

# ALLIANCE TO CURE CAVERNOUS MALFORMATION



## 21st Annual International CCM Scientific Meeting & 1st Annual CCM Center Faculty Meeting

Nov. 6-7, 2025  
Ritz-Carlton, Atlanta

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**21st Annual International CCM Scientific Meeting**  
**Ritz-Carlton, Atlanta, Georgia**  
**November 6-7, 2025**

**Thursday, November 6th**

7:00 Check-in & Breakfast (Ballroom, lower level)

**Session I: Clinical Correlates and Approaches (Ballroom)**

**Moderator: Angela Glading**

8:10	Helen Kim	<i>CCM therapeutic use and risk of hemorrhage in the Brain Vascular Malformation Consortium CCM Project</i>
8:30	Kelly Flemming	<i>Prospective Untreated Outcomes in Patients with Cerebral Cavernous Malformation</i>
8:50	Jenny Lee	<i>Development of Automated Deep Learning Pipeline for Image-Based Lesion Characterization in Cerebral Cavernous Malformations</i>
9:10	Romuald Girard	<i>Short Term Changes in Quantitative Susceptibility Mapping During Prospective follow-up of Cerebral Cavernous Malformations</i>
9:30	Issam Awad	<i>Relevance of Lesion Volume as an Outcome in Cerebral Cavernous Malformation Drug Trial: Exploratory Analyses in the AT CASH EPOC Trial</i>
9:50	Discussion	
10:10	Break	

**Session II: Inflammation in CCM (Ballroom)**

**Moderator: Romuald Girard**

10:30	Kyra Brewer	<i>The Role of TRPM7 in Microlia Activation and Microglia-Mediated Cerebral Endothelial Cell Injury</i>
10:40	Andrea Perrelli	<i>Identification of the adhesion GPCR ADGRL4/ELTD1 and Galectin-3 as potential prognostic biomarkers for Cerebral Cavernous Malformation</i>
11:00	Favour Onyeogaziri	<i>Targeting NETs in cerebral cavernous malformations</i>
11:20	Madison Yuan	<i>Microbiome/Tryptophan Interactions Modulate Lesion Burden in a Murine Model of Neurovascular Injury</i>
11:40	Shantel Weinsheimer	<i>Circulating Plasma Biomarkers Associated with Disease Phenotypes in Familial Cerebral Cavernous Malformation</i>
12:00	Cao Yong	<i>Single-cell transcriptomic and BCR repertoire analysis reveals inflammatory and angiogenic microenvironment in human cerebral cavernous malformations (CCMs)</i>
12:20	Discussion	
12:40	Lunch (Ballroom)	

**Session III Pre-clinical Treatment Testing and Clinical Trials (Ballroom)**  
**Moderator: Shantel Weinsheimer**

2:00	Delaney Fisher	<i>Pioneering Gene Replacement Therapy for CCM3 Mutations using Novel, Cross-Species AAV Vectors</i>
2:20	Connie Lee	<i>A Pilot Study of Image-Guided, Low-Intensity Focused Ultrasound for the Treatment of Cerebral Cavernous Malformation</i>
2:40	Zongze Li	<i>Hemorrhagic brainstem Cavernous malformation treatment with siroLiMus (CALM): protocol for a Randomized, Placebo-controlled Pilot Trial</i>
3:00	Bader Ali	<i>Recurrent Symptomatic Hemorrhage after Discontinuation of Atorvastatin versus Placebo in Randomized Trial of Hemorrhagic Cerebral Cavernous Malformations</i>

3:20 Discussion

3:45 Group Photo – Location TBD

**4:00-5:30 Poster Presentations and Reception** - In the Georgian Room on the lobby level

- **Robert Ferguson** Expert-Informed Clinical Practice Patterns in Cerebral Cavernous Malformations: A Practice Current Model for Guideline Development in Asymptomatic Lesions
- **Georgeio Sader** Assessment of Functional Outcome Measures in the AT CASH EPOC Trial for Hemorrhagic CCM
- **Ionela Iacobas** Challenges in Medical Management of Patients with PIK3CA-positive Cerebral Cavernous Malformations (CCMs)
- **Sandi Lam** Post Intensive Care Syndrome (PICS) in Pediatric Neurovascular Patients: A pilot study of parent experiences
- **Leander Brooks** Pediatric RASA1-Positive CM-AVM Syndrome: A Narrative Case Report with Literature Review & Expert Insights
- **Andrea Perrelli** CCM2 regulates KRIT1 subcellular localization and expression level
- **Malavika Desai** Endothelial PIK3CA Gain Of Function and CCM Deletion Drive Peripheral Vascular Lesions with Motor Dysfunction
- **Miguel Lopez-Toledano** A Phase 1, First-in-Human, Single-Ascending Dose Study to Assess Safety, Tolerability, and Pharmacokinetics of a ROCK inhibitor (NRL-1049) in Healthy Volunteers
- **Gabriela Seplovich** Effects of Hyper-Phosphorylated Tau on the Blood-Brain-Barrier
- **Patient Posters**

