Cystic Fibrosis pathology (*FFC#4/2017, *Extension)*

- **Taccetti G** *Final clinical commentary (5 min.)*

19;00 – 20;00

Informal meeting on Clinical Research

Chairmen: **Carlo Castellani**, **Roberto Buzzetti**

Looking for ideas to improve independent clinical research
Free discussion based on topics suggested by the chairmen, also with reference to the introduction of the Plenary Session 1
Chairmen will propose a concluding summary.

> At the same time, small group meetings can be held in the poster showroom.

Friday, October 27th

8;30 – 10;30

Plenary Session 4

SUPPORTING AND STABILIZING CFTR FUNCTION

Chairmen: **Nicoletta Pedemonte**, **Rita Padoan**

16. **Gambari R, Corradini R**
    MicroRNA Therapeutics in CF: Targeting CFTR and inflammation networks (MICRORNA-CF) (FFC#3/2016, *Pilot completed*)
17. **Atlante A**
    Relationship between mitochondria and F508del-CFTR in Cystic Fibrosis (FFC#1/2015, Completed)
18. **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, Completed)
19. **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,, Completed)
20. **Piacentini M, Maiuri L**
    Dissecting the role of TG2 in cystic fibrosis pathogenesis: identification of possible novel therapeutic targets (FFC#8/2015, Completed; *presented by* **Rossin F**)

- **Padoan R** *Final clinical commentary (5 min.)*

10;30 – 11;00 *Coffee break and Poster view*

11;00 – 13;10

Poster Session 1

CF INFLAMMATION

Chairmen: **Cristina Dechecchi**, **Laura Minicucci**

21. **Romani L**
    Anakinra in cystic fibrosis: from targeting pathogenic inflammation to correcting CFTR defect (FFC#9/2016, *In progress*)
22. **Recchiuti A**
    Resolvin D1 for Targeting Chronic Lung Inflammation, Infection, and Damage in Cystic Fibrosis (FFC#19/2016, *In progress*)

23. **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, *New*)
24. **Signorelli P**
Myriocin potential as a phenotype-modifying therapeutical in Cystic Fibrosis (FFC#11/2016, *In progress*)
25. **Ungaro F, Merkel OM**
Enabling pulmonary delivery of siRNA in cystic fibrosis lung inflammation: therapeutic potential of hybrid lipid/polymer nanoparticles (FFC#23/2017, *New*)

- **Minicucci L** *Final clinical commentary* (5 min.)

NEAR CLINICAL APPROACHES

Chairmen: **Natalia Cirilli**, **Donatello Salvatore**

26. **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*)
27. **Pistocchi AS**
In vivo validation of phage therapy against Pseudomonas aeruginosa infections using the new zebrafish (Danio rerio) animal model (FFC#22/2017, *New*)
28. **Battezzati A, Colombo C, Lucidi V, Magazzù G, Mari A**
Italian multicenter study of glucose tolerance defects in cystic fibrosis (FFC#20/2016, *In progress*)
29. **Signoretto C**
Environmental and human reservoirs of Pseudomonas aeruginosa and other bacterial species colonizing the lower airways of cystic fibrosis patients (FFC#22/2016, *In progress*)
30. **Nosotti M**
Extracorporeal photopheresis as induction therapy to prevent acute rejection after lung transplantation in cystic fibrosis patients (FFC#24/2017, *New*)
31. **Leal T, Ceri S, Nguyen-Khoa T**
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, *In progress; presented by* **Melotti P**)
32. **Melotti P**
Human intestinal organoids for detecting CFTR rescue molecules in human plasma (FFC#7/2016, *In progress*)

- **Salvatore D** *Final clinical commentary (5 min)*

13;10 – 14;10 *Lunch bag and Poster view*

14;10 – 16;10

Plenary Session 5

STATE OF THE ART AND NEW PERSPECTIVES ON CF DISEASE MODELS

Chairmen: **Mario Romano**, **Vincenzina Lucidi**

Alessandra Bragonzi. *Animal models of Cystic Fibrosis: setting the stage and directing the workforce for the future (Lecture, 30 min.)*

Discussion (10 min.)

Luis Galietta. *An overview of in vitro and ex vivo CF disease models (Introduction, 30 min.)*

Contributions (7 min. each, 6 slides) will follow on proposals and personal experiences of in vitro & ex vivo models for research and therapy support.

- **Netti P** *Artificial full thickness airway tissue*
- **Sorio C** *Monocytes*
- **Castaldo G** *Nasal epithelial cells*
- **Romano M** *Human periodontal ligament stem cells*
- **Melotti P** *Intestinal organoids*

Discussion (15 min.)

