

ALLIANCE TO CURE

CAVERNOUS MALFORMATION



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

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

Thank you to our sponsors!



NEURELIS™



Recursion



**Boston
Children's
Hospital**



Stanford
HEALTH CARE



AT THE FOREFRONT
**UChicago
Medicine**

Where the world comes for answers

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 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 | Yong Cao | <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 Malavika Desai *Endothelial PIK3CA Gain Of Function and CCM Deletion Drive Peripheral Vascular Lesions with Motor Dysfunction*

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
Katie Garbarini an overview of the network, growth strategy, and procedural updates.*

Consensus Priorities for Center Collaboration

1:20 Ed Smith *Focused discussion on identifying and aligning shared priorities for
Kelly Flemming 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 *University of New Mexico Health Sciences CCM Center of Excellence:
Tarun Girotra 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 *Brief summary of key takeaways and next steps for center
Katie Garbarini collaboration.*

Attendee Contact List

Bader Ali

University of Chicago
Chicago, Illinois USA
bader.ali@bsd.uchicago.edu

Issam Awad

University of Chicago
Chicago, Illinois USA
iawad@uchicago.edu

Megan Barzallo

Ann & Robert H. Lurie Children's Hospital
Chicago, Illinois USA
Mbarzallo@luriechildrens.org

Ranya Benchaaboune

Morehouse School of Medicine
Atlanta, Georgia
rbenchaaboune@msm.edu

Robin Boyd

Neurelis, Inc.
San Diego, CA USA
Rboyd@neurelis.com

Kyra Brewer

Morehouse School of Medicine
Atlanta, Georgia USA
Kbrewer@msm.edu

Leander Brooks

Morehouse School of Medicine
Atlanta, Georgia USA
Lee.brooks224@gmail.com

Karol Budohoski

University of Utah Health
Salt Lake City, Utah USA
Karol.budohoski@hsc.utah.edu

Yong Cao

Beijing Tiantan Hospital, Capital Medical University
Beijing, China
Caoyong@bjtth.org

Mikayla Carlson

Barrow Institute
Mikayla.carlson90@commonspirit.org

Chien-Hui Chang (Virtual)

University of Toronto
Toronto, Ontario Canada
Chienhui.chang@mail.utoronto.ca

Ellie Chilcott

Women's Health Center
University College London
London, England UK
e.chilcott@ucl.ac.uk

Jocelyn Cooper

Black Women's Health Imperative
Atlanta, Georgia USA
JCooper@bwhi.org

Brent Derry (Virtual)

The Hospital for Sick Children
Toronto, Ontario Canada
brent.derry@sickkids.ca

Malavika Bimal Desai (Virtual)

Uppsala University
Uppsala, Sweden
Desaimalavika@gmail.com

Helen Evans (Virtual)

Cavernoma Alliance UK
Dorchester, England UK
Helen@cavernoma.org.uk

Eva Faurobert (Virtual)

Institute for Advanced Biosciences
Grenoble, France
Eva.fauRobert@univ-grenoble-alpes.fr

Kiyanie Fedrick

Morehouse School of Medicine
Atlanta, Georgia US
Kfedrick@msm.edu

Robert Ferguson Jr.

University of Virginia School of Medicine
Charlottesville, VA USA
Robbyfergjr2@gmail.com

Delaney Fisher

Duke University
Durham, North Carolina USA
Delaney.fisher@duke.edu

Kelly Flemming

Mayo Clinic
Rochester, Minnesota USA
Flemming.Kelly@mayo.edu

Konstantin Gaengel

Uppsala University
Uppsala, Sweden
konstantin.gaengel@igp.uu.se

Francesca Galeffi

Duke University
Durham, North Carolina USA
Francesca.galeffi@duke.edu

Monica Gallego-Bonhomme (Virtual)

Centro de Investigaciones Biológicas Margarita Salas (CSIC)
Madrid, Spain
Monica8gb@gmail.com

