ALLIANCE TO CURE CAVERNOUS MALFORMATION

IMPACT REPORT

FY 2024-2025 *(Oct. 1, 2023 - Sep. 30, 2025)*

Our impact, *together*

Connie Lee, CEO
Alliance to Cure
Cavernous Malformation

David Borland, Chair, Board of Directors
Alliance to Cure
Cavernous Malformation

Thank you.

You most likely know someone who has cavernous malformation (cavernoma, CCM). You know how life-changing better treatments and a cure would be.

As you'll see in this impact report, with your support in FY2024-2025, we are closer to better treatments and a cure for CCM than ever before.

Thank you for your commitment to drive a cure and improve lives for all affected by CCM. ***Thank you*** for being part of our alliance.

Together, we will create a permanent cure for CCM – available, accessible, and affordable for all.

What is cerebral cavernous malformation (CCM), and why do we need a cure?

- Cerebral cavernous malformations (CCMs) are abnormal blood vessels in the brain and spinal cord that can cause strokes, seizures, and debilitating neurological symptoms.
- One in 500 people has CCM, and while most of the time, CCM does not cause symptoms, there are approximately 160,000 children and adults in the U.S. with symptoms.
- There is no treatment for CCM besides brain or spinal cord surgery to remove lesions. If the lesions are located deep in the brain, surgery is not an option.

FY2024 - FY2025 Impact at a Glance

DRIVING RESEARCH FOR A CURE

- **Led planning of a clinical trial for low-intensity focused ultrasound, a noninvasive treatment that halted the growth of 94% of CCMs in mice** *(Nature Biomedical Engineering, 2025)*
- Launched the first coordinated research program **dedicated solely to accelerating CCM drug discovery** *(CCM CureDriver™ Lab)*
- Began negotiations for transfer of assets from Recursion Pharmaceuticals following SYCAMORE – **CCM's first industry-developed drug trial** – which showed promising results in a subset of patients
- **Convened 100+** CCM scientists at our 20th International CCM Scientific Meeting, the largest and longest-running conference dedicated to advancing CCM research
- **5** new treatments entered the global CCM clinical pipeline

IMPROVING CARE

- **3,000** patients seen at our network of recognized CCM Centers of Excellence and Clinical Centers
- **60** updated and expanded recommendations for CCM diagnosis, monitoring, and treatment published in *Guidelines for the Diagnosis and Clinical Management of Cavernous Malformation (Neurosurgery, 2025)*

EMPOWERING PATIENTS

- **75,000** CCM patients reached through education programs, support groups, community events, and online resources

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ON THE COVER: Young members of the cerebral cavernous malformation (CCM) community at our first-ever CCM Family Weekend in 2024.

NOTE: This publication reports on Alliance to Cure Cavernous Malformation's fiscal years 2024 and 2025, from October 1, 2023 - September 30, 2025. For information beyond the scope of this reporting period, please visit alliancetocure.org.

Our Vision

A world with a permanent cure for cavernous malformation – available, accessible, and affordable for all.

Our Mission

Drive a cure and improve lives for all affected by cavernous malformation.

Our Programs

- Drive research for a cure
- Strengthen clinical care and improve access
- Support and empower patients and caregivers
- Lead & partner to increase impact

Our Values

THINK BIG AND ACT BIG. We are in urgent pursuit of finding a permanent cure for CCM. We will persevere until no one ever suffers from CCM again. We innovate, collaborate, and aim high to achieve our goal.

EARN TRUST, ALWAYS. We strive to be a trusted partner who can always be counted on to do the necessary work, and to act with transparency, discretion, and integrity in everything we do. We believe that a culture of trust makes us more productive and better collaborators.

CHAMPION EACH OTHER. Patients and their families are our North Star. Every day we aim to support each other as we face the many challenges from CCM in all its forms. Whether it is with resources, support, or our vision for a cure and better treatments, we hold our community close.

SEIZE OPPORTUNITIES. We believe that developing a cure requires science and so much more. So, we listen for ideas from endless sources–from global experts to individual families–to synthesize viewpoints from this broad base and understand the big picture. This is our superpower: we identify and create sweet spots where science, patients, and partners converge to move us forward to the cure.

Advancing promising noninvasive CCM treatments and cures

A non-invasive potential treatment for CCM families: *Focused Ultrasound*

We accelerated the development of low-intensity focused ultrasound (LIFU) for CCM, a potentially groundbreaking noninvasive treatment. Preliminary data presented at our 2023 International CCM Science Meeting showed that LIFU halted the growth of 94% of CCM lesions in a CCM mouse model. In 2024, we began planning a clinical trial of LIFU to determine if LIFU could one day be an effective treatment – or even a cure– for our CCM families, especially those with currently inoperable CCM lesions.

