16th CONVENTION OF FFC INVESTIGATORS IN CYSTIC FIBROSIS
22-24 November, 2018
Camera di Commercio Verona, Congress Center
Corso Porta Nuova, 96

Final program

Thursday, November 22nd

9:00 – 11:00 Registration and poster display
11:00 – 11:20 Introduction and greetings
11:20 – 13:20

Plenary Session 1

OVERCOME THE RESISTANCE OF PSEUDOMONAS AERUGINOSA

Chairs: Bevivino A, Ross M

1. Leoni L (Univ. Roma Tre)
   Drug repurposing for antivirulence therapy against Pseudomonas aeruginosa (FFC#17/2018, New), 8’

2. Biavasco F (Univ. Politecnica Marche)
   Induction of viable but non-culturable forms, possibly responsible for treatment failure, in in vitro biofilms of Pseudomonas aeruginosa. Role of antibiotics & antibiotic concentrations (FFC#13/2017, In progress), 8’

3. Sanguinetti M (Univ. Cattolica, Roma), Vitali A (ICRM, CNR, Milano), Iafisco M (ISTEC, CNR, Faenza), Catalucci D (IRGB, CNR, Milano)
   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, New), 8’
   Discussion (10’)

4. Fraziano M (Univ. Tor Vergata, Roma)
   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, Concluded), 15’

5. Visca P (Univ. Roma Tre), Peri F (Univ. Milano Bicocca), Sorrentino R (Univ. “Federico II”, Napoli)
   Exploiting the potential of gallium for the treatment of Pseudomonas aeruginosa pulmonary infection (FFC#18/2017, Concluded), 15’

6. Pistocchi AS (Univ. Milano)
In vivo validation of phage therapy against Pseudomonas aeruginosa infections using the new zebrafish (Danio rerio) animal model (FFC#22/2017, Concluded), 15'

Discussion (10’)

UNEXPLORRED AREAS IN CF LUNG INFECTION

7. **Bragonzi A** (San Raffaele Inst., Milano), **Corvol H** (Inserm, Parigi)
   Cystic fibrosis modifier genes related to Pseudomonas aeruginosa lung disease (FFC#15/2016, Concl.), 15’

8. **Antonelli G** (Univ. La Sapienza, Roma)
   Ex vivo study on Type I and III interferon response and virus–bacteria interactions in CF patients: a new approach to try to develop alternative therapeutic strategy (FFC#14/2018, New), 8’

Discussion (8’)

13:20 – 14:20 Lunch bag and Poster view
14:20 – 16:20

**Plenary Session 2**

TOWARDS NEW MODULATORS OF F508del-CFTR

Chairmen: Galietta LJV, Braggion C

9. **Bandiera T** (IIT, Genova), **Galiotta LJV** (TIGEM, Napoli), **Pedemonte N** (Ist. Gaslini, Genova)
   The lead corrector ARN23765 towards its preclinical development (FFC#TFCF & FFC#TFCF extension), 20’

Discussion (5’)

10. **Ghigo A** (Univ. Torino)
    Development of a P13k-derived peptide as a novel F508del-CFTR potentiator (FFC#11/2017, Concluded), 15’

11. **Hirsch E** (Univ. Torino)
    In depth characterization of the molecular mechanisms underlying P13k-mediated regulation of CFTR (FFC#8/2018, New), 8’

12. **Millo E, Cichero E** (Univ. Genova)
    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, Concluded), 15’

Discussion (13’)

13. **Signorelli P** (Univ. Milano)
    Myriocin potential as a phenotype-modifier in Cystic Fibrosis (FFC#11/2016, Concluded), 15’

14. **Barraja P** (Univ. Palermo), **Scudieri P** (TIGEM, Napoli)
    Towards the discovery of new correctors based on nitrogen heterocyclic systems (FFC#4/2018, New), 8’

15. **Rusnati M** (Univ. Brescia), **Fossa P** (Univ. Genova), **Orro A** (ITB, CNR, Milano)
    Rescuing defective CFTR applying a drug repositioning strategy based on computational studies, surface plasmon resonance and cell-based assays (FFC#11/2018, New), 8’

Discussion (13’)

16:20 - 16:50 Coffee break and Poster View
16:50 – 19:10

**Plenary Session 3**

Chairmen: Romani L, Lucidi V, Volpi S

CLINICAL PERSPECTIVES

16. **Castellani C** (Centro FC, Ist. G. Gaslini, Genova)
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, Concl.), 15'
Discussion (5’)

