ALLIANCE TO CURE CAVERNOUS MALFORMATION MAGAZINE | FALL 2025

# CCM CURE

## PATIENT VOICES DRIVING BETTER TREATMENTS AND A CURE

p. 5

# Letter from the Board

Welcome to the inaugural edition of *CCM Cure*.

Our goal with *CCM Cure* is to update you on our progress towards nonsurgical treatments and a cure for cavernous malformation (CCM) while recognizing the individuals in our community who are making a difference.

Our feature story this Fall 2025 issue, *Patients Powering Change*, describes how the Alliance has changed minds at the Food and Drug Administration (FDA) so that clinical trials of promising CCM treatments can happen faster. Written by our CEO Connie Lee, the article emphasizes the power of patient voices, like those of Rachel Paverman *(cover)*.

You'll see more evidence of how patients and caregivers are driving change in the story of patient Brandi Chavez *(After a cavernous malformation bleed, a New Mexico woman searches for answers.)* Brandi's resilience is a common thread that ties our patient community together.

Our 21st Annual International CCM Scientific Meeting happens Nov 6-7 in Atlanta, and in these pages, you'll meet Science Officer, Dr. Angela Glading, who attended the Scientific Meeting for years as a CCM researcher before joining our organization. We are grateful to have her expertise as we plan our focused ultrasound clinical trial and test promising drugs in our CCM CureDriver™ Lab.

Today, we have more opportunities than ever before on the path to a cure. The Alliance is actively funding more research to accelerate progress – but this journey is only possible with your support and expanded awareness. Check out the photos of *Shine Bright*, our *Strides to Cure CCM* volunteers, and the list of individuals and institutions whose generosity is funding a cure.

Together, we are driving a cure and improving lives for all affected by CCM.

David Borland, Board Chair
Alliance to Cure Cavernous Malformation


**ALLIANCE TO CURE**
**CAVERNOUS MALFORMATION**

977 Seminole Trail PMB 367
Charlottesville VA 22901

info@alliancetocure.org

AllianceToCure.org

# 21st Annual International CCM Scientific Meeting to convene CCM experts, speed up a cure

*The Alliance's annual meeting will take place this year Nov 6-7 in Atlanta*

Each year, the Alliance brings together the leading CCM scientists to share unpublished research data and form collaborations. Nearly every significant advance in disease understanding and treatment development has roots in this annual meeting.

This year, we are pleased to hold the inaugural CCM Center of Excellence and Clinical Center Faculty Meeting on Nov. 7, so that the nation's top CCM doctors can share best practices for improving access to expert CCM care.

The meetings will be held at the same time and place as our annual Patient Conference.

We are honored that the following institutions will be represented at the CCM Scientific and Clinical Meetings:

- Beth Israel Deaconess Medical Center
- BioAxone BioSciences
- Black Women's Health Imperative
- Boston Children's Hospital
- Capital Medical University, China
- Duke University
- Lurie Children's Hospital
- Mayo Clinic
- McGill University, Canada
- Morehouse School of Medicine
- Neurelis
- Northwestern University
- Texas Children's Hospital
- University College London, UK
- University of California, Los Angeles
- University of California, San Diego
- University of California, San Francisco
- University of Chicago
- University of New Mexico
- University of Rochester Medical Center
- University of Utah Health
- University of Virginia School of Medicine
- Uppsala University , Sweden
- Yale University School of Medicine

*Together, we are accelerating better treatments and a cure for CCM.*

## MEET SCIENCE OFFICER

# ANGELA GLADING, PH.D.

Dr. Glading began researching CCM in 2004 as a postdoc in Dr. Mark Ginsberg's lab at UCSD. She continued her research at the University of Rochester, where she had her own CCM lab for more than 15 years. We are fortunate to have her leadership and expertise as we drive a cure.

**What attracted you to CCM research? What do you like about working in this field?**

I've always been interested in how individual cells 'decide' how to behave in response to their environment. I was first intrigued by CCM as the molecules identified as being responsible for causing the disease didn't conform to our expectations of the type of molecule that would drive changes in blood vessel development. It seemed to me like an opportunity to uncover a new piece of the puzzle.

I stayed with CCM research because of the communities involved, both scientific and patient, that I got to know through the work of the Alliance. Their support and desire for answers really motivated me to try to make a difference.

**How has your research helped scientists better understand CCM biology and affected treatment development?**

I was part of the team that first identified why CCM lesions are leaky and pointed to Rho kinase activity as the cause. This led to preclinical studies and clinical trials of Rho kinase inhibitors that are still ongoing.

My lab also discovered that vascular endothelial growth factor (VEGF) expression was an important factor driving the severity of CCM. Existing VEGF inhibitors have serious side effects, so they have not been considered for clinical trials yet. It's possible that better VEGF inhibitors, or targeted drug delivery, could make this feasible in the future.

More recently, we have been looking at the role of the CCM proteins in other cell types, including immune cells, where they appear to regulate immune cell function- which likely contributes to CCM disease progression.

