

November 15-16th 2021

h. 9.30 – Seminar Room INGM Policlinico, Padiglione Invernizzi via Sforza 35, Milano



GENOME EDITING OF STEM CELLS

FOR ADVANCED DISEASE MODELLING AND TREATMENT
THEORETICAL AND PRATICAL COURSE

SPEAKERS:

Dario Besusso^{1,2}
Maura Galimberti ^{1,2}
Simona Lodato ³
Bernhard Schmierer ⁴

- 1. Department of Biosciences, University of Milan, Milan, Italy.
- 2. Istituto Nazionale Genetica Molecolare, Milan, Italy.
- 3. Department of Biomedical Sciences and Istituto di Ricovero e Cura a Carattere Scientifico (IRCCS) Humanitas Clinical and Research Center, Humanitas University, Milan, Italy.
- 4. Department of Medical Biochemistry and Biophysics, Karolinska Institute, Stockholm, Sweden.

ORGANIZERS:

Elena Cattaneo^{1,2}
Dario Besusso^{1,2}





November 15th PROGRAM



9:30 - 9:35 Opening & Welcome

Chairs: Elena Cattaneo, Dario Besusso

9:35 – 11:00 Dario Besusso History of disease modelling in a dish: from descriptive to predictive modelling

Introduction to the concept of *in vitro* disease modelling using conventional and advanced stem cell-based systems.

11:00 - 12:30 Maura Galimberti

A review of 3D dimensional cultures: organoids and assembloids for advanced disease modelling and treatment for brain, heart and liver diseases

To reflect the complexity of mammalian organism, the complexity of organoids is being ever increased and they can now include different cell types allowing to model cross-tissue interactions

Lunch Break

14:30 - 16:00 Dario Besusso

CRISPR-engineered cells for tailored disease modelling

An overview on how genetic modifications can help design better cell models.

16:00 – 17:30 Bernhard Schmierer Stem cell platforms for gene perturbation and CRISPR screenings

An overview of the potential of CRISPR/Cas9 screening in PSCs for the simultaneous interrogation of thousands of protein coding genes, IncRNAs or other genetic elements for involvement in biological and pharmacological processes of interest.



November 16th PROGRAM



9:30 – 11:00 Simona Lodato CRISPR in vivo gene perturbation to understand gene function and disease risk

Examples on how to leverage in vivo model to investigate gene function and disease risk using CRISPR technology

11:00 - 13:00 Dario Besusso

Gene editing for new pharmacological interventions

The lesson will review how CRISPR can be harnessed to generate new biomolecules and pharmacological products for the treatment of human diseases

Lunch Break

14:30 – 18:00 Dario Besusso, Maria Lidia Mignogna, Gianluca Damaggio

Practical lesson

Hands on the use of the third-generation sequencing technology Oxford Nanopore for DNA long-reads analysis

