

ANGIOMA ALLIANCE NEWSLETTER

because brains shouldn't bleed

Angioma Community Alliance Program Brings Our Mission To You

Angioma Alliance is growing. To guide our growth, we are in the process of developing an "Angioma Community Alliance" program as a structured, positive way to connect our members to others in their area. Everyone is invited to join. Community Alliances organize grassroots fundraising, support, education, and awareness initiatives in their local communities to support the Angioma Alliance mission.

Angioma Alliance staff and Board are here to support you and your developing Community Alliance every step of the way.

What is an Angioma Community Alliance?

An Angioma Community Alliance is an informal, structured group of volunteers in a specific geographical area (multi-state, statewide, or metropolitan), who work closely with Angioma Alliance to find and engage those affected by cavernous angioma, improve care through interactions with medical professionals, raise public awareness, and raise funds for a cure. An Angioma Community Alliance fosters stronger links between Angioma Alliance and families around the country.

Angioma Community Alliances can play a role in addressing some of the most common needs we hear:

"Are there other people with CCM near me who want to meet up?"

"Can anyone recommend a neurosurgeon in my area?"

"I wish there were a family conference or fundraising event near me."

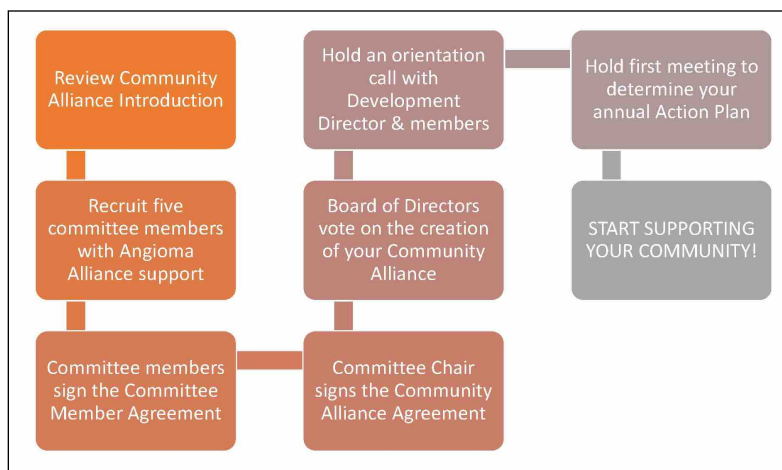
To do this, we are facilitating groups of our members—a minimum of 5 members per group are required as a starting point—who will meet, set goals, and work with us to grow Angioma Alliance's impact around the United States. We already have groups forming in MD/DC, Florida, the New York Tristate area, Southern California, and the Pacific Northwest.

How can an Angioma Community Alliance help?

In many ways! Angioma Community Alliances can deliver support and education programs, including finding patients, organizing informal adult or family meet-ups, disseminating patient education and resource information, and engaging local health care providers and the general public. Angioma Community Alliances can raise revenue to drive research in the diagnosis, cause, management, and care of cavernous angioma. Angioma Community Alliances may organize conferences and serve as ambassadors to nearby Angioma Alliance Centers of Excellence.

First Steps

Are you ready to work together to create a Community Alliance in your area? This flowchart shows the goals we can work towards:



This effort is being led by Stephanie Alband, Development Director, who can answer questions and connect you with others. Her email address is salband@angioma.org. We are hosting multiple Zoom video conferences to share information and answer questions. Find a schedule on our website at Angioma.org/local. Please join us!

Connie Lee

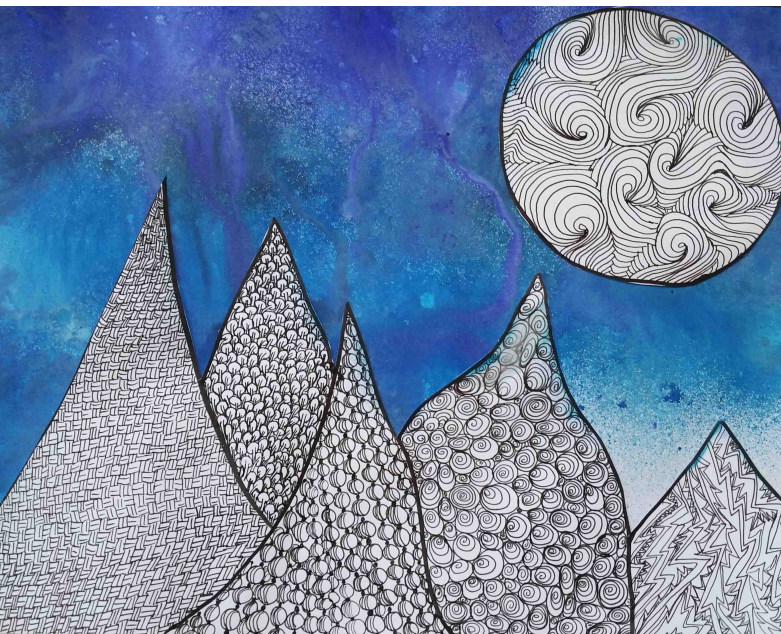
News

International artists are joining forces for Angioma Alliance & Cavernoma Alliance

