



MARCH 2023

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# FIRST MEETING AND WORKSHOP

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***COST Action CA21113 Genome  
Editing to Treat Humans Diseases  
(GenE-Humdi)***



**Funded by  
the European Union**

### **Local Organizing committee**

**Karim Benabdellah**, GENyO, *Fundación Andaluza Progreso y Salud* (FPS)

**Francisco Martín Molina**, GENyO, *Universidad de Granada* (UGR)

**María Bazuelo**, GENyO, *Fundación Andaluza Progreso y Salud* (FPS)

**F. Javier Molina Estévez**, GENyO, *Fundación para la Investigación Biosanitaria de Andalucía Oriental-Alejandro Otero* (FIBAO).

### **Scientific Committee**

Alessia Cavazza (United Kingdom)

Paula Rio (Spain)

Merita Xhetani (Albania)

Rasmus Bak (Denmark)

Karim Benabdel Lah (Spain)

Toni Cathomen (Germany)

Ayal Hendel (Israel)

Yonglun Luo (Denmark)

Marcello Maresca (Sweden)

Francisco Martín (Spain)

Javier Molina Estévez (Spain)

Lluís Montoliu (Spain)

Giandomenico Turchiano (United Kingdom)

Carsten Werner Lederer (Cyprus)

# Monday 13<sup>th</sup> March

Registration and COST sign up	8:00 am
GENyO, Entrance Hall	8:40 am
GeneHumdi kick-off meeting foreword and Institutional Welcome	8:40 am 9:00 am
Keynote session co-sponsored by the Spanish society of gene therapy	9:00 am 9:30 am

Keynote: **Shengdar Q. Tsai**

St Jude Children Research Hospital, USA.

Progress towards potentially curative genome editing therapies.



<b>Session1: Improving Gene Editing Technologies I (WG2)</b>	9:30 am
Chairs: R. Bak & F. Martín	11:00 am

OR01: **Rasmus Bak**

Aarhus University, Aarhus, Denmark

The ever-evolving CRISPR/Cas toolbox for genetic engineering

OR02: **Marc Güell**

Pompeu Fabra University, Barcelona Spain

Precise gene writing in mammalian genomes

OR03: **Giedrius Gasiunas**

CasZyme, Vilnius, Lithuania

Harnessing the diversity of CRISPR-Cas proteins for genome editing.

OR04: **Raúl Torres**

CNIO/CIEMAT IIS-FJD. Madrid, Spain

CRISPR-based strategies to diagnose and target cancer-related fusion oncogenes.

<b>Networking &amp; Coffee break</b>	10:45 am 11:15 am
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<b>Session 1: Improving Gene Editing Technologies II (WG2)</b>	11:15 am
Chairs: R. Bak & F. Martín	12:30 pm

OR05: **Lluís Montoliu – remote connection.**

Centro Nacional de Biotecnología, CNB. Madrid, Spain

Resurrecting CRISPR-associated nucleases from tens to thousands of millions of years ago and validating them as genome editing tools.

OR06: **Julian Grünwald**

Technical University of Munich. Germany

Engineering CRISPR base and prime editors

OR07: **Carsten Werner Lederer**

The Cyprus Institute of Neurology and Genetics; Nicosia, Cyprus

Use cases for therapy development and reverse genetics by genome editing in hematopoietic cells

OR08: **Artur Gittos**

Synthego

Choosing the right GMP partner for your CRISPR-enabled cell and gene therapy

**Improving Gene Editing Technologies III: short talks and round table.**

12:30 pm

1:00 pm

ST01 **Guillaume Pavlovic**

Institut Clinique de la Souris -ICS, PHENOMIN, IGBMC, France

ST02 **Lydia Teboul**

The Mary Lyon Centre, MRC Harwell, UK

ST03 **Duško Lainšček**

National Institute of Chemistry – Slovenia (SI)