**In the years that you've been part of the CCM science community, what role have you seen the Alliance play?**

The Alliance has been an integral part of the CCM science community. They have helped build very successful collaborations that have contributed significantly to the field. In addition, they provide a platform for young scientists (like myself at one time) to gain recognition for their work. I don't think that the CCM field would be as cohesive or as productive without their support.

**How do you feel about joining the Alliance team? What are you looking forward to?**

I am both super excited and very humbled to join this great Alliance team. I am most looking forward to helping to make a real difference in patients' lives. I know it sounds corny, but as a lab scientist, everything I did was to add another piece of knowledge to this puzzle we are building together. By working with the Alliance, I hope to help put the puzzle together, so that we can find a cure.

# Patients Powering Change

## How educating the FDA about cavernous malformation – and the lives it changes – has quickened cure development

By Connie Lee

*Connie is the Founder and CEO of the Alliance to Cure Cavernous Malformation and a CCM caregiver.*

As I was driving along the Virginia country roads on a very hot afternoon this past August, I considered how best to report on our visit to the Food and Drug Administration (FDA) the previous month. I realized this one-hour meeting in July was a small part of the eight-year story of how we—cavernous malformation (CCM) patients and caregivers—have shaped the FDA's understanding of CCM. The story is long but important: it's a key reason why non-surgical treatments for CCM are within reach.

### The FDA: a partner in treatment development

To move a drug from the laboratory to your medicine cabinet, it must go through a series of tests and trials, all of which must be approved by the FDA. This process takes years and millions of dollars.

I used to think of the FDA as a judge at the end of the process, presiding over decisions about whether treatments are safe and effective enough to warrant approving them for sale and keeping them on the market. Over the years, I've learned that's a small part of their role.

FDA's involvement in drug development starts much earlier. Even before treatments are developed for a condition, if asked, the agency can offer advice to help prepare for clinical trials. They consult on topics such as clinical endpoints—what a trial for a particular disease should measure to show whether a drug has an effect and trial design.

There are thousands of diseases, and FDA staff can't be expected to intimately understand all of them. This is where patient advocacy organizations, like the Alliance to Cure Cavernous Malformation, can have a major impact in speeding the treatment approval process. In contrast to companies or academics who are running trials, we can come to FDA


with questions and presentations that are neutral, covering the disease in general rather than advocating for a specific treatment or manufacturer.

In the case of CCM, the Alliance to Cure Cavernous Malformation has been holding meetings with the FDA since 2017. In that time, we've learned a few things about how to make clinical trials happen.

### Arguing over what to measure in a clinical trial

In 2017, the pharmaceutical companies Recursion and BioAxone were developing new drugs for CCM and beginning to think about launching clinical trials to test their drugs in human volunteers. To do so, they would eventually need FDA approval. But these companies -- indeed, any company developing a new treatment for CCM - faced the challenge of finding things to measure in clinical trials that would show a treatment is working. In other words, they needed a clinical endpoint that was feasible for them and acceptable to the FDA.

Ideally, a CCM drug trial's endpoint would be whether the drug reduced

hemorrhage, the most severe of CCM symptoms. However, in the natural course of CCM, hemorrhage is a rare event. To show a drug's benefit, a trial would need to be very large or very long. Either choice is costly and not acceptable to drug companies or universities sponsoring trials.

We were exploring alternative endpoints to hemorrhage. "Surrogate biomarkers," for example, are biological measures, like iron deposits seen on MRI or blood levels of specific proteins, that, ideally, change when hemorrhage risk increases or decreases or symptoms improve or worsen. If a drug affects the surrogate biomarker, it could be *fit for purpose* to judge whether a treatment impacts how a person *feels, functions, or survives*. (I've italicized these words because we hear these phrases in every meeting with the FDA.) Another way to measure whether a treatment has an effect is to monitor changes or stability in function – does someone's vision or walking improve or, at least, not get worse? We can also ask patients how they feel using carefully constructed and validated patient-reported outcomes measures.

Our first meeting with FDA, a "Critical Path Innovation Meeting" (CPIM) in 2017, was intended to gain FDA's support for clinical endpoints beyond hemorrhage, thus opening the door to feasible clinical trials. Our group, representing researchers, pharmaceutical company representatives, and Alliance to Cure staff, entered the meeting with all the hope in the world for straight answers and support for surrogate biomarkers and patient-reported outcome measures. We offered a comprehensive presentation outlining the challenge presented by low hemorrhage rates. We described the efforts of the field to address these, including MRI studies that could measure the amount of iron deposited in and around a lesion and blood biomarkers that might change to reflect different disease states. Finally, we presented the idea of a patient-reported outcomes measure. We believed such a tool could be the most important indicator of a treatment's efficacy because our patients' goal with any treatment is to feel better, or at least not feel worse.

The response of the FDA surrogate biomarker specialists in the room was not what we wanted to hear.

"Hemorrhage is frequent enough to be an endpoint," they said.

Instead of surrogate biomarkers and a PROM, they suggested the Modified Rankin Scale (mRS) as an additional trial endpoint. The mRS is a general measure of disability in which a patient receives a score on a scale of 0-6 points, where 0 is no disability and 6 is no longer living. The scores reflect the level of dependence on others for care. Most CCM patients who are at least 6 months from hemorrhage function at an mRS score of 1 or 2, reflecting some symptoms, but little dependence. Patients with midbrain, brainstem, or spinal bleeds can fare much worse, but their numbers are smaller. The mRS was not designed for CCM patients – our scores don't change that much over time. Any trial based on mRS or hemorrhage as its primary outcome would be very expensive because of its length or the number of patients needed. A drug company would not find it worth their time.

