



ANGIOMA **ALLIANCE**

Presents the 14th Annual

CCM SCIENTIFIC **MEETING**

THE DOUBLETREE BY HILTON HOTEL

SILVER SPRING, MD

NOVEMBER 8-9, 2018

Day 1 | Thursday, November 8th, 2018

- 7:30 Registration & Continental Breakfast, Connection Room
8:15 Welcome & Opening Remarks, Pinnacle Grand Ballroom

SESSION I – CLINICAL & BIOMARKERS

Moderated by Issam Awad, University of Chicago

- 8:20 Invited talk – *FDA perspective for Biomarker Qualification*
Christopher Leptak, Center for Drug Evaluation and Research, FDA
- 8:50 *Microbiome Signatures in Cerebral Cavernous Malformations*
Le Shen, University of Chicago
- 9:10 *Spinal Cord Cavernous Malformations in the Familial Cerebral Cavernous Malformations Cohort: High prevalence and positive correlation with brain cavernous malformations*
Marc Mabray, University of New Mexico
- 9:30 *Plasma Biomarkers of Cavernous Angioma with Symptomatic Hemorrhage (CASH)*
Seán Lyne, University of Chicago
- 9:50 *A Multi-Site Validation of MRI Biomarkers of Vascular Leak and Hemorrhage for Forthcoming Clinical Trials*
Nick Hobson, University of Chicago
- 10:10 **DISCUSSION**
- 10:30 **BREAK**

SESSION II – TRANSLATIONAL STUDIES

Moderated by Doug Marchuk, Duke University

- 10:50 *Cerebral Cavernous Malformations Form and Anticoagulant Vascular Domain*
Miguel Alejandro Lopez-Ramirez, University of California San Diego
- 11:10 *A kinase inhibitor inhibits MEKK3-KLF signaling and prevents initiation and progression of cerebral cavernous malformations*
Xiangjian Zhen, Centenary Institute
- 11:30 *A PDCD10 gut-brain axis exacerbates cerebral cavernous malformations*
Alan Tang, University of Pennsylvania
- 11:50 **DISCUSSION**
- 12:15 **LUNCH - CONNECTION**

SESSION III – TRANSLATIONAL STUDIES & CLINICAL TRIALS

Moderated by Rustam Al-Shahi Salman, University of Edinburgh

- 1:20 *Propranolol Repurposing and High Throughput Screening for treatment of Cerebral Cavernous Malformations*
Joppe Oldenburg, Uppsala University
- 1:40 *VE-cadherin targeted restoration of vascular integrity rescues cerebral cavernous malformation*
Jennifer Gamble, Centenary Institute
- 2:00 *Translation of ROCK2 inhibition to treat cavernous angioma*
Lisa McKerracher, BioAxone BioSciences
- 2:20 *Update on the clinical development for REC-994 (Tempol)*
Tim Considine, Recursion Pharmaceuticals
- 2:40 *Atorvastatin Treatment of Cavernous Angiomas with Symptomatic Hemorrhage Exploratory Proof of Concept (AT CASH EPOC) Trial*
Sean Polster, University of Chicago
- 3:00 *Treat CCM Clinical Trial – A multicenter randomized clinical trial on Propranolol in Cerebral Cavernous Malformation (CCM)*
Roberto Latini, Istituto Di Ricerche Farmacologiche Mario Negri
- 3:20 **DISCUSSION**
- 3:50 **GROUP PHOTO**

POSTER SESSION | DISCOVERY ROOM (4-5:30)

BA-1049 for Cavernous Angioma: target engagement and barrier function
Matthew Abbinanti, BioAxone BioSciences

CCM3, a protein mutated in cerebral cavernous malformation, is a signal transduction adapter
Kento Abe, University of Toronto

The Effect of Gut Microbiome on Chronic Models of CCM Lesion Formation
Christian Benavides, Duke University Medical Center

Ponatinib (AP24534) inhibits MEKK3-KLF signaling and prevent the initiation and progression of cerebral cavernous malformations
Jaesung Choi, Centenary Institute

Posterior Location and Inflammatory Comorbidities Increase Odds of Sporadic, Brain Cavernous Malformation Development
Kelly Flemming, Mayo Clinic

Low fluid shear stress conditions contribute to activation of cerebral cavernous malformation signaling pathways

