

Welcome
to the
10th Anniversary

ANGIOMA ALLIANCE
CCM SCIENTIFIC MEETING

BETHESDA NORTH MARRIOTT
HOTEL & CONFERENCE CENTER
NOVEMBER 6-7, 2014



ANGIOMA ALLIANCE

because brains shouldn't bleed

Day 1 | Thursday, November 6th, 2014

- 8:00 Registration Check in & Continental Breakfast, *White Oak A Conference Room*
8:55 Welcome – Amy Akers, Angioma Alliance

SESSION I | THERAPEUTIC TARGETS AND PRECLINICAL STUDIES IN CCM

- Moderator – Doug Marchuk, Duke University
- 9:00 *Targeting mTOR Signaling for the Treatment of Stroke*
Joanne Chan, Hampton University
- 9:20 *Novel insights into the role of oxidative stress in CCM pathogenesis*
Francesco Retta, University of Torino
- 9:40 *B-Cell Depletion Decreases Lesion Burden in a Murine Model of CCM Disease*
Changbin Shi, University of Chicago
- 10:00 **COFFEE BREAK**
- 10:10 *β -catenin signaling in CCM3 null endothelial cells contributes to Cerebral Cavernous Malformation pathology*
Luca Bravi, IFOM
- 10:30 *A Comparison between Fasudil and Simvastatin Treatment in Two Murine Models with CCM*
Robert Shenkar, University of Chicago
- 10:50 30-Minute Discussion
- 11:30 **LUNCH & SPECIAL GUEST SPEAKER**
WHITE OAK B
Drug Development in an Academic Lab
Susan Slaughaupt, Massachusetts General Hospital/Harvard Medical School

SESSION II | GENETIC & CLINICAL VARIATION IN CCM

- Moderator – Helen Kim, University of California San Francisco
- 12:40 *PDCD10 Gene Mutations in Multiple Cerebral Cavernous Malformations*
Silvana Penco, Niguarda Hospital
- 1:00 *Polymorphisms in DNA mismatch repair genes associated with severity of cerebral cavernous malformation type I*
Helene Choquet, University of California at San Francisco
- 1:20 *Adrenal Gland Calcifications Associated with Familial Cerebral Cavernous Malformations in Carriers of the CCM1 Common Hispanic Mutation*
Corinne Strickland, Beth Israel Deaconess Medical Center
- 1:40 15-Minute Discussion

Session III | Clinical Research Tools - Biomarkers & Clinical Guidelines

- Moderator – Issam Awad, University of Chicago
- 1:55 *Correlation of Quantitative Susceptibility Mapping in Cerebral Cavernous Malformations with Clinical and Conventional MRI Features*
Huan Tan, University of Chicago

2:15 *Proposed Methodology for the Development of Guidelines for the Clinical Management of Cerebral Cavernous Malformations: Consensus Recommendations of the Angioma Alliance Scientific Advisory Board Based on Systematic Literature Review*
Tania Rebeiz, University of Chicago

2:35 10-Minute Discussion

2:45 **COFFEE BREAK**

Session IV | Clinical Trial Panel Discussion

Moderator – Amy Akers, Angioma Alliance

2:55 Amy Akers – Introduction and Overview

3:00 Connie Lee – *Patient Engagement & Recruitment*

3:10 Issam Awad – *Models of Potential Clinical Trials, Imaging Biomarkers & Outcomes*

3:20 Kevin Whitehead – *Animal models & Physiological Biomarkers*

3:30 Claudia Moy – *Study Design & funding opportunities*

3:40 Kathryn O’Connell – *FDA Requirements for IND submission and trial initiation: are we ready?*

3:50 Lisa McKerracher – *From your experience, what makes a successful public-private partnership?*

4:00 Leslie Morrison – *Effect of statin treatment on Permeability with CCM1-CHM, Future studies*

4:10 Open Discussion

5:00 **END OF DAY 1 REGULAR SESSIONS**

6:30 **DINNER & KEYNOTE ADDRESS, WHITE OAK B**

Dr. Francis Collins, National Institutes of Health Director

Sponsorship provided by:



**ANGIOMA
ALLIANCE**



*NINDS
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Day 2 | Friday, November 7th, 2014

7:30 Registration Check in & Continental Breakfast, *White Oak A*

Session V | Signaling & Vascular Biology

Moderator – Angela Glading, University of Rochester

8:30 *CCM Proteins control endothelial B1 integrin dependent response to shear stress*
Eva Faurobert, INSERM Grenoble

8:50 *KRIT1 depletion modifies endothelial cell behavior via increased VEGF signaling*
Peter DiStefano, University of Rochester

9:10 *CCM3 regulates EC/pericyte interactions and vascular integrity*
Huanjiao Jenny Zhou, Yale

9:30 **COFFEE BREAK**

9:40 *The cerebral cavernous malformation pathway controls cardiac development through regulation of MEKK3*
Zinan Zhou, University of Pennsylvania

10:00 *The CCM proteins are required for cardiac outflow tract development*
Kevin Whitehead, University of Utah

10:20 20-Minute Discussion

Session VI | CCM protein Structure, Function & Signaling Networks

Moderator – Titus Boggon, Yale

10:40 *RhoA mediated cytoskeletal defects disrupt cell adhesion and cytokine signaling leading to CCM*
Adriana Beltran, UNC-Chapel Hill

11:00 *Defining the kri-1/ccm1 apoptosis pathway in C. elegans*
Eric Chapman, University of Toronto

11:20 *Mapping and Determining the Functional Significance of the CCM2:CCM3 Interaction*
Kyle Draheim, Yale

11:40 15-Minute Discussion

Noon **LUNCH, WHITE OAK B**

Session VI | CCM protein Structure, Function & Signaling Networks

Moderator – Brent Derry, The Hospital for Sick Children

1:00 *Loss of ccm-3 affects multiple signaling pathways regulating C. elegans germline development: a possible explanation for excessive aggressiveness associated with CCM3 mutation*
Swati Pal, The Hospital for Sick Children

1:20 *The role of CCM3 in gliogenesis*
Angeliki Louvi, Yale

1:40 *CCM3-Mst4-Erk1/2 axis in modulation of brain endothelial barrier integrity and CCM3 lesion formation*
Anuska Andejelkovic, University of Michigan

2:00 *A comprehensive proteomic characterization of CCM3 Complexes*
Anne-Claude Gingras, Lunenfeld-Tanenbaum Research Institute

2:20 20-Minute Discussion

2:40 Closing Remarks – Amy Akers, Angioma Alliance

2:45 End of 2014 CCM Scientific Meeting