Our contingent left the CPIM meeting deflated, wondering whether a company developing a drug for CCM could ever have a clinical trial. For a long time, we retreated into our separate spaces to reconsider our options.

## After hearing patient stories, FDA budges

The unsatisfactory outcome of the CPIM was surprising, but it made clear to me that the FDA needed additional education about our disease.

At the CPIM, FDA staff understood CCM hemorrhage, but they didn't understand the other aspects of CCM: the way symptoms can come and go with oozing, the anxiety, fatigue, disabling headache, and brain fog. The FDA needed to hear from our patients. Perhaps then, they would understand our urgency in conducting clinical trials and appreciate the need for flexibility in clinical endpoints.

With over 10,000 diseases in its portfolio, the FDA relies on meetings called Patient Listening Sessions to gain familiarity with the lived experience of patients. In November 2019, we organized a Listening Session. Five of our patients and I visited the FDA's main campus with the goal of painting a broader picture of CCM's effects, beyond hemorrhage.

As we waited to get the meeting started, we watched the room fill and overfill with strangers whom we knew had enormous power over our ability to get treatments. Every seat in the large conference room was filled, and additional staff were standing around the sides. We were told another twenty were listening via a conference line.

We began. Each of the five Alliance members, one after another, shared stories of times they or their affected family member had a worsening of symptoms despite an MRI showing no hemorrhage.

One of our presenters shared how his 21-year-old son became symptomatic years after a brainstem hemorrhage. Recent MRIs had shown no evidence of a re-hemorrhage. The father's voice cracked, and he began to cry as he described the fear he felt watching his son lose functioning for the second time.

Another presenter described her unrelenting, incapacitating headaches caused by multiple, non-hemorrhaging lesions. She shared that she and her husband had chosen not to have children because she worried that she would be unable to care for them.

I shared, in tribute, the story of one of our young patients who had a thalamic hemorrhage when she was only 16 that left her severely disabled. Though she didn't have another hemorrhage, she experienced seizures, and her condition deteriorated over the next 5 years. Her parents captured a picture of her last smile, just hours before she passed. Some FDA attendees gasped, some wiped tears when I shared the photo, and they learned of the loss of such a beautiful young woman.

At the end of the meeting, I asked Dr. Billy Dunn, the head of the Neuroscience Division at the time, "Can FDA accept a patient-reported outcome measure as a primary clinical endpoint for a CCM trial?"

"We'll work with any sponsor of a trial to be as flexible as possible," he responded. "They should consult with us early, and we'll figure out endpoints that are mutually agreeable."

It wasn't a yes, but it was far more hopeful than the CPIM two years before.

## A clinical trial endpoint informed by patient experience

In 2019, we were getting closer to a "mutually agreeable" clinical trial endpoint. Recursion Pharmaceuticals was close to bringing REC-994, a drug specifically for CCM, into trials and wanted a CCM-specific measure to be part of the trial. They offered to fund the development of a patient-reported outcomes measure (PROM) – an endeavor that costs hundreds of thousands of dollars and takes multiple years to create -- which was to be called the CCM Health Index or CCM-HI. Recursion chose the University of Rochester as the academic center to develop the CCM-HI.

Over the next three years, we recruited 500 patients to participate in CCM-HI development. Our patients were interviewed and completed iteration after iteration of the tool to refine it. In its first serious draft, the tool asked one question: "How much does the following impact your life now?" Participants were asked to rate different symptoms on a 6-point scale, which was divided into categories like fatigue, difficulty thinking, headaches, and difficulty walking. CCM is a disease with a wide range of symptoms, and the draft included over 100 items to be rated. It would identify even subtle differences between patients or over time.

In 2021, I returned to the FDA when Recursion and the University of Rochester presented the tool for feedback in what is known as a Type C meeting, which Recursion had organized as part of its process to secure FDA approval to launch a trial. My part in the meeting was to share patient perspectives on whether the tool captured the patient experience and priorities.

At this point in the pandemic, FDA meetings were conducted virtually via Zoom. When I entered the Zoom room, I was surprised to find that FDA staff kept their cameras off for the entire meeting while we kept ours on. The participant list was not viewable. It felt like we were addressing an invisible Greek chorus. This was a stark contrast from the standing-room-only experience of our Patient Listening Session or even the CPIM.

Like at the CPIM, the FDA challenged our presenters. They questioned whether the tool was "fit for purpose" for use in a clinical drug trial because it was long and included categories that a drug could not directly impact, like participation in activities outside of the home. They also questioned whether measuring a patient's perception of the impact of a symptom was as useful as whether the symptom objectively increased or decreased. They asked the University of Rochester to revise the tool to address their concerns. In the meantime, they again encouraged Recursion to use the Modified Rankin Scale as the primary endpoint for their trial.

