19th ECFS Basic Science Conference

20 – 23 March 2024, Valletta, Malta

Programme

Chairpersons: Alexandre Hinzpeter (Paris, FR), Patrick Harrison (Cork, IE) & Marie Egan (New Haven, US)

Wednesday, 20 March 2024 (Day 1)		
17:30-18:00	**Official Opening of the Conference by the Conference Chairpersons**	
18:00-19:00	**Opening Keynote Lecture:**	
	CFTR biogenesis and misfolding correction; the role of allosteric domain-domain coupling - Gergely Lukacs (CA)	
19:00-19:45	**Welcome Reception**	Level 6
19:45-21:30	*Dinner*	Level 2 - Floriani Hall

Thursday, 21 March 2024 (Day 2)		
07:30-08:45	*Breakfast*	Level 4 – Spice Island
08:45-10:30	**Symposium 1 – CFTR protein structure and function**	
	Chairs: Nicoletta Pedemonte (IT) / Gergely Lukacs (CA)	
08:45-09:10	CFTR structure, dynamics, and gating through the in silico microscope - Tamás Hegedűs (HU)	
09:10-09:35	Molecular mechanism of Cl^- conduction the open state of human CFTR - Régis Pomès (CA)	
09:35-10:00	CFTR NBD2 folding requires its assembly with TMDs rather than folded NBD1 - Ineke Braakman (NL)	
10:00-10:10	Abs. 02 - Exploring the activating effects of the inhibitors of type IV ABC transporters on CFTR gating – Paola Vergani (UK)	
10:10-10:20	Abs. 05 - Mechanism of action and binding site characterization of corrector *ARN23765* via Photo-Affinity Labeling (PAL) approach in live cells - Fabio Bertozzi (IT)	
10:20-10:30	Abs. 04 - Understanding the conformational landscape of NBD1 from CFTR: from normal function to cystic fibrosis - Arina Svoeglazova (BE)	
10:30-11:00	*Coffee break* & **Poster viewing**	
11:00-12:45	**Symposium 2 – CFTR genetics & CFTR expressing cells**	
	Chairs: Anna Cereseto (IT) / Batsheva Kerem (IL)	
11:00-11:25	Modulator responsiveness of 656 CFTR variants: from predicting patient treatability to structural insights - Martin Mense (US)	
11:25-11:50	Single-cell analyses of the human airways in health and respiratory diseases - Laure-Emmanuelle Zaragosi (FR)	
11:50-12:15	*CFTR* regulation in time and space - Ann Harris (US)	
12:15-12:25	Abs. 22 - Pre-clinical data demonstrates great promise for AAV gene therapy, one dose of AAV1 or 6-Δ27-264 CFTR successfully ameliorated clinical symptoms in G551D ferrets	

	after 5-weeks - Liudmila Cebotaru (US)	
12:25-12:35	Abs. 25 - Novel CFTR+ lung progenitor cells contribute to the dynamic developmental origins of fetal epithelial cell lineages - Amy Wong (CA)	
12:35-12:45	Abs. 20 - A modelling framework for epithelial airway fluid and ion transport with multiple cell types: implications for success or failure in gene therapies for cystic fibrosis - Omar Hamed (UK)	
12:45-14:30	*Lunch*	Level 5 – Admiral's Landing
14:30-15:30	**Flash Poster Session (even numbers)**	
	Chair: Neeraj Sharma (US)	
15:30-16:00	*Coffee break* & **Poster viewing**	
16:00-17:45	**Symposium 3 – Gene editing to correct any CFTR mutation**	
	Chairs: Stephen Hart (UK) / Ann Harris (US)	
16:00-16:25	Modulation of Double Strand Breaks repair to promote Cas9 and PEn dependent DNA insertions - Marcello Maresca (SE)	
16:25-16:50	Strategies to correct whole exons in CFTR by gene editing - Patrick Harrison (IE)	
16:50-17:15	Identification and evolution of novel CRISPR -Cas systems from the human microbiome - Anna Cereseto (IT)	
17:15-17:25	Abs. 28 - Rescuing G542X by Adenine Base Editing: A guide to restore function - Lucia Nicosia (IE)	
17:25-17:35	Abs. 33 - Site-specific gene targeting of chromosomal safe harbor and CFTR locus for correcting any CFTR mutation - Jim Hu (CA)	
17:35-17:45	Abs. 32 - CRISPR-ABE: a new strategy for the temporospatial control of editing to correct the W1282X mutation in the CFTR gene - Immacolata Zollo (PT)	
20:00-21:30	*Dinner*	Level 2 - Floriani Hall
21:30-23:00	**Evening Poster Session:** Posters with even numbers	
Friday, 22 March 2024 (Day 3)		
07:30-08:45	*Breakfast*	Level 4 – Spice Island
08:45-10:30	**Symposium 4 – Targeting stop codons and splicing defects**	
	Chairs: Jane Davies (UK) / Margarida Amaral (PT)	
08:45-09:10	Stabilization of RNA is critical to achieving functional level restoration of nonsense mutations by CFTR modulators - Neeraj Sharma (US)	
09:10-09:35	Reading through nonsense mutations with engineered tRNAs - Zoya Ignatova (DE)	
09:35-10:00	Targeting splicing mutations using oligonucleotides - Batsheva Kerem (IL)	
10:00-10:10	Abs. 43 - Identification of novel pharmacological inhibitors of nonsense-mediated RNA decay to rescue CFTR with premature termination codons - Arianna Venturini (IT)	
10:10-10:20	Abs. 42 - Upregulation of a nonsense mediated decay (NMD) insensitive CFTR mRNA isoform has therapeutic potential for the treatment of 3' CFTR PTC variants - Normand Allaire (US)	
10:20-10:30	Abs. 38 - Comparative study of readthrough molecules - Fabrice Lejeune (FR)	