16;10 – 16;30 *Coffee break and Poster view*

16;30 – 18;20

Poster Session 2

CF MICROBIOLOGY/INFECTION

Chairmen: **Paolo Visca**, **Cesare Braggion**

33. **Pini A, d'Angelo I**
Development of inhalable particles for optimal delivery of a potent antimicrobial molecule in P. aeruginosa infected lungs (FFC#16/2016, *In progress*)
34. **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, *New*)
35. **Fraziano M**
Preclinical study of a host-directed therapy based on Metformin and bioactive liposomes for the control of multidrug resistant P. aeruginosa infection (FFC#14/2017, *New*)
36. **Mangoni ML, Ferrera L**
Frog skin-derived peptides for treatment of Pseudomonas aeruginosa lung infection and bronchial epithelial repair: advanced in vitro and in vivo characterization and development of polymeric nanoparticles for lung delivery (FFC#15/2017, *New*)
37. **Pizzo E**
Pre-clinical effectiveness of three human cryptic anti-biofilm peptides (GVF27, HVA36 and IMY47): efficacy against lung pathogens and studies in animals (FFC#16/2017, *New*)
38. **Sabatini S**
Identification of new efflux pumps inhibitors able to contrast nontuberculous mycobacterial infections in cystic fibrosis patients (FFC#17/2017, *New*)
39. **Bragonzi A, Corvol H**
Cystic fibrosis modifier genes related to Pseudomonas aeruginosa lung disease (FFC#15/2016, *In progress*)
40. **Bevivino A, Mengoni A, Segata N**
A longitudinal metagenomic analysis to uncover microbial signatures of lung disease: unraveling host-microbial community interactions in humans and animal models (FFC#19/2017, *New*)

- **Braggion C** *Final clinical commentary* (5 min)

19;30 – 23;00 **Twenty-Year FFC Celebration Dinner**
(*Palazzo della Gran Guardia, Piazza Brà)*

Saturday, October 28th

9;00 – 11;00

Plenary Session 6

IS THERE REALLY A WAY TO CONTAIN OR PREVENT CF INFLAMMATION?

Chairmen: **Giulio Cabrini**, **Giuseppe Magazzù**

41. **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, Completed)
42. **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, Completed)
43. **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, Completed)
44. **Rimessi A**
    Mitochondrial quality control machinery a role in the P. aeruginosa-triggered inflammatory response in Cystic Fibrosis (FFC#20/2015, Completed)
45. **Cirillo DM**
    Impact of anti-Staphylococcus aureus treatment on Pseudomonas aeruginosa-induced lung damage (FFC#15/2015, Completed; *presented by* **Riva C**)
46. **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; Completed)

- **Magazzù G** *Final clinical commentary* (5 min)

11;00 – 11;20 *Coffee break*

11;20 – 13;30

Poster Session 3

TOWARDS CF BASIC DEFECT THERAPY

Chairmen: **Oscar Moran, Marco Cipolli**

47. **Cereseto A, Debyser Z, Arosio D**
    SpliceFix: fixing splicing defects in the CFTR gene through CRISPR/Cas9 technology (FFC#1/2017, *New*)
48. **Moran O**
    Identification of the binding sites of CFTR correctors (FFC#8/2016, *In progress*)
49. **Chilin A**
    New generation trimethylangelicin (TMA) analogues for selective modulation of defective CFTR or inflammation (FFC#1/2016, *In progress*)
50. **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, *New*)
51. **Millo E, Cichero E**
    Pharmacophore and pharmacokinetic filtering tools guiding for the design and synthesis of novel thiazole-containing and VX-809 hybrid derivatives as F508del correctors (FFC#6/2017, *New*)
52. **Pedemonte N, Cavalli A**
    RNF5 inhibitors as potential drugs for Cystic Fibrosis basic defect (FFC#9/2017, *New*)
53. **Ferrera L**
    Properties of airway mucus in cystic fibrosis: their modification by changes in the activity of CFTR and after application of bicarbonate-continuation (FFC#12/2016, *In progress*)
54. **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, *In progress*)

55. **Galietta LJV**
    Identification of deubiquitinases and ubiquitin ligases that affect mutant CFTR rescue (FFC#2/2017, *New*)
56. **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, *New*)
57. **Messina G**
    Dissecting the potency of human Mesoangioblasts to differentiate into CFTR-expressing epithelial cells: a step forward to an innovative cell-based therapy for Cystic Fibrosis disease (FFC#5/2017, *New*)

- **Cipolli M** *Final clinical commentary (5 min.)*

13;30 – 13;40 Closing remarks
13;40 – 14;00 Poster detachment

NOTES.

- The official language will be Italian. However, foreign researchers will be able to present their projects or to intervene in discussion in English
- The completed projects will be presented in plenary sessions: the presentation should not exceed 14 minutes, with a maximum of 12 slides; it will be followed by 5 min. discussion.
- The completed pilot projects will be presented in plenary session together with the approved extension project: a total of 14 minutes (12 slides) followed by 5 minutes of discussion.
- The extension projects must also be exhibited with a poster (without oral presentation in the poster session).
- Current (in progress) and new projects will be presented both as posters and as brief communications. The last ones will be held in special plenary sessions. The oral presentation should not exceed 7 minutes (maximum 6 slides) and will be followed by 4 minutes of discussion.
- The slides will be written in English, will have a simple setting and be legible from a distance (please, prove in advance the distance effect). It is recommended that the Powerpoint slides are in 16/9 format.
- The posters (printed on one sheet, with simple setup and clearly legible up to 2 meters) will have width of 90 cm and height of 140 cm and will be displayed in the poster hall before the beginning of the works.
- Speakers and moderators are invited to comply with the times indicated: this makes it possible to avoid calls, forced interruptions and above all not to compromise the smooth running of the work.
- 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 other research collaborators.
- The presence of a clinical chairman in all the sessions intends to contribute in the discussion to identify the binding of basic study with possible clinical perspectives.
- It is strongly recommended to all participants to attend all sessions, including the two ones on Saturday, October 28. It should be recalled that the Convention was thought to be an opportunity for the FFC Network to take a look at everything that moves in this area and to interweave exchanges and collaborative contacts.