Mapping and Evaluating Delivery Methods for Gene Editing in Clinical Applications

-a WG3 (Delivery Strategies) & WG5 (Translation into the clinic) joint meeting

September 25, 2025 | Ankara, Turkey

Organized by the European COST Action

"Genome Editing to Treat Human Diseases" (GenE-HumDi; action CA21113)

An EU-funded network connecting researchers and innovators across Europe and beyond

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Content

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1 Organizing committee

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Center for the Technologies for Gene and Cells Therapies (CTGCT),
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Local Organizers:

1. **Fatma Zehra Hapil Zevkliler, Akdeniz University**
2. **Gunhan Gurman, LÖSEV-LÖSANTE Hospital**
3. **Arzu Celalifer Ekinci, LÖSEV Foundation for Children with Leukemia**

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2 Welcome Messages

Welcome from the Chair of GeneHumdi COST Action

Dear colleagues and friends, GenE-HumDi was launched as a COST Action with the mission of building bridges between gene editing actors, connecting biotechnology and clinical research, and fostering collaboration across North and South, East and West. Our goals include enhancing inclusiveness by engaging young researchers, Near Neighbour Countries (NNC) and Inclusiveness Target Countries (ITC), advancing the regulatory framework for gene-edited Advanced Therapy Medicinal Products (ATMPs), and promoting public dissemination to increase awareness of our field. We expect that these one to two days of intensive brainstorming will open the door to new interactions and collaborative projects, and, most importantly, that these efforts will ultimately be reflected in better outcomes for patients in need of advanced therapies. Finally, hosting this Clinical Gene Editing Working Group Meeting in parallel with the LÖSEV - LÖSANTE 3rd International Cancer and Life Symposium further strengthens scientific exchange, fostering dialogue and creating new opportunities for collaboration across disciplines and countries. We sincerely thank you for attending and for sharing your knowledge with the community, which makes this meeting possible and impactful.
Warm regards,

Karim Benabdellah: Chair of GeneHumdi COST Action Fundación Pública Progreso y Salud (FPS); Centre for Genomics and Oncological Research (GENYO) Granada, Spain

Welcome from the WG3 (Delivery Strategies) Leader

CRISPR-Cas9 gene editing has revolutionized the field of molecular biology, offering unprecedented potential for treating genetic disorders, cancers, and infectious diseases. However, the successful translation of CRISPR technology from the laboratory to clinical practice heavily depends on efficient, safe, and targeted delivery systems. These systems are responsible for transporting the CRISPR components—namely the Cas9 enzyme and guide RNA—into specific cells or tissues, ensuring precise gene editing with minimal off-target effects.

Effective delivery is critical to overcoming biological barriers such as cellular membranes, immune responses, and tissue-specific challenges. Various delivery methods, including viral vectors, lipid nanoparticles, and physical techniques like electroporation, have been developed and optimized for different clinical scenarios. Each approach offers unique advantages and limitations in terms of efficiency, safety, and duration of gene editing activity.

Advancements in delivery systems are essential to enhance editing accuracy, reduce immune reactions, and improve patient outcomes. Tailoring delivery strategies to specific diseases and tissues will enable personalized therapies and minimize adverse effects. As we progress, innovative delivery platforms will play a pivotal role in unlocking the full therapeutic potential of CRISPR technology, ultimately transforming the landscape of precision medicine.

The importance of this meeting lies in addressing these critical challenges and exploring innovative solutions to optimize CRISPR delivery systems. Discussions here will be instrumental in shaping the future direction of gene editing therapies and their successful translation into clinical practice.

Dusko Lainscek
Department of Synthetic Biology and Immunology, National Institute of Chemistry (NIC); Centre for the Technologies for Gene and Cell Therapies (CTGCT), Ljubljana, Slovenia

Welcome from the WG5 (Translation into the Clinic) Leader

On behalf of the Gene-HumDi COST Action consortium, it is our great pleasure to welcome you to this symposium dedicated to advancing the delivery of therapeutic gene editors and the clinical translation of gene editing. The emergence of precise and versatile gene editing technologies represents one of the most transformative developments in modern biomedical science. With the potential to correct the underlying causes of genetic diseases, these approaches offer new hope for patients and families who, until now, have had limited or no therapeutic options. At the same time, turning this promise into reality requires overcoming significant challenges—scientific, clinical, regulatory, and ethical—that can only be addressed through collaborative effort and open dialogue.