10:30-11:00	*Coffee break* & **Poster viewing**	
11:00-12:45	**Symposium 5 – Gene(tic) therapies: are we ready for clinical research?** a patient organisation-initiated symposium	
	Chairs: Sylvia Hafkemeyer (DE) / Jeffrey Beekman (NL)	
11:00-11:25	Statements of people with CF regarding genetic therapies	
	A video on statements will be presented	
11:25-11:50	Enabling true 'informed' consent by empowering participants - Lorna Allen (UK)	
11:50-12:15	Delivery: What is feasible according to research and how does it fit to the expectations of people with CF – Stephen Hart (UK)	
12:15-12:40	Considerations to enhance patient access to genetic therapies - Stefano Zancan (IT)	
12:40-12:45	Final discussion and concluding remarks	
12:45-14:00	*Lunch*	Level 5 – Admiral's Landing
14:00-18:30	**Free Afternoon**	
18:30-19:30	**Flash Poster Session (odd numbers)** **Chair: Ineke Braakman (NL)**	
20:00 -21:30	*Dinner*	Level 2 – Floriani Hall
21:30-23:00	**Evening Poster Session:** Posters with odd numbers	
Saturday, 23 March 2024 (Day 4)		
07:30-08:45	*Breakfast*	Level 4 – Spice Island
08:45-10:30	**Symposium 6 – Targeting protein partners and alternative channels**	
	Chairs: Alexandre Hinzpeter (FR) / David Sheppard (UK)	
08:45-09:10	Mutant CFTR impacts Insulin-receptor signaling and regulates localization of tight junction proteins, disrupting the airway glucose barrier - Nael McCarty (US)	
09:10-09:35	Targeting ubiquitination to enhance modulator treatments - Nicoletta Pedemonte (IT)	
09:35-10:00	Targeting CFTR protein partners and alternative channels - Margarida Amaral (PT)	
10:00-10:10	Abs. 46 - Targeting ATP12A proton pump provides new therapeutic opportunities for cystic fibrosis - Giulia Gorrieri (IT)	
10:10-10:20	Abs. 58 - Enhancing apical loop currents in airway epithelia carrying CFTR non-sense mutations - Nathalie Baumlin (US)	
10:20-10:30	Abs. 47 - SLC26A9 modulators identified through High Throughput Screening (HTS) - Mohsen Esmaeili (CA)	
10:30-11:00	*Coffee break* & **Poster viewing**	

11:00-12:45	**Symposium 7 – Cell – bacteria relations: infection and inflammation in the era of modulators**	
	Chairs: Geneviève Héry-Arnaud (FR) / Dean Madden (US)	
11:00-11:25	Multi-lobe bronchoscopy reveals ETI's effects on regional lung infection and inflammation - Samantha Durfey (US)	
11:25-11:50	Effects of CFTR modulators on airway mucus, infection and inflammation in cystic fibrosis - Marcus Mall (DE)	
11:50-12:15	A Scottish exploration of the effects of Tez/Iva and Elex/Tez/Iva on systemic and pulmonary inflammation - from bench to bedside - Nicola Robinson (UK)	
12:15-12:25	Abs. 64 - GM1 ganglioside: new insight on its immunomodulatory capacity in CF - Dorina Dobi (IT)	
12:25-12:35	Abs. 65 - Exploring the relationship between *Pseudomonas aeruginosa* infection and SLC6A14 in cystic fibrosis - Manon Ruffin (FR)	
12:35-12:45	Abs. 72 - Differential effects of CFTR modulators on SARS-CoV-2 infectivity in cultured nasal, bronchial, and intestinal epithelia of people with cystic fibrosis - Loes den Hertog - Oosterhoff (NL)	
12:45-14:15	*Lunch*	Level 5 – Admiral's Landing
14:15-16:00	**Symposium 8 – Cell models, organoids in the enhanced life expectancy of patients**	
	Chairs: Marie Egan (US) / Patrick Harrison (IE)	
14:15-14:40	Functions of the pulmonary ionocyte in the proximal ferret airways - John Engelhardt (US)	
14:40-15:05	Intestinal organoids, CFTR function and tissue-specific disease - Jeffrey Beekman (NL)	
15:05-15:30	Role of enteroendocrine cell differentiation in the pathogenesis of CFRD - Daniel Zeve (US)	
15:30-15:40	Abs. 85 - Investigation of CFTR function and epithelial barrier properties at single cell resolution using multi-electrode array technology - Marjolein Ensinck (BE)	
15:40-15:50	Abs. 88 - Endometrium-derived organoids from cystic fibrosis patients to study the endometrial factor in the disease-associated fertility deficiency - Ellen De Pauw (BE)	
15:50-16:00	Abs. 79 - Elexacaftor/Tezacaftor/Ivacaftor treatment partially normalizes osteoclasts phenotype in cystic fibrosis-related bone disease - Johan Sergheraert (FR)	
16:00-16:30	*Coffee Break*	
16:30-17:30	**Closing Keynote lecture**	
	Bridging the Gap: scientific discovery to clinical benefit – Jane Davies (UK)	
20:00	*Dinner / Social Event*	