

**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

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 (Giando & Antonio)

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**

09:30 – 10:45 Lecture *From bench to bedside: gene editing for clinical applications pt. 1*
(A. Cavazza, L. De Rosa, A. Recchia, C. Mussolino, G. Turchiano, A. Carusillo)

10:45-11:10 Coffee break

11:10-12:45: Lecture *From bench to bedside: gene editing for clinical applications pt. 2*
(A. Cavazza, L. De Rosa, A. Recchia, C. Mussolino, G. Turchiano, A. Carusillo)

12:45-13:00 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.

