Mitocon

Insieme per lo studio e la cura
delle malattie mitocondriali

10th Italian Meeting on Mitochondrial Diseases

October 9 - 10, 2020

Virtual edition

Organised by Mitocon - Insieme per lo studio e la cura delle malattie mitocondriali Onlus

WITH THE SUPPORT OF:

FONDAZIONE Telethon

MITOCHONDRIAL MEDICINE SOCIETY

UNIAMO FEDERAZIONE ITALIANA MALATTIE RARE Rare Diseases Italy

SIMMESN

Sin SOCIETÀ ITALIANA DI NEUROLOGIA

AND WITH THE SUPPORT OF THE ITALIAN NATIONAL HEALTH INSTITUTE

Scientific programme committee

Enrico Bertini, Neuromuscular and Neurodegenerative Disorders, Bambino Gesù Children's Research Hospital, Rome

Valerio Carelli, IRCCS Institute of Neurological Sciences (ISBN), Bellaria Hospital, Bologna/University of Bologna

Massimiliano Filosto, NeMO Clinical Center-Brescia for Neuromuscolar Diseases; Department of Clinical and Experimental Sciences, University of Brescia

Costanza Lamperti, Division of Medical Genetics and Neurogenetics, Mariani Foundation Center for the Study of Mitochondrial Pediatric Diseases, Foundation IRCCS Neurological Institute "C. Besta", Milan

Michelangelo Mancuso, AOUP, Neurological Institute, University of Pisa

Olimpia Musumeci, AOU Policlinico "G.Martino", University of Messina

Serenella Servidei, Neurology Institute, Catholic University of the Sacred Heart, Rome

Valeria Tiranti, Division of Medical Genetics and Neurogenetics, Mariani Foundation Center for the Study of Mitochondrial Pediatric Diseases, Foundation IRCCS Neurological Institute "C. Besta", Milan

Massimo Zeviani, Department of Neurosciences, University of Padua

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FRIDAY OCTOBER 9

9:30 am- 10:00 am (CEST TIME)

Opening: P. Santantonio, President Mitocon Onlus
Video: patients' experiences

Session 1:
Accelerating high tech involvement in daily practice

Chairmen:

O. Musumeci, AOU Policlinico"G. Martino", University of Messina

M. Filosto, NeMO Clinical Center-Brescia for Neuromuscolar Diseases; Department of Clinical and Experimental Sciences, University of Brescia

10:00 am - 10:30 am

Artificial intelligence in the diagnosis of mitochondrial disease, H. Prokisch, Technical University Munich

10:30 am - 11:00 am

Telemedicine during Covid-19, C. Lamperti, Division of Medical Genetics and Neurogenetics, Mariani Foundation Center for the Study of Mitochondrial Pediatric Diseases, Foundation IRCCS Neurological Institute "C. Besta", Milan

11:00 am - 11:30 am

Epigenetics of pregnancy: when DNA is not the boss, D. Zuccarello, Department of Lab Medicine, Unit of Clinical Genetics and Epidemiology, University Hospital of Padua

11:30 am - 12:00 am

Overview of biological biomarkers. A. Suomalainen, Department of Neurosciences, University of Helsinki

12:00 pm - 12:30 pm

Questions & answers

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Session 2: Current trials and industry – flash presentation part I

Chairmen:

V. Carelli, IRCCS Institute of Sciences, Neurological (ISBN), Bellaria Hospital, Bologna/University of Bologna

E. S. Bertini, Neuromuscular and Neurodegenerative Disorders, Bambino Gesù Childrens Research Hospital, Rome

12:30 pm - 12:50 pm

PTC743 Mitochondrial Epilepsy Trial Update, M. Klein, Global Head Gene and Mitochondrial Therapies, PTC Therapeutics.

12:50 pm - 1:10 pm

Abliva's innovative drug development programs for primary mitochondrial diseases – status update and goals. M. Hansson, Chief Medical Officer, Vice President Preclinical & Clinical Development, Abliva

1:10 pm - 1:30 pm

MAT: Minovia's journey with a Personalized Therapy- A. Gill, Head of Medical EU, Minovia.

