

ALLIANCE TO CURE 
CAVERNOUS MALFORMATION
2022 International CCM Scientific Meeting Agenda

Privacy Policy

We would like to reiterate to everyone the “workshop” nature of this very special gathering, and the unique multidisciplinary opportunity to cross germinate ideas and form collaborations. A community of trust and respect for our privacy policy is essential to the success of the CCM Scientific Meeting.

To maintain privacy and encourage presentation and discussion of unpublished data, Angioma Alliance does not publicize written abstracts and it is not permissible to copy or disseminate unpublished data or results presented in presentation videos or at the live meeting.

Violations to the privacy policy will be reviewed by the organizing committee and will result in denied Meeting attendance for at least one year.

Thursday, November 17: The Human Condition, Treatment, and Trials

7:00 **Breakfast and Registration – Presidents I**

PLENARY – President’s Ballroom

8:30 Welcome – Amy Akers, Alliance to Cure Cavernous Malformation Chief Scientific Officer

8:40 Session I – Natural History and Biomarkers

Panel Moderators: Helen Kim and Jorge Marcondes de Souza

Female hormone therapy and risk of intracranial haemorrhage from cerebral cavernous malformations: a multicentre observational cohort study – Kelly Flemming, Mayo Clinic

Association Of Quality of Life Domains And Clinical Symptoms In Familial Cerebral Cavernous Malformation Patients – Cynthis Tsang, UCSF

Blood prognostic signature for progression risk of hemorrhagic CCMs – Jun Zhang, Texas Tech University Health Center

Biomarker assessments in Treat_CCM – Francesca Lazzaroni, Policlinico of Milan

Discussion

10:20 Break

10:50 Session II – Trial Readiness

Panel Moderators: Issam Awad and Rustam Al-Shahi Salman

The Cerebral Cavernous Malformations Health Index (CCM-HI): Development and Validation of a Novel Disease-Specific Patient Reported Outcome Measure for Clinical Trials – Jennifer Weinstein, University of Rochester

Prospective Symptomatic Hemorrhage Rates and Functional Outcomes in Trial Eligible Cavernous Angiomas with Symptomatic Hemorrhage – Kelly Flemming, Mayo Clinic

Changes in Quantitative Susceptibility Mapping on Magnetic Resonance Imaging During Prospective Follow-Up of Cavernous Angiomas with Symptomatic Hemorrhage in Trial Readiness Project – Stephanie Hage, University of Chicago

CARE-PREP: Planning a Multi-Site International Platform Trial – Rustam Al-Shahi Salman, University of Edinburgh

Non-Invasive Focused Ultrasound Platform for Biologics Delivery to Cerebral Cavernous Malformations – Delaney Fisher, University of Virginia

12:30 Discussion

12:50 Lunch – Presidents I

2:10 Updates: Alliance to Cure Cavernous Malformation – Connie Lee

2:20 Session III – Clinical Trials

Panel Moderator: Ed Smith and Roberto Latini

Cavernomas: A Randomised Effectiveness (CARE) pilot trial, to address the effectiveness of treatment with surgery (neurosurgery or stereotactic radiosurgery) versus treatment without surgery in people with symptomatic CCM – Rustam Al-Shahi Salman, University of Edinburgh

Single and Multiple Ascending Dose Studies of REC-994 in Healthy Volunteers – Glenn Morrison, Recursion Pharmaceuticals

Propranolol for familial cerebral cavernous malformations (Treat_CCM): a phase 2, randomised, open-label, blinded endpoint pilot trial – Roberto Latini, Mario Negri Instituto

Impact of Socioeconomic Status and Race on the Access to Specialized Care and Enrollment/Adherence in Clinical Trial in Cerebral Cavernous Malformations – Stephanie Hage, University of Chicago

AT-CASH EPOC Trial Update – Issam Awad, University of Chicago

3:50 Discussion

4:20 Group Photo

4:30 – Poster Session & Reception – Presidents Gallery

A role for the Golgi protein USO-1 in biological tube development – Tony Cheng, University of Toronto

Correlation of MRI and Histological Features of Individual Lesions in a Chronic Mouse Model of Cerebral Cavernous Malformations – Delaney Fisher, University of Virginia