ST04 **Araceli Aguilar**

GENyO, Spain

**R. Bak & F. Martín WG02 impressions and wrap up**

**Networking & Lunch Break**

1:00 pm

2:00 pm

Lunchboxes will be available on site for those who pre-ordered/prepaid them

**Session 2: Delivery Strategies I (WG3)**

02:00 pm

Chairs: Y Luo & Benabdel Lah K

03:30 pm

OR09: **Yonglun Luo**

Aarhus University. Aarhus, Denmark

High throughput quantification of on-target and off-target CRISPR gene editing efficiency in cells

OR10: **Axel Schambach**

Hannover Medical School, Hanover Germany

Novel VLPs for delivery of CRISPR-Cas9

OR11: **Rajeevkumar Nair**

Kavli Institute for Systems Neuroscience, Trondheim Norway

State-of-the art viral vector tools and their application in life science research.

OR12: **Rosario Sanchez**

GENYO-UGR. Granada, Spain

Nanotechnology based strategies for delivery of gene editing tools

OR13: **Peng Ling**

Centre Interdisciplinaire de Nanoscience de Marseille Aix-Marseille Université, CNRS. Marseille, France

Modular and adaptive self-assembling dendrimers for nucleic acid delivery

**Networking & coffee break**

3:30 pm

4:00 pm

**Session 2: Delivery Strategies II (WG3)**

4:00 pm

5:00 pm

Chairs: Y Luo & Benabdel Lah K

OR14: **Mazzini Loubna- remote connexion.**  
Technological, Medical and Academic Park (TMAP), Marrakech,  
Morocco  
Mesenchymal stem cells derived exosomes as a gene therapy  
delivery vehicle

OR15: **Kyriakos Kypreos**  
University of Patras Medical School Panepistimioupolis, Rio Greece.  
  
High density lipoprotein in atherosclerosis, obesity, and type II  
diabetes mellitus

OR16: **Virginia Arechavala Gomeza**  
Biocruces Health Research Institute. Bizkaia, Spain.  
The delivery hurdle in the field of Nucleic Acids Therapeutics

OR17: **Natalia Oliva Teles**  
Centro Hospitalar do Porto. Porto, Portugal.  
How can Medical Genetics contribute to GeneHumdi Dissemination  
and Translational Research.

<b>Networking</b>	5:00 pm
<b>&amp; Coffee break</b>	5:30 pm

<b>Session 2: Delivery Strategies II (WG3) short talks and round table.</b>	5:30 pm
Chairs: Y Luo & Benabdel Lah K	6:00 pm

ST05 Julie Petersen  
Aarhus University - Denmark (DK)

ST06 Paula García Sacristan  
GENyO, Spain

ST07 Belén Prado  
Centro Nacional de Investigaciones Cardiovasculares (CNIC, Spain)

ST09 Jan Prochazka  
Institute of Molecular Genetics - Czech Republic (CZ)

**Y Luo & K Benabdel Lah WG03 impressions and wrap up**

<b>Welcome Reception &amp; Networking Activities</b>	6:00 pm
Networking Activities Sponsored by Synthego	7:00 pm