During Brain Awareness Week this year (March 12-18, 2018), two events will take place in parallel to help raise awareness for cerebral cavernous malformations (CCMs)/cavernoma:

An online art auction (on Facebook) with all funds raised going to Angioma Alliance: www.facebook.com/ArtforAngiomaAlliance.

An art exhibition in London, UK, and an online art sale, with funds raised going to Cavernoma Alliance UK: www.cavernoma.org.uk/landscape-mind-art-sale.



- Artwork by Karen Swartz for Angioma Alliance

I. Art for Angioma Alliance online auction

On March 11, 2018, we will reveal artwork donated by both famous and aspiring artists on our Art for Angioma Alliance Facebook page. Make sure to follow us to get notified: www.facebook.com/ArtforAngiomaAlliance.

The auction will take place within an online photo album, and you can bid by posting a comment next to a picture. The auction will close on Saturday, March 17 at:

- 11am PDT (US West Coast)
- 12pm MDT (US Mountain Time)
- 1pm CDT (US Central)
- 2pm EDT (US East Coast)
- 7pm BST (London, UK)
- 8pm CEST (Central Europe)

Make sure to mark your calendars as we expect skyrocketing bids just before the auction closes!

During Cavernous Angioma Awareness month in June, 2017, we raised a total of \$2,760 for Angioma Alliance with our art auctions. Please help us top these numbers this year and spread the word. While you wait for the new artwork to be revealed, feel free to check out our videos from the previous auctions: www.facebook.com/ArtforAngiomaAlliance/videos/310844756036867/

If you are an artist and would like to contribute some of your artwork to this auction, send an email to: jana.bergholtz@gmail.com to get detailed instructions. Photos of your artwork can be submitted until March 10, 2018. Artists are responsible for shipping their art to the highest bidder once the auction has closed. Our auction is not anonymous; every artist gets a chance to display links to their webpage, Instagram, YouTube, etc. next to their artwork during the auction.

2. Landscape of the Mind Art Sale hosted by Cavernoma Alliance UK

Cavernoma Alliance UK is holding an Art Sale during Brain Awareness week to raise funds: www.cavernoma.org.uk/landscape-mind-art-sale

Art by both famous and aspiring artists will be sold at a fixed price of £75, making art accessible to everyone. But it'll be first come, first served, so be quick. The exhibition will take place from March 12-18th at the Artful Pelican Gallery (143 Lee Road, London, England, SE3 9DJ) with a special VIP preview night on March 10th, 2018, with drinks and chocolates for £5 per ticket. Art will also be online for those of you who can't come to the gallery, but you'll have to pay postage on top of the £75 purchase price. Art will be exhibited anonymously until sold. Each piece will come with a short note or description from the artist, so the buyer will have something unique and personal. We hope you can join us either online or at the exhibition.

If you are an artist and you want to contribute some of your artwork to the UK exhibition and sale, artwork can be submitted until March 3, 2018. It must be sent to the UK. Check here for more information: www.cavernoma.org.uk/landscape-mind-art-sale/

If you want to fundraise for an Angioma Alliance or Cavernoma Alliance/Association/Charity in other countries during Brain Awareness Week, please contact us. If we can't make it this year, we will make it next year. The more charities participate, the more awareness we can raise for cavernoma.

The Baca Family Historical Project

The mission of the Baca Family Historical Project, an Angioma Alliance program operating in New Mexico and surrounding states, is to foster community among the descendants of Cristobal Baca for better health outcomes. We know CCM runs in the families descended from this original founding family, and our goals are to find at-risk families, offer genealogical research and genetic testing, and connect them for support. We also raise awareness of the illness among the public and teach the greater medical community.

Nora Chavez, Community Engagement Specialist, and Joyce Gonzales, staff genealogist, were busy in 2017, our founding year, hosting workshops around New Mexico, talking to the media, and creating materials to spread the word.

In 2018, they have begun sharing information with legislators. Ten Baca Family members attended a meeting of the state legislature's Health Committee, and three were allowed to testify. We have also been working with the state Board of Health, and we plan to have a meeting with the Secretary of Health very soon. The more state resources we can bring to bear for physician education and genetic testing, the more Baca family members we will reach, and the more CCM care will improve.