**Dinner – 6:30-9:30 Ray's in the City, 240 Peachtree St NW**

## Friday, November 7th

7:15 Breakfast with Patients and Clinicians  
8:30 Welcome and Alliance to Cure Cavernous Malformation Updates  
8:50 Keynote Address (hybrid): Neal Kassell, Founder of Focused Ultrasound Foundation  
10:00 Break with Patients and Clinicians

**\*Clinicians will move to the Plaza Ballroom at 10:25 - 12:10 for a shared session with patients.**

### Session IV: New Genetic Discoveries and Models (Ballroom)

**Moderator: Delaney Fisher**

10:30 Doug Marchuk *Mosaic Chromosome Alteration (MoChA) analysis of Cerebral Cavernous Malformation tissue reveals recurrent copy number alterations across distinct chromosomes and chromosomal regions*  
10:50 Monica Gallego-Bonhomme *Cellular and Molecular Characterization of a Hereditary Cerebral Cavernoma: Towards Personalized Medicine*  
11:10 Jathin Rao *Discovery and Functional Characterization of a Hereditary Cerebral Cavernoma: Towards Personalized Medicine.*  
11:30 Konstantin Gaengel *Cavernous Malformations of the Sciatic Nerve Induce Motor Deficits Independent of Cerebral Hemorrhage*  
11:50 Discussion  
12:10 Lunch with Patients and Clinicians

**\*CLINICIANS WILL MOVE TO CONGRESS ROOM FOR REMAINDER OF THE AFTERNOON.  
MEETING AGENDA SEE PAGE 4.**

### Session V: Advances in CCM Models and Mechanisms (Ballroom)

**Moderator: Andrea Perrelli**

1:20 Amanda Norren *Intravital Imaging of Disease Mechanisms in a Mouse Model of CCM Skin Lesions*  
1:40 Dariush Skowronek *High-throughput differentiation of human blood vessel organoids reveals overlapping and distinct functions of the cerebral cavernous malformation proteins*  
2:00 Eva Faurobert *Over expressed mechanosensitive calcium channels cooperatively trigger endothelial defects in Cerebral Cavernous Malformations*  
2:20 Mike Wang *Mitochondrial integrated stress signaling mediates communications between mutant and wild type ECs during CCM lesion progression*  
2:40 Jian Ren *Angiogenic Switching in Cerebral Cavernous Malformations Driven by MAP3K3/PIK3CA Synergy*  
3:00 Discussion  
3:20 Afternoon break and end of Scientific Meeting

**1st Annual Center of Excellence and Clinical Center  
Faculty Meeting  
Congress Room, 3rd Floor**

**Friday, November 7**

**Welcome and Alliance to Cure Updates**

1:10 Connie Lee  
Katie Garbarini *Brief updates on key aspects of the CCM Centers program, including an overview of the network, growth strategy, and procedural updates.*

**Consensus Priorities for Center Collaboration**

1:20 Ed Smith  
Kelly Flemming *Focused discussion on identifying and aligning shared priorities for collaboration across CCM Centers. The goal is to establish a consensus on key areas for joint effort—such as collaborative research, clinical care initiatives, and addressing gaps in care.*

2:10-2:25pm **Break – Reset / Stretch / Snack**

**Clinical Research Presentations**

2:20 Aryan Wadhwa *Beth Israel Deaconess Medical Center CCM Clinical Center: Optimal Imaging Follow-up Strategies for Conservatively Managed Cerebral Cavernous Malformations: A Cost-Effectiveness Analysis.*

2:35 Robert Ferguson *University of Virginia CCM Center of Excellence: Expert-Informed Clinical Practice Patterns in Cerebral Cavernous Malformations: A Practice Current Model for Guideline Development in Asymptomatic Lesions.*

2:50 Georgeio Sader *University of Chicago CCM Center of Excellence: Assessment of Functional Outcome Measures in the AT CASH EPOC Trial for Hemorrhagic CCM.*

3:05 All attendees **Break: Seventh Inning Stretch!**

**Clinical Topics and Cases**

3:10 Leslie Morrison  
Tarun Girotra *University of New Mexico Health Sciences CCM Center of Excellence: Leadership from the UNM CCM clinical team will share recent insights from a community studio patient engagement event. They will also poll faculty meeting attendees on best practices for incorporating CCM community feedback into clinical care, research, and center activities.*

3:35 Kelly Flemming *Mayo Clinic Rochester CCM Center of Excellence: Clinical case presentation*

3:45 Sean Polster *University of Chicago CCM Center of Excellence: Clinical case presentation*

**Summary and Meeting Adjournment**

3:55 Connie Lee  
Katie Garbarini *Brief summary of key takeaways and next steps for center collaboration.*