## Seven years after our first FDA meeting, an industry-sponsored trial

Recursion incorporated the Modified Rankin Scale, but it also incorporated the CCM-HI -- now somewhat shorter and with questions about seizure and headache frequency -- and other patient-reported measures as exploratory endpoints in their Phase 2 safety trial. This meant the trial itself could serve as a testing ground, comparing which measure best reflected changes in patients taking the drug.

The FDA approved this design, and the first participants were enrolled in the REC-994 SYCAMORE trial in March 2022.

The approved trial design was a victory because it included secondary and exploratory endpoints that moved far beyond hemorrhage. Any future industry-sponsored trial could use the same endpoints. Our work as explainers and advocates at the FDA had borne fruit. We could be proud, and we could step back for a bit.

## New Technology: Focused Ultrasound

This past spring, I knew it was time to go back to the FDA.

About a year earlier, around the New Year of 2024, Dr. Richard Price, the primary CCM focused ultrasound (FUS) researcher, and I had a conversation confirming that we were both thinking there was enough pre-clinical evidence to move to a clinical trial of focused ultrasound.

Focused ultrasound is a non-invasive technology that uses focused, pulsed sound waves to treat disease. For CCM, the technology has been tested in combination with small gaseous bubbles called microbubbles. The microbubbles hit against the walls of the brain blood vessel when they receive focused ultrasound waves and create very small openings in the blood-brain barrier that close after a few hours. It appeared from Dr. Price's mouse studies that the microbubbles' impact triggers a wound-healing response that can stabilize and perhaps even shrink lesions. Additionally, mice did not develop new lesions in brain areas that had been treated.

With this compelling evidence, our first task was to organize a group of collaborators to plan a trial. We drove the formation of a group with the University of Virginia (UVA) neurosurgery and clinical trial protocol-writing teams, the manufacturer of a UVA-housed FUS machine, and the Focused Ultrasound Foundation, which we hoped would fund the trial. We decided on a trial design, and by this spring, we felt it was time to consider a meeting with the FDA. Ultimately, we would need the FDA's approval to launch a clinical trial of FUS in CCM patients, and we wanted to engage them early.

There are nine Centers of the FDA, each overseeing different types of treatments. Despite our years-long relationship with the FDA divisions responsible for drugs like Recursion's REC-994, the division responsible for medical devices, the Center for Devices and Radiological Health (CDRH), had no reason to know what CCM was. We had to talk to CDRH because they oversaw focused ultrasound approvals.

As a working group, we had three choices regarding how to approach CDRH, with the goal of eventually securing approval for the FUS trial.

One choice was to go ahead and submit an Investigational Device Exemption application, the authorization we'd need to get from the FDA to launch the trial. A meeting naturally follows the application. This could be quickest, but only if the FDA boned up on CCM and agreed with our trial plan. It was high-risk: if CDRH didn't know about CCM, they would likely reject our application.

Another option was to request a pre-IDE meeting to discuss our trial plan before submitting the application. This could provide valuable feedback to allow for a better initial application. It would extend the timeline to a trial because, by law, the FDA has several months to even respond to a request, let alone schedule a meeting. However, in the end, it might save time by preventing the need for a resubmission.

Finally, we could find a way to hold a meeting with the FDA before we were ready for either the IDE or pre-IDE meeting. This could allow us to introduce the CDRH to CCM and to gauge which path they thought would be best. The only way to do this was if the request came from the Alliance to Cure Cavernous Malformation, rather than

the researchers or manufacturer. The meeting would need to focus on CCM itself, not a particular trial or machine.

I decided to reach out to CDRH with a meeting request.

## Is anyone there?

Our timing could not have been worse.

In April 2025, I wrote my first message to the CDRH Assistant Director for Neurointerventional Devices that UVA knew, Dr. Sara Thompson, introducing our organization and asking for a meeting. When I got no response despite repeated attempts over the course of few weeks, I had to consider alternatives.

As you remember, this spring, the Department of Government Efficiency had made significant cuts in the FDA staffing, but the staff lists usually available online had not been updated. I wasn't sure whether Dr. Thompson was still employed by the Agency. I needed information.

In a somewhat unconventional approach, I turned to Reddit. On a Health and Human Services (HHS) subreddit, I posted a question: how would I find out whether a specific assistant director was still employed?

I got a thread of enlightening responses. The FDA staff didn't know which co-workers were still employed, and staff were not allowed to add an autoresponder to their email to indicate they were gone. None of these responses helped me find the right person. However, I did receive an anonymous private message that essentially said, "I'm a CDRH Division Director. Who are you looking for?" I learned that Dr. Thompson was indeed still at the Agency but had been out with illness.

When Dr. Price's focused ultrasound research was published in the prestigious *Nature Biomedical Engineering* journal the first week of May, I tried again, with a link to the paper. I received a nearly immediate response from Dr. Thompson, who expressed her interest in a meeting. At that time, with all the changes, we weren't sure what kind of meeting was possible. I expected it would be a conversation between Dr. Thompson and me. She scheduled us for July 17 to give some time for things to settle in case this availed us of more options.