In 2017, our conference focus was on northern New Mexico, with events in Albuquerque, Santa Fe, Las Vegas, and Taos. This year, we'll revisit these locations, but we'll also be looking south. In the Spring, we will be educating health workers along the border of New Mexico, Texas, and Chihuahua, Mexico. The Common Hispanic Mutation was present in these areas before there were borders, and we are fortunate to be able to reach providers in Chihuahua who can one day help their patients participate in clinical drug trials along with their extended Texas and New Mexican families. We have much to gain from each other.

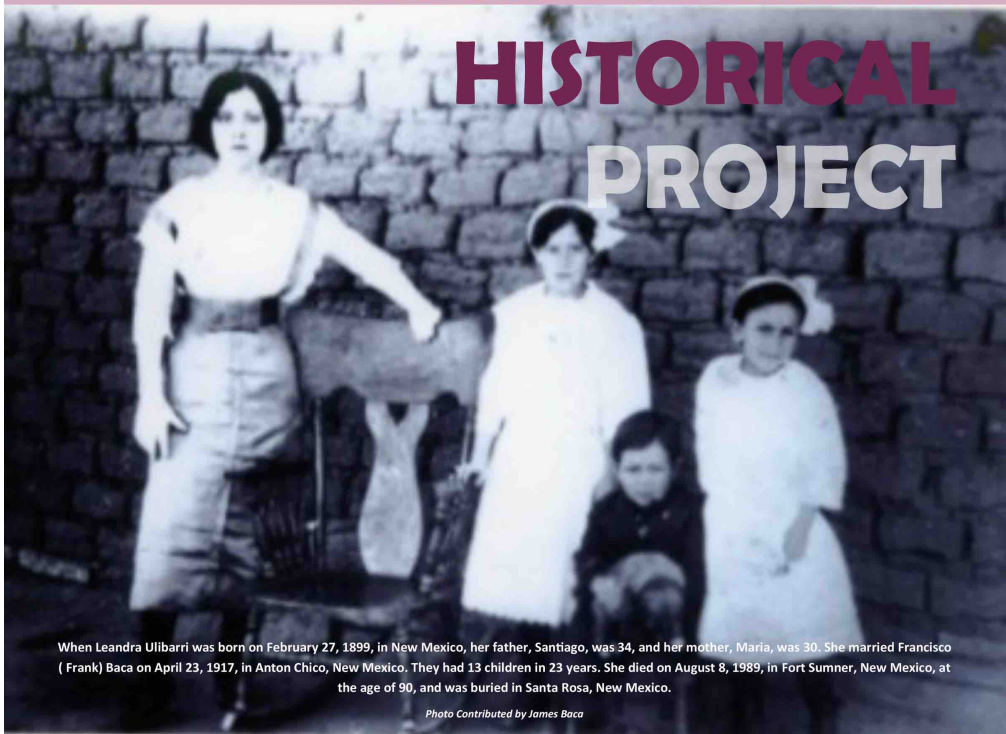
In May, we will hold two conferences back-to-back: Albuquerque, NM, on Friday, May 18, 2018, and Los Lunas, NM, on Saturday, May 19, 2018. We love presenting at regional historical society meetings. If you would like us to speak in your New Mexico or southern Colorado community, please contact us at nchavez@angioma.org.

Finally, we are excited to announce that the Angioma Alliance Baca Family Historical Project has received a \$20,000 matching grant from the Julian Grace Foundation. The foundation will match new or incremental donations restricted to the project dollar-for-dollar. Your \$100 gift becomes \$200 of potential for us, enough to fund one Common Hispanic Mutation genetic test. This grant and your matching contributions will allow us to help 200 at-risk families, a considerable benefit to the CCM community. Please help us meet the challenge!

**CONFERENCE
FRIDAY, MAY 18
2018**

LIVING WITH CEREBRAL CAVERNOUS ANGIOMA
NEW MEXICO'S MEDICAL MYSTERY

BACA FAMILY HISTORICAL PROJECT



When Leandra Ulibarri was born on February 27, 1899, in New Mexico, her father, Santiago, was 34, and her mother, Maria, was 30. She married Francisco (Frank) Baca on April 23, 1917, in Anton Chico, New Mexico. They had 13 children in 23 years. She died on August 8, 1989, in Fort Sumner, New Mexico, at the age of 90, and was buried in Santa Rosa, New Mexico.

