

2nd COST Action CA21113 Conference, Working Group Meeting
and Workshop - Genome Editing to Treat Human Diseases (GenE-HumDi)

Therapeutic Genome Editing: From Conception to Commercialization



8-10 April 2024 / Pefkos City Hotel, Limassol, Cyprus

Funding Body



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the European Union

Local Organiser



THE CYPRUS INSTITUTE OF
NEUROLOGY & GENETICS

Project



GenE-
HumDi

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Information



Meeting
Secretariat



Oral Presentations Speakers



Basma Naiisseh, The Cyprus Institute of Neurology & Genetics, PhD student, Cyprus

Gonzalo Martinez-Navajas, Pfizer - Universidad de Granada - Junta de Andalucía Centre for Genomics and Oncological Research, PhD student, Spain

Iris Ramos Hernández, Centro Pfizer-Universidad de Granada-Junta de Andalucía de Genómica e Investigación Oncológica, PhD student, Spain

Kiriaki Paschoudi, School of Biology, Aristotle University of Thessaloniki, PhD student, Greece

Melita Vidakovic, Institute for Biological Research "Sinisa Stankovic", Serbia

Petros Patsali, The Cyprus Institute of Neurology & Genetics, Cyprus

Working Groups Leader and CO-Leader



Francisco Martin Molina, Working Group 2 Chair, Fundación Pública Progreso y Salud - GENYO, Spain

Yonglu Luo, Working Group 3 Chair, Aarhus University, Denmark

Ayal Hendel, Working Group 4 Chair, Bar-Ilan University, Israel

Alessia Cavazza, Working Group 5 Chair, University College London, United Kingdom

Carsten W. Lederer, Working Group 6 Chair, The Cyprus Institute of Neurology & Genetics, Cyprus

Javier Molina Estévez, Working Group Chair 7, Fundación Pública Progreso y Salud - GENYO, Spain

Programme



DAY 1 - PUBLIC CONFERENCE

- 08:55-09:00 Short welcoming from the Action chairs, **Karim Benabdellah, Alessia Cavazza**
- 09:00-10:20 Introduction to GE tool and their application
Chair: **Karim Benabdellah, Petros Patsali**
- 09:00-09:20 Current gene editing tools and trends - an overview, **Carsten W. Lederer**,
The Cyprus Institute of Neurology & Genetics, Cyprus
- 09:20-09:40 Mitigation of on-target mutagenesis with HDR-CRISPR, **Claudio Mussolino**,
University Hospital Freiburg, Germany
- 09:40-10:00 Off-target effects, **Eivind Valen**, Oslo University, Norway
- 10:00-10:20 Viral and non-viral delivery methods, **Stephen Hart**, University College London, UK
- 10:20-11:00 **Coffee Break / Discussions**
- 11:00-12:00 **Welcome ceremony & keynote lecture**,
Chair: **Carsten W Lederer, Francisco Javier Molina Estevez**
- 11:00-11:05 Welcome to Cyprus and the conference, **Carsten W Lederer**,
The Cyprus Institute of Neurology & Genetics, Cyprus
- 11:05-11:10 Introduction to COST Action CA21113, **Karim Benabdellah**,
Fundacion Progreso y Salud, Spain
- 11:10-11:20 Research and innovation in Cyprus, **Theodoros Loukaidis**,
Deputy Ministry of Research, Innovation & Digital Policy
- 11:20-11:30 Postgraduate education at The Cyprus Institute of Neurology & Genetics,
Kyproula Christodoulou, The Cyprus Institute of Neurology & Genetics
- 11:30-12:00 Keynote "Women in science, business and Cyprus", **Marianna Prokopi-Demetriades**,
RSL-Labs |TheraMir| ProMedBioscience, Cyprus
- 12:00-13:30 **Selected ITC & ESR Abstracts**,
Chair: **Alessia Cavazza, Laura Torella**
- 12:00-12:15 Enhancing safety and transgene expression for effective paracrine cross-correction with
HSPCs gene editing, **Iris Ramos-Hernández** GENyO, University of Granada, Spain
- 12:15-12:30 Context base editing for splice correction of HBB^{IVS1-110(G>A)} thalassemia,
Basma Naitseh, The Cyprus Institute of Neurology & Genetics, Cyprus
- 12:30-12:45 Preclinical validation of HBB^{IVS1-110(G>A)}-specific gene editing as advanced therapy
for thalassaemia, **Petros Patsali**, The Cyprus Institute of Neurology & Genetics, Cyprus
- 12:45-13:00 Towards the clinical practice: Bernard-Soulier Syndrome type C,
Gonzalo Martinez-Navajas GENyO, University of Granada, Spain
- 13:00-13:15 Use of epigenetic editing tools for direct cellular reprogramming or targeted gene
silencing as a potential pre-therapeutic approach, **Marija Đorđević**,
University of Belgrade, Serbia
- 13:15-13:30 Base editing mediated in vivo induction of -113 A>G HPFH mutation efficiently reactivates
HbF in humanized mouse models of β -hemoglobinopathies, **Kyriaki Paschoudi**,
Aristotle University of Thessaloniki, Greece
- 13:30-14:50 **Lunch Break / Poster Session**