This symposium has been designed as a dynamic forum where experts from research and clinical practice can come together to exchange knowledge and perspectives. We are convinced that the interactions taking place here, through presentations, discussions, and informal conversations, will help shape innovative strategies, stimulate new collaborations, and accelerate progress toward safe and effective clinical applications of gene editing. We are especially enthusiastic to welcome participants from across Europe and beyond, whose diverse expertise and experience will enrich the conversations and ensure a truly multidisciplinary perspective. It is this collective engagement that will drive impactful outcomes: from clarifying translational pathways, to identifying common challenges, to creating new opportunities for cooperation that extend well beyond this meeting.

We thank you warmly for joining us and for contributing your time, insights, and passion to this endeavor. Together, we can advance the responsible translation of gene editing and move closer to delivering life-changing therapies for patients

Alesia Cavazza Co-Chair of GeneHumdi COST Action Institute of Child Health, University College London (UCL), London, United Kingdom

Welcome from the Local Organizers

Dear colleagues and friends,

It's a great pleasure to welcome you to Ankara. As a member of the organizing committee and a local host, it's a true honor to be part of this event. While I'm a faculty member at the University of Miami now, Ankara is my hometown, and there's no better feeling than welcoming you all to a place that is so special to me. We're also incredibly grateful to the LÖSEV Foundation and LÖSANTE Hospital for their generosity in hosting us at this inspiring venue, which perfectly connects scientific advancement with patient care.

It feels especially meaningful to host all of you here, in the heart of Anatolia. There's a famous saying from the wise and humorous folk hero, Nasrettin Hoca, who once said, "Here in Anatolia, we're standing in the center of the world." I think that quote perfectly captures the spirit of our meeting—we've come from different corners of the globe to stand together at the center of innovation and collaboration in our field.

My own work in transplantation and cellular therapy, specifically T-cell engineering, has shown me firsthand the incredible power these advanced therapies hold. This meeting is a fantastic opportunity to bridge the gap between groundbreaking research and clinical application. By focusing on critical areas like delivery strategies and clinical translation, we're tackling the very challenges that will determine the future of gene editing as a viable treatment for human diseases.

I hope you find the program not only scientifically stimulating but also personally rewarding. Please don't hesitate to reach out to me or the other local organizers if you need anything at all.

Warm regards,

Erden Atilla, MD, PhD, Division of Transplantation and Cellular Therapy, Department of Medicine, University of Miami Miller School of Medicine, Sylvester Comprehensive Cancer Center, Miami, FL, United States of America.

It is a great pleasure to welcome you to Ankara for the joint meeting of WG3 (Delivery Strategies) and WG5 (Clinical Translation). As a faculty member of Akdeniz University, it is truly an honor for me to be part of the organization as one of the local hosts. We are very grateful to LOSEV's LÖSANTE Hospital for kindly providing the venue, which reflects their inspiring mission of connecting science with patient care.

This meeting is also very meaningful for us in Turkiye. Genome editing and gene therapy are recognized as priorities in our national development plan, and as an Inclusion Target Country in the COST framework, Turkiye is committed to building strong scientific collaborations across Europe. Many groups in our country, -including my own- are actively engaged in gene and cell therapy research, and this meeting is a wonderful opportunity to share ideas, build connections and strengthen our network.

I warmly welcome you all, and I hope you will find this meeting both scientifically rewarding and personally enjoyable.
With kind regards,

Fatma Zehra Hapil Zevkliler, PhD, Department of Basic Medical Sciences, Medical Biology, Faculty of Medicine, Akdeniz University

3 Meeting Overview

The upcoming joint meeting of Working Group 3 (Delivery) and Working Group 5 (Translation into the clinic) of the GeneHumdi COST Action is dedicated to fostering collaboration and sharing the latest advancements in genome editing delivery technologies and their translation into clinical applications. Aligned with the objectives of the GenE-HumDi action, the primary goal of this event is to review recent progress, identify remaining challenges, and develop strategic initiatives to enhance the safety, efficiency, and scalability of gene editing therapies. This meeting will serve as a vital platform for multidisciplinary exchange, promoting innovative delivery solutions, both viral and non-viral delivery methods—such as AAV vectors, nanoparticle-based systems, VLPs, and EVs—and exploring their pathways toward clinical implementation. Key topics include GMP scale-up, regulatory considerations, and strategies to accelerate the transition from laboratory research to patient treatments. By aligning efforts across disciplines, the event aims to advance genome editing therapies with a direct and meaningful impact on human health, ultimately accelerating the journey from research to effective clinical solutions for genetic diseases.