≡ **SYNTHEGO**

## Tuesday 14<sup>th</sup> March

<b>Registration and COST sign up</b>	8:30 am 9:00 am
GENyO, Entrance Hall	
<b>Session 3: Safety issues: Monitoring and standardization I</b>	9:00 am 11:30 am
Chairs: A. Hendel & G. Turchiano,	
OR18: <b>Ayal Hendel</b> Bar-Ilan University, Israel CRISPR gene correction of human HSPCs for SCID therapy: efficacy and safety challenges towards clinical applications.	
OR19: <b>Marcelo Maresca</b> Astrazeneca, Molndal Sweden. SPOT-ON is a Cas9 nuclease with no detectable off targets and reduced chromosomal translocations in vivo	
OR20: <b>Jan Gorodkin</b> University of Copenhagen, Denmark CRISPR-Cas9 on- and off-target design	
OR21: <b>Karim Benabdellah</b> GENyO, Fundación Progreso y Salud Fueling cancer immunotherapy	
OR22: <b>Simone Massalini</b> GeneScript Not only Gene Synthesis. CRISPR services from Research to GMP	
<b>Networking &amp; coffee break</b>	11:00 am 11:30 am
<b>Session 3: Safety issues: Monitoring and standardization II</b>	11:30 am 12:30 pm
Chairs: G. Turchiano & J. Molina Estévez	
OR23: <b>Juan R. Rodríguez-Madoz – remote connection.</b> Hemato-Oncology Program, Cima Universidad de Navarra; IdiSNA; CIBERONC. Pamplona, Spain Universal allogeneic CAR T cells combining CRISPR and transposon-based technologies to overcome dysfunctionalities of autologous CAR T cells.	
OR24: <b>Manuel Gonçalves</b> Leiden University, Leiden, The Netherland Expanding the targeting range of gene-editing tools in human cells	
OR25: <b>Giandomenico Turchiano</b> University College of London, London, United Kingdom Comprehensive genome stability characterization in ex-vivo gene edited primary cells	
OR26: <b>Stefan Seemann</b> University of Copenhagen, Copenhagen, Denmark On- and off-target assessment of RNA-seq data in CRISPR-Cas9 edited cells with CRISPR roots	

<b>Session 3: Safety issues: Monitoring and standardization III short talks and round table.</b>	12:30 pm 1:00 pm
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ST09 Orli Knop  
Bar Ilan University - Israel

ST10 Nechama Kalter  
Bar-Ilan University - Israel

ST11 Luka Bockor  
Institute for Anthropological Research - Croatia

<b>A. Hendel &amp; G. Turchiano WG04 impressions and wrap up</b>
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Networking & Lunch Break	1:00 pm 2:00 pm
Lunchboxes will be available on site for those who pre-ordered/prepaid them	

<b>Session4: Translation into the clinic I (WG5)</b>	2:00pm 3:30pm
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Chairs: Alessia Cavazza & Paula Rio

OR27: **Alessia Cavazza**  
University College of London, London, United Kingdom  
Hematopoietic stem cell gene editing for the treatment of blood disorders

OR28: **Gal Cafri**  
Sheba Medical Center, Ramat Gan, Israel  
Preclinical and Clinical T-Cell therapy programs at Sheba Medical Center

OR29: **Annarita Miccio**  
INSERM, Paris, France  
Genome editing approaches to beta-hemoglobinopathies

OR30: **Michael Schmück-Henneresse**  
BCRT, Charité-Universitätsmedizin Berlin, Berlin, Germany  
Gene editing for immunotherapy and remedies to overcome Cas9 immunogenicity

OR31: **José Bonafont**  
DanausGT - Spain (ES)  
Unlocking gene editing: the road from bench to bedside

Networking & coffee break	3:30 pm 4:00 pm
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<b>Session4: Translation into the clinic II (WG5)</b>	4:00 pm 5:30 pm
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Chairs: Alessia Cavazza & Paula Rio

OR32: **Paula Rio**  
CIEMAT, Madrid Spain  
Therapeutic Application of Base and Prime Editing in Fanconi Anemia

OR33: **Neli Kachamakova-Trojanowska**  
Jagiellonian University, Krakow, Poland  
Application of CRISPR/Cas9 for the generation of monogenic