Photo Contributed by James Baca

2018 ALBUQUERQUE Conference
Botts Auditorium, Special Collections Library
Albuquerque NM

Rare Disease Day is February 28

International Rare Disease Day is a day to raise awareness among the general public and decision-makers about rare diseases and their impact on patients' lives. This year's theme is "Research," which goes hand in hand with Angioma Alliance's 2018 "CURE" theme. The 10th Annual Rare Disease Day is an opportunity for rare disease patients and advocates to call upon researchers, universities, students, companies, policy makers, and clinicians to do more research and to make them aware of the importance of research for the rare disease community.

On this day, we ask you to show your support in the following ways on social media:

1. **Upgrade Your Profile:** Go to rarediseaseday.org and click on Download Materials to change your social media profile picture to represent Rare Disease Day.

2. **Share & Tag:** Post a short video or picture on social media, share your rare disease story, and tag @angiomaalliance & @rarediseaseday. Tagging is essential to help us raise awareness and have the greatest reach.

3. **Donate or Fundraise:** Donate or create a fundraising campaign for Angioma Alliance at crowdrise.org/angiomaalliance.

Recruiting Committee Members for Upcoming Events!

These events are currently recruiting committee members! To find out how you can be involved, get in touch with Stephanie Alband, salband@angioma.org.

- Cincinnati Reds Event, Monday, August 13, Cincinnati, OH
- Wine Tasting, September 14, Torrington, CT
- Flo Jo Walk & 5K, Sunday, September 24, Mission Viejo, CA
- Malibu Walk, October/November, Malibu, CA

Do you want to host an event in your community? Reach out to us to start planning.

Monthly Giving

Monthly gifts can be beneficial for you and help support the mission of Angioma Alliance in a more significant way than a one-time donation. Your monthly donation will allow you to give more on an annual basis, but the distribution of payments across the year makes the commitment more manageable. Also, a monthly donation enables Angioma Alliance to sustain and grow by having a clear financial forecast for the future. Here's how your monthly donation makes a difference:

- **\$10 per month** provides patient information booklets to 40 newly-diagnosed families.
- **\$25 per month** funds enrollment of two people into the CCM Microbiome Project.
- **\$50 per month** offers four young researchers the opportunity to attend our annual Scientific Meeting, nurturing the next generation of cavernous angioma researchers.
- **\$100 per month** provides up to six families with genetic testing, which may save lives.
- **\$500 per month** funds two regional patient meetings, which feature cavernous angioma education as well as opportunities for patients to meet others with the diagnosis, often for the first time in their lives.

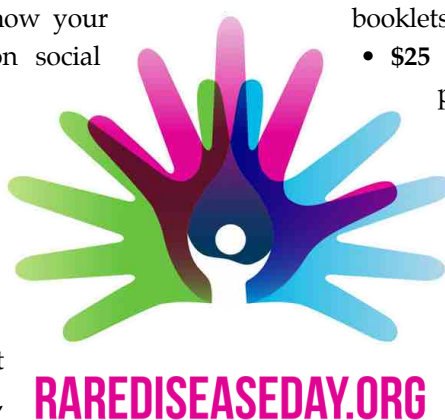
Save the Dates for Patient Conferences

May 12, 2018: Cerebrovascular Family Day at Boston Children's Hospital, with special sessions on CCM. Childcare will be available. Register here: www.eventbrite.com/e/warner-cerebrovascular-family-day-tickets-42542436573

June 2, 2018: San Mateo, CA. Details TBA.

August 13, 2018: Cincinnati, OH. Details TBA.

November 9, 2018: Angioma Alliance's 2018 National Patient Conference will be held in Silver Spring, MD in conjunction with the CCM International Scientific Meeting. Meet our scientists over lunch, attend the keynote address, and learn from experts. More program and registration info to follow.



Angioma Alliance Recognizes Mayo Clinic as Center of Excellence

Mayo Clinic has been named a Center of Excellence by Angioma Alliance for treatment and research into cerebral cavernous angiomas.

“Cavernous malformations are rare; even to a neurologist or neurosurgeon,” says Kelly Flemming, M.D., a Mayo Clinic neurologist, medical director of Mayo’s Center of Excellence and a member of the Angioma Alliance Scientific Advisory Board. “Having coordinated care by providers familiar with the disease is very important to patients.”

“It is important for patients to be evaluated in centers where there is in-depth expertise in all of the different aspects of their care,” says Giuseppe Lanzino, M.D., a Mayo Clinic neurosurgeon and co-director of Mayo’s Center of Excellence.

Criteria for certification of Centers of Excellence were created by Angioma Alliance staff with input from patients. Centers of Excellence offer multidisciplinary care for patients who often are burdened with coordinating multiple appointments with different medical specialists.

