

Coupling Genome editing tools with Delivery systems

Institut d'Investigació Biomèdica de Bellvitge

Barcelona, Spain

3rd and 4th October 2024

Organized by the European COST action “Genome Editing to Treat Human Diseases” (GenE-Humdi; action CA21113), an EU-funded network that connects researchers and innovators across Europe and beyond



PROGRAM AGENDA

Thursday, 3.10.24

Friday, 4.10.24

PROGRAM

Thursday October 3rd 14:00–20:00

14.00–14.30 Registration

14.30-14.45 Welcome from the GeneHumdi COST action Karim Benabdel Lab)

14.45-15.00 Objectives of the workshop (Julian Cerón Madrigal)

Mapping and Advancing Gene Editing Technologies (Chair, Marc Güell, Jan Gorodkin)

15.00-15.30 **Talk 1** Marc Güell, *Novel natural and synthetic gene writers*

15.30-16.00 **Talk 2** Giedrius Gausinas, Caszyme, *Engineering Compact CRISPR Nucleases for Genome Editing*

16.00-16.20 **Talk 3** Julián Cerón, *Exploring the potential of novel Cas9 nucleases*

16.20-16.40 **Talk 4** Carles J. Ciudad, Ph. D. *Endonuclease-independent platforms*

16:40–18:00 Coffee break, posters, networking and Group Photo

Exploring viruses for the CRISPR/Cas system delivery (Chair, Karim Benabdellah)

18:00-18.30 **Talk 5** Alessia Cavazza. In vivo base editing via Virus-Like Particles for the treatment of lysosomal storage disorders.

18:30-18:50 **Talk 6** Gloria Gonzalez Aseguinolaza, Implications of delivering CRISPR Cas9 editing tools using AAV vectors *in vivo*.

19:10–19:50 Selected Short Talks (8 + 2 minutes) (Chair Fco. Javier Molina Estevez)

Short talk 1 Araceli Aguilar González. CRISPNA, a new tool for genome editing and diagnosis

Short talk 2 Ana Pilar Gómez Escribano. Endonuclease-Free RNA Editing Tools for Treating Inherited Retinal Dystrophies

Short talk 3 Merve Nur DUZGUN. Development of Microfluidic-Based Targeted Tumor Suppressor Gene Delivery Systems for Cancer Gene Therapy

Short talk 4 Marcel McCullough Figueras. Combinatorial CRISPR/Cas12a Knockout Panel Reveals Novel DNA Repair Interactions by Mutational Spectra Analysis

19:50-20:20 **Talk 16** Karen O´Hanlon Cohrt (CRISPR Medicine News)

Crucial Role of Dissemination and Public Engagement in Gene Editing Projects

20:30 networking dinner

(The organization will propose a restaurant, but participation is optional, and the expenses are not covered by the organization).

Friday October 4th (9:30 to 14:30)



Keynote opening (Chair, Carla Fuster)

9:30-10:00 **Talk 9** Vittoria Raffa, CRISPR/Cas9 in the era of nanomedicine

Session 4: Advances in Gene Editing: Ex vivo applications (HSCs, T cells and Ips)

10:00-10:15 **Talk 10** Karim Benabdellah Karim; *Fulling cancer immunotherapy through gene editing (T cells).*

10:15-10:45 **Talk 11** Carla Fuster and Jan Gorodkin, CRISPRroots on and off-target assessment of CAR T cells with CD19 antigen knockout

10:45-11:00 **Talk 11** Fco. Javier Molina Estevez, *KI-Ep, a new genome editing platform for transcriptional control of gene expression through naturally occurring epigenetic modifications.*

11:00-11:15 **Talk 12** Laura Batlle Morera; *iPS-Gene editing platform for disease modeling*

11:15-12:00 Coffee break and networking

12:00: 12:30 Selected Short Talks (8 + 2 minutes) (Chair, Francisco Martin)

Short talk 5 Tommaso Ferrari. CRISPRa system to improve the production of rAAV

Short talk 6 Daniela Benati. CRISPR/Cas9-mediated TCR replacement to target MAGE-A1+ metastatic melanoma cells

Short talk 7 Victor Ronco Díaz. Second-generation CARs targeting CLL-1 with CD28 and 4-1BB co-stimulation in AML cell line models

Advances in Gene Editing: In Vivo Applications (Chair, Alessia Cavazza)

12:30-12:50 **Talk 13** Cecilia Jiménez-Mallebrera and Mariana Köber; *Targeting muscle cells to treat Neuromuscular Disorders*

12:50-13:10 **Talk 14** Lourdes R Desviat, Centro de Biología Molecular Severo Ochoa UAM-CSIC; *Targeting hepatocytes for the treatment of liver diseases*

13:10-13:30 **Talk 15** Lorea Blázquez, Department of Neurosciences, Biogipuzkoa Health Research Institute; *Targeting the brain to tackle neurological disorders.*

13:30-14:00 Julian Cerón, **Closing remarks**