YOUR 2024-2025 IMPACT:

- In early 2024, we organized the stakeholders – the University of Virginia pre-clinical and clinical trials teams, the manufacturer NaviFUS, and the Focused Ultrasound Foundation – to introduce the idea of a clinical trial and create a plan.
- We initiated a meeting with the Food and Drug Administration (FDA) – approver of the trial – to educate them about CCM and the need to test LIFU. Only the Alliance to Cure could do this.
- The University of Virginia and the Alliance to Cure developed and revised the LIFU study protocol (the design of the clinical trial) to ensure FDA permission to move forward with a trial. Pending FDA approval, we will enroll CCM patients in a safety trial of LIFU by early 2027.

CCM CureDriver™ Lab: Expediting preclinical discovery and de-risking industry investment

CCM drug development has traditionally faced two systemic barriers: 1) access to CCM mouse models and 2) lack of incentives to test repurposed drugs for CCM. We launched our CCM CureDriver™ Lab in 2024 to bypass these roadblocks by developing a CCM mouse colony and conducting preclinical testing on repurposed drugs. Such programs are rare for patient-advocacy organizations to undertake because of the scientific expertise and expense involved, and we experienced setbacks in our first year of the program (2024). In 2025, however, we made progress in our program to expedite preclinical research.

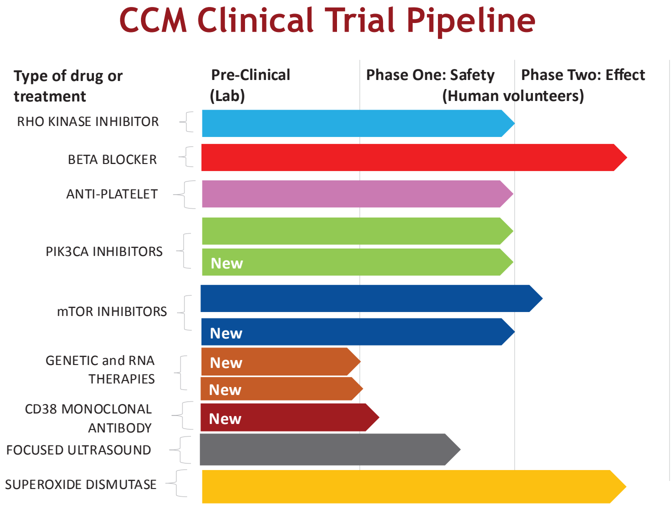
FY2025 HIGHLIGHTS:

- We laid the groundwork for developing a CCM mouse colony in 2026 at a leading nonprofit biomedical research organization so that drug companies can more easily test their compounds without undertaking the time-consuming process of developing their own CCM mice. Our previous attempt in 2024 to establish a mouse colony at a different scientific research organization was unsuccessful. The colony is expected to be fully developed by the end of 2026.
- We contracted with an experienced CCM researcher to develop a 10-arm study protocol for testing five repurposed drugs and drug combinations in CCM mouse models to generate data for advancing these drugs through the clinical trial pipeline. (These drugs are known to interact with proteins and pathways involved with CCM.) Results are expected by the end of 2026.

A deep pipeline of potential treatments, built with our guidance and research tools

The CCM Clinical Trial Pipeline shows all known CCM treatments under development worldwide. Since 2023, five new treatments have entered the pipeline.

In 2024-2025, we drove treatment development through ongoing technical guidance and three critical resources: our International CCM Scientific Meeting, Patient Registry, and Biobank.


CCM Clinical Trial Pipeline

International CCM Patient Registry Our Patient Registry is overseen by an external International Review Board. It is at ccmregistry.org.

IN 2024-2025:

- 3,352+ surveys completed by patients for researchers
- 826 patients enrolled in our Registry, representing a 150% increase since 2023

CCM Bioank. Patients can consent to donate biosamples like blood, saliva, and resected cavernous malformation lesions for research when they join the Patient Registry. For scientists, the biobank is a powerful resource - a treasure.

IN 2024-2025:

- 158 patients consented to donate biosamples, growing by 24% since 2023
- 44 patients donated samples to our biobank, bringing the total to 1173

International CCM Science Meeting

In July 2025, we held our 20th International CCM Science Meeting in Toronto, convening more than 100 leading CCM scientists to share preliminary data, form collaborations, and deepen CCM understanding. Most major CCM research breakthroughs from the last 20 years have grown out of these meetings.