Patients with CCM are seen at Mayo Clinic sites in Rochester, Phoenix, and Jacksonville, Florida. Because of patient volume criteria, Mayo Clinic’s Rochester campus received the Center of Excellence designation.

“We are pleased to recognize the Center of Excellence team at Mayo Clinic for the outstanding care they have been providing to our patients,” says Connie Lee, Psy.D., president and CEO, Angioma Alliance. “We also appreciate the investment they have made in researching cerebral cavernous malformations, paving the way for better treatments in the future. Angioma Alliance sets a high bar for Centers of Excellence, and Mayo Clinic joins a select group that has surpassed our expectations.”

Susan Barber Lindquist



Clinical Trials Readiness

The Chair of Angioma Alliance’s Scientific Advisory Board and longest-term advisor, Dr. Issam Awad has taken the lead in developing the tools needed to prepare for clinical trials. Dr. Awad recently received funding from NIH for a study called, “Trial Readiness in Cavernous Angiomas with Symptomatic Hemorrhage,” affectionately abbreviated, CASH.

It is known that a CCM lesion that has previously bled is more likely to bleed again within the next few years, as compared to those that have never bled. As a result, treatments that prevent hemorrhage are ideally suited for individuals with previous hemorrhage (including familial and sporadic CCM with lesions in any location).

Hemorrhage is a devastating but rare event. Given the infrequency of CCM diagnosis and scarcity of those with hemorrhagic lesions, multi-center trials will be a necessity. We also need to understand the number and locations of CCM patients who would be eligible for a

CASH trial, and we need to validate the tools to be used to measure drug effects.

The CASH Trial Readiness Project aims to take on these challenges. This study includes CCM research and clinical experts from the University of Chicago, Mayo Clinic, UCSF, UNM, and John Hopkins University. Across sites, the study team will:

- Analyze patient populations local to each site,
- Standardize advanced MRI techniques across sites, and
- Investigate hemorrhage rates and quality of life in CASH CCM patients.

Angioma Alliance looks forward to supporting this effort. Dr. Connie Lee serves on the project’s Advisory Committee, and Dr. Amy Akers will be sharing progress of the project as it becomes publicly available.

Amy Akers, Ph.D.

Announcing Our New Webinar Series

Angioma Alliance has begun hosting a series of webinars with experts who will share their understanding of cavernous angioma with our community. Recently, Dr. Ed Smith, Director of Pediatric Cerebrovascular Neurosurgery at Boston Children's Hospital, discussed surgical management of CCM. Upcoming, we will feature members of our Scientific Advisory Board and other experts discussing upcoming clinical drug trials, scientific advances, and current treatment guidelines.

The following is a transcript of the member question and answer portion of Dr. Smith's webinar. You can watch his entire presentation on our YouTube channel (youtu.be/L4fWHGWGE2I). In the full webinar, Dr. Smith speaks about advanced surgical techniques, including laser surgery, about creating 3D models to prepare for complex surgeries, and offers general information about cavernous angioma illness.

Connie Lee: Is there ever a situation in which a CCM resection would be performed where the approach is through the nose?

Dr. Ed Smith: In general, you never say never, but it would be highly unlikely you would do a cavernous malformation resection through the nose. That's called a transsphenoidal surgery. Most of the time, the approach through the nose is limited to specific parts of the brain: the pituitary gland, the under-surface of the eye nerves. It's an area that is not typically where cavernous malformations grow. That said, there are isolated case reports. In the right anatomy, it would be the right approach. But, by and large, that's not a typical means to approach most cavernous malformations simply because they're not in that part of the brain in most patients.

CL: Is there ever a time or situation where it's appropriate to remove multiple lesions in one surgery?

ES: Certainly, you can remove multiple lesions in one surgery. I've done it; others have. Most of the time, for people who have multiple lesions, you really want to understand what the purpose of surgery is. Every time you do more operating, you increase risk. Multiple resections are usually predicated on two things. Either you have multiple lesions that are growing or causing symptoms, and, therefore, it's

justified to take them out. Or, you're taking one out that's causing a problem, and it is so immediately adjacent to another one, that it will really not add to the risk of removing it. On the other hand, just taking them out because they're there is probably not going to benefit the patient, especially if they have numerous malformations. You really want to limit your surgical intervention to the ones that are causing problems, because it's likely you may be doing other surgeries down the road. The more surgeries you do, the more scarring there is, and the harder subsequent surgeries are.

CL: This is a two-part question. First, in a child or adult with seizure, how do you go about deciding which of the lesions to remove? Second, if the seizure disorder is not resolved by the surgery and the hemosiderin had not been removed, do you ever go back in and take additional brain tissue out?

