

4th PISA MUSCLE AWARD

Multisystem involvement
in Neuromuscular Diseases

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Giuseppe Vergaro, *Pisa*

Sede evento
Palazzo della Sapienza | Via Curtatone e Montanara, 15, 56126 Pisa

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L'evento è stato accreditato dal Provider ECM EDU.co n.1357
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2 - Linee guida - protocolli - procedure

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4th PISA MUSCLE AWARD

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in Neuromuscular Diseases

13 - 14
novembre
2026

Aula Magna Storica
Palazzo della Sapienza
PISA

Responsabili
Scientifici

Michelangelo Mancuso
Giulia Ricci
Gabriele Siciliano

Con il patrocinio di



UNIVERSITÀ
DI PISA



4th
PISA

MUSCLE AWARD

Multisystem involvement
in Neuromuscular Diseases

RATIONALE

The 4th Pisa Muscle Award (PMA) will focus on the theme “Multisystem involvement in neuromuscular diseases”, expanding the perspective of previous editions, which mainly focused on basic pathways of muscle damage in neuromuscular disorders (NMD) such as muscle degeneration, muscle inflammation and muscle remodeling. Built up on new acquisition in this topic, this edition aims to explore the interactions between muscle, brain, heart and other organs, emphasizing those peculiarities of pathogenic molecular mechanisms for extramuscle manifestations of NMD, among which cell structural damage, metabolism derangement, mitochondrial dysfunction and RNA toxicity. Particular attention will be devoted to the identification of multisystem biomarkers for diagnosis, prognosis and especially treatment monitoring, emphasizing those clinical challenges posed by systemic involvement in NMD. International experts in clinical and translational research will present the latest advances, including precision medicine and innovative therapies. By fostering interdisciplinary collaboration, the aim is to bridge pathophysiology and clinical practice, improving outcomes for patients with neuromuscular diseases. The 2026 edition of the Pisa Muscle Award will be granted on behalf of the AIM (Associazione Italiana di Miologia).

Friday 13 November

14:45 - 15:00 Welcome and Introduction
V. Nigro and G. Siciliano

Session 1 - Brain Involvement in Neuromuscular Diseases
Chairs: *T. Mongini, A. Toscano*

15:00 - 15:20 CNS involvement in Pompe disease:
from glycogen storage to neurodegeneration
O. Musumeci

15:20 - 15:40 Neurological manifestations of mitochondrial diseases: beyond myopathy
C. Lamperti

15:40 - 16:00 Sleep-cognitive features in myotonic dystrophies
V. Sansone

16:00 - 16:20 Brain-related comorbidities in congenital muscular dystrophies
A. Berardinelli

16:20 - 16:40 Cognitive and neurodevelopmental disorders in spinal muscular
atrophy type I at the time of disease-modifying therapies
F. Ricci

16:40 - 17:00 Brain pathology in Duchenne muscular dystrophy:
the dark side of the moon
L. Bello

17:00 - 17:20 **Coffee break and poster views | NO CME Session**

Session 2 - Therapeutic corner
Chairs: *E. Procopio, G. Ricci*

17:20 - 17:40 Comparative treatment strategies towards motorneuron rescue in SMA
C. Bruno

17:40 - 18:00 Thymidine kinase 2 deficiency (TK2d): a treatable mitochondrial disease
T. Klopstock

18:00 - 18:20 Mitochondrial medicine and treatments
A. Karaa

18:20 - 18:40 Lysosomal diseases: when CNS is the odd player
M. Filosto

18:40 - 19:00 Targeting CNS with AAV gene therapy in NMD
G.P. Comi

NO CME Session

19:00 - 19:30 **Pisa Muscle Award Ceremony** - Chair: *C. Angelini, G. Siciliano*
**Winner Lecture - Muscle Channelopathies: New advancements
in diagnosis and treatment**

Saturday 14 November

08:30 - 09:30 **Breakfast with poster view | NO CME Session**
Chair: *D. Baldessari, R. Battini*

09:30 - 10:00 **Key note lecture:** Tissue-specificity of myopathy pathomechanisms
signatures in organoid models
S. Tedesco
Introduction: *M. Mancuso*

Session 3 - Heart involvement in Neuromuscular Diseases
Chairs: *M. Emdin, L. Politano*

10:00 - 10:20 Laminopathies: a paradigm of cardio-muscular disease
G. Lattanzi

10:20 - 10:40 Titinopathies: genotype-phenotype correlations
A. Fornaro

10:40 - 11:00 Autonomic nervous system involvement in CIDP
V. Donadio

11:00 - 11:20 Prevalence and prognostic issues of cardiac involvement
in myotonic dystrophy 1
V. Russo

11:20 - 11:40 **Coffee Break**

Session 4 - Therapeutic corner
Chairs: *A. Barison, S. Servidei*

11:40 - 12:00 Treatment challenges tailoring heart in Duchenne muscular dystrophy
R. Adorisio

12:00 - 12:20 Advances in the treatment of myasthenia gravis
M. Maestri Tassoni

12:20 - 12:40 aTTR amyloidotic cardiomyo-polyneuropathy:
current treatments in real word
G. Vergaro

12:40 - 13:00 Registry and biomarkers for new personalised medicine
H. Prokisch

13:00 - 13:30 **Poster winner prize, greetings and conclusions**
NO CME Session
Presenting: *M. Mancuso, G. Ricci, G. Siciliano*