ANGIOMA ALLIANCE

Presents the 13th Annual

CCM SCIENTIFIC MEETING

THE WESTIN GEORGETOWN – WASHINGTON, DC

OCTOBER 26-27, 2017
Day 1 | Thursday October 26, 2017

8:15  Registration & Continental Breakfast, Washington Ballroom
9:00  Welcome & Opening Remarks

SESSION I | LESION GENESIS

Moderated by Mark Kahn, University of Pennsylvania

9:10  Uncovering molecular pathways involved in cerebral cavernous malformations by pharmacological suppression
Cecile Otten - Potsdam University

9:25  Inactivation of RhoA-kinase in Ccm3+/- mice suggest a role for the RhoA-kinase pathway in CCM3-lesion genesis and maturation
Amy Peiper - Duke University

9:40  Thrombospondin1 (TSP1) controls development of cerebral cavernous malformations
Miguel Alejandro Lopez-Ramirez - University of California, San Diego

9:55  Endothelial TLR4 and the gut microbiome drive cerebral cavernous malformations
Alan Tang - University of Pennsylvania

10:10  Vascular Endothelial Growth Factor is required for CCM lesion initiation
Kevin Whitehead - University of Utah and Angela Glading - University of Rochester

10:25  DISCUSSION & BREAK

SESSION II - CCM SIGNALING & PATHOBIOLOGY

Moderated by Mark Ginsberg, University of California San Diego

11:00  The role of Polycomb group Proteins in CCM pathology
Matteo Malinverno, Institute of Molecular Oncology, Milan

11:15  A conserved CCM complex promotes apoptosis non-autonomously by regulating zinc homeostasis in C. elegans
Eric Chapman - University of Toronto

11:30  The role of Ccm2l1 in Cerebral Cavernous Malformations in neonatal mouse brain
Jaesung Choi - Centenary Institute, Sydney

11:45  CCM3 deficiency increases levels of multiple tyrosine kinase receptors
Juan Zalvide - Universidade de Santiago de Compostela

12:00  DISCUSSION

12:30  LUNCH - PROMENADE

SESSION III - CLINICAL MANAGEMENT AND PATIENT REPORTED OUTCOMES

Moderated by Helen Kim, University of California at San Francisco

1:30  Review of a Large Registry of Pediatric Familial CCM
Leslie Morrison - University of New Mexico
1:45  *Minimally Invasive Resection of Rolandic Cavernomas in Children using a Novel Navigable Tubular Retractor System*
Erin Kiehna – Novant Presbyterian Health System, Hemby Children’s Hospital

2:00  *Mental Health Outcomes in Pediatric Cavernoma*
Tiffany Campbell - Cincinnati Children's Hospital Medical Center

2:15  *Quality of Life Measures in Familial Cerebral Cavernous Malformation Patients*
Jeff Nelson - University of California San Francisco

2:30  **DISCUSSION**

2:50  **GROUP PHOTO**

**POSTER SESSION | PROMENADE (3 – 4:30)**

*Pre-symptomatic treatment of Sturge-Weber Syndrome with aspirin and anticonvulsants*
Anne Comi – Kennedy Krieger Institute

*Pathologic/Radiologic Correlation and Findings in fCCM Lesions in Different Tissues*
Blaine Hart – University of New Mexico

*Role of lamotrigine in management of commonly encountered symptoms in patients with cerebral cavernous malformations*
Asad Ikram - University of New Mexico

*Exploring the ccm-3 Network*
Benjamin Lant – The Hospital for Sick Children

*The Baca Family Historical Project: Paradigm-Shifting Approach to Patient Engagement*
Connie Lee – Angioma Alliance

*Up-regulation of NAPH oxidase-mediated redox signaling contributes to the loss of barrier function in KRIT1 deficient endothelium*
Nicholas Nobiletti – University of Rochester

*The Effect of Gut Microbiome on Chronic Models of CCM Lesion Formation*
Heidy Pardo – Duke University

*Development of the pectoral fin vasculature in zebrafish embryos*
Scott Paulissen – NIH/NICHD

*Characterizing the role of RHOA in regulating cranial vascular integrity*
Laura Pillay – NIH/NICHD

*ROCK2-selective inhibitors and their effects on key features of the biology of cerebral cavernous malformations*
Jörg Ruschel – BioAxone Biosciences

*A Novel Perivascular Cell Population in the Zebrafish Brain*
Marina Venero Galanternik – NIH/NICHD

*Novel functions of CCM1 delimit the relationship of PTB/PH domains*
Jun Zhang – Texas Tech University Health Sciences Center

**WELCOME DINNER | NICK’S RIVERSIDE GRILLE (7-9 PM)**

*3050 K St NW, Washington, DC*
Day 2 | Friday, October 27, 2017

**SESSION IV - CELL BIOLOGY**

Moderated by Brant Weinstein, NICHD/NIH

9:00  *The Cerebral cavernous malformation disease causing gene KRIT1 participates in epithelial barrier maintenance and regulation*
Le Shen - University of Chicago

9:15  *Glycolytic metabolism and VEGFR3 signaling are required for lymphangiogenesis*
Joanne Chan - Hamilton University

9:30  *Molecular regulation of vascular smooth muscle cell recruitment to arteries during development*
Amber Stratman – NIH/NICHD

9:45  **DISCUSSION**

10:00  Keynote address, Introduction by Doug Marchuk, Duke University
Victoria Bautch - University of North Carolina at Chapel Hill

10:45  **BREAK**

11:00  Angioma Alliance Updates
Connie Lee - Angioma Alliance

**SPECIAL SESSION | PEDIATRIC CCM: SPECIAL CONSIDERATIONS FOR TREATMENT & TRIALS**

Moderated by Ed Smith, MD, Boston Children’s

*Barry Mangum, PharmD - CEO Paidion Research, Inc (Trial Design Considerations)*
*Ronald Farkas MD PhD - Vice President PAREXEL International (Regulatory Considerations)*
*Sudhakar Vadivelu, MD - Cincinnati Children’s Hospital and Medical Center (Clinical Management)*

12:20  **LUNCH - PROMENADE**

**SESSION V - CLINICAL TRIAL READINESS**

Moderated by Issam Awad, University of Chicago

1:30  *Plasma Biomarkers of Inflammation and Angiogenesis Predict Cerebral Cavernous Malformation Symptomatic Hemorrhage or Lesional Growth*
Romuald Girard - University of Chicago

1:45  *Developing a new drug for CCM: the path from bench to bedside*
Ken Rosen - BioAxone BioSciences

2:00  *Recent Developments in Advancing Tempol as a Treatment for CCM*
Tim Considine - Recursion Pharmaceuticals

2:15  *CASH (Cavernous Angiomas with Symptomatic Hemorrhage) Trial Readiness*
Sean Polster – University of Chicago

2:30  **DISCUSSION**

2:45  *2017 CCM Meeting Summary and Closing Remarks*
Issam Awad - University of Chicago

3:00  **CLOSE OF MEETING**