Programme



DAY 1 - CONFERENCE (Open Access)

- 14:50-16:10 **Regulatory and Commercialization**
Chair: **Carla Fuster García, Carsten W Lederer**
- 14:50-15:10 Base-Edited 'universal' CAR7 T cells for T-cell malignancies, **Christos Georgiadis**, University College London, UK
- 15:10-15:30 TBD Commercial products for clinical translation, **Marianna Romito**, Maxcyte, MD, USA
- 15:30-15:50 TBD Commercial products for clinical translation, **Eleni Papanicolaou**, Miltenyi Biotec, Germany
- 15:50-16:10 Non-viral DNA delivery coupled to TALEN gene editing efficiently corrects the sickle cell mutation in long-term HSCs, **Julien Valton Collectis**, France
- 16:10-17:00 **Group Photographs / Coffee Break / Discussions**
- 17:00-18:40 **New Developments**,
Chair: **Francisco Martin Molina, Carlos J Ciudad**
- 17:00-17:20 Improving the efficiency of the CRISPR/Cas system, **Duško Lainšček**, National Institute of Chemistry, Slovenia
- 17:20-17:40 Engineered EVs for biotherapeutic delivery, **Dhanu Gupta**, University of Oxford, UK
- 17:40-18:00 Efficient and specific in vivo genetic engineering of human hematopoietic stem/progenitor cells, **Luca Biasco**, University College London, UK
- 18:00-18:20 Engineering Designer-Recombinases for therapeutic genome editing, **Frank Buchholz**, Technical University Dresden, Germany
- 18:20-18:40 Computational CRISPR gRNA design: From Cas9 to base editing and Cas12a, **Jan Gorodkin**, University of Copenhagen, Denmark
- Transfer to Dinner Venue
- 19:30-21:30 Centrally organised dinner

DAY 2 WG Brain Storming

- 09:00-09:30 Overview and targets for days 2 and 3, **Karim Benabdellah**
- 09:30-10:30 **WG2 - Improvement of GE technology**
Chair: **Francisco Martin Molina**
- 09:30-09:50 Presentation of WG2 and 4 key challenges/goals to address during brainstorming sessions, **Francisco Martin Molina**
- 09:50-10:00 Challenge 1: Improving efficacy, specificity, and safety (ESS) of new GE tools, **Dimitri Ivancic**
- 10:00-10:10 Challenge 2: Improving efficacy of HDR/Repair strategies, **Claudio Mussolino**
- 10:10-10:20 Challenge 3: To avoid immune responses to GE tools, **Julian Ceron**
- 10:20-10:30 Challenge 4: Limitations to advance GE into clinics, **Laura Torella**

Programme



DAY 2 WG Brain Storming

10:30-10:50	Coffee Break / WG2 lead write-up
10:50-11:50	WG3 - Delivery Strategies Chair: Yonglun Luo
10:50-11:10	Presentation of WG3 and 4 key challenges/goals to address during brainstorming sessions, Yonglun Luo
11:10-11:20	Challenge 1: Efficiency of Delivery Systems, Alvaro Somoza, Dr. Manuel Goncalves
11:20-11:30	Challenge 2: Specificity and Off-Target Effects, Yongluo
11:30-11:40	Challenge 3: Delivery to Specific Cell Types and Tissues specificity, in vivo, Annarita Miccio, Lorea Blazquez
11:40-11:50	Challenge 4: Scale-up and manufacturing of delivery systems translation, Gloria González Aseguinolaza
11:50-12:10	Coffee Break / WG3 lead write-up
12:10-13:10	WG4 - Safety issues: Monitoring and standardization Chair: Ayal Hendel
12:10-12:22	Presentation of WG4 and 4 key challenges/goals to address during brainstorming sessions, Ayal Hendel
12:22-12:30	Challenge 1: Off-target effects and where to find them: Challenges in harmonization and standardization of off-target identification tools, Ciaran Lee
12:30-12:38	Challenge 2: Functional consequences of on/off-target site repair outcomes, Ayal Hendel
12:38-12:46	Challenge 3: Finding the best gRNA: Bioinformatics software for gRNA design and selection, Jan Gorodkin
12:46-12:54	Challenge 4: Detection of structural variations in on/off-target sites, Carla Fuster Garcia
12:54-13:02	Challenge 5: Matching treatment to patients: the need for personalized genome editing studies, Nechama Kalter
13:02-13:10	Challenge 6: Genomic stability assessment for Genome Editing enhancers, Giandomenico Turchiano
13:10-14:10	WG4 lead write-up / Lunch break
14:10-15:10	WG5 - Translation into the clinic Chair: Alessia Cavazza
14:10-14:25	Presentation of WG5 and 3 key challenges/goals to address during brainstorming sessions, Alessia Cavazza
14:25-14:40	Challenge 1: From bench to bedside: defining a consensus on the preclinical studies required to move to the clinic, Paula Rio
14:40-14:55	Challenge 2: Hurdles in the manufacturing and release of gene editing-based ATMPs, Jose Bonafont
14:55-15:10	Challenge 3: Improving patient access to gene therapies, Alessia Cavazza
15:10-15:30	Coffee Break / WG5 lead write-up

