Clinical Applications of In Vivo Gene Editing: Methods, Challenges, and Solutions

Duration: Four days; 17rd to 20rd September 2024

Department of Biomedicine, Aarhus University, Denmark

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

Organizer

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2. Dr. Karim Benabdellah, Department of Genomic Medicine, Pfizer-University of Granada-Andalusian Regional Government Centre for Genomics and Oncological Research (GENYO), Granada, Spain

GenEHumDi Support:

1. Dr. Francisco Javier Molina Estevez, SCC
2. Raquel Soriano Martinez, Grant Holder Manager

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Overview:

In this course, we investigate into the complex landscape of gene editing within a clinical framework, focusing on the generation of a comprehensive map detailing current delivery methodologies employed for *in vivo* gene editing across various animal models and clinical scenarios, with some insight for *ex vivo* studies. The course includes a meticulous comparison of efficacy and specificity data, elucidating the diverse array of gene editing tools administered through different delivery methods within specific tissues and organs of interest.

Main Objectives:

1. To become familiar with CRISPR techniques: Participants will gain a thorough understanding of CRISPR technology, including its mechanisms, applications, and potential for genetic modification.

2. To learn about AAV (Adeno-Associated Virus) delivery systems: The course will cover the use of AAV vectors in gene editing, focusing on their design, delivery, especially as Donor template for Gene editing approaches as well for transient endonuclease delivery

3. To explore LNP (Lipid Nanoparticle) systems: Attendees will delve into the principles and applications of LNPs in gene editing, particularly their use in delivering mRNA and other gene editing tools

4. To understand the application of LVs (Lentiviral Vectors): The program will provide insights into the use of lentiviral vectors for gene editing.

5. finally case studies and practical examples of their use in treating different pathologies such as, cancer (Immunotherapy) Neuromuscular Disorders (NMDs), neurological disorders, and Retinal, Pancreatic, and Cardiac Diseases.

Output:

1. **Evaluation Report on Delivery Methods**: Students will create a detailed assessment report analysing various techniques used to deliver gene editing tools in living organisms across diverse research models and medical contexts. This report will delve into the effectiveness, precision, safety, and practicality of each method.
2. **Profile of Gene Editing Effects in Specific Tissues/Organs**: Students will construct a comprehensive profile outlining how different gene editing tools perform when delivered through various methods in particular tissues and organs. This profile aims to shed light on the real-world applications and constraints of gene editing within different biological environments.
3. **Proposal for Experimental Designs**: Students will develop proposals for experimental designs aimed at refining the delivery methods of gene editing tools for specific therapeutic targets in clinical scenarios. These proposals will consider factors such as the accessibility of target tissues, the efficiency of delivery systems, and safety considerations.
4. **Analysis of Case Studies**: Students will examine case studies of gene editing applications in clinical settings. They will critically assess the outcomes,

including the effectiveness of treatments, unintended effects, and long-term implications. Through this analysis, students will gain insights into the practical hurdles and accomplishments of gene editing in clinical practice.

5. **White papers**: Students will draft a White paper based on the recent achievement of in vivo gene editing application based on the recent literature and those described within the course.

Time	Day 1	Day 2	Day 3	Day 4
Topic	CRISPR therapy overview	In vivo therapy with AAV	In vivo therapy with LNP	In vivo therapy with other methods like LVs
9-12	Lectures	Lectures, with one in vivo case	Lectures, with one vivo case	Lectures, with one in vivo base
Lunch break				
13-16	Workshop & lab	Workshop & lab	Workshop & lab	Workshop and project presentation
After TC network activities	Welcome drinks	Network dinner	City tour	End of TS
Notes				

Support:

GeneHumdi finantial support will be available for selected applicants. For Further details visit www.genehumdi.eu or contact the Action Grant Holder Manager Raquel Soriano, the Action Chair Karim Benabdellah orGeneHumdi SCC Javier Molina.

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