Finding a path forward after the REC-994 trial

In 2024, Recursion Pharmaceuticals shared results from their Phase 2 SYCAMORE trial, the first industry-sponsored clinical trial of a drug developed for CCM. The drug, REC-994, was found to be safe and well-tolerated, and there were encouraging exploratory efficacy trends, including decreases in lesion volume and improvement in overall function and CCM-associated symptoms. We had worked alongside Recursion for a decade to make this trial happen, from advising on trial design and educating the FDA, to mobilizing patients to enroll. However, in the spring of 2025, Recursion discontinued the REC-994 trial. Intent on understanding why encouraging trends were observed in some patients, we immediately began negotiations with Recursion to inherit assets from the trial. We finalized the negotiations in 2026 and will begin conducting analyses as to why a portion of trial volunteers responded well to REC-994. Our hope is that further analysis could provide a rationale for re-evaluating REC-994 as a viable treatment for some groups of patients.

More, **better** care options for CCM families

A network of experienced, multidisciplinary CCM care teams

Our CCM Center program recognizes 22 U.S. hospitals that we've designated as CCM Centers of Excellence or CCM Clinical Centers. CCM Centers offer experienced, multi-disciplinary care for adults and children affected by CCM. The program makes it easier for families to locate specialists with a deep understanding of CCM.

YOUR 2024-2025 IMPACT:

- 3,000+ CCM patients served at CCM Centers annually *(alliancetocure.org/ccmcenters)*.
- We recognized three more hospitals as CCM Centers: Texas Children's, Seattle Children's, and Mayo Clinic in Florida.

AJ, 6, three months after his brain surgery at a CCM Center of Excellence.

AJ's family traveled 1,750 miles so that AJ could get treatment for his CCM seizures at a CCM Center in 2025. AJ's parents learned about the experienced, multi-disciplinary CCM care at CCM Centers after connecting with the Alliance.

> "We know how lucky he is to have had such a great medical team, surgeon, and advocates at the Alliance."
>
> – Jennifer, AJ's mom

Health provider outreach and education

We strive to offer health providers the most up-to-date information on the diagnosis and care of CCM families. In this reporting period, we published *Guidelines for the Diagnosis and Clinical Management of Cavernous Malformations of the Brain and Spinal Cord: Consensus Recommendations Based on a Systematic Literature Review by the Alliance to Cure Cavernous Malformation Clinical Advisory Board Experts Panel in Neurosurgery* (May 2025). The updated guidelines reflect new research on CCM and diet, hormones, pregnancy, the safety of everyday medicines, and more. They are our #1 website download (alliancetocure.org/careguidelines).

LIVES CHANGED IN 2024-2025

We provided support, information, and community to approximately 75,000 individuals affected by CCM in 2024-2025 through support groups, informational webinars, our comprehensive website (alliancetocure.org), online communities, and in-person events.

KEY ACHIEVEMENTS

- Our first CCM Family Weekend: In the summer of 2024, we hosted 70+ children and adults living with CCM at a lodge in the Adirondacks for our inaugural CCM Family Weekend. Participants met others with CCM for the first time and left with lasting friendships.
- 1:1 Patient Navigation launched in April 2025: We have already connected nearly 100 individuals with complex cases to necessary care and resources.
- Key support groups added: In addition to our long-standing twice-weekly patient support groups, we began holding regular groups specifically for individuals affected by spinal cord CCM, individuals affected by brainstem CCM, young adults, women, men, and caregivers.

In 2025, Karen began experiencing urgent CCM symptoms and was being advised to undergo an invasive, high-risk procedure by her local providers. Through our newly-launched **Patient Navigation program**, we helped connect Karen to a Center of Excellence for timely evaluation so she could better understand her options and make an educated decision about next steps.

Through Alliance to Cure, I've finally found my community. Here, I'm understood, informed, and connected in ways that have transformed my life. For anyone newly diagnosed, I'd say: join the Alliance. Learn. Connect. Find your safe place to share. You don't have to go through this alone.

– Maria, CCM stroke survivor

"I'm very grateful to the Alliance."

– Ben (CCM stroke survivor) and his wife, Nicole, at our first CCM Family Weekend in upstate New York in 2024

Awareness-Raising & **Advocacy**

State and Federal Advocacy Highlights (2024-2025)

- Secured an additional $300,000 in New Mexico state funding to improve care for CCM patients in New Mexico, leading to a total of $1 million allocated to CCM care since 2023. New Mexico has the highest concentration of CCM patients in the world.
- Organized CCM families to share their stories with U.S. Senate offices, leading to the correction of a critical mistake in the 2022 Inflation Reduction Act that had discouraged rare disease drug development.
- Successfully advocated the U.S. Department of Labor to include clinical trial participation in the Family and Medical Leave Act (FMLA).

