

2023 IMPACT REPORT

ALLIANCE TO CURE

CAVERNOUS MALFORMATION



Dear friends,

As family members of cavernous malformation patients, we are in urgent pursuit of better treatments and a cure for this rare disease that causes so much suffering and anxiety.

Cavernous malformation is a cerebrovascular disease where leaky blood vessels form in the brain or spinal cord and bleed, leading to debilitating symptoms in an estimated 138,000 Americans. Patients with this diagnosis may suffer symptoms like migraines, seizures, and strokes, at any age. The only treatment is brain or spine surgery, which can lead to additional deficits.

But we are hopeful: with your generous support in 2023, we are closer than ever to better treatments and a cure.

The Alliance to Cure Cavernous Malformation is the leading global organization driving research, expanding access to care, and supporting patients. Since our founding in 2002, we have served as a hub that connects researchers, clinicians, patients, and pharmaceutical and government leaders working to find a cure for cavernous malformation.

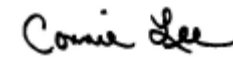
You'll see in this impact report that 2023 was a landmark year. We drove research forward for 10 possible treatments, expanded expert care to 20 Centers of Excellence and Clinical Centers, provided support and education to 25,000

patients and their families, and received recognition for our rare disease organization leadership.

We invite you to read more to celebrate the impact you have made. None of this would be possible without your support.

Together, we will make better treatments and a cure for cavernous malformation a reality.

In gratitude,



Connie Lee
Founder and CEO
Alliance to Cure Cavernous Malformation



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



2023 Highlights

Better access to care. Better patient outcomes. More treatments in development than ever before.
With your support, the Alliance to Cure Cavernous Malformation is in urgent pursuit of a lasting cure.

Operating budget was \$1 million for fiscal year ending Sep 30, 2023

Informed and supported
25,000+
Patients

Supported research of
10 Possible Treatments

Drove full enrollment of
60 Patients in the 1st Industry-sponsored Clinical trial

for cavernous malformation drug REC-994 by Recursion

2023 Recipient of the
Abbey S. Meyers Leadership Award

Given by the National Organization for Rare Disorders (NORD)

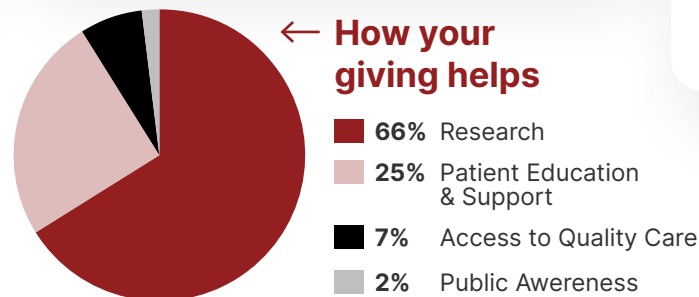
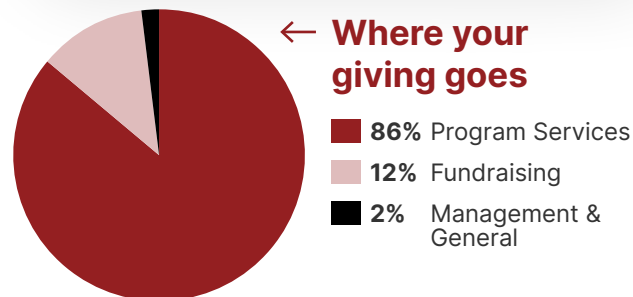
Expanded our network to
20 Centers of Excellence and Clinical Centers

