

Day 1 | Thursday, November 19, 2015

8:00 Registration Open, *Metropolitan East/West at The Liaison Hotel*
8:30 Welcome – Amy Akers, Angioma Alliance

SESSION I | PATHOBIOLOGY OF CCM PART 1 – LESION GENESIS & VASCULAR BIOLOGY

Moderator – Doug Marchuk, Duke University

8:45 *micro-CT Imaging of Cerebral Cavernous Malformations (CCM) in Mouse Models*
Xiangjian Zheng, Sydney Medical School, University of Sydney

9:05 *Nogo-B receptor deficiency causes cerebral cavernous malformations-like defects during blood vessel development in mice*
Robert Miao, Children's Research Institute, Medical College of Wisconsin

9:25 *Toward a unifying pathogenic mechanism for CCM disease: Identification of defective autophagy as a key feature of CCM disease pathogenesis*
Eliana Trapani, University of Torino

9:45 COFFEE BREAK

10:00 *Regulation of B1 integrin-Klf2-mediated angiogenesis by CCM proteins*
Cecile Otten, University of Potsdam

10:20 *KLF4 is a key determinant of development and progression of Cerebral Cavernous Malformations*
Noemi Rudini, FIRC Institute of Molecular Biology

10:40 *CCM1/2 complex controls endothelial integrity by regulating the balance between ROCK1 and ROCK2 activities*
Eva Faurobert, INSERM U823, Grenoble

11:00 Discussion

11:45 **LUNCH**, *The Hill at The Liaison Hotel*
INVITED TALK – *Orphan Drug Regulatory Process in the US*
Timothy Coté, Coté Orphan LLC

SESSION II | DRUG DEVELOPMENT & CLINICAL TRIALS: PLANNING, PROGRESS AND UPDATES

Moderator – Elisabeth Tournier-Lasserre, INSERM Paris

1:20 *Development of BA-1049 for treatment of recurrent bleeding in CCM*
Lisa McKerracher, BioAxone BioSciences Inc

1:40 *Propranolol treatment of cavernous malformations with symptomatic hemorrhage*
M. Yashar Kalani, Barrow Neurological Institute

2:00 *Randomized simvastatin trial in CCM1-CHM*
Leslie Morrison & Blaine Hart, University of New Mexico

2:20 *Atorvastatin Therapy in Cerebral Cavernous Malformation (AT-CCM): A Proof of Concept Phase I/II Trial*
Maged D. Fam, University of Chicago

2:40 *Vitamin D3 and Tempol for CCM - an Update*
Christopher Gibson, Recursion Pharmaceuticals

3:00 Discussion

3:20 COFFEE BREAK

3:30 **INVITED TALK – Fasudil as a Novel Therapeutic for Parkinson's Disease**
Jeff MacKeigan, Van Andel Research Institute

4:15 **END OF DAY 1 REGULAR SESSIONS**

POSTER SESSION & WELCOME DINNER | JOHNNY'S HALF SHELL RESTAURANT

JOHNNY'S HALF SHELL – 400 NORTH CAPITOL STREET, NW, WASHINGTON, DC

5:30 *Endothelial cells derived from induced-pluripotent stem (iPS) cells as a model for CCM*
Adriana Beltran, University of North Carolina at Chapel Hill

Development of ROCKII selective BA-1049 for treatment of cerebral cavernous malformations
Lisa Bond & Thomas Carsillo, BioAxone Biosciences

Origin of the Common Hispanic Q455X/KRIT1 Founder Mutation Causing Cerebral Cavernous Malformation Type 1
Hélène Choquet, University of California San Francisco

Familial Cerebral Cavernous Angiomas: molecular diagnostic improvement through MLPA
Maria Sole Cigoli, Niguarda Ca' Granda Hospital

Targeting Vascular Malformations Using the Zebrafish as a Discovery Model
Joanne Chan, Hampton University

The C. elegans CCM-3 regulatory network
Benjamin Lant, The Hospital for Sick Children

Inhibition of Rho-kinase in CCM3+/- mice suggests involvement of CCM3 in the Rho-kinase pathway during disease progression
Autumn Rorrer, Duke University Medical Center

A cerebral Cavernous Malformation Phenotyping and Human Tissue Research Core Unit
Robert Shenkar, University of Chicago

Endothelial cell genetic disruptions and CCM pathology
Taline Shishoian, University of California Los Angeles

Vascular Permeability and Quantitative Susceptibility Magnetic Resonance Imaging as Biomarkers of Clinical Activity in Longitudinal Follow-up of CCM Patients
Hussein Zeineddine, University of Chicago

6:30 **DINNER, Johnny's Half Shell Restaurant**

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PREVENTION GENETICS
DISEASE PREVENTION THROUGH GENETIC TESTING

Day 2 | Friday, November 20, 2015

8:00 *Angioma Alliance Updates* - Connie Lee, Angioma Alliance

SESSION III | PATHOBIOLOGY OF CCM PART 2 – SIGNALING

Moderated by Anne-Claude Gingras, Lunenfeld-Tanenbaum Research Institute

8:15 *Recent Structural Studies on CCM Proteins*
Titus Boggon, Yale University School of Medicine

8:35 *Heart of Glass is a Physical Nexus for Rap1 Signaling in Cardiovascular Function*
Bart-Jan de Kreuk, University of California San Diego

8:55 *ICAP1/KRIT1 Signaling Regulation Through Cellular Localization*
Kyle Draheim, Yale University

9:15 Discussion

9:30 COFFEE BREAK

SESSION IV | CLINICAL STUDIES

Moderated by Leslie Morrison, University of New Mexico

9:40 *Automated Small Lesion Counting Algorithm for Cerebral Cavernous Malformation*
Xiaowei Zou, University of California San Francisco

10:00 *Spinal cord cavernous malformations in a CCM1 population*
Mary Bartlett & Blaine Hart, University of New Mexico

10:20 *Seasonal Variation in ICH/FND in Patients with Intracerebral cavernous malformation*
Kelly Flemming, Mayo Clinic

10:40 *Peripheral Plasma Vitamin D and Non-HDL Cholesterol Reflect the Severity of Cerebral Cavernous Malformation Disease*
Romuald Girard, University of Chicago

11:00 Discussion

11:20 **LUNCH & TRAINEE NETWORKING SESSION**, *The Hill at The Liaison Hotel*

12:45 **INVITED TALK** – *From CCM proteins to preclinical and clinical trials*
Elisabeth Tournier-Lasserre, INSERM Paris

SESSION V | DISCUSSION PANEL

How Animal Models and Cell-Specific Data are Informing Therapeutic Approaches

Moderator – Issam Awad, MD

1:30 Mark Kahn – *Introduction and Overview*

1:40 Kevin Whitehead – *Role of endothelial cells*

1:50 Angeliki Louvi – *Role of neural cells*

2:00 Joanne Chan – *Role of pericytes*

2:10 Brent Derry – *Role of nonautonomous signaling*

2:20 Open Discussion

3:00 **END OF 2015 CCM SCIENTIFIC MEETING**