Minutes of the GenE-HumDi COST Action Workshop Co-organized with CRISPRMED25

COST Action: CA21113 – GeneHumDi

Activity: GenE-HumDi COST Action Workshop co-organized with CRISPRMED25

Date: 14 April 2026

Venue: Øksnehallen, Vesterbro, Copenhagen, Denmark

Attendance: Face-to-face

Time: 09:00–15:00

By Karim Benabdellah



1. Background and objectives

The GenE-HumDi COST Action co-organised a series of interactive workshops within the framework of **CRISPRMED26**, held in Copenhagen, Denmark, on 14 April 2026. The workshops were designed to provide participants, particularly students and early-career researchers, with an interactive and practice-oriented learning environment covering key aspects of genome editing and its translation towards therapeutic applications. The activity followed a **"learn by doing"** approach, combining expert presentations with discussion, case-based examples, practical activities and opportunities for direct interaction with experts in the field. The workshops aimed to complement the scientific programme of CRISPRMED26 by providing participants with practical knowledge that could be transferred to their own research activities. **The activity contributed to several GeneHumDi objectives**, including strengthening the network, promoting scientific exchange and collaboration, improving research and training capacity, and disseminating knowledge on genome-editing technologies.

2. Workshop programme

Three thematic workshops were organized covering complementary aspects of the development of genome-editing technologies:

1. ***Tools: Choosing the Right Gene-Editing Approach***
2. ***Delivery: Navigating the CRISPR Delivery Landscape***
3. ***Safety: Pre-Clinical Safety Analyses***

Together, the three workshops addressed key stages in the development of genome-editing strategies, from the **selection of the appropriate editing technology**, through **delivery to target cells**, to the **assessment of safety and specificity**.

3. Workshop 1 – Tools: Choosing the Right Gene-Editing Approach

Facilitators

- **Dr. Claudio Mussolino**, Universitätsklinikum Freiburg, Germany
- **Dr. Antonio Carusillo**, Università di Modena e Reggio Emilia, Italy

Content

The workshop introduced participants to different genome-editing technologies, focusing on their underlying mechanisms, advantages and limitations.

Particular attention was given to recent developments in genome-editing approaches that avoid conventional DNA double-strand breaks (DSBs), including **base editing and prime editing**.

The session highlighted the importance of selecting the most appropriate genome-editing technology according to the **disease, target and therapeutic objective**.

Different therapeutic objectives were considered, including:

- Gene replacement

- Gene restoration
- Gene insertion
- Correction of disease-associated variants
- Modulation of gene expression

The workshop also discussed the potential applications of newer editing approaches in therapeutic development. For example, the material presented during the session highlighted the potential of **base editing** for correcting specific nucleotide changes and other targeted modifications, while **prime editing** provides a broader editing capacity for different types of sequence alterations. The workshop also introduced **epigenome editing** as an alternative approach to regulate gene expression without directly modifying the DNA sequence.

Practical component

A hands-on component was included in which participants were encouraged to apply the concepts discussed during the workshop to the design of genome-editing experiments.

The practical activities were aimed at developing participants' ability to:

- Select an appropriate editing technology.
- Consider the therapeutic objective of an intervention.
- Evaluate the advantages and limitations of different editing strategies.
- Design an appropriate experimental approach.
- Consider potential safety and ethical implications.

Ethical and safety considerations were integrated into the discussion, with emphasis on regulatory requirements and best practices.

The workshop was highly interactive, with questions and discussion incorporated throughout the session. The training material also illustrated the application of different editing strategies to therapeutic development, including examples related to **sickle cell disease and thalassemia**, demonstrating how the choice of editing strategy can depend on the underlying genetic defect and therapeutic objective.

4. Workshop 2 – Delivery: Navigating the CRISPR Delivery Landscape

Facilitators

- **Dr. Juan Antonio Marchal**, University of Granada, Spain
- **Dr. André Görgens**, Karolinska Institutet, Sweden

Content

The workshop focused on the current landscape of **viral and non-viral delivery systems** for CRISPR-based genome-editing technologies.



Dr. **Juan Antonio Marchal** provided an overview of the different delivery systems available for genome-editing applications, discussing their principal characteristics, advantages and limitations. Particular emphasis was placed on **extracellular vesicles (EVs)** as emerging non-viral delivery systems. The presentation addressed the potential of EV-based platforms for the delivery of genome-editing components and their possible application in therapeutic development. The presentation highlighted the importance of considering the characteristics of the delivery platform when designing a genome-editing strategy, particularly in relation to the target cell and the intended therapeutic application.

Dr. **André Görgens** focused specifically on the **characterisation of extracellular vesicles**,



addressing important considerations associated with the evaluation of EV-based delivery systems. His contribution highlighted the importance of appropriate EV characterisation in assessing the properties and quality of EV preparations and in supporting the development of EVs as delivery platforms.