17. **Battezzati A** (DeFENS, Univ. Milano), **Colombo C** (Centro FC, Policl. Mangiagalli, Milano), **Lucidi V** (Osp. Bambino Gesù, Roma), **Magazzù G** (Univ. Messina), **Mari A** (IN, CNR, Padova)
   Italian multicenter study of glucose tolerance defects in cystic fibrosis (FFC#20/2016, Concluded), 15'

18. **Signoretto C** (Univ. Verona)
   Environmental and human reservoirs of Pseudomonas aeruginosa and other bacterial species colonizing the lower airways of cystic fibrosis patients (FFC#22/2016, Concluded), 15'

19. **Taccetti G** (Centro FC, Osp. “A Meyer”, Firenze)
   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, Concluded), 15'

Discussion (8’)

20. **Terlizzi V** (Centro FC, Osp. “A. Meyer”, Firenze), **Padoan R** (Centro Supp. FC, Spedali Civili, Brescia), **Antonella T** (Centro FC, Univ. “Federico II”, Napoli), **Claut LE** (IRCCS Ca’ Granda, Milano)
   Cystic Fibrosis screen positive inconclusive diagnosis (CFSPID): an Italian multicenter survey evaluating prevalence, clinical data, management and outcome (FFC#30/2018, New), 8'

Discussion (5’)

**INTEGRATED RESEARCH METHODOLOGY: FACE-TO-FACE LABORATORY & CLINICS**

21. **Romano M**, **Lanuti P** (Univ. Chieti-Pescara)
   Identification and validation of circulating microvesicle analysis as a new ex vivo assay to monitor cystic fibrosis disease (FFC#29/2018, New), 12’
   **Braggion C** (Centro FC, Osp. “A. Meyer”, Firenze)
   Critical comments and proposals. What part of the research project shall clinicians be involved in: the original idea, the design or the study implementation? (12’)

Discussion (25’)

---

**Friday, November 23rd**

8:30 – 10:40

**Plenary Session 4**

Chairmen: Pedemonte N, Cipolli M

**NEW TARGETS AND RESCUE MECHANISMS OF F508del-CFTR**

22. **Luini A** (IBP, CNR, Napoli)
   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, Concluded), 15'

23. **Moran O** (IBF, CNR, Genova)
   Identification of the binding sites of CFTR correctors (FFC#8/2016, Concluded), 15'

24. **Pedemonte N** (Ist. G. Gaslini, Genova), **Cavalli A** (Univ. Bologna)
   RNF5 inhibitors as potential drugs for Cystic Fibrosis basic defect (FFC#9/2017, In progress), 8'

Discussion (10’)

25. **Cozza G** (Univ. Padova), **Tosco A** (Centro FC, Univ. “Federico II”, Napoli), **Ferrari E** (IERFC, Milano)
Alternative strategies for F508del-CFTR repair: novel targets for drug discovery approach in Cystic Fibrosis (FFC#10/2017, Concluded), 15’
26. **Salvi M** (Univ. Padova)
   Modulation of protein kinases in the regulation of chaperone machinery leading F508del-CFTR fate (FFC#12/2017, Concluded), 15’
27. **Galietta LJV** (TIGEM, Napoli)
   Identification of deubiquitinases and ubiquitin ligases that affect mutant CFTR rescue (FFC#2/2017, In progress), 8’
28. **Ferrera L** (Ist. G. Gaslini, Genova)
   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), 8’

Discussion (12’)

29. **Armirotti A** (IIT, Genova)
   Proteomic profiling of F508del-CFTR cells to identify new pharmacological targets for CF (FFC#1/2018, New), 8’
30. **Baroni D** (IBF, CNR, Genova)
   Dissecting the rescue mechanisms mediated by CFTR correctors (FFC#3/2018, New), 8’
31. **Gambari R** (Univ. Ferrara), **Corradini R** (Univ. Parma)
   Revealing the microRNAs-transcription factors network in cystic fibrosis: from microRNA therapeutics to precision medicine (CF-miRNA-THER) (FFC#7/2018, New), 8’

Discussion (10’)