Nkkezi George

Morehouse School of Medicine
Atlanta, Georgia USA
Nkgeorge@msm.edu

Romuald Girard

University of Chicago
Chicago, Illinois USA
Rgirard@uchicago.edu

Tarun Girotra

University of New Mexico
Albuquerque, New Mexico USA
Tagirotra@salud.unm.edu

Destiny Gordon

Morehouse School of Medicine
Atlanta, Georgia USA
Dgordon@msm.edu

Blaine Hart

University of New Mexico
(emeritus)
Blhart@gmail.com

Justine Hsu

University of Chicago
Chicago, Illinois USA
Justinelee89@bsd.uchicago.edu

Ionela Iacobas

Texas Children's Hospital
Houston, Texas USA
ixiacoba@texaschildrens.org

Helen Kim

University of California, San Francisco
San Francisco, California USA
helen.kim2@ucsf.edu

Monique Latif

Morehouse School of Medicine
Atlanta, Georgia USA
Mlatif@msm.edu

Jenny Lee

University of California
San Francisco, California USA
jenny.lee@ucsf.edu

Zongze Li

Huashan Hospital, Fudan University
Shanghai, China
lizongze666@aliyun.com

Rhonda Lightle

University of Chicago
Chicago, Illinois USA
rlightle@uchicago.edu

Miguel Lopez-Toledano

Neurelis, Inc.
San Diego, California USA
mtoledano@neurelis.com

Douglas Marchuk

Duke University School of Medicine
Durham, North Carolina USA
douglas.marchuk@duke.edu

Samuel McClugage

Texas Children's Hospital
Houston, Texas USA
Sgmcclug@texaschildrens.org

Lisa McKerracher

BioAxone BioSciences
Xxm MA
Lmck@bioaxonebio.com

Wilson Miller

University of Virginia
Charlottesville, VA USA
Gwm2n@virginia.edu

Leslie Morrison

University of New Mexico
Albuquerque, New Mexico USA
lmorrison56@gmail.com

Nitha Muntu

University of Chicago
Chicago, Illinois USA
Nitha.aimamuntu@bsd.uchicago.edu

Leock (Stella) Ngo

Neurelis, Inc
San Diego, California USA
Ngo_stella@neurelis.com

Vivian Nguyen

University of Chicago
Chicago, Illinois USA
Vsnguyen@uchicagomedicine.org

Amanda Norren (Virtual)

Uppsala University
Uppsala, Sweden
Amanda.norren@igp.uu.se

Amanda Norren (Virtual)

Uppsala University
Uppsala, Sweden
Amanda.norren@igp.uu.se

Nebechi Okpala

Morehouse School of Medicine
Atlanta, Georgia USA
Neybaychi@gmail.com

Favour Onyeogaziri

Uppsala University
Uppsala, Sweden
Favour.onyeogaziri@igp.uu.se

Andrea Perrelli

University of Rochester
Rochester, New York USA
andrea.perrelli@unito.it

Robin Pilz (Virtual)

University of Medicine
Greifswald
Greifswald, Germany
Robinalexander.pilz@med.uni-greifswald.de

Sean Polster

University of Chicago
Chicago, Illinois USA
spolster@uchicago.edu

Matthew Potts

Northwestern University
Chicago, Illinois USA
Mpotts@nm.org

Adrian Rabinowicz

Neurelis, Inc.
San Diego, California USA
Arabinowicz@neurelis.com

Aubrianna Ramsland

University of Chicago
Chicago, Illinois USA
Aubrianna.ramsland@uchicagomedicine.org

Jathin Rao

McGill University
Montreal, Quebec Canada
Raojathin@gmail.com

Matthias Rath (Virtual)

MSH Medical School
Hamburg, Germany
Matthias.rath@medicalschoo-hamburg.de

Jian Ren

Xuanwu Hospital
Beijing, Beijing China
renjian1129@126.com

Elodie Rey (Virtual)