1:30 pm - 2.30 pm

Break

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Session 2: Current trials and industry – flash presentation part II

Chairmen:

V. Carelli, IRCCS Institute of Sciences, Neurological (ISBN), Bellaria Hospital, Bologna/University of Bologna

E. S. Bertini, Neuromuscular and Neurodegenerative Disorders, Bambino Gesù Childrens Research Hospital, Rome

2:30 pm - 2:50 pm

Lumevoq Gene Therapy in Leber Hereditary Optic Neuropathy (LHON), M. Taiel, MD Chief Medical Officer, GenSight Biologics

2:50 pm - 3:10 pm

Highlights on the state of the art of Idebenone therapy in LHON, X. Llòria, Global Program Leader Raxone, Santhera Pharmaceuticals

3:10 pm - 3:30 pm

Overview of REN001 Clinical Development Program, A. Dorenbaum, Chief Medical Officer, Reneo Pharmaceuticals.

3:30 pm - 3:50 pm

Khondrion's Sonlicromanol: progress update, J. Smeitink, CEO Khondrion

3:50 pm - 4:10 pm

Questions & answers

4:10 pm - 4:20 pm

Break

4:20 pm - 4:25 pm

Special greetings from Salvatore Di Mauro

4:25 pm - 5:00 pm

Special lecture: the mitochondrial journey. Celebrating the 80th years of Billi Di Mauro. M. Zeviani, Department of Neurosciences, University of Padua

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Session 3: Gene therapy and editing

Chairmen:

M. Zeviani, Department of Neurosciences, University of Padua

C. Lamperti, Division of Medical Genetics and Neurogenetics, Mariani Foundation Center for the Study of Mitochondrial Pediatric Diseases, Foundation IRCCS Neurological Institute "C. Besta", Milan

5:00 pm - 5:30 pm

Changing mtDNA heteroplasmy with DNA editing enzymes, C. T. Moraes, University of Miami Miller School of Medicine.

5:30 pm - 6:00 pm

Experimental models for mtDNA maintenance defects. C. Viscomi, Department of Biomedical Sciences, University of Padua

6:00 pm - 6:30 pm

Treatment of mitochondrial diseases with AAV: the proposal of gene therapy for MNGIE, R. Martí, Research group on neuromuscular and mitochondrial diseases, Vall d'Hebron Research Institute (VHIR), Barcelona

6:30 pm - 7:00 pm

A bacterial cytidine deaminase toxin enables CRISPR-free mitochondrial base editing, B. Y. Mok, Department of Chemistry and Chemical Biology, Harvard University, Cambridge MA

7:00 pm - 7:30 pm

Questions & answers

7:30 pm

Closing: P. Santantonio, President Mitocon Onlus

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SATURDAY OCTOBER 10

Session 4:
Mitochondrial disease, natural history and cohorts

Chairmen:

E. S. Bertini, Neuromuscular and Neurodegenerative Disorders, Bambino Gesù Childrens Research Hospital, Rome

S. Servidei, Neurology Institute, Catholic University of the Sacred Heart, Rome

9:00 am - 9:15 am (CEST TIME)

Opening: P. Santantonio, President Mitocon Onlus
Video: patients' experiences

9:15 am - 9:45 am

Outcome measures in primary mitochondrial myopathies. M. Mancuso, AOUP, Neurological Institute, University of Pisa

9:45 am - 10:15 am

LHON - role model for mitochondrial therapies, T. Klopstock, Department of Neurology, University of Munich

10:15 am - 10:45 am

Clinical course of MELAS. Can we predict it?, R. McFarland, Wellcome Trust Centre for Mitochondrial Research

10:45 am - 11:15 am

Questions & answers

11:15 am - 11:30 am

Break

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Session 5: Pathophysiology and models of mitochondrial dysfunction

Chairmen:

V. Tiranti, Division of Medical Genetics and Neurogenetics, Mariani Foundation Center for the Study of Mitochondrial Pediatric Diseases, Foundation IRCCS Neurological Institute "C. Besta", Milan

M. Mancuso, AOUP, Neurological Institute, University of Pisa

11:30 am - 12:00 am

Inflammation and mitochondrial diseases. D. Martinelli, Genetics and Rare Diseases Research Division, Unit of Metabolism, Bambino Gesù Children's Research Hospital, Rome

12:00 pm - 12:30 pm

iPS models for OPA1 and POLG associated with parkinsonism. V. Broccoli, CNR - National Research Council, Institute of Neuroscience, Milan

12:30 pm - 1:00 pm

Inhibition of autophagy corrects the visual defects in a model of ADOA, L. Scorrano, Department of Biology & VIMM, University of Padua

1:00 pm - 1:30 pm

Questions & answers

1:30 pm

Closing: P. Santantonio, President Mitocon Onlus

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