Placing most Americans within a reasonable travel distance to expert care

For the second year in a row, donors like you gave

\$1 million

to drive progress towards a cure



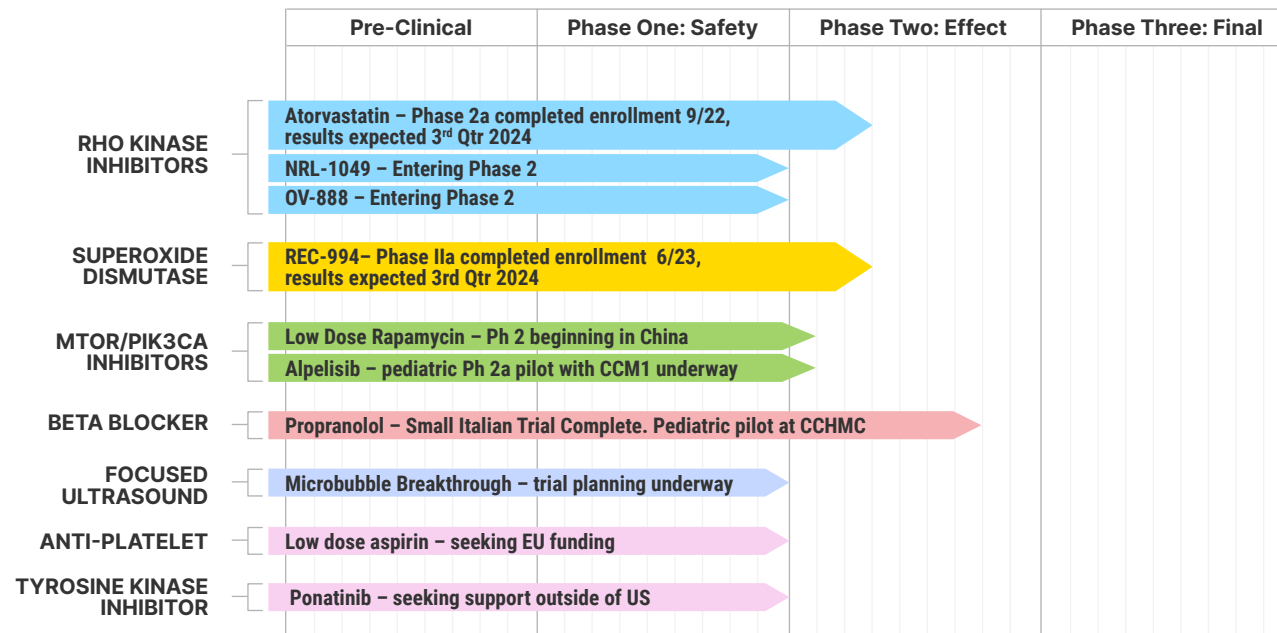
Driving Research for Better Treatments and a Cure

Brain or spinal cord surgery is the only way to treat cavernous malformation patients with severe symptoms because there is no FDA-approved drug treatment for the condition. Alliance to Cure works with researchers in academic labs and within the pharmaceutical and biotech industries to develop safe, effective treatment options for patients. Over the last two decades, we have been at the forefront of deepening cavernous malformation understanding and bringing promising non-invasive treatments to clinical trials.

10

Potential cavernous malformation treatments in the clinical trial pipeline, a doubling in the last five years.

Cavernous Malformation Treatment Pipeline



Trent enrolls in the first industry-sponsored clinical trial of a drug treatment for cavernous malformation, Recursion's REC-994, at Penn Medicine. Trent enrolled in the trial after registering with Alliance to Cure's international Cavernous Malformation Patient Registry, a database of 4,000 patients that enables researchers to find clinical trial participants.

2023 Research Successes

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After partnering with biotech company Recursion to launch the first industry-sponsored clinical trial of a cavernous malformation treatment in 2022, we mobilized our patient community to participate, leading to an **ahead-of-schedule full trial enrollment in 2023, with expected results in 2024**. Efficient trial enrollments incentivize companies to invest in rare disease drug development. We have also continued working closely with Neurelis, and Ovid Therapeutics and Graviton sought us out to initiate a drug development and clinical trial partnership in 2023.

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Our partnerships with research institutions helped advance promising treatments. University of Virginia researchers studied focused ultrasound's effect on cavernous malformation and reported the non-invasive treatment's potential to **prevent lesion development and growth**. The *Lancet Neurology* published findings from an Italian trial showing propranolol's safety and possible benefit in treating cavernous malformation. The University of Chicago's atorvastatin trial continued, with results expected in 2024, and additional studies of existing treatments began, including Duke University's gene therapy study and the University of Pennsylvania's research into drug delivery via nanoparticles. Alliance to Cure shaped all these studies as a funder, advisor, patient liaison, or facilitator.

