



# Optimizing Gene Editing with Advanced Omics: Safety, Off-Target Detection, and Cell Phenotyping

11<sup>th</sup> - 14<sup>th</sup> March 2025

# Centro Pfizer-Universidad de Granada-Junta de Andalucía de Genómica e Investigación Oncológica (GENyO)

*Organized by the European COST action "Genome Editing to Treat Human Diseases" (GenE-HumDi; action CA21113)* 











# Coordinated by

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#### Scientific Committee (SciC)

Carla Fuster, PhD Yonglun Luo, PhD Erden Atilla, MD Juan Roberto Rodríguez, PhD *Raúl Torres, PhD* Karim Benabdellah, PhD

## Industry Specialists (IS)

Lumeng Ye, PhD Juliana Campo Georg Papoutsoglou

## Local Organizing Committee (LoC)

Javier Molina, PhD Paulina Rybakowska, PhD Kristina Pavlovic, PhD Marina Cortijo, PhD Victor Ronco María Ortiz Rafael Campos, PhD

# <u>Grant Holder Manager</u>

Raquel Soriano









# Summary

# Session 1: Principles of CRISPR/Cas Gene Editing

This session will provide an overview of gene editing, covering core concepts and principal platforms. Participants will gain insights into selecting the appropriate gene editing platform and candidate sites, as well as learning the process of guide RNA design for optimal outcomes. Risks associated to unspecific activity of gene editors will also be addressed, including available methods for off-target detection and mitigation strategies.

# Session 2: Gene Editing in Immunotherapy

Focusing on the role of gene editing in cancer treatment, this session will explore cell-based treatments and the enhancement of immunotherapies through gene editing. The generation of CAR T cells will be a central focus, examining approaches to optimize their generation and efficacy.

## Session 3: Harnessing the gene editing potential: optimization, delivery & selection

This session will explore how different editing and delivery strategies can be tailored to meet specific product development needs. Participants will learn about the challenges of gene delivery, cell selection processes for high-quality outcomes, while also addressing GMP compliance issues for clinical applications.

## Session 4: Precision assessment

Emphasizing the evaluation of gene editing performance, this session will explore singlecell analysis techniques to assess targeted editing outcomes at both the genomic and protein levels, including the simultaneous screening for potential genomic aberrations.

## Field trip: Visit to GMP facilities

This guided visit will offer participants a unique opportunity to explore firsthand the GMP facilities at Reina Sofia Hospital and the IMIBIC research center in Córdoba.









| Tuesday (March 11 <sup>th</sup> , 2025) |  |  |                             |  |
|---|--|--|-----------------------------|--|
| 8:30-9:00                               | Check-in and Accreditation   |  |                             |  |
| 9:00 - 9:15                             | <b>Welcome &amp; Overview of the Workshop</b><br><i>Marta Alarcón Riquelme</i> , MD PhD, Scientific Director of GENYO.<br><i>Karim Benabdellah PhD, Carla Fuster PhD</i> |  |                             |  |
|   | Session 1: P   | rinciples of CRISPR/Cas Gene Editing                                       |                             |  |
| 9:15 - 10:00                            | CRISPR system: a powerful tool for genome modification<br>(Lumeng Ye, Novel Therapeutic Material Development<br>GenScript <sup>®</sup> )                                 |  |                             |  |
| 10:00 - 10:45                           | CRISPR-based gene editing design<br>(Yonglun Luo, PhD)   |  |                             |  |
| 10:45 - 11:15                           | Coffee break   |  |                             |  |
| 11:15 - 12:00                           | Gene editing genotoxicity<br>(Carla Fuster, PhD)   |  |                             |  |
|   | Session 2  | : Gene Editing in Immunotherapy  |                             |  |
| 12:00 - 12:45                           | T Cells & CAR-T Cells<br>(Erden Atilla, MD)  |  |                             |  |
| 12:45 - 13:30                           | Fueling CAR-T with gene editing<br>(Karim Benabdellah, PhD)  |  |                             |  |
| 13:30 - 14:30                           | Lunch  |  |                             |  |
|   | Pr   | actical Training (Session A)   |                             |  |
| 14:30 - 15:15                           | Introduction   | to Practical Training (Javier Molina, PhD,                                 | )                           |  |
| 15:15 - 18:00                           | Group 1  | Integrating GMP Compliance<br>(IAVANTE) que no irán a Córdoba              |                             |  |
|   | Group 2  | gRNA Design<br>(Yonglun Luo, PhD & Carla Fuster, PhD)                      | Coffee Break<br>17:00-17:30 |  |
|   | Group 3  | FACS Analysis of Gene-Edited Cells<br>(Victor Ronco & Marina Cortijo, PhD) |                             |  |