Plasma Proteins in Correlation with Lesional Iron Content and Permeability Imaging in Clinical Trial of Cavernous Angioma with Symptomatic Hemorrhage – Stephanie Hage, University of Chicago

Blocking the $\beta 1$ adrenergic receptor prevents Cerebral Cavernous Malformations – Wenqing Li, UCSD

Transcriptomic signatures of individual cell types in cavernous angioma – Ying Li, University of Chicago

Inflammation and neutrophil extracellular traps in cerebral cavernous malformation – Peetra Magnusson, Uppsala University

Be Brave Foundation Micro Grant Treat_CCM Bridge Observational Study – Jennifer Meessen, Mario Negri Instituto

CCM disease from a redox perspective: KRIT1 acts as a traffic warden at the busy crossroads between redox signaling and CCM pathogenesis – Andrea Perrelli, University of Rochester

In Vivo Permeability of the Aging Brain with Cerebral Microbleeds – Sharbel Romanos, University of Chicago

A Case of Brainstem Cavernous Malformation Treated with Off-Label Atorvastatin and Propranolol Combinatorial Therapy – Jorge Marcondes de Souza, UFRJ

Time-resolved endothelial transcriptome of mouse cerebrum and cerebellum reveals regional differences and demonstrates delayed gene regulation in cerebellar endothelium – Ross Smith, Uppsala University

Multiplexed Histopathological Characterization of Cerebral Microbleeds in the Aging Brain – Abhinav Srinath, University of Chicago

C-X3-C motif chemokine receptor 1 (CX3CR1) gene variants associated with lesion burden in Cerebral Cavernous Malformation – Shantel Weinsheimer, UCSF

7:00 – Dinner **Washington Duke Hotel, Ambassador Ballroom**

Friday, November 18: Pathogenesis

Breakfast – Presidents I

PLENARY – Presidents Ballroom

8:30 *Welcome* – Amy Akers, Alliance to Cure Cavernous Malformation Chief Scientific Officer

8:40 – Keynote

Keynote Introduction – Issam Awad, University of Chicago

Keynote Address to Patients, Families, and Investigators – Doug Marchuk, Duke University

9:45 – Break with Family Conference attendees

10:10 Session IV – Pre-Clinical Targets and Lesion Development

Panel Moderators: Angeliki Louvi and Peetra Magnusson

Pharmacologic Rescue of Cerebral Cavernous Malformations (CCMs) by Rapamycin, A Pre-clinical Study using FDA-approved mTOR inhibitor – Lun Li, University of Pennsylvania

Fluvastatin and zoledronate therapy in mouse models of CCM – Angeliki Louvi, Yale

Mechanisms of hypoxia-induced immunothrombosis and exacerbation of cerebral cavernous malformation disease – Miguel Lopez-Ramirez, UCSD

Immunothrombosis and cerebral hypoxia in cerebral cavernous malformation – Peetra Magnusson, Uppsala University

11:50 Discussion

12:10 Lunch with Family Conference attendees – Presidents I

1:40 Session V – Genetics & CCM Signaling

Panel Moderators: Doug Marchuk and Angela Glading

*kri-1 and ccm-3 converge on the innate immune system to maintain viability in *Caenorhabditis elegans** – Sam Krempel, University of Toronto

Cerebral cavernous malformation development in chronic mouse models driven by dual recombinases induced gene deletion in brain endothelial cells – XJ Zheng, University of Sydney

Heterozygous Loss of KRIT1 in Mice Affects Metabolic Functions of the Liver, Promoting Hepatic Oxidative and Glycative Stress – Andrea Perrelli, University of Rochester

Somatic Loss of Heterozygosity in CCM Lesions – Andrew Ressler, Duke University

Dysregulation of the Ephrin B2/EphB4 Ratio in Human Cerebral Cavernous Malformations is Associated with Endothelial Cell Dysfunction in vitro – Julie Sesen, Boston Children's Hospital

2:50 Discussion

3:10 Closing Remarks – Issam Awad & Doug Marchuk

3:20 Break with Family Conference attendees

3:40 End of meeting