

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

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| 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

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|-------|---------------------|---|
| 10:30 | Kyra Brewer | <i>The Role of TRPM7 i Microglia 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

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| 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 *Brief updates on key aspects of the CCM Centers program, including an overview of the network, growth strategy, and procedural updates.*
Katie Garbarini

Consensus Priorities for Center Collaboration

1:20 Ed Smith *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.*
Kelly Flemming

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 *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.*
Tarun Girotra

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 *Brief summary of key takeaways and next steps for center collaboration.*
Katie Garbarini