| Wednesday (March 12 <sup>th</sup> , 2025)   |   |  |                             |  |  |  |  |
|---|---|--|-----------------------------|--|--|--|--|
| 9:00 - 9:15   | Presentation of the Day's Agenda<br>(Carla Fuster, PhD)   |  |                             |  |  |  |  |
| Session 3: Harnessing the gene editing potential:<br>optimization, delivery & selection |   |  |                             |  |  |  |  |
| 9:15 - 10:00  | CRISPR applications in cancer: Modeling disease and advancing therapies <i>(Raúl Torres, PhD)</i>                           |  |                             |  |  |  |  |
| 10:00 - 10:45   | Delivering CRIPSR systems: options and GMP implications (Juan Roberto Rodríguez, PhD)                                       |  |                             |  |  |  |  |
| 10:45 - 11:10   | Coffee Break  |  |                             |  |  |  |  |
| 11:10 - 11:50   | Accelerating Cell and Gene Therapy Success with cGMP-compliant<br>Gene Editing Components<br>(Juliana Campo, GenScript®)    |  |                             |  |  |  |  |
| 11:50 - 12:30   | CyTOF application in immune monitoring studies<br>(Paulina Rybakowska, PhD)   |  |                             |  |  |  |  |
| 12:30 - 12:50   | Case Study: Purification of CD19-Allogeneic CAR-T Cells<br>(Kristina Pavlovic, PhD)   |  |                             |  |  |  |  |
| 12:50 - 13:30   | CAR-T Cell production optimization: Memory T Cell using Miltenyi<br>Biotec platforms (María Lobo & Elena Torres, Miltenyi®) |  |                             |  |  |  |  |
| 13:30 - 14:30   | Lunch   |  |                             |  |  |  |  |
| Practical Training (Session B)  |   |  |                             |  |  |  |  |
| 14:30 - 17:00   | Group 3   | gRNA Design<br>(Yonglun Luo, PhD & Carla Fuster, PhD)                      |                             |  |  |  |  |
|   | Group 1   | Integrating GMP Compliance<br>(IAVANTE)                                    | Coffee Break<br>17:00-17:30 |  |  |  |  |
|   | Group 2   | FACS Analysis of Gene-Edited Cells<br>(Victor Ronco & Marina Cortijo, PhD) |                             |  |  |  |  |









| Thursday (March 13 <sup>th</sup> , 2025) |   |  |  |  |  |
|--|---|--|--|--|--|
| 9:15 - 9:30                              | Presentation of the Day's Agenda<br>(Karim Benabdellah, PhD)  |  |  |  |  |
| Session 4: Precision assessments         |   |  |  |  |  |
| 9:30 - 10:15                             | Omics approaches for advanced gene editing characterization <i>(Yonglun Luo, PhD)</i>   |  |  |  |  |
| 10:15 - 11:45                            | Profiling efficiency and accuracy with single-cell analysis<br>(MissionBio® representative)   |  |  |  |  |
| 11:45 - 12:15                            | Coffee Break  |  |  |  |  |
| 12:15 - 13:15                            | <b>Roundtable Discussion I</b> : Gene Editing:<br>Emerging Platforms, Delivery Systems and Safety concerns<br><i>Moderated by Yonglun Luo, Carla Fuster and Lumeng Ye</i>         |  |  |  |  |
| 13:15 - 14:30                            | Lunch   |  |  |  |  |
| 14:30 - 15:30                            | <b>Roundtable Discussion II</b> : Cancer and Inmunotherapy:<br>Innovations, Challenges and Future Directions<br>Moderated by Erden Atilla, Raúl Torres and Juan Roberto Rodríguez |  |  |  |  |
| 15:30- 16:00                             | Coffee Break  |  |  |  |  |
| 16:00 - 17:00                            | Closing Remarks & Group Photos  |  |  |  |  |









Sociedad Española

| Friday | (March | 14 <sup>th</sup> , | 2025) |
|--------|--------|--------------------|-------|
|--------|--------|--------------------|-------|

## **Visit to GMP Facilities**

(Only for attendees with travel grant)\*

Pick-up point: GENyO main entrance (

| Location: Hospital R<br>Center Visit Lead: In | eina Sofía, Córdoba <b>Terapia Génica ș Celular</b><br>maculada Herrera, MD, PhD |  |
|---|--|--|
| 9:00 - 11:00                                  | Travel to Córdoba  |  |
| 11:00 - 12:30                                 | Discussion with Experts on GMP & CAR-T Therapy Development                       |  |
| 12:30 - 13:30                                 | Lunch Break  |  |
| 13:30 - 14:30                                 | Guided Visit: GMP Facilities & CAR-T Cell Production Units                       |  |
| 14:30 - 15:00                                 | Closing Discussion & Certificate Distribution                                    |  |
| 15:00 - 16:00                                 | Wrap-up & Feedback Collection  |  |
| ETD 19:00                                     | Estimated Departure (ETD) – Return to GENYO (Granada)                            |  |

\*Additional slots subject to availability









Special thanks to:

• *The Spanish Society for Gene and Cell Therapy (SETCG) for their commitment.* 



· Iavante; GenScript and Miltenyi for their collaboration.











Gene Editing for the treatment of human disease (<u>GeneHumdi</u> | <u>COST action</u> <u>CA21113</u>) is a network Funded by the European Union. For more information visit our website <u>https://www.genehumdi.eu/</u> and follow us on