Jennifer Gamble, Centenary Institute

Investigation of a novel mouse model for Cerebral Cavernous Malformations using Bub 1 b heterozygosity as a genetic sensitizer

Erin Griffin, Duke University Medical Center

Elucidation of mrck-1 pathways in tube development and embryogenesis of Caenorhabditis elegans

Evelyn Popiel, Sickkids

Genetic investigation of five Japanese CCM cases by whole-exome sequencing

Hiroki Hongo, The University of Tokyo

Multiple Bleeds, Lower Cranial Nerve Dysfunction and Gait Dysfunction Predict Lesser Employment Status after Brainstem Cavernous Malformation Diagnosis

Shivram Kumar, Mayo Clinic

Novel Derivatives of Fasudil that Inhibit ROCKII with Enhanced Potency and Kinase Selectivity

Matthew Lee, Cervello Therapeutics

Development of the pectoral fin vasculature in zebrafish embryos

Scott Paulissen, NICHD/NIH

Hemorrhagic Risk Factors in Cerebral Cavernous Malformations

Myranda Robinson, University of New Mexico

Regulation of endocytic trafficking and VEGFR2 receptor availability by a component of the microtubule motor dynein

Amber Stratman, NICHD/NIH

Artificial intelligence-powered drug discovery: using machine learning to identify novel therapeutic targets for CCM

Andrea Taddei, BenevolentAI

Introduction of Ranger Biotechnologies

Christina Udesen, Ranger Biotechnologies

Comorbidities and Cerebral Vascular Burden In Hereditary Hemorrhagic Telangiectasia

Ashley Wegele, University of New Mexico

Cause of Death in Familial Cerebral Cavernous Malformations: An Analysis of Prospective Database

Atif Zafar, University of New Mexico

5:30 **BREAK**

WELCOME DINNER | MRS. K'S RESTAURANT & CELLAR (7-9 PM)

9201 Colesville Road, Silver Spring, MD

Day 2 | Friday, November 9th, 2018

CONCURRENT SESSION SCIENTIFIC MEETING & FAMILY CONFERENCE

7:30 Registration & Continental Breakfast, Connection Room

8:30 Welcome & Introduction, Pinnacle Ballroom

8:40 **PLENARY PRESENTATION**

Rustam Al-Shahi Salman, University of Edinburgh

9:30 **BREAK**

SESSION IV – VASCULAR SIGNALING STUDIES

Moderated by Angela Glading, University of Rochester

9:50 *Characterizing the function of RHOA signaling in regulating cranial vascular integrity and development*

Laura Pillay, NICHD/NIH

10:10 *Moving toward prognostic biomarkers and therapeutic strategies for CCM disease: KRIT1 loss-of-function causes increases in protein S-glutathionylation*

Andrea Perrelli, University of Torino

10:30 *Role for a Hippo-like pathway in Cerebral Cavernous Malformations?*

Amin Ghabrial, Columbia University College of Physicians and Surgeons

10:50 *Investigating the molecular interaction and modulation within CSC complex*

Akhil Padarti, Texas Tech University Health Science Center

11:10 *Novel and known genes elucidated in cerebral cavernous malformation through comparative transcriptomic analysis of multiple model species and human microdissected lesional endothelial cells*

Janne Koskimäki, University of Chicago

11:30 **DISCUSSION**

11:45 **LUNCH – CONNECTION**

Lunchtime speaker: Issam Awad, University of Chicago, *Content and Collaboration*

SESSION V – VASCULAR DEVELOPMENT & LESION GENESIS

Moderated by Angeliki Louvi, Yale University

1:00 *Studying the origin and function of novel brain vascular-associated cells*

Maria Venero Galanternik, NICHD/NIH

- 1:20 *MicroRNA-mediated control of developmental lymphangiogenesis*
Hyun Min Jung, NICHD/NIH
- 1:40 *Understanding the role of CCM3 in endothelial development and disease*
Tvisha Misra, Sickkids
- 2:00 *Cerebral Cavernous Malformations Develop through Clonal Expansion of Mutant Endothelial Cells*
Matt Detter, Duke University Medical Center
- 2:20 *Cerebral cavernomas arise from clonal expansion of endothelial cells*
Matteo Malinverno, IFOM, FIRC Institute of Molecular Oncology Foundation
- 2:40 **DISCUSSION**
- 3:00 **CLOSE OF MEETING**

Thank you to our sponsors!



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