

20-21 June 2024 - Milan, Salone PIO XII - via Sant'Antonio 5

Congress of the SPOKE 9 The Pharmacology of RNA Drugs: An Unmet Pharmacological Need Tackled by the National Centre of RNA Drugs.

PROGRAMMA Thursday **20th June 2024**

9.00 – 9.30 **REGISTRATION**

9.30 – 9.50 *Welcome by the authorities*

Prof. Elio Franzini, Rector of the University of Milan

Prof. Adriana Maggi, National Center for Gene Therapy and Drugs based on RNA Technology

9.50– 10:00 **Opening Remarks (1° Day): Prof. Paolo Ciana**, University of Milan

Session: RNA Drug Pharmacokinetics

Chair: Prof. Cristiana Perrotta, University of Milan

10.00 – 10:30 *Prof. Tommaso Bellini*, University of Milan

“Silencing of the R4496C mutation in the RYR2 gene: analysis of the sequence dependence of siRNA-mRNA hybridization by label-free biosensors”.

10.30 – 11:00 *Dr. Electra Brunialti*, University of Milan

“Multidimensional reporter platform: a streamlined approach for profiling innovative drug effects”.

11:00 – 11:15 *Prof. Luisa Ottobrini*, University Milan

“Development of non-invasive procedures for the study of RNA-based drugs biodistribution: preliminary in vitro results”.

11.15 – 11.45 *Coffee break*

Session: RNA Drug Pharmacodynamics

Chair: Prof. Massimiliano Pagani, University of Milan

11.45 – 12.15 *Dr. Elisa Di Pasquale*, IRGB-CNR

“Assessment of biocompatibility of the allele-specific RyR2-mRNA silencing through CaP-U10 nanoparticle formulations in cardiac cellular models.”

12.15 – 12:45 *Dr. Mattia Toninelli*, IFOM

“Charting the immune landscape and communication axes in metastatic colorectal cancer with spatial transcriptomics.”

12.45 – 13:00 *Prof. Simona Lodato*, Humanitas University

“Investigating Neurodevelopmental Origins and RNA-based Therapeutic Interventions in C9ORF72 ALS Patient-Derived 3D Cortical Organoids”.

13:00 – 14.30 *Lunch & Poster session*

14.30 – 15.00 **Special guest: Prof. Giorgio Valentini**, University of Milan

“An RNA Knowledge Graph to support AI-boosted research on the RNA world”.

15.00 – 16:00 **Roundtable - the unmet pharmacological need in the design, discovery and development of RNA drugs**

Chair: Dr. Fulvio Ferrara, CDI Centro Diagnostico Italiano S.p.A., CDI and **Prof. Paolo Ciana**, University of Milan

Panelists: Dr. Giancarlo Aquino, Chiesi Farmaceutici S.p.A; **Dr. Franca Cattani**, Dompé Farmaceutici S.p.A.; **Dr. Giandomenico Turchiano**, Astra Zeneca.

16.00 – 17.30 *Coffee break and One-to-One meetings*

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PROGRAMMA Friday 21st June 2024

9.00 – 9.30 REGISTRATION

Session: Toxicology and Immunoreactivity of RNA drugs

Chair: Prof. *Emanuela Corsini*, University of Milan

9.30 – 10:00 Prof. *Domenico Mavilio*, University of Milan

“In vitro characterization of the effect of nucleic acid therapeutics on immune cells”.

10.00 – 10:30 Prof. *Laura Rigon*, University of Padua

“Exploring ER Stress and UPR Pathway Activation as Biomarkers for Liver Toxicity in Liver-Targeted Gene Therap”.

10.30 – 10:45 Prof. *Marco Peviani*, University of Pavia

“In vivo platform to investigate predictive biomarkers of the CNS-specific immune-toxicological effects induced by NA-loaded NPs”.

10:45 – 11:10 Coffee break

Session: Regulatory framework

Chair: Prof. *Paola Minghetti*, University of Milan

11:10 – 11:35 Dr. *Sara Mucherino*, University of Naples Federico II

“Outcome research as a key in the regulatory process: Cost-Effectiveness Of Treatment with Advanced Therapy Medicinal Products”.

11:35 – 12:00 Dr. *Sara Manellari*, University of Milan

“Nucleic acids as medicinal products: the regulatory landscape”.

12:00 – 13:00 Roundtable – Fast track to reduce toxicity and speed the market authorization of RNA drugs

Chair: Dr. *Roberto Orsenigo*, Novartis Farma S.p.A. and Prof. *Paola Minghetti*, University of Milan

Panelists: Dr. *Laura Berardi*, Stevanato Group; Dr. *Stefano Baila*, Eurofins Biolab Srl;
Dr. *Fabio Selis*, Thermo Fisher Scientific; Dr. *Vincenzo Salvatore*, University of Insubria

13.00 – 14.00 Lunch & Poster session

14.00 – 15.00 One-To-One

15.00 – 15:15 Concluding Remarks
Prof. *Paolo Ciana*, University of Milan