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We made clinical trials more feasible by streamlining data-gathering. Our partners at the University of Rochester finished the CCM Health Index, a tool that allows researchers to track changes in patients' reported health outcomes during clinical trials. The Trial Readiness Project ended, **providing useful data** to research centers preparing future clinical trials. And ongoing work by the University of Chicago to evaluate whether biomarkers in blood samples can be used instead of MRI to track hemorrhages is enabling more efficient clinical trials.

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Our patient community continued to make better treatments and a cure possible. They identified research priorities in our Patient-Driven Research Map, and **700 new patients joined our 4,000-member Patient Registry**. More than 100 patients consented to donate tissue or DNA for researchers' future use, adding to a biobank program that overall has enabled the donation of nearly 250 samples.



Attendees gather for Alliance to Cure's 19th Annual International CCM Science Meeting held in Miami in July 2023. A collaborative setting for researchers, clinicians, and representatives from industry and government, the CCM Science Meeting has evolved from a forum for sharing basic cavernous malformation science research to one that focuses on potential treatments.

Improving Cavernous Malformation Care

Many cavernous malformation patients face medical challenges that are common within the rare disease community, including delayed diagnoses and ineffective treatment plans. We work to improve access to quality care for all cavernous malformation patients by removing barriers to diagnosis, equipping clinicians with evidence-based care guidelines, and connecting our community members to doctors with cavernous malformation expertise within our network of recognized Centers of Excellence and Clinical Centers.



Pediatric Cerebrovascular Neurosurgeon Dr. Sandi Lam and Alliance to Cure CEO and Founder Connie Lee hold a plaque commemorating Lurie Children's Hospital's recognition in 2023 as a Clinical Center for cavernous malformation.



Before 2016, no U.S. hospitals were recognized as centers of excellence for cavernous malformation. Thanks to Alliance to Cure's Centers of Excellence program, patients today can receive expert care at **20 Centers of Excellence and Clinical Centers across the country.**

Lurie Children's Hospital in Chicago joined Alliance to Cure's network of Clinical Care Centers in July 2023.

Photo credit: Lurie Children's.

Access to Quality Care Successes



In 2023, we added **three more hospitals to [our network of 20 recognized Centers of Excellence and Clinical Centers](#)**, placing most Americans within a reasonable travel distance of expert cavernous malformation care. Treating a combined total of approximately 2,000 cavernous malformation patients a year, doctors at these centers provide specialized, interdisciplinary care and uphold national clinical care guidelines created by Alliance to Cure's Scientific Advisory Board.



We provided free genetic testing to families who encountered barriers to diagnosis so they could know whether their symptoms were caused by a hereditary form of cavernous malformation. Over the last eight years, Alliance to Cure has enabled **400 families** access to this knowledge, which can influence patient treatment plans.



We connected patients from communities that are underrepresented in the Cavernous Malformation Patient Registry to better treatment through outreach, patient education, genetic testing, and referrals to Centers of Excellence. An example of our efforts is our Breaking Barriers initiative, which has led to a **400 percent increase** in the number of Black patients on the Patient Registry since 2021.



Our [Clinical Care Guidelines](#), cited in **more than 300 scientific papers** since their publication in *Neurosurgery* in 2017, continue to enable clinicians around the globe to provide evidence-based care and guidance to their patients.



Alliance to Cure brought patients, family members, clinicians, researchers, and industry representatives together at the 2023 Patient and Science Conference.

Connecting patients to expert care

After years of numbness, aches, and misdiagnoses, an excruciating bout of pain brought Ana to a doctor's office in Anchorage, Alaska, in March of 2023. An MRI showed a bleeding cavernous malformation in Ana's spinal cord, and Ana's doctor recommended she have an experienced neurosurgeon remove the lesion.

Ana searched online about her condition. "I found the Alliance through Facebook, thank God," she says. She also found the list of Alliance to Cure's recognized Centers of Excellence, which includes Stanford Health in Palo Alto, California. Ana referred herself there to find out more.

"I have never worked with a medical center that was so on top of things," says Ana. Centers of Excellence have specialized teams that follow the national Cavernous Malformation Clinical Care Guidelines. They also treat more cavernous malformation patients than typical hospitals, and Ana learned that the Stanford team was experienced in performing spinal cord surgeries, the rarest type of lesion.

Even at a Center of Excellence, "to make that decision to have surgery is really difficult," Ana

explains. A risk of spinal cord surgery was paralysis from the waist down, but opting not to have surgery could have resulted in a paralyzing stroke if Ana's cavernous malformation continued to hemorrhage.

