



Transformative Advances in Neuromuscular Treatment:

Next Generation Therapies

Outline Workshop AIM 2024

NOVEMBER 29, Hotel nhow Milan, Via Tortona 35

9:20 - 9:30 Introduction

Giacomo Pietro Comi (Milano) | AIM President

9:30 - 11:00 Basic tools

Chair: Stefania Corti (Milano), Michelagelo Mancuso (Pisa)

Andrea Bandin

The BioRobotics Institute and Department of Excellence in Robotics and AI, Scuola Superiore Sant'Anna, Pisa, Italy

Telemonitoring and digital technology to study neuromuscular disorders

Arianna Tucci

Medical Research Council, Queen Mary University of London, UK

Whole genomic sequencing in large populations and rare disorders:
the UK 100 K genome project

Irene Faravell

Department of Stem Cell & Regenerative Biology, Harvard University and Stanley Center for Psychiatric Research, Broad Institute of MIT and Harvard, Cambridge, MA, USA

Modelling individual variation to disease susceptibility using human brain Chimeroid

11:30 - 12.30 Therapeutics I

Chair: Adele D'Amico (Roma), Vincenzo Nigro (Napoli)

Scott Harper

Center for Gene Therapy Abigail Wexner Research Institute, Columbus, OH, USA AAV-gene therapy in neuromuscular disorders

Zoya Ignatova

Biochemistry and Molecular Biology, University of Hamburg, Hamburg, Germany RNA therapeutics for genetic diseases

14:00 - 15:30 Therapeutics II

Chair: Federica Ricci (Torino), Luca Bello (Padova)

Eugenio Maria Mercuri

Pediatric Neurology, Department of Woman and Child Health and Public Health, Child Health Area, Universita Cattolica del Sacro Cuore, and Centro Clinico Nemo Fondazione Policlinico Gemelli IRCCS, Rome, Italy

What next in DMD?

Valeria Sansone

The NEMO (NEuroMuscular Omniservice) Clinical Center, and University of Milan, Italy.

The new therapeutic wave in DM1: adaptive platform trial will do?

Lorenzo Maggi

Neuroimmunology and Neuromuscular Diseases Unit, Fondazione IRCCS Istituto Neurologico "Carlo Besta", Milan, Italy

Engaging new targets in myasthenia gravis

15:30 - 15:40 Concluding remarks

15:40 - 16:30 AIM General Assembly