ES: About a third of patients show up with seizure as their presenting problem. This is a very common issue. If you have more than a single lesion, you want to go to a center that has a multi-disciplinary core that treats cavernous malformations and has an epilepsy or seizure team.

Part of the evaluation before any proposed surgery would be to have the epilepsy team evaluate the patient. They will probably do an EEG, and they can sometimes do something called MEG, which is a fancy magnetic study. They can localize where the seizure is coming from. Then, they can look at the anatomy and see if the location where the seizure originates matches where a lesion is. It may be that the two coincide, and then you can figure out which malformation is the bad one that you need to remove. There are situations where people can have seizure for other reasons or have what is known as multi-focal epilepsy, where there are multiple spots that cause seizures. In those cases, taking out a cavernous malformation may not have any impact on the seizures or may reduce some of the seizure threshold, but not all of it. Really getting a full epilepsy evaluation targeting seizure focus, coupled with anatomy, is the first step to answering that question.

The second question is what happens if you had a cavernous malformation you thought was causing the seizures and you remove it; do you ever go back? Do

you ever have to do more? As mentioned, if you've carefully selected beforehand, you have a 90% chance of curing or markedly reducing the seizures. That leaves 5-10% of people who are still going to have problems after. In those cases, there may be a need to go back for further surgery.

The two issues you want to address in that case are, first, is the seizure actually being caused by the location where the cavernous malformation used to be? Sometimes people have seizures in other areas aside from the lesion that was removed that were missed if they've been masked or difficult to find. If you're operating on the left side and the seizure's coming from the right, taking out more hemosiderin will not help things. First thing is to be sure that the area where you had the surgery is still the problem spot. If you are sure you have the right spot, removing additional tissue many times can help remove or reduce seizures; about two thirds to 80% of the time.

One of the key things to think about, in some cases, is that the reason some of the tissue was left behind might have been because it was in a very high-risk area of the brain. If it's in a motor area, if it's in a deep part of the brain, it might cause really undesirable side effects to go back in a second time. That may be where you have to think about medication therapy. Or, you may have to think about an "awake" operation where you can monitor the child, or the adult, awake. Or, you may decide to do the surgery, but it's higher risk. Bottom line, going back in can help, but only if you've carefully selected the patient, and you've had a good talk about the risks and benefits.

CL: A final question is about going in and finding out only a partial resection is possible. Do you think doing a partial resection is enough to reduce the risk of re-hemorrhage?

ES: Thankfully, that's the vast minority of cases. Almost always, due to the nature of cavernous malformations, you can get them all out or get the vast majority of one out. Clearly, if you get out 90%+ of a cavernous malformation, you're going to markedly reduce the danger of a physical mass pushing on the brain. You're going to markedly reduce the risk of severe hemorrhages that might cause life-threatening problems. One thing that's important to distinguish is, a little bit of bleeding—a tiny or a small amount—probably is a lot safer than a lot of bleeding. A surgeon may speak to the family beforehand and say, "You know, I may get out 80% or 90%, but if it's stuck to an important blood vessel, if it's in a deep part of the brain, I may deliberately leave a little bit behind. But, by reducing the so-called mass effect, the pressure on the brain, the nerves around there may work better because they've got better blood supply. They're not squished." While there still may be a risk of bleeding or growth in the future, the general size of the overall problem is so much smaller, the quality of life is improved. That said, while the bleeding amount may be less, the bleeding rate may be the same. It's just that it's so much smaller, that life is better. It may also be that by cutting off the blood supply and making the cavernous malformation so tiny, it may actually bleed less often as well. I think the literature is very, very spotty on this. My personal experience with this is that generally it helps. It's just a matter of being clear what the expectations are with the family.

Angioma Alliance Resources for Care and Research

Patient Registry: We boast the largest registry of cavernous angioma patients in the world (Angioma.org/Registry). If you're not in it, you should be! The registry is one way we communicate with you, and it provides aggregate information about the illness for research. We hope to upgrade the registry in the coming year to make it even more useful.

Clinical Care Guidelines (Angioma.org/CCMGuidelines): Does your doctor need more information? The Angioma Alliance Scientific Advisory Board has created peer-reviewed clinical care consensus guidelines that provide the information needed to inform standard of care.

Patient Booklets (Angioma.org/documents/2017PatientBooklet.pdf): Looking for the best portable information? Our Patient Booklet answers 20 questions about your illness in simple language.

DNA/Tissue Bank (Angioma.org/DNA): Have an upcoming surgery? Because our members donate their lesions at the time of surgery, Angioma Alliance is the major supplier of cavernous angioma tissue to researchers around the world.

