



# **ANGIOMA** **ALLIANCE**

*Presents the 12<sup>th</sup> Annual*

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## **CCM SCIENTIFIC MEETING**

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**THE DUPONT CIRCLE – WASHINGTON, DC**

**NOVEMBER 10<sup>TH</sup> & 11<sup>TH</sup>, 2016**

## Day 1 | Thursday, November 10, 2016

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8:30 Registration & Continental Breakfast, Glover Park Ball Room

8:50 Welcome & Opening Remarks

### SESSION I | LESION GENESIS & THERAPEUTIC TARGETS

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Moderator – Doug Marchuk, Duke University

9:00 *High-throughput screening: An approach for potential therapies for CCM*  
Angeliki, Louvi, Yale School of Medicine, New Haven, CT

9:20 *KRIT1 loss-of-function sensitizes cells to oxidative stress- and inflammation-mediated molecular and cellular dysfunctions by affecting major redox-sensitive mechanisms: towards a unifying mechanistic scenario for CCM disease pathogenesis and treatment*  
Francesco Retta, University of Torino, Torino, Italy

9:40 *Cerebral cavernous malformations arise from endothelial gain of MEKK3-KLF2/4 signaling*  
Alan Tang, University of Pennsylvania, Philadelphia PA

10:00 *Augmented exocytosis and angiopoietin-2 secretion contribute to cerebral cavernous malformations*  
Huanjiao Jenny Zhou, Yale University School of Medicine, New Haven, CT

10:20 **DISCUSSION**

10:40 **DISCUSSION & BREAK**

### SPECIAL SESSION | TRAINEE PLENARY PRESENTATION

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10:50 *Thrombospondin1 (TSP1) replacement prevents cerebral cavernous malformations*  
Miguel Alejandro Lopez-Ramirez, UCSD, San Diego, CA

11:30 **GROUP PHOTO & BREAK**

12:00 **LUNCH** – Café Dupont

### SESSION II | BIOMARKERS & CLINICAL TRIALS

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Moderator – Issam Awad, University of Chicago

1:00 *Plasma Biomarkers of inflammation Reflect Seizures and Hemorrhagic Activity of Cerebral Cavernous Malformations*  
Romuald Girard, University of Chicago, Chicago, IL

1:20 *Brain Lesion Count in Familial Cerebral Cavernous Malformation Type 1 (CCM1) Patients is Associated with Risk of Intracranial Hemorrhage*  
Jeffrey Nelson, UCSF, San Francisco, CA

1:40 *Differential Expression of Immunoglobulin Genes in Blood and Lesion Burden in Familial Cerebral Cavernous Malformation Type 1 (CCM1) Patients*  
Helen Kim, UCSF, San Francisco, CA

2:00 *Investigating the potential of BA-1049 for treatment of CCM*  
Lisa McKerracher, BioAxone BioSciences Inc., Cambridge, MA

2:20 *A proposed trial of oral propranolol for symptomatic cerebral cavernous malformation not amenable to surgical resection*  
Yashar Kalani, University of Utah School of Medicine, Salt Lake City, UT

2:40 **DISCUSSION**

3:00 **BREAK (END OF DAY 1 REGULAR SESSIONS)**

## **SPECIAL SESSION | CLINICAL TRIAL READINESS (3:15 – 5 PM)**

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Moderated by Amy Akers & Connie Lee, Angioma Alliance

Connie Lee – *Patient community needs & considerations for recruitment/enrollment*  
Jim Koenig – *NIH requirements for trial funding*  
Helen Kim – *Design considerations and questions*  
Kelly Flemming – *Clinical data elements*

Blaine Hart – *Imaging data elements*  
Issam Awad – *Biomarkers and their validation*  
Michael Lawton – *Treatment vs follow-up; outcomes*  
Lisa McKerracher – *Industry needs to move to PhI/II*

## **POSTER SESSION | FOXHALL BALLROOM (4:30-6 PM)**

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*BA-1049 reverses ROCK activation in brain*

Matthew Abbinanti, BioAxone BioSciences, Inc. Cambridge, MA

*Behavioral and Emotional Difficulties and Family Burden in Children and Adolescents with Cerebral Cavernous Malformations*