Proud to be members of:

American Brain Coalition

Coalition to Transform Clinical Trial Engagement.

CZI Rare as One

Global Genes Leadership Council

NORD

Rare Disease Diversity Coalition

Rare Disease Legislative Advocates

Rare Diseases Clinical Research Network

Rare Epilepsy Network

Save Rare Treatments Task Force

Minneapolis's I-35W Saint Anthony Falls Bridge illuminated on June 13, 2025 for all families affected by CCM. Our annual Shine Bright campaign takes place each June, during Cavernous Malformation Awareness Month.

Leadership & *Financials*

BOARD OF DIRECTORS (2024-2025)

David Borland *(Chair)*
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Helen Kim, MPH, PhD
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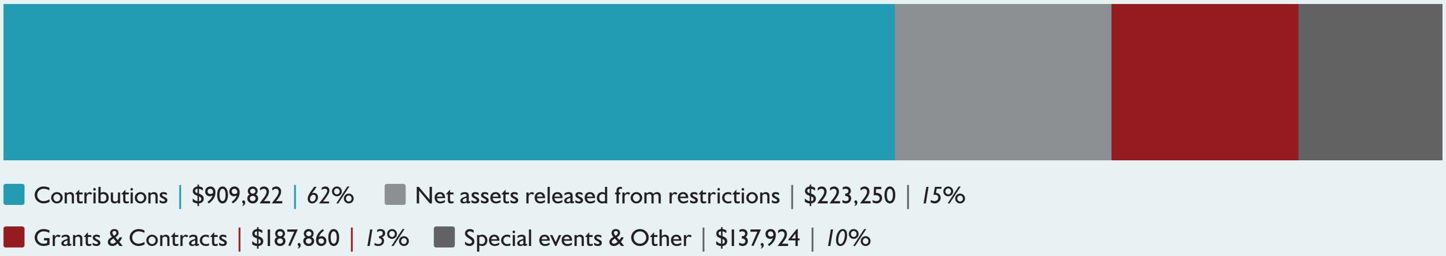
Kelly Flemming, MD

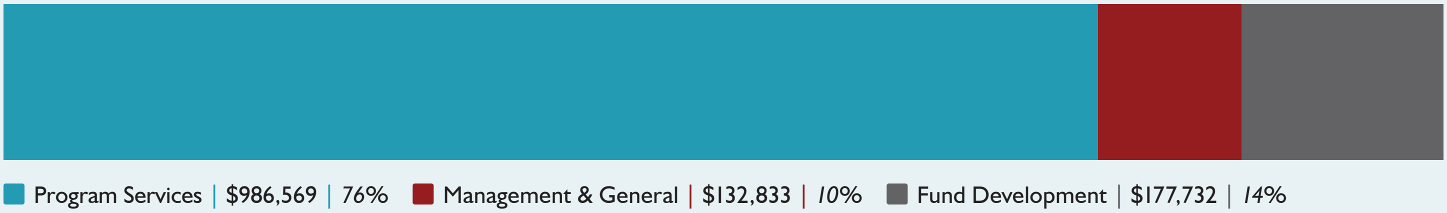
Financials Highlights - FY25

For full audited statements for FY25 and FY24, visit *alliancetocure.org/financials*

FY25 TOTAL REVENUE | $1,458,856



EXPENSES (FOR YEAR ENDING SEP 30, 2025) | $1,297,134



Recursion

Ovid Therapeutics

graviton bioscience

Boston Children's Hospital

NEURELIS™

Stanford HEALTH CARE

AT THE FOREFRONT UChicago Medicine

Barrow Neurological Institute

Texas Children's®

Major Fundraisers

Chip-in for a Cure Golf Tournament (MD)

Colorado Alliance to Cure Cavernous Malformation Charity Golf Scramble (CO)

Elle's Angels Holy Smokin' Bar-B-Que Bash (GA)

Orange County Walk (CA)

Heine Strong Benefit Golf Tournament (MO)

MadoroM Annual Wine Release Dinner & Charity Auction (CA)

Ocean State Makos Memorial Golf Tournament (RI)

Rocking for a Cure (MD)

Saberseminar (IL)

Strides to Cure CCM (nationwide)

Swinging for a Cure Golf Tournament (CA)

Torrington Wine Tasting (CT)

ALLIANCE TO CURE
CAVERNOUS MALFORMATION

977 Seminole Trail PMB 367
Charlottesville VA 22901
info@alliancetocure.org
AllianceToCure.org

A copy of the latest financial report, registration filed by this organization, and a description of our programs and activities may be obtained by contacting us at: Alliance to Cure Cavernous Malformation, 977 Seminole Trail, PMB 367, Charlottesville, VA 22902, info@alliancetocure.org. If you are a resident of one of the following states, you may obtain financial information directly from the state agency. **Angioma Alliance d/b/a Alliance to Cure Cavernous Malformation, EIN 02-0600697**, complies with state charitable registration requirements.