CRISPR-Delivery-Cell matchmaking

A central concept discussed during the workshop was the importance of selecting the appropriate delivery system according to both the **genome-editing tool and the target cell**. The workshop introduced the concept of **"CRISPR-Delivery-Cell (CDC) matchmaking"**, highlighting that successful genome editing requires consideration of the compatibility between:

- The genome-editing system.
- The delivery platform.
- The target cell.
- The intended therapeutic application.

This provided participants with an integrated framework for considering delivery as a key component of genome-editing design rather than as an independent technological step.

The workshop therefore connected the selection of genome-editing tools with the biological and technological requirements associated with their delivery and potential therapeutic use.

5. Workshop 3 – Safety: Pre-Clinical Safety Analyses

Facilitators

- **Dr. Ciaran Lee, PhD**, University College Cork, Ireland
- **Prof. Yonglun Luo**, Aarhus University, Denmark

Content

The workshop focused on the potential safety risks associated with genome-editing technologies, with particular emphasis on unintended genomic modifications. The session addressed the risks associated with the unspecific activity of CRISPR-based platforms and their potential impact on both the **safety and efficacy** of genome-editing approaches. Participants were introduced to different types of potential genotoxic events and to methods for monitoring unintended genomic modifications.

The main topics addressed included:

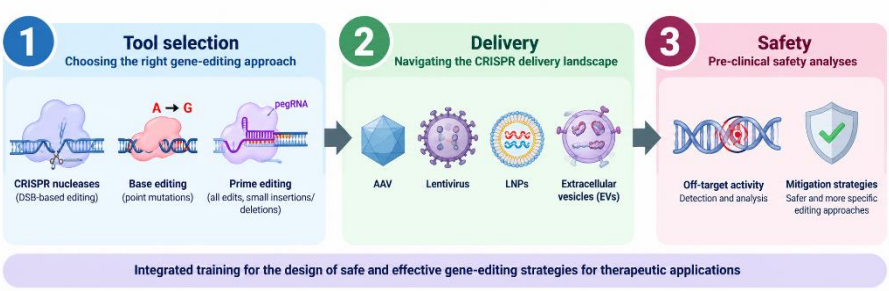
- **Off-target activity of genome editors**
- **Genotoxicity**
- **On-target and off-target genomic aberrations**
- **Methods for evaluating off-target activity**
- **Strategies for mitigating unintended effects**
- **Specificity prediction**

Practical training

The workshop included **basic hands-on training on specificity prediction**, allowing participants to become familiar with approaches used to assess the potential specificity of genome-editing systems. The session emphasised the importance of incorporating safety assessment into the development of genome-editing strategies, particularly when technologies are being considered for translational and clinical applications.

6. Integrated learning across the workshops

The three workshops provided complementary training covering different stages of genome-editing development. The overall structure allowed participants to consider the development of a genome-editing strategy from three interconnected perspectives:



1. Tool selection → 2. Delivery → 3. Safety

The first workshop addressed how to select the appropriate genome-editing approach according to the disease and therapeutic objective. The second workshop addressed how the selected editing system can be delivered efficiently to the relevant target cells, with particular attention to viral and non-viral systems and the emerging role of extracellular vesicles. The third workshop addressed how the resulting genome-editing strategy can be evaluated from a safety perspective, particularly regarding unintended genomic modifications and off-target activity. This integrated approach provided participants with a broader understanding of the factors that need to be considered when designing genome-editing strategies for therapeutic applications.

7. Training and capacity building

The workshops contributed directly to the GeneHumDi objective of improving the quality of scientific and research training in genome editing.

Participants were exposed to practical knowledge covering:

- Selection of genome-editing technologies.
- Base and prime editing.
- Alternative approaches to DNA editing.
- Delivery technologies.
- Extracellular vesicle-based delivery.
- EV characterisation.
- CRISPR-delivery-cell matching.
- Off-target activity.
- Genotoxicity.
- Specificity assessment.
- Safety considerations.

The training therefore covered both **discovery-oriented and translational aspects** of genome editing.

8. Contribution to the GeneHumDi GP Goals

The activity contributed to four associated GeneHumDi GP Goals.

GP Goal 1 – Strengthening and expanding the GeneHumDi network the workshops brought together members of the GeneHumDi community with experts from academic institutions and different areas of genome-editing research. The activity strengthened interactions within the network and created opportunities for new scientific and professional connections.

GP Goal 2 – Linking ongoing gene-editing research The workshops facilitated the exchange of knowledge across different areas of expertise, including genome-editing technologies, delivery systems and safety assessment. By addressing these areas within a common training programme, the activity promoted a more integrated understanding of genome-editing research and its translation.

GP Goal 3 – Enhancing scientific and research training This was a central objective of the activity. The workshops provided participants with an interactive and practical learning environment covering both fundamental and translational aspects of genome editing.The **hands-on and case-based approach** allowed participants to develop practical competencies that could be transferred to their own research activities.

GP Goal 4 – Dissemination and public engagement The activity contributed to the dissemination of knowledge on genome-editing technologies and their potential applications in human disease. By providing accessible training and promoting interaction between researchers and experts, the workshops supported the broader dissemination and understanding of developments in genome editing.