Ana decided to have her surgery done by the specialized team at Stanford in August 2023, rather than wait for a potential emergency surgery scenario in Alaska. The Stanford neurosurgery team removed the lesion without serious complications. Nine days after the surgery, Ana was able to walk with a walker.

"Everyone was floored by how soon I was walking," she said. "I think a lot of that is going to Stanford, going to the best."

Ana is also thankful for *Alliance to Cure*. "There's so much good information," she says. "The support groups, those have really been a lifesaver for me."

Seven months post-surgery, Ana is back in Anchorage. She is still recovering, but she is able to walk with a cane, and she feels thankful. "I lived," she says. "Somebody went into my spinal cord and I survived it." ●



After learning about *Alliance to Cure* and its recognized Centers of Excellence, Ana had surgery at Stanford Health to remove a hemorrhaging spinal cavernous malformation that threatened paralysis. Stanford is part of Alliance to Cure's network of Centers of Excellence and Clinical Centers, 20 hospitals recognized by Alliance to Cure for providing high-quality, interdisciplinary care for cavernous malformation patients.

Empowering patients through education

Alliance to Cure helps cavernous malformation patients lead their healthiest lives possible. Through education programs like our Patient-Expert Course, CCM-101 virtual class, monthly webinars, and comprehensive website, alliancetocure.org, patients learn how to manage their disease through recommended care-seeking practices and lifestyle choices. As rare disease patients, they also learn the critical role they can play in finding a cure by participating in clinical trials.



Published in 2023, our children's book *Biscochito* tells the story of a boy and his grandmother, who helps him understand her cavernous malformation diagnosis. *Biscochito* will soon be available in more than 100 school libraries in New Mexico.



Our clinical trial education campaign mobilized a diverse patient group to participate in the Recursion trial, leading to a completed, ahead-of-schedule enrollment in 2023.

During a May 2023 *Alliance to Cure* webinar, hemorrhage and brain surgery survivor Ty shares the knowledge he learned from Alliance to Cure's Patient-Expert Course that helped him manage his diagnosis and treatment plan. Patient-experts are certified by Alliance to Cure to educate community members about basic disease knowledge and patient resources.



Patient education successes

In 2023, our monthly online Introduction to Cavernous Malformation (CCM-101) course and in-depth online [Patient-Expert Certification](#) course taught 100 newly diagnosed and experienced patients and caregivers recommended care practices, cavernous malformation biology, and lifestyle choices to help mitigate symptoms. Over the years, patient-experts have shared best practices online and with their own families and communities, **collectively touching thousands of lives.**

We helped patients stay on top of the latest cavernous malformation research and care recommendations through monthly webinars hosted live and on our [YouTube channel](#), which logged **nearly 32,000 views and over 2,200 viewing hours** in 2023. Popular topics included women's health and cavernous malformation, headaches and pain management, and surgical perspectives on ruptured brainstem cavernous malformations.

Our website, alliancetocure.org, continues to be the one-stop-shop for patients and caregivers around the world to find comprehensive cavernous malformation information, resources, and research news.

CASE STUDY

Patient empowerment through knowledge

David's symptoms began when he was working from home in North Carolina during the summer of 2021, typing an email.

"I had a weird vision change, like my world shifted slightly," he says. "It kind of felt like my brain was short-circuiting."

David was having a stroke. At the ER, doctors identified a hemorrhaging sporadic cavernous malformation on his brainstem. David's symptoms began to resolve, and surgery to remove the lesion was scheduled for two months later. At home, recovering from his stroke and awaiting surgery, David searched online to learn about his new diagnosis.

"Thank God I found the Alliance to Cure", he says.

Through Alliance to Cure's patient-education resources, which do not offer personalized medical advice but do provide a general understanding of the disease and its management, David learned that brainstem surgery carries serious risks that must be properly considered. He sought second opinions from surgeons, some from Alliance to Cure's recognized Centers of

Excellence, who agreed that David's cavernous malformation could be conservatively managed for the time being.

David opted not to go through with the surgery. Instead, he established care at Duke University Hospital's Center of Excellence in Durham, North Carolina, and made changes to his lifestyle that he had learned from Alliance to Cure's patient-education resources.