- Florida – A COPY OF THE OFFICIAL REGISTRATION AND FINANCIAL INFORMATION MAY BE OBTAINED FROM THE DIVISION OF CONSUMER SERVICES BY CALLING TOLL-FREE, WITHIN THE STATES, 800-435-7352 (800-HELP-FLA) OR BY VISITING www.800helpfla.com. REGISTRATION DOES NOT IMPLY ENDORSEMENT, APPROVAL OR RECOMMENDATION BY THE STATE. Florida Registration CH20096
- Georgia – A full and fair description of our programs and our financial statement summary is available upon request at our office and email indicated above.
- Colorado – Colorado residents may obtain copies of registration and financial documents from the office of the Secretary of State, 303-894-2860, www.sos.state.co.us/ Reg. No. 20063003635.
- Maryland – For the cost of copies and postage, from the Office of the Secretary of State, State House, Annapolis, MD 21401.
- Michigan – MICS # 35000
- New Jersey – INFORMATION FILED WITH THE ATTORNEY GENERAL CONCERNING THIS CHARITABLE SOLICITATION AND THE PERCENTAGE OF CONTRIBUTIONS RECEIVED BY THE CHARITY DURING THE LAST REPORTING PERIOD THAT WERE DEDICATED TO THE CHARITABLE PURPOSE MAY BE OBTAINED FOR THE ATTORNEY GENERAL OF THE STATE OF NEW JERSEY BY CALLING 973-504-6215 AND IS AVAILABLE ON THE INTERNET AT: http://www.state.nj.us/lps/ca/charfrm.htm. REGISTRATION WITH THE ATTORNEY GENERAL DOES NOT IMPLY ENDORSEMENT.
- New York – Upon Request, Attorney General Charities Bureau, 102 Broadway, New York, NY 10271
- North Carolina – Financial information about this organization and a copy of its license are available for the State Solicitation Licensing Branch at 919-807-2214. This is not an endorsement by the state.
- Pennsylvania – The official registration and financial information of Angioma Alliance may be obtained from the Pennsylvania Department of State by calling toll-free within Pennsylvania 800-732-0999. Registration does not imply endorsement.
- Virginia – State Division of Consumer Affairs, Department of Agriculture and Consumer Services, PO Box 1163, Richmond, VA 23218.
- Washington – Secretary of State at 800-332-4483 or http://www.sos.wa.gov/charities/. REGISTRATION WITH A STATE AGENCY DOES NOT CONSTITUTE OR IMPLY ENDORSEMENT, APPROVAL OR RECOMMENDATION BY THAT STATE.

OUR MISSION is to drive a cure and improve lives for all affected by cavernous malformation.

OUR VISION is a permanent cure for cavernous malformation – available, accessible, and affordable for all.

WAYS TO GIVE

By check, mail to this address:
Alliance to Cure Cavernous Malformation
977 Seminole Trail Box 367
Charlottesville VA 22901

Through a donor advised fund (DAF)
Our EIN: 02-0600697
Our legal name: Angioma Alliance d/b/a Alliance to Cure Cavernous Malformation
The address associated with our account:
Alliance to Cure Cavernous Malformation
977 Seminole Trail PMB 367
Charlottesville VA 22901

Stock
Our brokerage firm: Fidelity
Our account number: Z40135019
DTC number: 0226
Our account title: Alliance to Cure Cavernous Malformation
The address associated with our account:
Alliance to Cure Cavernous Malformation
977 Seminole Trail PMB 367
Charlottesville VA 22901
Please email dwinchester@alliancetocure.org when donating stock

By credit card (3% fee applies)
Visit alliancetocure.org/donate

By wire/ACH, or other inquiries
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Alliance to Cure Cavernous Malformation is a non-profit 501(c)3 tax-exempt organization under the legal name Angioma Alliance. All contributions are tax-deductible. Our EIN is 02-0600697.

Charity Navigator
FOUR-STAR

Platinum Transparency 2026
Candid.