Savannah Cheek, University of New Mexico, Albuquerque, NM

*Is the common CCM2 exon 2-10 deletion due to a founder mutation in the US population?*

Matthew Detter, Duke University, Durham, NC

*MRI Sequence Sensitivity for the Detection of Spinal Cavernous Malformations*

Blaine Hart, University of New Mexico, Albuquerque, NM

*Genome-wide sequencing reveals differentially expressed microRNAs in Cerebral Cavernous Malformations*

Souvik Kar, International Neuroscience Institute, Hannover, Germany

*Micro-Computed Tomography in Murine Models of CCM as a Paradigm for Brain Disease*

Thomas Moore, University of Chicago, Chicago, IL

*Human brain endothelial cell ROCK1/2 protein interactions*

Michael Moritz, UCLA David Geffen School of Medicine, Los Angeles, CA

*Genetic Inactivation of RhoA-kinase in Ccm3<sup>+/-</sup> mice suggest a role for the RhoA-kinase pathway in CCM3-lesion genesis and maturation*

Amy Peiper, Duke University, Durham, NC

*Cerebrovascular angiominin mediates convergent CCM protein signaling*

Taline S. Shishoian, UCLA David Geffen School of Medicine, Los Angeles, CA

*UK Priorities for Research into Cavernoma*

David White, Caveroma alliance UK

## **WELCOME DINNER | THE GRYPHON RESTAURANT (6:30 - 9 PM)**

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*The Gryphon, 1337 Connecticut Avenue, NW*

2016 CCM Scientific Meeting Welcome Dinner is Sponsored by DSG, Inc. (Document Solutions Group)



## Day 2 | Friday, November 11, 2016

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### SESSION III | THINKING OUTSIDE THE BRAIN – CCM MECHANISMS

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Moderated by Angeliki Louvi, Yale

- 9:00 *Deciphering the roles of CCM proteins inside and outside the CCM-pathway*  
Claudia Jasmin Rödel, University of Potsdam, Potsdam, Germany
- 9:20 *C. elegans CCM-3 mediated PARTitioning is crucial for germline lumen formation and embryo development*  
Swati Pal, The Hospital for Sick Children, Toronto, Ontario
- 9:40 *KLF4 overexpression due to the loss of CCM genes results in congenital heart disease with outflow tract defects*  
Kevin Whitehead, University of Utah, Salt Lake City, UT
- 10:00 **DISCUSSION & BREAK**

### SPECIAL SESSION | INVITED KEYNOTE ADDRESS

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- 10:30 *Vascular Disease and the Mouse Model – Lessons from HHT Investigations*  
S. Paul Oh, Professor of Physiology and Functional Genomics, University of Florida, Gainesville, FL
- 11:30 **BREAK**
- 12:00 **LUNCH & TRAINEE NETWORKING SESSION**

### SESSION IV | IMAGING & MOUSE MODELS

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Moderated by Kevin Whitehead, University of Utah

- 12:40 *Vertebral Hemangiomas in a CCM1 population*  
Blaine Hart, University of New Mexico, Albuquerque, NM
- 1:00 *Micro-CT imaging of cerebral cavernous malformations in mouse models*  
Jaesung Choi, Centenary Institute & Sydney Medical School, Sydney, Australia
- 1:20 *Phenotypic Features of Murine Models of Cerebral Cavernous Malformations*  
Issam Awad, University of Chicago, Chicago, IL
- 1:40 **DISCUSSION & BREAK**

### DISCUSSION | CCM & CANCER: OVERLAPPING BIOLOGY & CROSS-FERTILIZATION (2-3:30 PM)

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Moderated by Brent Derry, SickKids Hospital

Anne-Claude Gingras – *Protein Networks*  
Angela Glading – *Adhesion & Permeability*

Maria Grazia Lampugnani – *EndMT*  
Brad St. Croix – *Angiogenesis & Cancer*



National Institute of Neurological Disorders and Stroke



RECURSION  
pharmaceuticals



PREVENTION GENETICS

DISEASE PREVENTION THROUGH GENETIC TESTING