"I basically tried to do everything I possibly could to help my brain heal by eating a brain-healthy diet and eliminating emulsifiers," he says. "I really think that all of that stuff has helped me get to where I am today."

Two years after his stroke, David has not had another bleed. He now educates other patients and caregivers about cavernous malformation and how to cope with the anxiety that often accompanies the diagnosis. He has made lasting friendships with cavernous malformation patients and actively participates in Alliance to Cure's virtual support group.

"I feel like [Alliance to Cure] saved my life," he says. "I am so grateful for the Alliance for providing all the information."



After taking Alliance to Cure's Patient-Expert Course and connecting with other patients through Alliance to Cure's support groups, David made lifestyle changes to manage his cavernous malformation diagnosis.

Fostering a caring community

Many cavernous malformation patients and their caregivers feel alone when first diagnosed. The disease is rare and not well known by many doctors, and finding others going through the same thing is difficult. Alliance to Cure improves patients' well-being by [building a community](#) that provides support, understanding, and friendship.

Patient support successes

We continued to host twice-weekly virtual support groups that brought together more than **200 patients from around the world**. The support group encourages participants to share their progress and setbacks in a safe setting, and many attendees find comfort simply by listening. Common questions and topics include disease symptoms, treatment options, insurance challenges, and coping strategies.

Our 7,600-member Facebook group, Alliance to Cure Cavernous Malformation (cavernoma, cavernous angioma), grew by 10 percent in 2023. The group is an active forum where patients and caregivers share their experiences and ask questions throughout the day. Staff members and



Cavernous malformation patients and family members gather at the 2023 Patient and Science Conference in Miami, FL on July 6-8, 2023. Patient conferences bring patients and family members together to find information, support, and community.

certified patient-experts provide answers, encouragement, and guidance for finding resources.

We helped patients and caregivers find a community close to home through our U.S. regional collaboration groups. Hosted virtually each month, these groups provide a space for patients and caregivers to meet online and in person. Groups have met to tour their local Centers of Excellence, share meals, or plan fundraisers. **In 2023, we expanded this program to 15 regions throughout the U.S.**

We hosted a national Patient Conference alongside our Science Conference in Miami in July 2023. The family-friendly agenda allowed many children and adults to meet others with cavernous malformation **for the first time**. Patients also had the opportunity to share their stories with researchers and clinicians. ●



Our 2023 Patient Conference included activities for younger patients and family members to have fun and make new friends.



“Our members are there for each other during the support groups, and they’re there for each other all week long.”

- Darla Clayton, Alliance to Cure Cavernous Malformation National Community Relations Specialist

What does **Support Group** mean for patients?



"The support that we get, it's a lot different [than] talking to your regular friends. When you talk to people who actually have it, it really gets to your heart."

- Amanda



"It is really positive to have a community of people who understand the process that we all go through because having [cavernous malformations] in your brain is not common."

- Anna



"A lot of times the doctors don't understand what you have or what you're going through, but it's so nice to have this group because someone usually does, and they know exactly how it is to go through it. You get answers."

- Kristen



"I'm an older person and I basically have no relatives or friends close by and the support group has become my family. It's really important."

- Jeffrey



"The support group felt like a family to me and made me feel like I wasn't alone after getting a terrible diagnosis of having a vascular malformation that might bleed sometime."

- David



"The support group lets me calm myself. It shows me that I'm not alone. That we're all on this journey together. Someday we'll find a cure."

- Allison



"You can talk to anybody about anything. I learn stuff every week. I just love being here every week, twice a week."

- Rox



"We're actually from several different parts of the world and it feels really good to have the support. [Cavernous malformation] is a rare disease so it's most likely more valuable for a person who has a rare disease to have this opportunity to meet other persons. It's very important."

- Sussie

Driving systemic change through advocacy and awareness-raising

In the policy world, rare diseases like cavernous malformation are often overlooked and lost without a champion. We are proud to be driving systemic change forward in our mission to find a lasting cure.

Our decades-long education campaigns and partnerships in New Mexico laid the groundwork for **securing more than \$700,000 in 2023-2024 from the New Mexico state legislature to improve cavernous malformation care.** This region has the highest concentration of cavernous malformation patients in the world due to a hereditary genetic mutation called the CCM1 Common Hispanic Mutation.