diabetes disease model based on isogenic human iPSCs lines

OR34: **Alex Garanto**

Nijmegen, Netherland

Therapeutic genome editing for Stargardt disease

OR35: **Cristina Maccalli**

Sidra Medicine, Doha Qatar

The development of CAR-T and NK cell therapy program at Sidra Medicine

OR36: **Joanna Vella**

University of Malta. Centre of Molecular Medicine and Biobanking. Msida, Malta

Gene Editing to study mitochondrial disease in HeK293T

### Translation into the clinic III (WG5) short talks and round table

05:30 pm

06:00 pm

ST12 Elma Mrehic

Genome4life - Bosnia and Herzegovina

ST13 Daniela Ježová

Institute of Experimental Endocrinology BMC SAS - Slovakia

ST14 Noelia Maldonado Pérez

GENyO | UGR. Granada - Spain

ST15 Laura Torrella

CIMA, University of Navarra -, Spain

**Alessia Cavazza & Paula Rio WG05 impressions and wrap up**

CORE GROUP MEETING

6:00 pm

7:00 pm



**20:00 – Evening Networking Activity  
(to be booked, and paid for, individually)**

8:00 pm

11:00 pm



## Wednesday 15<sup>th</sup> March

<b>Registration/ signing COST Attendance</b>		8:30 am
GENyO, Entrance Hall		9:00 am
Gene-Humdi	<b>Maria Jose Bazuelo</b> GeneHumdi Grant Holder Administrative, FPS. Granada, Spain How to successfully submit your expenses claim in e-COST	
<b>Session 5: Regulatory Issues</b>		9:15 am
Chairs: Toni Cathomen		11:30 am
OR37:	<b>Toni Cathomen</b> Medical Center-University of Freiburg, Freiburg, Germany Regulatory considerations when performing off-target analyses	
OR38:	<b>Maria José del Pino – remote connection.</b> Clinical Advisor, Agencia Española de Medicamentos y Productos Sanitarios (AEMPS), Madrid, Spain Gene Editing products as seen by AEMPS, Guidance and resources for researchers and clinicians seeking Clinical approval	
OR39:	<b>Rafi Emmanuel Emendo</b> Biotherapeutics Rehovot, Israel EMD-101, an Autologous HSC-based Product for Treating ELANE-related Severe Congenital Neutropenia	
OR40:	<b>Gloria Carmona</b> Scientific Coordinator. Qualified Person Production and Reprogramming Cell Unit of Seville. Red Andaluza de diseño y traslación de Terapias Avanzadas. Fundación Progreso y Salud. Seville, Spain Quality Assessment and GMP facilities	
OR41:	<b>Javier Alonso</b> Litek Pharma, Bilbao, Spain Preparation of Advanced Therapies Medicinal Product under Isolator	
<b>Networking &amp; coffee break</b>		11:00 am
		11:30 am
<b>Session 5: Technology transfer and industry</b>		11:30 am
Chairs: Monica Paul and Phillip, du Chateaux		12:30 pm
OR42:	<b>Monica Paul</b> Caszyme, Vilnius, Lithuania Novel Applications and limitations of CRISPR based gene therapies	
OR43:	<b>Francisco Martín</b> <b>CRISPNA</b> CRISPNA Bio; a new start-up based on a new tool that combine Cas proteins with PNAs	
OR44:	<b>Karim Benabdellah</b> <b>EVOSTem (EXo CAR)</b> Cell-free immunotherapy-based approach	
<b>Translation into the clinic III (WG5) Round table</b>		12:30 pm

<b>Phillip de Chateau and Monica Paul wrap up</b>		<b>1:00 pm</b>
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OR45: <b>SHORT TERM SCIENTIFIC MISSION</b> and ITCs conference		
Chair Merita Xhetani		
University of Tirana - Albania (AL)		
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OR46: <b>GeneHumdi Communication Brief and Photo Op</b>		
Francisco Javier Molina Estévez		
GeneHumDi SCC		
Fundacion de Investigación Biomedica de Andalucía Oriental (FIBAO)		
<b>FINAL REMARKS &amp; CLOSING:</b>		<b>1:00 pm</b>
		<b>1:30 pm</b>
<b>Karim Benabdel Lah Chair and Alessia Cavazza Co-Chair</b>		

We thank the following for their support of our networking events around the GeneHumdi (COST Action CA21113) Meeting and Training School.

**Diamond:**



**Gold:**



**Silver:**



**Local Organisers:**



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