At the end of June, Dr. Thompson let me know that she could host a 1-hour hybrid meeting and that Division Directors and any interested staff would be invited to attend. It would be held on the FDA campus in Bethesda, MD, and we could include any speakers we wanted. While that left me scrambling to create a program instead of just having a conversation, I was thrilled.

## A meeting with a multi-tasking Device Division

On July 17, four Alliance to Cure representatives visited the FDA campus in person, and two others participated virtually. Alliance to Cure Science Officer Dr. Angela Glading, long-time Alliance member and advocate Rachel Paverman, her mother Stacey, and I were on campus to share patient lived experience and needs.

It was a hot, windy day. We rode from the hotel to FDA in Stacey's car, talking about our nervousness and determination to represent the greater patient community well.

We arrived at the FDA campus with time to spare. We entered Building 66, the home of CDRH, into a voluminous 3-story atrium. We later learned that, in addition to recent staff reductions, some FDA staff were still working remotely because they live in other parts of the country and were hired as remote employees during the pandemic, leaving the building emptier than it might have otherwise been.

After going through security and a short wait, Dr. Thompson came and offered a friendly greeting. She led us to our conference room, and we started our tech setup.

Before the meeting, I had had great technology trepidation because the meeting's hybrid portion would use Microsoft Teams, and our folks were not as familiar with the platform. I had invited two remote presenters: neurosurgeon and Alliance to Cure Board Member Dr. Joseph Zabramski, who would offer clinical information, and Dr. Richard Price, who would share the pre-clinical focused ultrasound research. In the days before the meeting, I had scheduled a tech check with them to ensure they knew how to log in and present slides. I had also scheduled our combined presentations to run exactly 45 minutes, to leave enough time for a five-minute delayed start and questions. I thought we were as ready as we could be.

I hadn't realized that every A/V staff member at the FDA had been let go.

Dr. Thompson's boss, the Director of Neurointerventional Devices, Dr. Jaime Raben, and Dr. Thompson had to try to figure out the hybrid situation. It turned out we'd been given the wrong Teams link, and the new link wouldn't work for our remote presenters. Chaos and stress ensued.

We worked out the access issue fifteen minutes into our scheduled start time, so my 15-minute buffer for a late start and questions was eaten. The room was full, and we could see more attendees online.

## A powerful patient voice

The meeting started with Rachel Paverman, well-known in our community, describing the CCM hemorrhages and brain surgeries that left her with multiple physical deficits interfering with mobility, vision, hearing, speech, and the use of her right side. With assistance from Stacey, Rachel moved from her mobility scooter to a seat at the presenter's table. She read her presentation from index cards written in large print. Her message was powerful.

Rachel's world was turned upside down when she was 25, working as an event planner for professional sports teams. In her book, *To the Abyss*, she tells the story of how her life changed:

"I had five strokes, I was paralyzed, lost my voice, lost the ability to balance, lost my best friend in the world, but here I am...How I look at things is just different now - I have to be ok. I have no other choice but to be ok."

After Rachel's presentation, I followed with additional patient stories that illustrated how CCM impacts life trajectories. I also presented the results of a patient survey we administered a couple of years ago, when we asked what patients would prefer in a treatment. A treatment like focused ultrasound – non-invasive and potentially curative for some – was high on the list.

Drs. Zabramski and Price offered the technical components (with Dr. Thompson running the audio and Angela Glading running the slides).

In the end, thirty-two FDA staff were introduced to CCM, and their questions made it clear that our introduction was needed. Even more than our presentation, the hallway conversation after the meeting with Dr. Raben and Dr. Thompson, now relieved of A/V duty, provided the most insight.

They encouraged us to schedule a pre-IDE meeting rather than jumping straight to an application. They would help us structure the trial, probably as an adaptive trial, since this would be the first time FUS would be used for CCM. An adaptive trial would allow us to adjust the trial design if we found something was not working as well as we expected, instead of starting an entirely new trial.

After the meeting, Dr. Thompson emailed and thanked us for coming to campus. "Everyone was quite moved by Rachel's story," she wrote. She emphasized just how important the patient stories are in helping the FDA understand.

## Still driving, but closer to our destination

The quick update on our latest FDA meeting is this: our July 17 meeting met its objectives and provided the next steps we need to move the focused ultrasound work forward. We will schedule a pre-IDE meeting when the clinical trial protocol is finalized, and, pending FDA approval, we will enroll patients in a clinical trial in 2026.

But the long and winding story – not unlike the road I drove while planning this report – is about time, planning, setbacks, strategy, and most importantly, patient voices. Because the FDA understands our disease well, we have been granted flexibility in clinical trial designs that reflect the variability of CCM symptoms. This means that clinical trials happen more quickly and feasibly, and that a cure may happen sooner. None of these gains would have happened without our patients sharing their stories.

Together, we are driving a cure.

# CCM Center Patient Experience

We established the CCM Center of Excellence and CCM Clinical Center designation program in 2016 to improve diagnosis and care for CCM patients. At a CCM Center, be it a Center of Excellence or a Clinical Center, patients can expect high-quality CCM care provided by a multi-disciplinary clinical team. Centers of Excellence also conduct CCM research.

