



National Center for Gene Therapy and Drugs based on RNA Technology

Spoke 3

Schedule Meeting 5th-07th October 2025

RNA-based innovative therapies for neurological disorders

Day 1 5th of October

17.00-17.15 Welcome Gustincich - IIT

17.15 – 17.30 Welcome Rizzuto – CN3, UniPD

Theme 1 : RNA Metabolism and therapeutics (Chairs Gustincich Oliviero)

17.30- 18.30 Plenary Lecture

Prof. Adrian R. Krainer

St. Giles Foundation Professor
Cancer Center Program Co-Leader
Cold Spring Harbor Laboratory
USA

18.30 – 19.30 Poster Session (sala Colonna)

19.30 Apericena Buffet

Day 2 6th of October

9.00 – 9.20 Prof. Oliviero Salvatore. – UniTO

“Non-coding RNAs in Brain Developmental Diseases”

9.20 – 9.40 Dr. De Pietri Tonelli Davide – IIT

“piRNA Pathway Functions in the Aging Brain: From Synaptic Plasticity to Neuroinflammation”

9.40 – 10.00 Prof. Tartaglia Gian Gaetano– IIT

"Breaking Boundaries in Biotechnology: Designing RNA molecules for Diagnostics and Therapeutics"

10.00 – 10.20 Dr. Siciliano Velia. – IIT

“RNA-based Bioengineering Approaches to Therapy”

10.20 – 11.00 Coffee break

Theme 2 : Neurodev (Chairs Bonora Cancedda)

11.00 – 11.20 Prof. Presutti Carlo- UniROMA1

To be determined.

11.20 – 11.40 Dr. Barberis Andrea – IIT

“SINEUPs for the Treatment of Genetic Epilepsies Linked to Impaired GABAergic Inhibition”

11.40 – 12.00 Prof. Gustincich Stefano.– IIT

“multiSINEUPs for the Treatment of 22q11.2 deletion syndrome”

12.00 – 12.20 Dr. Cancedda Laura – IIT

“NKCC1 miRNA Gene Therapy as a Potential Treatment for Normal Pressure Hydrocephalus”

12.20 – 12.50 Invited Speaker

Prof. Gaia Novarino

Executive Vice President, Institute of Science and Technology Austria (ISTA)

Vienna

Austria

“From Molecular Programs to Neural Systems: Navigating the Complex Landscape of Autism Spectrum ”

12.50 – 14.00 Buffet Lunch + poster session

Theme 3 : AD/PD/ALS (Chairs Antonini Frigeri)

14.00 – 14.20 Prof. Rosa Alessandro - UniROMA1

“The RNA-binding Protein ELAVL4/HuD: from Basic Molecular Mechanisms to a New Possible Target in ALS”

14.20 – 14.40 Prof. Ballarino Monica - UniROMA1

To be determined.

14.40 – 15.00 Prof. Denti Michela – UniTN

“Therapeutic Modulation of RNA Splicing for the Treatment of 4R Tauopathies”

15.00 – 15.20 Prof. Antonini Angelo- UniPD

“Clinical, Biological and Genetic Characterization of the PADUA-CESNE Cohort”

15.20 – 15.50 Invited Speaker

Prof. Piero Fratta

Professor of Cellular and Molecular Neuroscience
Department of Neuromuscular Diseases
University College London
London, UK

15.50 – 16.30 Coffee break

Theme 4 : Expanded repeats (Chairs Poletti Provenzani)

16.30 – 16.50 Prof. Poletti Angelo – UniMI

“Targeting Expanded Repeat-Derived Toxic Proteins: Emerging Interventions for Motor Neuron Disorders”

16.50 – 17.00 Prof. Corti Stefania– UniMI

“Therapeutic Targeting of C9ORF72-G4C2 Repeats: An Innovative Approach to Mitigate Neurotoxicity in ALS/FTD”

17.00 – 17.20 Prof. Biagioli Marta – UniTN

“The Conserved *circHTT*(2,3,4,5,6) in Huntington’s Disease: From Pathological Insight to Therapeutic Innovation”

17.20 – 17.40 Prof. Martello Graziano- UniPD

“Testing the Therapeutic Potential of MTF1 Gene Delivery in Human *in vitro* Models of Huntington’s Disease”

17.40 – 18.00 Dr. Maglione Vittorio - NEUROMED

“Nanoparticle-mediated Delivery of Therapeutic RNA in Pre-clinical Models of Neurodegenerative Diseases”

18.00 – 18.30 Invited Speaker

Prof. Davide Trotti – Jefferson University, USA

Professor | Dept. of Neuroscience
Thomas Jefferson University
Director of Research | Weinberg ALS Center
Vickie and Jack Farber Institute for Neuroscience

“Genome Instability and Innate Immune Stress Response in Neurodegeneration”



18.30 – 20.0 Poster Session

Restricted dinner (invited + steering committee)

Day 3 7th of October

Theme 5 : Stroke/Retina (Chairs Taglialatela Toni)

9.00 – 9.20 Prof. Surace Enrico Maria- UniNA

“Transcriptional Repression as a Disease-modifying Gene Therapy for Gangliosidosis”

9.20 – 9.40 Prof. Pignataro Giuseppe- UniNA

To be determined.

9.40 – 10.00 Prof. Toni Danilo- UniROMA1

To be determined.

10.00 – 10.20 Prof. Siciliano Gabriele – UniPI

“Retina-Gut-Brain Axis and Paths to Interfere at RNA level with Neurodegeneration in ALS and AD: from Mouse Models to Clinical Correlates”

10.20 – 10.40 Prof. Espinoza Stefano – UniPO

“SINEUP Targeting PRPF31 for Retinitis Pigmentosa 11: a Proof of Concept for an Innovative, Effective Therapy”

10.40 – 11.00 Prof. De Falco Sandro– CNR

“Proof-of-concept of Gene Editing Approach to Inhibit Pathological Neovascularization in Age-related Macular Degeneration”

11.00 – 11.30 Coffee break

11.30-12.30 Plenary Lecture

Prof. Michael Chopp

Vice Chairman for Research of the Department of Neurology
Scientific Director of the Henry Ford Neuroscience Institute
The Zoltan J. Kovacs Chair in Neuroscience Research
Detroit, USA

“Small Extracellular Vesicles-outsized Therapeutic Effects on Stroke and Neural Injury”

12.30 – 13.00 Concluding Remarks



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End at 13 00 pm