10:40 – 11:10 Coffee break and Poster view
11:10 – 13:00

**Plenary Session 5**

Chairmen: Moran O, Minicucci L

---

**OTHER TARGETS TO RESCUE AND STABILIZE CFTR**

32. **Aureli M** (Univ. Milano), **Tamanini A** (AOUI, Verona)
   Development of ganglioside GM1-based therapy to improve F508delCFTR rescue approaches (FFC#2/2018, New), 8’
33. **Piacentini M** (Univ. Tor Vergata, Roma), **Maiuri L** (IERFC, Milano), **Delogu G** (Univ. Cattolica, Roma)
   Dissecting the mechanism of action of the TG2 inhibitor cysteamine on Cystic Fibrosis (FFC#10/2018, New), 8’

Discussion (8’)

---

**CFTR MODULATORS: RESEARCH AND CLINICAL APPLICATION**

- **Galietta LJV** (TIGEM, Pozzuoli, NA)
  Close to the edge: state of the art of the F508del-CFTR modulators and new cell types, 30’
- **Salvatore D** (Centro FC, Osp. San Carlo, Potenza)
  Ivacaftor in Italian CF patients with residual CFTR function, 15’
- **Carnovale V** (Centro FC adulti, Az. Osp. “Federico II”, Napoli)
  Retrospective observational study in patients with cystic fibrosis homozygous for F508del, treated for compassionate use programme with Ivacaftor/Lumacaftor (Orkambi), 15’

Discussion (25’)

13:00 - 14:00 Lunch bag and Poster view
14:00 - 16:15

**Plenary Session 6**

Chairmen: Dechecchi C, Pradal U
### Towards New Potential Anti-Inflammatory Therapies

<table>
<thead>
<tr>
<th>Page</th>
<th>Authors</th>
<th>Title</th>
<th>Details</th>
</tr>
</thead>
<tbody>
<tr>
<td>35.</td>
<td>Bianchi ME (Osp. San Raffaele, Milano)</td>
<td>Preclinical Testing in Cystic Fibrosis of a Repurposed Molecule Targeting HMGB1 (FFC#22/2018, New)</td>
<td>8’</td>
</tr>
<tr>
<td>36.</td>
<td>Pasut G (Univ. Padova), Percudani R (Univ. Parma)</td>
<td>Therapeutic Potential of a Long-Acting Lung-Specific DNase (DNase2b) for the Treatment of CF (FFC#9/2018, New)</td>
<td>8’</td>
</tr>
</tbody>
</table>

Discussion (10’)

<table>
<thead>
<tr>
<th>Page</th>
<th>Authors</th>
<th>Title</th>
<th>Details</th>
</tr>
</thead>
<tbody>
<tr>
<td>37.</td>
<td>Recchiuti A (Univ. Chieti-Pescara)</td>
<td>Resolvin D1 for Targeting Chronic Lung Inflammation, Infection, and Damage in Cystic Fibrosis (FFC#19/2016, Concluded)</td>
<td>15’</td>
</tr>
<tr>
<td>38.</td>
<td>Bellet MM (Univ. Perugia)</td>
<td>Thymosin Alpha 1 in Cystic Fibrosis: From the Lung to the Gut (FFC#21/2018, New)</td>
<td>8’</td>
</tr>
<tr>
<td>39.</td>
<td>Dechecchi MC (AOUI Verona), Guaragna A (Univ. Federico II, Napoli)</td>
<td>Evaluation of Anti-Inflammatory Treatments for CF Lung Disease in Murine Models of Lung Infection in Vivo (FFC#23/2018, New)</td>
<td>8’</td>
</tr>
<tr>
<td>40.</td>
<td>Romani L, (Univ. Perugia)</td>
<td>Pharmacology and Therapeutics of Inhaled Indoles, as Aryl Hydrocarbon Receptor Ligands, in Cystic Fibrosis (FFC#24/2018, New)</td>
<td>8’</td>
</tr>
</tbody>
</table>

Discussion (10’)

### Anti-Inflammatory Agents with CFTR Recovery Action

<table>
<thead>
<tr>
<th>Page</th>
<th>Authors</th>
<th>Title</th>
<th>Details</th>
</tr>
</thead>
<tbody>
<tr>
<td>41.</td>
<td>Chilin A (Univ. Padova)</td>
<td>New Generation Trimethylangelicin (TMA) Analogues for Selective Modulation of Defective CFTR or Inflammation (FFC#1/2016, Concluded)</td>
<td>15’</td>
</tr>
<tr>
<td>42.</td>
<td>Romani L, (Univ. Perugia)</td>
<td>Anakinra in Cystic Fibrosis: From Targeting Pathogenic Inflammation to Correcting CFTR Defect (FFC#9/2016, Concluded)</td>
<td>15’</td>
</tr>
</tbody>
</table>

Discussion (10’)

