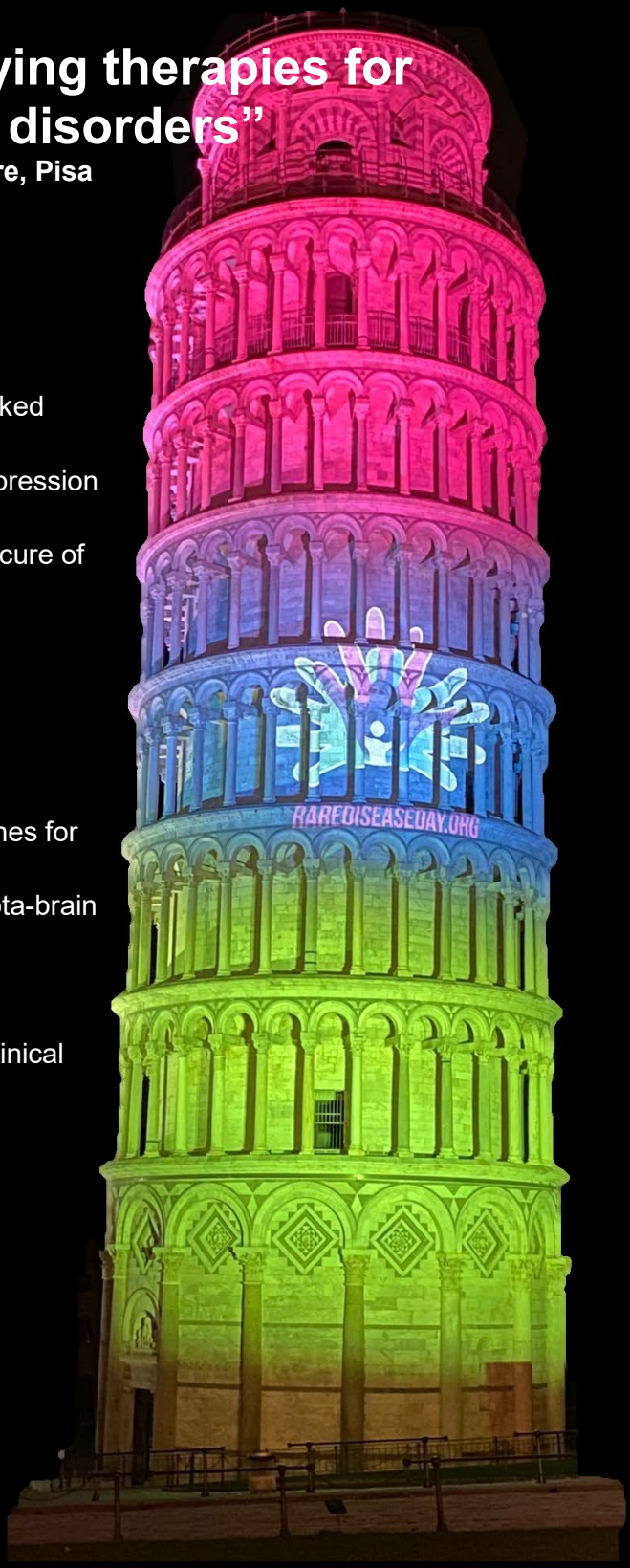
Workshop “Disease modifying therapies for neurodevelopmental disorders”

Scuola Normale Superiore, Pisa
August 31, 2023



I Session

Chair: **Stuart Cobb**

14.00 **Ralph Hector**, The tractability and challenges in the development of gene therapy in Rett syndrome

14.30 **Andrea Cerase** New targeted therapies to treat X-linked genetic disorders

15.00 **Paul Ross Single** gene circuit regulation of gene expression in Rett syndrome

15.30 **Laura Baroncelli** Gene replacement therapy for the cure of creatine transporter deficiency

16.00 coffee and poster session

II Session

Chair: **Marco Onorati**

17.00 **Sophie Thomson** Alternative gene therapy approaches for dosage sensitive and large genes

17.30 **Paola Tognini** the Exploring role of the gut- microbiota-brain axis in CDKL5 deficiency disorder

18.00 **Alessandro Gozzi** Biological subtyping of autism via cross-species fMRI

18.30 **Tommaso Pizzorusso** Novel tools for studying preclinical models of neurodevelopmental disorders.

19.00 end of meeting

Organizing committee: Laura Baroncelli, Tommaso Pizzorusso

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Poster Session

Microbiota manipulation as a potential therapeutic strategy to improve clinical symptoms in CDKL5 deficiency disorder

Francesca Damiani, Scuola Normale Superiore, francesca.damiani@sns.it

Zika virus induces FOXG1 displacement and downregulation in human neural progenitors: a possible cause of neurodevelopmental disorders

Beatrice D'Orsi, Neuroscience Institute, CNR, beatrice.dorsi@in.cnr.it

Modeling Pitt-Hopkins Syndrome and new pathogenetic variants of TCF4 by gene editing: a step forward toward personalized medicine (HOPeFOR)

Martina Orefice, Università di Pisa, martipih@gmail.com

Voluntary running ameliorates brain development and behavioral performance in a mouse model of CDKL5 deficiency disorder

Beatrice Uguagliati, Università di Bologna, beatrice.uguagliati@unibo.it

Cell fate decisions in Rent Syndrome: Boolean model of gene regulation network

Giuseppe Neri, Università di Pisa, g.neri19@studenti.unipi.it

Intranasal painless NGF rescues behavioral deficits and neuroinflammation in adult Mecp2+/- female mice

Giulia Borgonovo, Scuola Normale Superiore, giulia.borgonovo@sns.it

Mechanisms of synaptic dysfunction in the Angelman Syndrome

Federica Baronchelli, Humanitas University, federica.baronchelli@humanitasresearch.it

Pharmacological treatment with sphingosine analogues to correct sphingolipid metabolic imbalance in Rett syndrome models

Salvatore Fioriniello, Institute of Genetics and Biophysics "Adriano Buzzati Traverso", CNR, salvatore.fioriniello@igb.cnr.it

A Self-Regulating Gene Therapy for Rett Syndrome Gene transfer rescues audiogenic seizures in a mouse model of Fragile X Syndrome

Paul D Ross, Deanery of Biomedical Sciences, Edimburgh University, paul.ross@ed.ac.uk

Gene transfer rescues audiogenic seizures in a mouse model of Fragile X Syndrome.

Jim Selfridge, Deanery of Biomedical Sciences, Edimburgh University, stuart.cobb@ed.ac.uk