With the addition of Seattle Children's and Mayo Clinic in Florida this year, there are now 13 hospitals that have earned Center of Excellence designations and nine that have earned Clinical Center designations. We will continue to recognize additional hospitals that apply for the designation as we work to ensure high-quality CCM care for all.

**81%** of CCM patients/caregivers seen at a CCM Center within the last six months said the CCM care they received was better than most other healthcare experiences

**88%** of CCM patients/caregivers would recommend their CCM Center to others for CCM care

**65%** of CCM patients/caregivers who were familiar with the CCM Center network had heard of it from the Alliance to Cure Cavernous Malformation

*(Findings from 264 respondents of the July 2025 CCM Center Patient Experience Survey distributed through the CCM Patient Registry and Alliance to Cure social media, website, and email)*

*"I visited two Centers of Excellence when going through my initial second opinions. The ability to have a list of hospitals and doctors with the designation of a Center of Excellence was invaluable. Both of the Centers of Excellence gave me the same recommendations, which were opposite what my local neurosurgeon had told me. I'm eternally thankful for that. The Centers of Excellence program is such an important thing that the Alliance does. Because this condition is not well known by a lot of practitioners, being able to see doctors at the center means I know I am getting the best care I can for my condition."*

**- CCM CENTER PATIENT EXPERIENCE SURVEY RESPONDENT, JULY 2025**

*"The entire team treated us with compassion, respect, and professionalism. They took time to listen, explain, and involve us every step of the way. We truly felt cared for during an incredibly difficult time."*

**- CCM CENTER PATIENT EXPERIENCE SURVEY RESPONDENT, JULY 2025**

Do you have feedback about your experience at a CCM Center? Please email Clinical Programs Director Katie Garbarini at katie@alliancetocure.org, or take the next CCM Center Patient Experience Survey in early 2026 by enrolling in the CCM Patient Registry (ccmregistry.org) or signing up to receive our emailed updates (alliancetocure.org/sign-up-for-updates/)

# CCM Care Guidelines updated and available for free, improving access to latest CCM diagnosis and care recommendations

Any healthcare provider taking care of someone with CCM should know that their patient is at risk of headaches, seizures, or hemorrhages. They should know that lifestyle choices, like diet and use of hormones, can affect CCM activity. And they should know where to refer their patient for specialized care and second opinions.

As part of our physician education efforts, we led the two-year effort to update and publish the CCM Care Guidelines so that providers are equipped with the knowledge to deliver informed care and appropriate referrals to CCM patients.

The recommendations, "Guidelines for the Diagnosis and Clinical Management of Cerebral Cavernous Malformations of the Brain and Spinal Cord: Consensus Recommendations Based on Systematic Literature Review By the Alliance to Cure Cavernous Malformation Clinical Advisory Board Experts Panel," were published on May 21, 2025 in the journal Neurosurgery and are free to download from Alliance to Cure's website.

The original guidelines were published in 2017 and are now updated to include more recent evidence, including information about lifestyle factors impacting hemorrhage, spinal cavernous malformation, pediatric surgical considerations, diagnostic guidelines, and more.

We urge all CCM patients and caregivers to print out and share the guidelines with their healthcare providers by visiting: **alliancetocure.org/home/for-patients/newly-diagnosed/ccm-care-guidelines/**

## New CCM Centers expand access to expert CCM clinical care

We were honored to recognize Seattle Children's Hospital on Sep 12 as a CCM Center of Excellence, the first pediatric hospital in the western U.S. to earn the designation. On Aug. 7, we were pleased to name Mayo Clinic in Florida as a CCM Clinical Center. Hospitals with these designations offer expert CCM care, and Centers of Excellence also conduct research. We are proud that our CCM Centers program has grown to 13 Centers of Excellence and nine Clinical Centers since its beginning in 2016.

PATIENT STORY

# BRANDI CHAVEZ

## After a cavernous malformation bleed, a New Mexico woman searches for answers – and becomes an advocate

When the results of her CT scan came back, a week after she had begun experiencing fatigue and extreme dizziness in January 2023, Brandi Chavez heard an explanation that many patients eventually diagnosed with cavernous malformation hear on their first visit to an ER.

"The doctor said, 'I'm sorry I have to tell you,'" said Brandi, a 36-year-old mother of three from Albuquerque, New Mexico. "'You have a tumor in your brain, most likely cancer that's come from somewhere else in your body. You're having a craniotomy [in two days].'"

While Brandi and her family were grappling with her diagnosis and preparing for brain surgery, a routine pre-surgery MRI surfaced new information. Brandi's diagnosis changed: instead of metastatic cancer, she had a cerebral cavernous malformation (CCM), a mulberry-shaped cluster of capillaries that was bleeding in her cerebellum. The hemorrhaging CCM was irritating brain tissue and causing stroke-like symptoms.

Brandi and her husband were relieved to learn she didn't have cancer. But Brandi's CCM diagnosis did not provide much clarity on a prognosis. With no CCM treatment option besides a surgical resection, which Brandi's neurosurgeon deemed too risky given the CCM's location, Brandi's only course of treatment was to go home and wait for her brain to reabsorb the blood from her hemorrhage. Her doctor said her symptoms would slowly lessen as the cavernous malformation stabilized.