16:15 – 16:45 Coffee break and Poster view

16:45 – 18:30 Plenary Session 7

Chairmen: Leoni L, Castellani C

### Lung Transplantation

<table>
<thead>
<tr>
<th>Page</th>
<th>Authors</th>
<th>Title</th>
<th>Details</th>
</tr>
</thead>
<tbody>
<tr>
<td>44.</td>
<td>Palleschi A (IRCCS Fond. Ca’ Granda, Milano), Aliverti A (Politecnico, Milano)</td>
<td>Use of Multivolume MRI Instead of Ionizing Imaging Techniques for Surveillance in Young Patients after Lung Transplantation for Cystic Fibrosis (FFC#27/2018, New)</td>
<td>8’</td>
</tr>
<tr>
<td>45.</td>
<td>Rea F (Univ. Padova), Schena FP (Schena Foundation, Padova). Speak, Lunardi F</td>
<td>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, New)</td>
<td>9’</td>
</tr>
</tbody>
</table>

Discussion (12’)


NEW MOUSE MODELS AND NEW EXPLORATIONS IN CF MICROBIOLOGY

46. Lorè NI (San Raffaele Inst., Milano)
Phenotyping new genetically-diverse mouse models mirroring the complexity of the Cystic Fibrosis pathology (FFC#4/2017, In progress), 9'

47. Boschi F (Univ. Verona)
Testing the anti-inflammatory effects of matrix metalloprotease inhibitors in P. aeruginosa-infected CFTR-knockout mice by in vivo imaging techniques (FFC#21/2017, In progress), 8'

48. Bevivino A (ENEA Casaccia, Roma), Mengoni A (Univ. Firenze), Segata N (CIBIO, Trento)
A longitudinal metagenomic analysis to uncover microbial signatures of lung disease: unravelling host-microbial community interactions in humans and animal models (FFC#19/2017, In progress), 8'

49. Cigana C (San Raffaele Inst., Milano)
Off-target effects of CFTR-modulators in preclinical infection models (FFC#15/2018, New), 8'

Discussion (13')

20:00 – 22:30 Welcome Dinner

Saturday, November 24th

9:00 – 11:20

Plenary Session 8

Chairmen: Bragonzi A, Taccetti G

ANTIMICROBIAL PEPTIDES

Development of inhalable particles for optimal delivery of a potent antimicrobial molecule in P. aeruginosa infected lungs (FFC#17/2016, Concluded), 15'

51. Mangoni ML (Univ. La Sapienza, Roma), Ferrera L (Ist. G. Gaslini, Genova)
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, Concluded), 15'

52. Pizzo E (Univ. “Federico II”, Napoli)
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, Concluded), 15'

In vitro and in vivo efficacy of an antimicrobial and antibiofilm designed peptidomimetic against CF lung pathogens (FFC#18/2018, New), 8'

Discussion (13')

NON-TUBERCULOUS MYCOBACTERIA AND ASPERGILLUS IN CF

54. Sabatini S (Univ. Perugia)
Identification of new efflux pumps inhibitors able to contrast nontuberculous mycobacterial infections in cystic fibrosis patients (FFC#17/2017, Concluded), 15'

55. Tortoli E (San Raffaele Inst., Milano), Colombo C (Centro FC, IRCCS Policl. Mangiagalli, Milano), Di Serio MC (Univ. San Raffaele, Milano)
Establishment of animal model to investigate pathogenesis of infection by Mycobacterium abscessus complex members in cystic fibrosis patients (FFC#20/2017, Concluded), 15'

Discussion (10')

Preclinical evaluation of liposomes carrying bioactive lipids as an immune therapeutic tool against in vivo infection with Mycobacterium abscessus (FFC#16/2018, New), 8’

57. **Pasca MR** (Univ. Pavia)
    New weapons against Mycobacterium abscessus and other nontuberculous mycobacteria (FFC#19/2018, New), 8’

58. **Bartoloni A** (AOU Careggi, Firenze), **Viscoli C** (Univ. Genova), **Cariani L** (Fondaz. IRCSS Ca’ Granda, Milano), **Fiscarelli EV** (Osp. Bambino Gesù, Roma)
    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, New), 8’

Discussion (10’)

