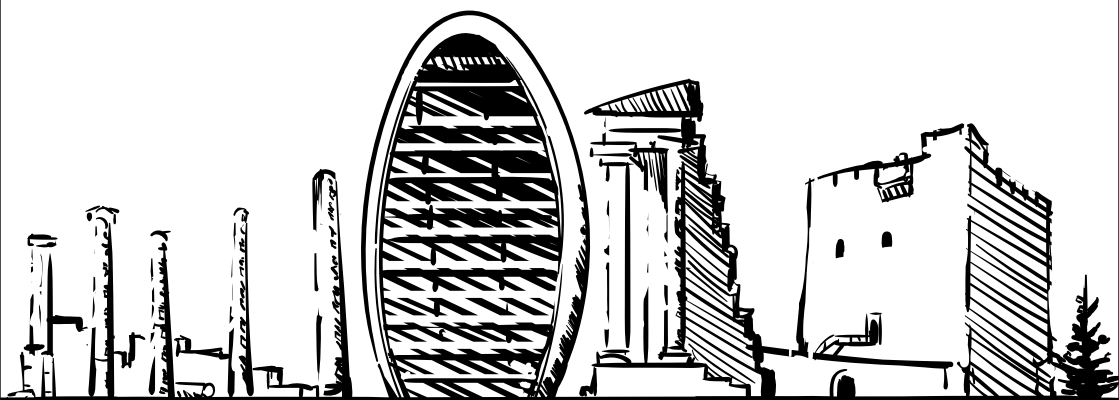


2nd COST Action CA21113 Conference, Working Group Meeting
and Workshop – Genome Editing to Treat Human Diseases (GenE-HumDi)

Therapeutic Genome Editing: From Conception to Commercialization



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

Funding Body



Funded by
the European Union

Local Organiser



THE CYPRUS INSTITUTE OF
NEUROLOGY & GENETICS

Project



GenE-
HumDi

With friendly support by



Information



Meeting
Secretariat





Dear Colleagues and Friends,

Welcome to the GenE-HumDi COST Action CA21113 2nd International Meeting in Limassol, Cyprus on 8 - 10 April 2024!

Recent breakthroughs in genome editing (GE) technologies have ignited new possibilities for treating diseases by precise and efficient genome modification. Our event will delve into the impressive results already achieved, be it in the creation of new tools, in efficiency and cell selectivity of delivery, in cellular or animal models, and across a wide spectrum of diseases. In our public conference on day 1 and our subsequent CA21113 working group meetings we will address the persisting challenges, which include harmonization of assessments and streamlining of regulatory procedures for novel treatments, translation of findings into treatments and company portfolios, and the development of an entrepreneur mindset in young researchers.

We look forward to welcoming you to Limassol, to move our Action closer to our goal of uniting efforts, increasing resilience and facilitating progress in the European GE sector across pharmaceutical and biotech companies, academic institutions, patient advocacy groups and regulatory bodies. Together, we will accelerate the translation of GE technologies for human disease treatment!

Join us for updates on the latest GE research, technology, and tools, as well as discussions with leading EU stakeholders. Let's pave the way toward routine clinical application of GE technology, while savoring the sights and flavors of beautiful Cyprus!

With kind regards,

Carsten Werner Lederer
Local Organizer

Petros Patsali
Local Organizer



Dear Colleagues,

We are pleased to extend our warmest invitation to all members of the GenE-HumDi COST Action CA21113 for our upcoming meeting. This gathering, scheduled to take place in Limassol, on 8-10 April 2024, marks a significant milestone in our collective endeavor to advance genome editing (GE) technologies for the betterment of human health.

As Chair and Co-chair of this esteemed action, it is our privilege to witness the remarkable progress we have achieved since the inception of GenE-HumDi. Our concerted efforts have been instrumental in breaking down barriers that once hindered the widespread application of GE technologies in treating human diseases. Through collaboration among pharmaceutical companies, academic institutions, regulatory agencies, and patient advocacy groups, our synergistic network aimed at accelerating the translation of GE technologies into tangible therapies.

This second annual meeting underscores our commitment to fostering collaboration, sharing knowledge, and ultimately, advancing the application of Genome Editing to benefit patients in need. This meeting presents a unique opportunity for our community to come together, exchange insights, and propel forward in our mission to revolutionize healthcare through Genome Editing.

Moreover, this gathering serves as a platform to communicate our progress and advancements to the broader society in an accessible language.

We encourage each of you to actively participate in the discussions, workshops, and networking sessions planned for the meeting. Your expertise, insights, and collaboration are indispensable as we strive to overcome challenges, establish standardized procedures, and pave the way for the safe and effective application of genome editing in clinical settings.

Together, let us seize this opportunity to reaffirm our commitment to excellence, innovation, and most importantly, to the patients who stand to benefit from our collective efforts. We look forward to welcoming you all to Limassol and to the fruitful exchanges that lie ahead.

Karim Benabdellah
Chair

Alessia Cavazza
Co-chair

Information



About the Meeting

Recent advances in genome editing (GE) technologies have opened up exciting possibilities for treating diseases by precisely modifying patients' genomes. Impressive results have been achieved in animal models for various genetic disorders, infectious diseases, and cancer. However, challenges remain, including inadequate integration of academic research into pharmaceutical company strategies, insufficient interest from academic institutions in regulatory science, and the lack of universal standards for risk-benefit assessment by regulatory agencies. To address public health needs, collaboration between basic and clinical research, regulatory bodies, and stakeholders is crucial.