In 2023, we were panelists at conferences sponsored by NORD, Global Genes Rare Equity Summit, the World Orphan Drug Congress, and the Chan Zuckerberg Initiative. Each of these opportunities allowed us to raise awareness about cavernous malformation and effective patient programming to hundreds of medical professionals, pharmaceutical executives, and other patient advocacy organizations. We received the 2023 Abbey S. Meyers

Leadership Award from the National Organization for Rare Disorders (NORD) for our work connecting patients to research and care. Representing more than 330 member organizations, NORD is the leading rare disease advocacy organization in the U.S. and was instrumental in passing the Orphan Drug Act of 1983, which encourages pharmaceutical companies to develop drugs for rare diseases. (Recursion's REC-994 drug, currently in clinical trials, was granted Orphan Drug status for cavernous malformation.)

We worked with Senator Ben Ray Lujan (NM) to **introduce federal legislation** called the [CCM-Care Act of 2023](#), which calls for an expansion and coordination of government agency efforts to find effective treatments and a cure for cavernous malformation. This legislation will be under consideration through the end of the session in December 2024.



With daughter Julia (left) and NORD Executive Vice President Pamela Gavin (right), Alliance to Cure CEO and Founder Connie Lee holds the Abbey S. Meyers Leadership Award at the 2023 Rare Impact Awards celebration on May 4, 2023 in Washington, D.C.

We **influenced rare disease policy** when we were asked to join a February 2023 8-member roundtable of national leaders representing the White House, NORD, the National Institutes of Health (NIH), Takeda, and academia. The result of the roundtable was a white paper published by Takeda, a pharmaceutical company leading the way in rare disease equity, and NORD, [providing recommendations to policymakers](#) about improving health equity and diagnostic journeys for rare disease patients. ●

Coming together for support, acting together for a cure

In 2023, volunteers worked hard to organize events across the country that increased public awareness and offered hope and empowerment to our community.



Arizona Walk/Run

Bella's Buddies

Biotrek Fundraiser

Colorado Golf Scramble

Cooking and Queens

Elan's Funding Hope Fundraiser

Elle's Angels BBQ

Heine Strong Golf Tournament

Malibu Beach Walk/Run

MadoroM

Ocean State Makos Golf Tournament

Orange County 5k Walk/Run

Rocking for a Cure

Saberseminar

Southern California Golf Scramble

Step to Cure New Mexico

Strides to Cure CCM

Torrington Wine Tasting





Our Strategic Plan

A permanent cure for cavernous malformation



Inform, support, and mobilize those affected by cavernous malformation and drive research for better treatments and a cure. Develop and execute strategic, creative, high-return interventions as a model for rare diseases.



Drive research & create care networks

Educate & support patients & caregivers

Mobilize patients & empower participation

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.



Empower and engage everyone. We know that solutions don't look the same for everyone, so we meet each community and individual where they are. Our diverse community is stronger and our research is more powerful when there is equity, access to information, and everyone is included and feels a sense of belonging.



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.

Leadership

For 12-month period ending September 30, 2023

2023 Board of Directors

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Cavernous Malformation Centers of Excellence

Barrow Neurological Institute
Boston Children's Hospital
University of California San Francisco
University of Chicago Medicine
Cincinnati Children's Hospital Medical Center
Duke University Hospital
Mayo Clinic Rochester
University of Miami/Jackson Health System
Penn Medicine
Stanford Health Care
University of New Mexico Health Sciences
University of Virginia Health System

Cavernous Malformation Clinical Centers

Ascension St. John Medical Center/St. John Neuroscience Institute
Baptist Medical Center/Wolfson Children's Hospital
Beth Israel Deaconess Medical Center
Lurie Children's Hospital
Thomas Jefferson University Hospitals
University of California Los Angeles
UW Medicine's Harborview Medical Center

Financials

Full audit is available at

www.alliancetocure.org/about-us/financials/





ALLIANCE TO CURE

CAVERNOUS MALFORMATION

Contact us

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Sign up to receive news and updates

Donate online at alliancetocure.org/donate



Alliance to Cure Cavernous Malformation is a non-profit 501(c)3 tax organization under the legal name Angioma Alliance. All contributions are tax-deductible. Our EIN is 02-0600697.