11:20 – 11:45 Coffee break and Poster view

**Plenary Session 9**

Chairmen: Hirsch E, Colombo C

<table>
<thead>
<tr>
<th>CORRECTIVE APPROACHES FOR NON-F508DEL-CFTR MUTATIONS AND GENE-CELL THERAPY</th>
</tr>
</thead>
<tbody>
<tr>
<td>59. <strong>Cereseto A</strong> (CIBIO, Trento), <strong>Debyser Z</strong> (Center for Molecular Medicine, KU Leuven), <strong>Arosio D</strong> (IBF, CNR, Trento)</td>
</tr>
<tr>
<td>SpliceFix: fixing splicing defects in the CFTR gene through CRISPR/Cas9 technology (FFC#1/2017, In progress), 8’</td>
</tr>
<tr>
<td>60. <strong>Lentini L</strong>, <strong>Pibiri I</strong> (Univ. Palermo)</td>
</tr>
<tr>
<td>Optimization of a new lead promoting the readthrough of nonsense mutations for the CFTR rescue in human CF cells (FFC#3/2017, In progress), 8’</td>
</tr>
<tr>
<td>61. <strong>Di Leonardo A</strong> (Univ. Palermo)</td>
</tr>
<tr>
<td>Investigating CRISPR-CAS13b as a tool for the RNA editing of CFTR mRNA with premature stop codons (FFC#5/2018, New), 8’</td>
</tr>
<tr>
<td>62. <strong>Messina G</strong> (Univ. Milano)</td>
</tr>
<tr>
<td>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, Concluded), 15’</td>
</tr>
<tr>
<td>Discussion (14’)</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>IN VIVO, EX VIVO AND IN VITRO PREDICTIVE TESTS AND MODELS TO EVALUATE THE CFTR FUNCTION</th>
</tr>
</thead>
<tbody>
<tr>
<td>63. <strong>Leal T</strong> (UC, Louvain), <strong>Ceri S</strong> (Univ. Milano), <strong>Thao NK</strong> (Necker-Enfants Malades Hosp., Paris)</td>
</tr>
<tr>
<td>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, Concluded), 15’</td>
</tr>
<tr>
<td>64. <strong>Netti P</strong> (IIT, Napoli), <strong>di Bernardo D</strong> (Univ. “Federico II”, Napoli)</td>
</tr>
<tr>
<td>A novel Full Thickness Cystic Fibrosis model on a microfluidic chip to study pathogenic mechanisms and evaluate therapeutic strategies (FFC#8/2017, In progress), 8’</td>
</tr>
<tr>
<td>65. <strong>Eramo A</strong> (Istituto Superiore di Sanità), <strong>Lucarelli M</strong> (Univ. La Sapienza, Roma)</td>
</tr>
<tr>
<td>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, New), 8’</td>
</tr>
</tbody>
</table>
| Discussion (10’)

66. **Melotti P** (Centro FC, AOUI Verona) |
    Human intestinal organoids for detecting CFTR rescue molecules in human plasma samples (FFC#7/2016, Concluded), 15’

67. **Sorio C** (Univ. Verona)
Testing intestinal organoids for the prediction of response to CFTR potentiators and correctors used in clinic (FFC#13/2018, New), 8’

68. Frulloni L (Univ. Verona), Lucidi V (Osp. Bambino Gesù, Roma), de Jonge H (Erasmus University Medical Center, Rotterdam)

Intestinal organoids for assessment and pharmacological correction of abnormalities in fluid transport and anion currents in patients affected by pancreatitis (FFC#6/2018, New), 8’

Discussion (10’)

14:00 - 14:05 Closing remarks
14:05 – 14:30 Poster detachments

ORGANIZATIONAL NOTES

General
- The official language will be Italian. However, foreign researchers will be able to present their projects or to intervene in discussion in English
- 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 all the sessions intends to contribute in the discussion to identify the binding of basic studies with possible clinical perspectives.
- It is strongly recommended to all participants to attend all sessions, including the two ones on Saturday, November 24th.

Presentations & slides
- The name of the presenter is underlined and, exceptionally, may not be that of the PI or Partner
- The presentation of completed projects must last no longer than 15 minutes. That of projects in progress or new no more than 8 minutes. Times will be strictly controlled by moderators. A signal will be given two minutes before the mandatory reporting time.
- The slides (to be delivered to the slide center before the start of the related session) will be written in English, will have a simple setting, with essential information and data, and be legible from a distance (please, prove in advance the distance effect). For optimal visibility, the slide format should be 16:9.

Posters
- A poster is required for all projects (included the completed ones), even if presented in plenary sessions.
- 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 and will be displayed in the poster hall before the beginning of the Convention and detached at the end of the works on Saturday, November 24th.