The GenE-HumDi COST Action CA21113 aims to unite pharmaceutical companies, academic institutions, regulatory agencies, biotechnology firms, patient advocacy associations, information technology and emerging scientific insights to accelerate the translation of GE technologies for human disease treatment. In this year's international meeting in Limassol, Cyprus, we will receive updates on the latest developments in GE research, technology and tools, on pre-clinical and clinical therapeutic application, and on successful commercialization of GE technology. With plenty of scope to also sample the sights and tastes of Cyprus, the event will combine a one-day conference showcasing recent achievements and young researchers, with two days of discussion by leading EU stakeholders to tackle challenges and solutions toward routine clinical application of GE technology.

Acknowledgement

This conference and meeting is based on support by COST (European Cooperation in Science and Technology) for COST Action CA21113 (Gene Editing for the Therapy of Human Diseases, GenE-HumDi). CA21113 is jointly coordinated by



Karim Benabdel Lah, Fundación Progreso y Salud,
Consejería de Salud y Consumo, Spain



Alessia Cavazza, University College London, UK

Information



COST description

COST (European Cooperation in Science and Technology) is a funding agency for research and innovation networks. Our Actions help connect research initiatives across Europe and enable scientists to grow their ideas by sharing them with their peers. This boosts their research, career and innovation.



Funded by
the European Union

Local Organising Committee

Carsten W Lederer - The Cyprus Institute of Neurology & Genetics

Petros Patsali - The Cyprus Institute of Neurology & Genetics



THE CYPRUS INSTITUTE OF
NEUROLOGY & GENETICS

Scientific Committee

Karim Benabdellah

Carsten W Lederer

Yonglun Luo

Cavazza Alessia

Ayal Hendel

Rasmus Bak

Paula Rio

Francisco Martin

Javier Molina Estévez

Event Dates & Venue

The Meeting and Workshop will take place on the 8-10 April 2024 at Pefkos City Hotel, Limassol, Cyprus.

Who can attend

Participation in the event is free. Day 1 is open to the general audience, whereas Days 2 & 3 are only open by prior invitation or to Participants of COST Action CA21113.

Participation Cost

The selection and criteria for reimbursing meeting participants will be in line with COST Principles, notably the Excellence and Inclusiveness Policy (see Annotated rules of COST actions).

Please, note that given the large size of our Action (>250 CA21113 WG members), not all members can be funded by our Action for their attendance of this meeting.

Information



Eligibility for funding has been determined based on each member's active involvement and participation in WG activities to date, by following the recommendations of each Working Group (WG) leader in the selection process. Selected members are expected to significantly contribute to the deliverables and activities of each WG during and after the meeting. This is in line with the goals outlined during the GenE-HumDi annual presentation and ensures a cost-effective and fair use of the available funding.

Twenty further members will be selected for funding and oral/ poster presentation, based on the evaluation of the submitted abstract, where the sole criterion for selection will be its scientific quality.

Action members eligible for reimbursement will receive a formal invitation from the COST portal.

Certificates of Attendance

Certificates of attendance will be issued after the event, based on confirmation by signatures on site and/or attendance log.

Accommodation

You can book your accommodation directly with Pefkos City Hotel, our meeting venue, claiming the below special rates. Send an email to the hotel reservations desk, info@pefkoshotel.com, mentioning the code CA21113.

Single Standard Room, Bed and Breakfast - €66.00 per night

Single Superior Room, Bed and Breakfast - €78.00 per night

Twin Standard Room, Bed and Breakfast - €79.00 per night

Twin Superior Room, Bed and Breakfast - €90.00 per night

About the Destination

Visitors arriving in Cyprus by air may enter the Republic of Cyprus only through the International Airports of Larnaka and Pafos for eligibility for potential reimbursement.

You can travel from the Airports to Limassol, either by bus or by taxi. You can see more information regarding travelling by bus at <http://enlimassolairportexpress.eu/>. Alternatively, see live bus updates in the Moovit App! available on Google Play Store.

Interested to know more about Limassol? Have a look in <https://www.visitcyprus.com/>, or the Limassol Tourism site!



Christos Georgiadis, UCL Great Ormond Street Institute of Child Health, United Kingdom

Claudio Mussolino, Medical Center - University of Freiburg, Germany

Dusko Lainscek, National Institute of Chemistry, Slovenia

Eivind Valen, Oslo University, Norway

Frank Buchholz, Technical University Dresden, Germany

Jan Gorodkin, University of Copenhagen, Denmark

Julien Valton, Collectis, France

Luca Biasco, University College London, UK

Marianna Prokopi-Demetriades, RSL Revolutionary Labs Ltd, Promed Bioscience Ltd and Theramir Ltd, Cyprus

Marianna Romito, MaxCyte, Inc., UK & Ireland

Stephen Hart, UCL Great Ormond Street Institute of Child Health, United Kingdom

Carsten Werner Lederer, The Cyprus Institute of Neurology & Genetics, Cyprus

Dhanu Gupta, University of Oxford, UK

Eleni Papanikolaou, Miltenyi Biotec, Germany

Karim Benabdellah, Pfizer - Universidad de Granada - Junta de Andalucía Centre for Genomics and Oncological Research, Spain

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, Fransisco 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 Naisseh , 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, [Yonglun](#)

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

Programme



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, Lluís 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. Lluís Montoliu
17:50-18:10	Departure / WG7 lead write-up
19:00-22:00	Guided night-time stroll thought 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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