Free Genetic Testing (Angioma.org/Testing): Do you have multiple lesions that can't be explained by a DVA? Are you having trouble obtaining genetic testing? You can request free genetic testing by signing up for our registering and indicating your interest.

The 13th Annual CCM Scientific Meeting

Angioma Alliance hosted the 13th annual CCM Scientific Meeting on October 26-27, 2017, in Washington, DC.

An essential feature of the CCM Scientific Meeting is the scientific diversity of those in attendance. We welcome researchers who study people, cells, proteins, and animal models; they all tackle CCM research from a different angle. When we all get together, our scientists create fantastic ideas and collaborations! Attendance grew again this year, and this was our largest meeting to date, with 92 participants from across the United States, Canada, Europe, and Australia.

No other scientific conference brings together our entire scientific community. CCM Meeting attendees use the opportunity to host project-specific meetings with collaborators. These satellite meetings happen outside of our regular meeting agenda - on the day before, at breakfast meetings, and between our day's presentations and dinner. Every minute of each day is jam-packed.

This year, teams met to discuss furthering the microbiome project, to provide updates about an ongoing molecular signaling study, and to discuss clinical trial readiness. Also, the Brain Vascular Malformations Consortium executive committee met to discuss better predictors of clinical course and disease severity in CCM.

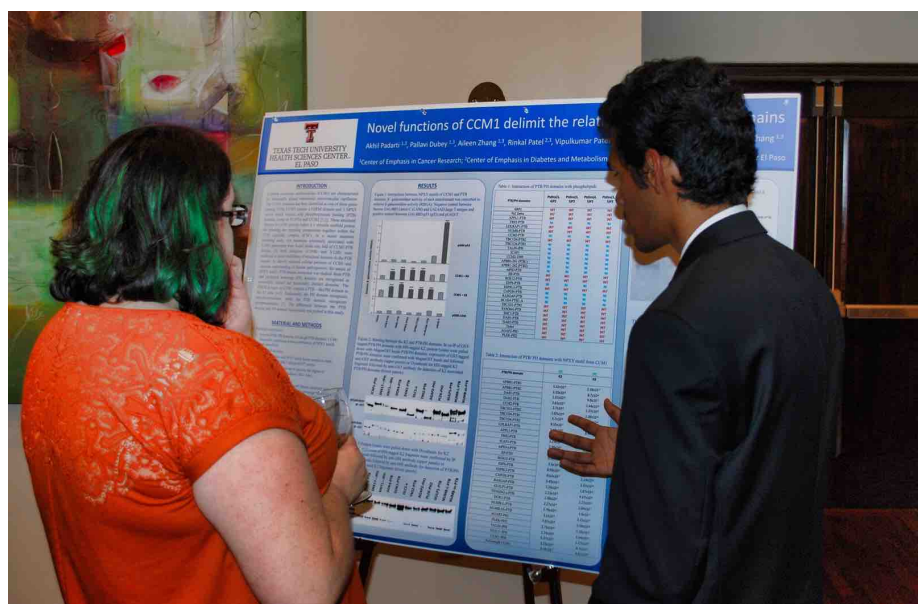
CCM Meeting Day 1

The morning of the first official meeting day focused on molecular biology, the research that addresses the question of what CCM proteins do in cells. Scientists also presented studies exploring the mechanism for how cavernous angiomas form and why they grow. Many of these lesion studies use mouse, fish, and worm experiments that aim to understand the biology of CCM disease with the goal of identifying new molecular targets for drug intervention.

Presentations in the afternoon clinical session stimulated discussions about surgical approaches, the UNM Statin Trial (data analysis of which is ongoing),

and quality of life for CCM patients. There are standard measurement tools used to access and quantify an individual's quality of life. None are specific for CCM disease, but we are working to determine which, if any, can be used effectively with our population.

Day One closed with an afternoon poster session. Poster sessions are less formal than oral PowerPoint presentations. Authors typically engage in discussion with a small group of individuals who move around a reception-style room. Posters are great platforms for early-stage studies and young investigators who are developing research projects and scientific communication skills.



CCM Meeting Day 2

The morning session on day two focused on the biology of tubes: blood vessels, lymphatic vessels, and intestines. Understanding the biology of a variety of tubes in the human body can help us determine how hereditary CCM might play a role in other kinds of illness. Also in this session was an excellent keynote delivered by Dr. Victoria Bautch of the University of North Carolina at Chapel Hill. Dr. Bautch is famous for her work investigating how blood vessels develop over time. While her work is not directly related to CCM, her talk brought a fresh perspective, and stimulated several hypotheses. As

she was leaving the conference she already was planning a new mouse experiment with one of our researchers. This type of thinking and collaboration-building is why we invite keynote speakers from fields related to, but not identical to, CCM research.