Institute for Advanced
Biosciences
Grenoble, France
Elodie.rey@univ-grenoble-alpes.fr

Georgeio Sader

University of Chicago
Chicago, Illinois USA
Georgeio.sader@bsd.uchicago.edu

Valeriia Saenko (Virtual)

Greifswald Medical University
Greifswald, Germany
Valeriia.saenko@med.uni-greifswald.de

Gabriela Seplovich

Morehouse School of Medicine
Atlanta, Georgia USA
Gseplovich@msm.edu

Shashank Shekhar

Duke University School of
Medicine
Durham, North Carolina USA
Shashank.shekhar@duke.edu

Robert Shenkar (Virtual)

University of Chicago
Chicago, Illinois USA
rshenkar@bsd.uchicago.edu

Caitlin Simmons

Morehouse School of Medicine
Atlanta, Georgia USA
Caitlinmsimmons98@gmail.com

Dariusz Skowronek (Virtual)

Greifswald Medical University
Greifswald, Germany
Dariusz.skowronek@med.uni-greifswald.de

Edward Smith

Boston Children's Hospital
Boston, Massachusetts USA
Edward.smith@childrens.harvard.edu

Breeana Snowball

Morehouse School of Medicine
Atlanta, GA USA
bsnowball@msm.edu

Abhinav Srinath

University of Chicago
Chicago, Illinois USA
Abhinavs@uchicagomedicine.org

Agnieszka Stadnik

University of Chicago
Chicago, Illinois USA
astadnik@bsd.uchicago.edu

Xavier Street

Morehouse School of Medicine
Atlanta, GA USA
xstreet@msm.edu

Sophie Su

University of Chicago
Chicago, Illinois USA
Yssu@uchicagomedicine.org

Sarah Thomas

Morehouse School of Medicine
Atlanta, GA USA
srthomas@msm.edu

Aryan Wadhwa

Beth Israel Deaconess Medical
Center
Boston, Massachusetts USA
Aryan.wadhwa@gmail.com

Min Wang

Yale University School of
Medicine
New Haven, Connecticut USA
mike.wang388@gmail.com

Shantel Weinsheimer

University of California San
Francisco
San Francisco, California USA
shantel.weinsheimer@ucsf.edu

Austin Williams

Morehouse School of Medicine
Atlanta, Georgia USA
Abwilliams@msm.edu

Lindsay Williams

Mayo Jacksonville
Jacksonville, Florida USA
Williams.lindsay@mayo.edu

Madison Yuan

University of Chicago
Chicago, Illinois USA
Mxyuan@uchicago.edu

Jenny Zhou

Yale University School of
Medicine
New Haven, Connecticut USA
Jenny.zhou@yale.edu

Alliance to Cure Cavernous Malformation Staff

Connie Lee

CEO

clee@alliancetocure.org

Angela Glading

Chief Scientific Officer

angela.glading@alliancetocure.org

Jianbo Hu

Industry Relations Specialist

jianbo.hu@alliancetocure.org

Holly Blei

Clinical Research Specialist

hblei@alliancetocure.org

Dinah Winchester

Director of Strategic Partnerships

dwinchester@alliancetocure.org

Lindsay Ramirez

Strategic Partnerships Specialist

lindsay@alliancetocure.org

Darla Clayton

Patient Affairs Director

darla@alliancetocure.org

Linda Fuchser

Community Development
Specialist

linda@alliancetocure.org

Katie Garbarini

Clinical Programs Director

Katie@alliancetocure.org

Jessica Biggs

Patient Navigator

Jessica.biggs@alliancetocure.org

Alliance to Cure Cavernous Malformation Board Members

David Borland

Chair

chair@alliancetocure.org

Ken Brevoort

Treasurer@alliancetocure.org

Tyler Fairbank

Tfairbank@fairbankgrp.com

Dorothy Robinson

Dorothy.robinson@yale.edu

Kandance Weems-Norris

kandance@alliancetocure.org