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DAY 2 WG Brain Storming


15:30-16:30	WG6 - Technology transfer and industry / Regulatory issues Chair: Carsten W Lederer
15:30-15:42	Presentation of WG6 and 4 key challenges/goals to address during brainstorming sessions, Carsten W Lederer
15:42-15:54	Challenge 1: Equity of patient access, Oliver Feeney
15:54-16:06	Challenge 2: Requirement for a separate pediatric regulatory framework, Alejandro Barquero
16:06-16:18	Challenge 3: Focus on safety of products and transparent reporting, Carla Fuster García
16:18-16:30	Challenge 4: Harmonization of regulation and data, Lluis Montoliu
16:30-16:50	Coffee Break / WG6 lead write-up
16:50-17:50	WG7 - Dissemination Chair: Javier Molina Estévez
16:50-17:00	Presentation of WG7 and 5 key challenges/goals to address during brainstorming sessions, Javier Molina Estévez
17:00-17:10	Challenge 1: Increase the reach of our dissemination among young and early career scientists and company professionals, Merita Xhetani
17:10-17:20	Challenge 2: GenE-HumDi visibility, interest to plan a Y3 member and media forum (editors, journalist, scientific writers), Josip Madunić
17:20-17:30	Challenge 3: Proceeding booklet summarizing all the main conferences of each WG. Orchestrating a special issue?, Neli Kachamakova-Trojanowska
17:30-17:40	Challenge 4: Missing a forum for conversations between members, work groups and external stakeholders. Luka Bockor
17:40-17:50	Challenge 5: Contacts and steps needed to propose an Intervention in scientific and medical Assembly at EU Commission. Lluis Montoliu
17:50-18:10	Departure / WG7 lead write-up
19:00-22:00	Guided night-time stroll through Limassol, for those interested / individual dinner

Programme



DAY 3 WG Summaries & Roadmaps | Administrative Issues | Sightseeing

09:00-09:15	Overview for day 3, Karim Benabdellah
09:15-10:45	Presentation of WG Outcomes and Deadlines Chair: Karim Benabdellah
09:15-09:30	WG2 - Improvement of GE technology, Francisco (Paco) Martin Molina
09:30-09:45	WG3 - Delivery Strategies, Yonglun Luo
09:45-10:00	WG4 - Safety issues: Monitoring and standardization, Ayal Hendel
10:00-10:15	WG5 - Translation into the clinic, Alessia Cavazza
10:15-10:30	WG6 - Technology transfer and industry / Regulatory issues, Carsten W. Lederer
10:30-10:45	WG7 - Dissemination, Javier Molina Estévez
10:45-11:05	Coffee Break
11:05-11:55	Final Remarks & Closing Chair: Karim Benabdellah, Alessia Cavazza
11:05-11:30	The CA21113 Roadmap 2024/2025, Karim Benabdellah
11:30-11:45	How-to: COST Claims for Travel Reimbursement, Maria Ángeles, Cayuso Jiménez
11:45-11:55	Conclusion and Sendoff
11:55-11:55	Departure of non-Core Group members / Preparation for sightseeing tour
11:55-12:55	WG Leadership / Core Group Meeting Chair: Karim Benabdellah, Alessia Cavazza
11:55-12:55	Strategic round table
12:55-14:30	Individually organised lunch
14:30-18:30	Sightseeing tour bus to Limassol Carsten W. Lederer, Petros Patsali



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