Once Brandi got home, though, her symptoms worsened.

"I ended up pretty much paralyzed on the right side," Brandi said. "I had to have assistance walking, going to the bathroom, getting showered. My husband would wash my hair, get me showered. Anytime my kids touched me or I moved, if I had a leg kind of tilted out, the room would start spinning. I just had to lie completely still."

Brandi's symptoms did slowly subside over the course of nine months, but calls to her neurosurgeon did not yield much information.

"I felt kind of lost, like there was no one I could go to to say, 'is this normal?'" she said. Brandi leaned on her faith and family to help her get through this time.

Within the first three months after her bleed, she made an important connection.

"I came across the Alliance website and on there was the Volunteer of the Month, and it was Christina, and I'm like, I know her!" Brandi said. Brandi grew up in Santa Rosa, New Mexico, where Alliance to Cure Board member Christina Campos and her husband, Joe, have advocated for better CCM care and awareness. Joe, along with tens of thousands of others with New Mexican heritage, has a familial CCM mutation called the CCM1 Common Hispanic Mutation. Like other inherited forms of CCM, which account for roughly 20 percent of the estimated three million cases of symptomatic CCM worldwide, the CCM1 Common Hispanic Mutation is passed down through generations and causes multiple cavernous malformations to form in the brain and/or spinal cord.

After Brandi reconnected with the Campos family, she wondered if she, too, had the same familial version. Her parents were from Santa Rosa, a CCM1 Common Hispanic Mutation hotspot, but her MRI had shown just one cavernous malformation.

In fact, because Brandi only had one lesion, four months after her bleed, her neurosurgeon had suggested that she consider stereotactic radiosurgery (gamma knife) to prevent future bleeds. For patients with sporadic CCM, where they have only one CCM that is not caused by a familial mutation, gamma knife can sometimes be used to help prevent future bleeding. For familial CCM, however, gamma knife is not recommended because the radiation can cause more CCM lesions to form.

Brandi met with the radiation oncology doctor to learn more about gamma knife.

"I asked him, I said, 'I'm worried that this is genetic, and if it can potentially cause harm down the road,'" she said. "He goes, 'I don't think yours is genetic. You only have one, so I think this is the right move. This is what I would do to my family, or what I would do to myself." Brandi went ahead with the gamma knife treatment.

Brandi's six-month gamma knife follow-up showed the radiated CCM looked slightly smaller, but two additional CCMs had developed.

Now suspecting that Brandi's CCM was familial and required longer-term monitoring, Brandi's doctors initiated a referral to the University of New Mexico (UNM) Health System, one of 22 Alliance to Cure – recognized CCM Centers of Excellence and Clinical Centers. Meanwhile, at the Campos family's suggestion, Brandi attended the annual UNM CCM Patient Conference in March 2024, where she met CCM experts and other patients. At the conference, she also received free genetic testing. The results confirmed she had the CCM1 Common Hispanic Mutation.

"I did my genetic testing because I wanted to know," Brandi said. "I wanted [my family members] to make their own choices. Even if they don't get genetic testing, if they go into the ER [with CCM symptoms], they can say, 'I have a family member with this; CCM could potentially be an option.'"

Today, Brandi has gotten more used to having CCM. She is prone to falls, and her handwriting is not as clear as it once was, but she has regained much of the lost function from her hemorrhage almost three years ago. She has six-month check-ups with the CCM team at UNM and follows a low-emulsifier diet.

She is also part of a close-knit network of CCM advocates in New Mexico.

"When I started to meet all these other [CCM community members], with their concern and care, that made all the difference," she said. "If I could be that for somebody else, that would be amazing."

In addition to educating others about CCM at community awareness events and serving on the Alliance's volunteer Community Engagement Committee, Brandi raises funds to support the Alliance's mission to drive a cure and improve lives for all affected by CCM. She was the top fundraiser for Alliance to Cure's annual community fundraiser, Strides to Cure CCM, this past June.

"I wanted to help raise money to find some sort of treatment or cure, not even for myself, but for anybody in my family who ends up having this as well," she said. "Working with the Alliance to hopefully find some sort of treatment, some sort of cure – I know they're working so hard for that, and that gives me so much hope, that there are people who care."

Strides 2025 to cure CCM

ALLIANCE TO CURE CAVERNOUS MALFORMATION

2025 Strides to Cure CCM participants: together you raised nearly **$50,000** for a CCM cure, making this year's Strides our most successful one yet!

Thank you for driving a cure and improving lives for all affected by CCM.

Stay tuned for Strides to Cure 2026 in June!





CAVERNOUS MALFORMATION
AWARENESS MONTH
ALLIANCE TO CURE



# Shine Bright for CCM

Shine Bright for CCM is our signature awareness-raising campaign during Cavernous Malformation Awareness Month. Shine Bright 2025 was our best year yet, with dozens of landmarks across the country shining red and white throughout June. Thank you to the dedicated team of volunteers led by the amazing Genise Oldenberg for coordinating this effort. If you'd like to raise awareness about cavernous malformation and the need for better treatments and a cure, please email volunteer@alliancetocure.org.

