

**Co-organised GenE-HumDi COST Action 21113 and UNIMORE Summer School**

## **GENE EDITING: FROM TOOLS TO THERAPEUTIC APPLICATIONS**

June 18-20 2025 @ TECNOPOLO UNIMORE. Via Pietro Vivarelli, 2 - 41125  
Modena, Italy



### **Local Organizers**

Prof Laura De Rosa, Prof Alessandra Recchia, Prof Alessia Cavazza

### **Trainers**

Dr Claudio Mussolino (University of Freiburg, Germany), Dr Giandomenico Turchiano (AstraZeneca, UK), Dr Antonio Carusillo (Alia Therapeutics, Italy), Prof Alessia Cavazza (UNIMORE, Italy; University College London, UK; Gene-HumDi COST Action co-chair)

## PROGRAMME

### WEDNESDAY 18 JUNE 14:00-17:30

#### **Welcome and Introduction**

14.00 – 14.30

- Welcome from the GeneHumdi COST action and PhD School in Molecular and Regenerative Medicine

- Overview and Objectives of the workshop

(A. Cavazza, L. De Rosa, A. Recchia )

#### **Session 1: Genome Engineering for Human Therapies**

14:30 – 15:30 Lecture: Gene Editing in a Nutshell: dusting off the DNA repair pathways  
(A. Cavazza)

15:30 – 15:40 Interactive Q&A with Mentimeter

#### **15:40-16:00 Coffee break**

#### **Session 2: Genome Engineering Tools**

16:00 – 16:30 Lecture: From Gen X to Gen Z: ZFN, TALEN and CRISPR-Cas (C. Mussolino)

16:30 – 17:00 Lecture: Epigenome editing (C. Mussolino)

17:00 – 17:20 Lecture: Base and Prime Editing (A. Carusillo)

17:20 – 17:30 Interactive Q&A with Mentimeter

### THURSDAY 19 JUNE 9:30-17:30

#### **Session 3: Delivery of genome editing components**

09:30 – 10:30 Lecture: Delivery of engineering tools and donor DNA templates for HDR  
(A. Cavazza)

10:30 – 10:40 Interactive Q&A with Mentimeter

#### **10:40-11:00 Coffee break**

#### **Session 4: Safety & Therapy design (G. Turchiano and A. Carusillo)**

11:00 – 11:45 Lecture: Assessing safety for genome editing (G. Turchiano)

11:45 – 13:00 Practical session Intro “design and defend your safe and efficient gene therapy strategy: one disease, many tools”

#### **13:00-14:00 Lunch**

**Session 4b**

14:00 – 16:00 Practical session: “Hands-on design and defend your safe and efficient gene therapy strategy: one disease, many tools”

**16:00-16:15 Coffee break****Session 4c**

**16:20-17:30 Discussion of gene editing approaches proposed by the working groups**

**FRIDAY 20 JUNE 9:30-13:00****Session 5: Clinical Application of Gene Editing**

09:00 – 11:00 Lectures *From bench to bedside: gene editing for clinical applications pt.1*  
*Ex vivo gene editing for blood disorders* (A. Cavazza, G Santilli)

*Ex vivo gene editing for cell-based Immunotherapy* (A. Carusillo, D Benati)

**11-11:30 Coffee break**

11:30 – 12:30 Lectures *From bench to bedside: gene editing for clinical applications pt.2*  
*In vivo gene editing for skin and ocular disorders* (L. De Rosa, A. Fabrizi, A. Recchia)

12:30 -12:45 Closing Remarks

**Details of hands-on sessions**

**Trainees are asked to bring along their own laptops for practical sessions and case studies.**

**Session 4**

Practical session: Trainers will illustrate software for the design of gRNAs, HDR template, base editors, epigenome editors, and assess the safety.

Case study “One disease, many tools”: trainees will be split into working groups. Each working group will propose a custom approach to tackle a given disease, taking into account potential efficacy and safety. Groups will prepare a short presentation and will present their proposed strategy.

