

Mitochondrial Medicine. The GENOMIT initiative

Mitochondrial disorders are now considered the most common forms of inherited human disease. In the recent years, amazing progress has been obtained in terms of pathophysiology, natural history and new treatments options for these diseases.

This conference, based on the experience of the GENOMIT consortium, funded by the European Joint Programme on rare diseases, aims to bring different international groups together, exploring the most recent advances in term of natural histories, artificial intelligence, clinical trial readiness and emerging treatment options in preclinical and clinical models of mitochondrial diseases.

with the unconditional support of

Aliveda  **Blueprint Genetics**

 **Tisento** THERAPEUTICS  **Pharma Nord**

 **Reneo** Pharmaceuticals  **KHONDRION**
The Mitochondrial Medicine Company 

REGISTRATIONS	until 01/09/2023	from 02/09/2023
Students - Residents - Post Doc	FREE	FREE
E-mit members	FREE €	50,00 €
NON E-mit members	100,00 €	150,00 €
PHARMA and STAKEHOLDERS	150,00 €	250,00 €

Registration link: genomit.myquadra.it

Registrations fees include: Access to the conference, Coffee breaks, congress KIT and certificate of attendance. Dinner not included, VAT included
The event is for maximum 70 participants and once this number of participants will be reached, registrations will be closed.

Course Venue

Aula Magna, Scuola Superiore Sant'Anna
Piazza Martiri della Libertà 33
56127 - Pisa - Italy

Patronages



European Reference Network
for rare or low prevalence complex diseases

Network
Neuromuscular Diseases (ERN EURO-NMD)

Coordinator
Assistance Publique - Hôpitaux de Paris, Hôpital Pitié-Salpêtrière - France

Comune di Pisa

Organising secretariat

 **FIRST CLASS**
EVENTS & CONFERENCES

First Class - Meetings and Conferences
Via Vittoria Colonna, 40 - 20149 Milano Italy
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Mitochondrial Medicine. The GENOMIT initiative

Pisa, November 17 - 18, 2023
Aula Magna, Scuola Superiore Sant'Anna



Mitochondrial Medicine. The GENOMIT *initiative*

Pisa, November 17 - 18, 2023

Scientific Committee:

Prof. Michelangelo Mancuso
Prof. Gabriele Siciliano
Prof. Silvestro Micera
Dr.ssa Giulia Ricci
Dr. Vincenzo Montano
Dr. Piervito Lopriore
Dr.ssa Adriana Meli

Faculty:

Andrea Bandini
Valerio Carelli
Patrick Chinnery
Fang Fang
Amel Karaa
Thomas Klopstock
Costanza Lamperti

Serena Massucci
Robert McFarland
Kei Murayama
Yi Ng
Holger Prokisch
Valeria Tiranti
Carlo Viscomi

November 17th

13.30 Registration

14.00 Greetings and welcome

Silvia Briani (Pisa)
Direttrice generale dell'Azienda ospedaliero-universitaria Pisana

Michele Emdin (Pisa)
Professore Ordinario presso Scuola Superiore Sant'Anna, Pisa

Gabriele Siciliano (Pisa)
Direttore della Clinica Neurologica dell'Azienda Ospedaliero Universitaria Pisana

Riccardo Zucchi (Pisa)
Rettore dell'Università di Pisa

14.30 - 17.30 Session 1. The GENOMIT Journey.

Chairs: *Holger Prokisch (Munich), Costanza Lamperti (Milan)*

14.30 The global registry on mitochondrial diseases. Where we stand.
Thomas Klopstock (Munich)

15.00 Advances in Multi-Omics Diagnostics.
Holger Prokisch (Munich)

15.30 Advances in natural history studies. Focus on MELAS
Costanza Lamperti (Milan)

16.00 Advances in natural history studies. Focus on MERRF
Yi Ng (Newcastle)

16.30 Advances in natural history studies. Focus on LHON and its treatment
Valerio Carelli (Bologna)

17.00 Advances in natural history studies. Focus on Leigh Syndrome
Robert McFarland (Newcastle)

17.30 Q&A

18.00 Closing day 1

November 18th

08.30 - 10.15 Challenging the future of mitochondrial medicine

Chairs: *Patrick Chinnery (Cambridge), Michelangelo Mancuso (Pisa)*

08.30 The role of mtDNA variation in common diseases.
Patrick Chinnery (Cambridge)

09.00 The role of AI in mitochondrial medicine.
Andrea Bandini (Pisa)

09.30 The global approach to mitochondrial medicine through GENOMIT
Fang Fang (China)

10.00 Q&A

10.15 - 10.30 Break

10.30 - 12.00 The new era of clinical trial readiness

Chairs: *Amel Karaa (Boston), Kei Murayama (Tokio)*

10.30 Exploring new treatments for MELAS. The experience of the Japanese Network
Kei Murayama (Tokio)

11.00 Selection, outcome measures and endpoints in PMDs clinical trials
Amel Karaa (Boston)

11.30 Developing Gene therapy for mitochondrial diseases
Carlo Viscomi (Padova)

12.00 - 13.00 The voice of the industries:

Chairs: *Michelangelo Mancuso (Pisa), Valeria Tiranti (Milano)*

Nourishing the Future: Aliveda Nutraceutical Solutions
Lucia Chico - Laboratori Aliveda

Clinical Trial Update
Madhu Davies - Reneo Pharma Ltd

Nupower Update
Gene Kelly - Stealth Biotherapeutics

An Introduction to Tisento Therapeutics and the Zagociguat MELAS Program
Chad Glasser - Tisento Therapeutics

Accredited pipeline for identification of genetic variants in mitochondria
Massimiliano Gentile - Blueprint Genetics

13.00 The patients perspective
Serena Massucci (MITOCON, Italy)

13.30 Q&A

14.00 Closing the meeting and light lunch