



Agenda

Renaissance Marriott in Harrison, NY
Co-Chairs: Darryl DeVivo, MD & Paola Leone, PhD

Saturday, June 4th

- 8:30 – 8:45am Welcome and Opening Remarks - Jordana Holovach, Jacob's Cure, NY
- 8:45 - 9:10am Morris Baslow, Ph.D.
The structural unit of the brain is the site of Canavan Disease pathology
- 9:10 - 9:35am Emile Van Schaftingen, PhD
Molecular identification of enzymes involved in the synthesis and breakdown of neurometabolites
- 9:35 - 10:00am Maria Traka, Ph.D.
Development of an vitro approach to identify molecular pathways of Canavan Disease
- 10:00 -10:25am Paola Leone, Ph.D.
Pathological Modeling of Canavan Disease and potential for Stem Cell Therapy
- 10:25 - 10:50am Coffee Break
- 10:50 - 11:15am Klaus-Armin Nave, PhD
Glial support of axonal energy metabolism - a role for NAA?
- 11:15 - 11:40am Patrizia Casaccia, Ph.D.
Protein acetylation in glial differentiation and neuronal function

Saturday, June 4th (Cont'd)

- 11:40 - 12:05pm John Moffett, Ph.D.
Histone hyperacetylation in the tremor rat model of Canavan disease is reversed by metabolic acetate therapy

- 12:05 -12:30pm Ronald Viola, Ph.D.
Exploring Enzyme Replacement as a Possible Therapy for Canavan Disease
- 12:30 - 1:45pm Lunch Break
- 1:45 - 2:10 pm Danilo Tagle, Ph.D.
Translational Research – Bridging the valley of Death
- 2:10 - 2:35 pm Heather Lau, M.D.
Diagnosing Canavan Disease: Clinical and Laboratory Dilemmas
- 2:35 - 3:00 pm Jean De Vellis, Ph.D.
ASPA gene mutation impacts epigenetic regulation involved in development of neural cells and their function in an animal model
- 3:00 - 3:25pm Gao Guangping, Ph.D.
Efficacious and safe preclinical Gene Therapy of Canavan disease: a scientific fiction or reality
- 3:25 - 3:45 pm Coffee Break
- 3:45 - 4:10 pm Lidia DeFilippis
Therapy of neurodegenerative diseases with neural stem cells: the challenge of Lysosomal Storage Diseases
- 4:10 - 4:30pm Maria Escolar, M.D.
Timing: The key to differential response to therapy in neurodegenerative conditions of childhood
- 4:30 - 4:55pm Ed Wirth, M.D., Ph.D.
Human Embryonic Stem Cell-Derived Oligodendrocyte Precursors for the Treatment of Neurodegenerative Diseases
- 4:55 - 5:20pm Evan Synder, M.D., Ph.D.
The intriguing role that gap junction coupling might play in stem cell-mediated rescue of degenerating neural cells
- 5:20 - 5:45 pm Coffee Break

Saturday, June 4th (Cont'd)

- 5:45 - 6:10 pm Yanhong Shi, Ph.D.
Studying Canavan Disease using an iPSC approach

- 6:10 - 6:35pm Dolan Sondhi, Ph.D.
Gene Therapy for the CNS Manifestations of Hereditary Disorders
- 6:35 - 7:00pm Bryan Delaney, PhD
Dietary Toxicology Studies Conducted in Rats with N-Acetylaspartic Acid

Sunday, June 5th

8:30- 9:30 am Breakfast with the families and patients (Uniting Scientists & Clinicians)

9:30am – 12:00pm Poster Session & Meeting with families

Required Departure: Sunday at noon or later (Car service available)

Poster Session

Prasanth Ariyannur, Ph.D.

title: TBA

Jeremy Francis, Ph.D.

title: TBA

Christopher Janson, Ph.D.

title: TBA

Shalini Kumar, Ph.D.

Molecular and Biochemical Characterization of observed CNS Developmental Alterations in a mouse model of Canavan disease

Wendong Li, Ph.D

title: TBA

Laura Rota Nodari, Ph.D

Characterization of GMP grade Human Neural Stem Cell lines

Elsa Wiame, Ph.D

Molecular dissection of the enzyme that synthesizes N-acetylaspartate