Main Objectives

The main objectives of the joint WG3 and WG5 meeting:

1. Review and Discuss the Current State of Delivery Methods for Gene Editing for Clinical Applications: This includes presentations on AAV-mediated in vivo gene editing, nanoparticle-based delivery systems, emerging technologies such as VLPs, EVs, and hybrid systems, and their respective challenges and limitations.

2. Explore and Share Advances in Translational and Clinical Applications of Gene Editing: Focusing on case studies like liver-directed gene editing and preclinical GMP scale-ups, as well as the regulatory considerations for bringing gene editing therapies into clinical practice.

3. Identify Key Challenges and Opportunities in the Development and Optimization of Delivery Systems: Through dedicated roundtable discussions, participants aim to pinpoint current obstacles and potential solutions in delivery methodologies and their clinical translation.

4. Facilitate Networking and Collaborative Opportunities Among Stakeholders: Encouraging dialogue among researchers, clinicians, regulators, and industry representatives to foster partnerships that will advance the safe and effective deployment of gene editing technologies in medicine.

4 Program Schedule

GenE-HumDi COST Action Working Group 3 and Working Group 5 Joint Meeting	
09:30-10:00	Opening Speech: **Overview of Delivery Methods for Gene Editing in Clinical Applications - challenge and limitations** Dusko Lainscek, *National Institute of Chemistry, Ljubljana, Slovenia*
Session 1 Delivery Methods for Gene Editing – State of the Art	
10:00-12:30	**10:00-10:30: AAV-Mediated in Vivo Gene Editing** Rajeevkumar Nair Raveendran, *Norwegian University of Science and Tech* **10:30-11:00: Nanoparticle-Based Delivery of Gene Editors-LNPs** Mariana Köber, *Institute of Materials Science of Barcelona, Spain* **11:00-11:30: Nanoparticle-Based Delivery of Gene Editors-Dendrimers and Other Nanocarriers** Ling Peng, *Centre Interdisciplinaire de Nanoscience de Marseille, France* **11:30-12:00: Emerging Technologies in Gene Editing Delivery: VLPs, EVs, and Hybrid Systems** Alessia Cavazza, *University College London, United Kingdom* **12:00-12:15: Nanoparticle-Assisted Gene Delivery Strategies for Huntington's Disease** *Emre Sefik Caglar, University of Health Sciences, Turkiye*
12:15-13:30	Lunch Break
Session 2 Clinical Applications of Gene Editing – Translational Insights	
13:30-14:30	**13:30-14:00: AAV-Mediated Liver-Directed Gene Editing** Gloria Gonzalez Aseguinolaza, *Director of the Gene Therapy for Rare Diseases program, Clinica Universidad de Navarra, Spain* **14:00-14:30: Preclinical GMP Scale-Ups And Orphan Drug Designation For Pediatric Genetic Diseases** Jose Carlos Segovia, *CIEMAT, Spain*
14:30-15:00	Coffee Break
Session 3 Roundtables	
15:00-15:45	**Roundtable 1: Key Challenges and Opportunities in Delivery Methods for Gene Editing**
15:45-16:30	**Roundtable 2: Clinical Translation of Gene Editing - Regulatory and Manufacturing Insights**
16:30-17:00	Closing Remarks
17:00-18:00	Networking Session

5 Modality of the Meeting-link

Hybrid Meeting

Venue: LÖSANTE Hospital Conference Hall; Kızılcaşar Neighborhood, 23 Nisan Avenue, 2705 Street, No:20, İncek, 06830 Gölbaşı, Ankara, Türkiye

Virtual meeting by Zoom Platform:

https://us06web.zoom.us/j/83797233313?pwd=GjpxQfsdUpySEO9vrpNlhqpcmhmIud.1

***Finally**, we would like to express our sincere gratitude to the **LÖSEV Foundation for Children with Leukemia** and **LÖSANTE Hospital** for their generosity in hosting this meeting in parallel with the 3rd LÖSEV–LÖSANTE International Cancer and Life Symposium.*