

*Il futuro della ricerca terapeutica
per curare la malattia di Huntington*

2021 CONVEGNO ANNUALE LIRH

ONLINE

3 DICEMBRE 2021 - H 15.00-17.30 CET

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PROGRAM

15.00-15.20

WELCOME AND KEYNOTE SPEECH

BARBARA D'ALESSIO

LIRH FOUNDATION

SIMONETTA MASSAFRA

CSS MENDEL INSTITUTE/IRCCS CASA SOLLIEVO DELLA SOFFERENZA

FERDINANDO SQUITIERI

CSS MENDEL INSTITUTE/IRCCS CASA SOLLIEVO DELLA SOFFERENZA

15.20-17.00

UPDATES ON CLINICAL TRIALS

BETH BOROWSKY

NOVARTIS

ANU BHATTACHARYYA

PTC THERAPEUTICS

DANIEL LEONARD AND SARA YING

UNIQURE

MICHAEL A. PANZARA

WAVE LIFE SCIENCES

MICHAEL R. HAYDEN

PRILENIA

17.00-17.30

Q&A - FINAL REMARKS

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**Barbara D'Alessio**

Presidente e CEO della Fondazione LIRH, laurea in giurisprudenza all'Università Sapienza di Roma, Master in Marketing e Master in Patients Advocacy Management presso l'Università Cattolica di Roma, ha oltre 20 anni di esperienza nel settore non profit. E' attiva nella Huntington Community dal 2012.

President and CEO of the LIRH Foundation, graduated in law from the Sapienza University of Rome, Master in Marketing and Master in Patients Advocacy Management from the Cattolica University in Rome, has over 20 years of experience in the non-profit sector. She has been active in the Huntington Community since 2012.

**Simonetta Massafra**

Responsabile UOC Ricerca Clinica Istituto Mendel IRCSS Casa Sollievo della Sofferenza
Direttore Sanitario Istituto CSS-Mendel di Roma.

*Head of Clinical Research Unit at Istituto Mendel - IRCSS Casa Sollievo della Sofferenza
Chief Medical Officer at Istituto Mendel - IRCSS Casa Sollievo della Sofferenza*

**Ferdinando Squitieri**

Neurologo, con un dottorato di ricerca in neurobiologia. Si occupa di malattia di Huntington, anche nella sua forma giovanile, da oltre 30 anni. E' principal investigator di tutti gli studi clinici sull'Huntington svolti in Italia.
Neurologist with a PhD in Neurobiology.

He has been studying and treating patients with HD and juvenile onset HD (JoHD) over the last 30 years. Dr Squitieri is Principal Investigator of all the therapeutic and observational HD studied being conducted in Italy.

**Michael R. Hayden**

Medico e scienziato di enorme esperienza, è Presidente e amministratore delegato di Prilenia Therapeutics. È Killam Professor e Senior Scientist presso il Center for Molecular Medicine and Therapeutics dell'Università della British Columbia (UBC), Canada. Il Dr. Hayden è l'autore più citato al mondo su ABCA1 e malattia di Huntington.

Dr. Michael R. Hayden, accomplished physician and scientist, is President and CEO of Prilenia Therapeutics. He is a Killam Professor at the University of British Columbia and Senior Scientist at the Center for Molecular Medicine and Therapeutics. He is also a Canadian Research Chair in Human Genetics and Molecular Medicine. Dr. Hayden is the world's most cited author on ABCA1 and Huntington's disease.

**Michael A. Panzara**

Direttore Medico e Capo della Ricerca e Sviluppo di Wave Life Sciences, ha più di 20 anni di esperienza nello sviluppo di terapie per i disturbi neurologici. Laureato in medicina alla Stanford University, si è specializzato presso il Massachusetts General Hospital, il Brigham and Women's Hospital e la Harvard School of Public Health.

Chief Medical Officer, Head of Therapeutics Discovery and Development at Wave Life Sciences, Dr. Panzara has over 20 years of experience developing therapies for neurological disorders. He received the medical degree from Stanford University, trained in neurology at Massachusetts General Hospital with post-doctoral training in immunology and rheumatology at Brigham and Women's Hospital, and received his Master of Public Health degree from the Harvard School of Public Health.

**Anu Bhattacharyya**

Executive Director Biology & NRD at PTC Therapeutics. Ha un dottorato di ricerca in Microbiologia presso la Columbia University e un post doc in Biologia Molecolare presso il National Institutes of Health. Ha condotto programmi di scoperta di farmaci per l'Alzheimer, il Parkinson, l'atrofia muscolare spinale (SMA), la malattia di Huntington e altre malattie a ripetizione di nucleotidi (NRD).

Executive Director Biology & NRD at PTC Therapeutics. She received her Ph.D. in Microbiology from Columbia University and completed a postdoc in Molecular Biology at the National Institutes of Health before joining PTC. As a project leader of multiple CNS projects, she has run drug discovery programs for Alzheimer's, Parkinson's, Spinal Muscular Atrophy (SMA), Huntington's Disease & other nucleotide repeat diseases (NRDs).

**Beth Borowsky**

Executive Director e Senior Global Program Clinical Head in Neuroscience presso Novartis Pharmaceutical Corporation dal 2018. Ha una lunga esperienza nello sviluppo clinico in diverse aziende farmaceutiche e una formazione in ambito psicologico, farmacologico e medico.

Executive Director and Senior Global Program Clinical Head in Neuroscience at Novartis Pharmaceutical Corporation, where she has been since 2018. Beth received a BS in Psychology and Neurobiology from Rutgers University, and a PhD in Pharmacology from Duke University Medical Center. She completed her post-doctoral training at the National Institute for Mental Health.

**Daniel Leonard**

E' il direttore senior di Global Patient Advocacy presso uniQure. Lavora a stretto contatto con le organizzazioni dei pazienti, sostenendo i loro bisogni. Ha oltre 21 anni di esperienza nel settore delle biotecnologie.

Senior Director of Global Patient Advocacy at uniQure. Dan works closely with Huntington's disease patient organizations. He serves as an ambassador to the patient community and as an internal advocate for patients' interests. Dan has spent over 21 years working in the biotech industry. Prior to joining uniQure, he spent 16 years at Genzyme.

The upcoming therapies to treat Huntington Disease

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DECEMBER 3, 2021 - H 15.00-17.30 CET

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