



10th SEPTEMBER 2024

HILTON ROME EUR LA LAMA Viale Europa, 287 00144 Rome, Italy



14:00-17.30

During the final meeting of the SCN1A-UP! project, funded by the 2020 EJPRD Program, the results of the collaborative research involving six European research groups will be presented and discussed. The overall objective of SCN1A-UP! has been to perform pre-clinical studies for developing more effective treatments for DS with a bottom up approach, targeting SCN1A loss of function, the primary cause of the disease, and pathologic remodeling that can develop at later stages, therapeutic concepts that could be applied also to other diseases.

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PROGRAMME

SCN1A - UP! FINAL MEETING

14:00-14:10 General introduction

Massimo Mantegazza - Institute of Molecular and Cellular Pharmacology, CNRS-Inserm-University Cote d'Azur, France

Simona Borroni - Gruppo Famiglie Dravet Aps

14:10-14:35 **Gene therapy approaches** Gaia Colasante - IRCCS San Raffaele Scientific

Institute, Italy

14:35-15:00 Repurposed drugs targeting secondary responses identified in Zebrafish

Danielle Copmans - University of Leuven, Belgium Eleonora Aronica - University of Amsterdam, The Netherlands

15:00-15:25 **Boosting a homeostatic** response as therapeutic approach

Massimo Mantegazza & Evgeniia Rusina - Institute of Molecular and Cellular Pharmacology, CNRS-Inserm-University Cote d'Azur, France

15:25-15:55 Break

15:55-16:20 iPS cell models for studying pathological mechanisms and selecting drugs

Nael Nadif Kasri - Radboud University, The Netherlands

16:20-16:45 Targeting secondary responses identified with transcriptomics in Dravet syndrome

Ulrike Hedrich & Stefano Iavarone - University of Tubingen, Germany

16:45-17:30 Scientific Round table

Discussion on overall results and their exploitation, ongoing and future collaborations and funding SCN1AUP! team

Rima Nabbout - Necker Hospital, France Lieven Lagae - University of Leuven, Belgium Patient Advocacy Organizations representatives



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Project funded by



Meeting realised with the support of











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