# Thank you to our community fundraisers!


*Interested in hosting an event for the Alliance? Email lindsay@alliancetocure.org*

SAVE THE DATE

## Family Weekend 2026

Mark your calendars! We are excited to announce our next Family Weekend, happening July 17–19, 2026, at the beautiful Ironwood Springs Ranch near Rochester, MN.

This casual and welcoming weekend is all about fun, laughter, and connection with others who understand the unique challenges of living with cavernous malformation. The ranch is a highly accessible, family-friendly camp with hotel-style lodging, private family sleeping rooms, and meals included.

You can look forward to fishing, swimming, nature walks, rock wall climbing, crafts, music, games, campfires, and even zip lining—with adaptive options available for all activities. Whether you're a child, adult, or whole family, this is a chance to relax, recharge, and make unforgettable memories together.

We can't wait to see you there!

ALLIANCE TO CURE
CAVERNOUS MALFORMATION

Online

# Support Groups

## WEEKLY SUPPORT

**Weekly Support Group**

Monday's
9 am PT/12 pm ET

Thursday's
4 pm PT/7 pm ET

**Care Partners**

Mondays
5 pm PT/8 pm ET

**Next Gen (Gen Z)**

Wednesdays
5 pm PT/8 pm ET

FIND ALL OUR ONLINE SUPPORT/EVENTS HERE:
ALLIANCETOCURE.ORG/EVENTS/

**1st Thursday**
- CCM 101 - 7:00 pm ET
- Regular Support Group - 8 pm ET

**2nd Thursday**
- Men's Group - 7 pm ET
- Women's Group- 7 pm ET

**3rd Thursday**
- Webinar - 7 pm ET

**4th Thursday**
- Brainstem - 7 pm ET
- Millenial Breakout during support group - 8 pm ET

**Last Friday of the Month**
- Writer's Club 2 pm ET

## PATIENT NAVIGATION

*Offering guidance and support to individuals and families affected by CCM*

Do you have questions about finding specialized care, navigating insurance, or understanding your CCM diagnosis? Email **help@alliancetocure.org** to connect with our Patient Navigator.

Here's how we can help:

- Finding Specialized Medical Care
- Understanding your Diagnosis
- Financial & Insurance Navigation
- Coping Strategies & Daily Living Support
- Emotional & Peer Support
- Clinical Trials & Research Information
- Recreation & Qualty of Life

What we cannot provide: medical advice or treatment decisions, legal services, replacement for your healthcare team, direct financial assistance, or emergency/crisis intervention.

For more information, visit
**alliancetocure.org/patient-navigation/what-is-patient-navigation/**


## 2025 CCM Patient Conference

Nov. 6-8
The Ritz-Carlton, Atlanta
Atlanta, GA

Register Here for Conference and Hotel

# CCM patients and care partners, join us for our signature in-person community event!

**Day 1**

### Get up to speed on CCM research

*Thu. Nov. 6*

- Tour of Emory's research facilities
- Late afternoon reception and poster session with scientists
- Panel with experts on CCM research and treatment development

**Day 2**

### Meet CCM doctors & learn best practices

*Fri. Nov. 7*

- Keynote on focused ultrasound as a potential CCM therapy
- Talks with docs from CCM Centers of Excellence and Clinical Centers
- Sessions on mental health, CCM healthy diet, and more

**Day 3**

### Connect with your community

*Sat. Nov. 8*

- Picnic lunch in Olympic Park
- Visit to the Atlanta Aquarium

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darla@alliancetocure.org
www.AllianceToCure.org

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# ROCKIN' FOR A Cure

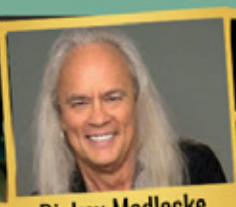
**IN SUPPORT OF GROUNDBREAKING RESEARCH TO CURE CAVERNOUS MALFORMATION**

Join us for an unforgettable evening of music, food, and celebration. This exclusive benefit will feature both live and silent auctions showcasing contributions from legendary artists including **Lynyrd Skynyrd, ZZ Top, Styx, Bret Michaels, Carrie Underwood, Lainey Wilson**, and many more. Honored guests from the world of sports have also generously contributed, with featured items from the **Mickey Mantle Estate**, **Doug Flutie**, the **Jacksonville Jaguars**, **Hendrick Motorsports**, and more.

## SPECIAL GUESTS INCLUDE


Johnny VanZant


Donnie VanZant


Rickey Medlocke


Brett Myers


Bobby Amaru


Doug Flutie


Josh Scobee


David & Danny Mantle

**NOV 5TH**
**2025**

## Porsche Jacksonville

11211 ATLANTIC BLVD., JACKSONVILLE, FL

**7 PM TO 9:30 PM** **TICKETS $150**

Sponsorships & Tickets available at **bit.ly/RockinCure** or contact Kathy Signorile at kathy@stmichaelssoldiers.org

Silent Auction opens **October 27** at **bit.ly/RockinCure**

ALLIANCE TO CURE CAVERNOUS MALFORMATION

HOMEGROWN KINDNESS PROJECT

FIELDS AUTO GROUP

ST. MICKEY'S SOLDIERS