The Food and Drug Agency (FDA) approves drugs that are effective in at least one of three areas: how a patient feels, functions, or survives. For the CCM patient community, how one individual feels and functions may be entirely different from the next person, even within families. This variability adds a unique level of challenge for designing clinical trials for CCM. This is further complicated by what we need to consider when we think about treating children versus adults. So, this year we held a special discussion session on Pediatric CCM: Special Considerations for Treatment & Trials. Dr. Ed Smith of Boston Children's Hospital moderated the discussion with participants Dr. Sudhakar Vadivelu of Cincinnati Children's and two industry representatives, Dr. Barry Mangum from Paidion Research, who shared his expertise on pediatric trial design, and Dr. Ron Farkas of PAREXEL, who spoke on regulatory considerations.

Key takeaway messages from the discussion include:

- A unique aspect of CCM in children, particularly those with the familial illness and with those with CCM3 mutations, is that they are just beginning to develop lesions and will (likely) continue to develop lesions for the rest of their

lives. Drugs that stop lesion formation (which are currently under development) are ideally suited for children as a preventative treatment.

- We might assume, for safety consideration, that drugs will first be tested on adults, then later on kids. But, if the unique aspects of pediatric disease make children the most appropriate target population for a new drug, then children may be tested first. The science of each potential medication will drive trial design and participant inclusion criteria.

- The role of patient advocacy is broad: we need to collaborate with our key opinion leaders in the field and engage with the FDA using their formal meeting platforms. We are already on this, as Angioma Alliance organized a Critical Path Innovation Meeting last spring, held at the FDA, to discuss CCM drug development challenges.

The meeting closed with a session on clinical trials. We heard from both Recursion Pharmaceuticals (who is working to bring Tempol to trial) and BioAxxon Biosciences (the group working on the ROCK inhibitor BA-1049). Both groups have made significant progress in the past year with animal studies and are now working their way through the regulatory process with FDA leading up to human trials. Stay tuned; there are many moving parts and we should all be excited about research progress and future trials. I know I am!

Amy Akers, Ph.D.



Cavernoma Alliance UK News

Cavernoma Alliance UK (CAUK) are experiencing a period of high activity raising awareness amongst GPs, lobbying medical schools and training institutions, and circulating information on cavernoma to as many neurologists as possible. This awareness raising has led to an increase in membership. As recipients of a grant from a local charity in Dorchester, we are able to send our information packs to new members and, at the same time, continue with our CaverCentres around the UK. Despite the loss of Debbs Urch, our Southern Community Worker, who left CAUK at the beginning of January for personal reasons, Ailsa Crowe our Northern Community Worker has been able to undertake some of the work in England. For example, during Brain Awareness Week this coming March, she will have meetings in Middlesbrough, Leeds, and Glasgow, and CAUK will be presenting a variety of speakers in London.

CAUK have been fortunate to receive a major grant in the spring from Global's Make Some Noise, a grant pioneered by Simona Stankovska, whose work as volunteer press officer has been a great asset to the cavernoma community. This grant is specifically for CaverFamilies, the branch of the charity that helps young people with cavernoma, leaving CAUK to apply for grants to keep the charity functioning.

The annual London CaverHub was held at the National Hospital for Neurology and Neurosurgery, Queen Square by Mr Daniel Walsh who gave a presentation on surgery and aspects of decision making involved in choosing surgery on their brain or spinal cavernoma.

A training day was also held in October for CaverBuddies. Nine new volunteer CaverBuddies took part, and the day included sessions on listening and communication skills, safeguarding and ethics, the wider consequences of cavernoma, and sources of advice and support.

CAUK now has 20 trained CaverBuddies throughout the country who provide one-to-one support for members who value the opportunity to discuss the physical, mental, and practical challenges they face with someone who has experienced life with cavernoma, whether as a patient, parent, partner, or carer. CAUK also held its first ever volunteer day, to thank our volunteers for their hard work and listen to sessions on social media, CaverBuddying, and fundraising.

CAUK continues raising awareness to the wider professional community with the British Association of Neuroscience Nurses, whose conference in October was attended by volunteer member Kristen Johnson. Further plans are made to attend the British Neurovascular Group, Interventional Group, and The Society of British Neurological Surgeons.

In December, CAUK, in partnership with Global's Make Some Noise, hosted a Christmas CaverHub in London for children aged 2-14 affected by cavernoma. Masterminded by Simona Stankovska, families throughout the UK were invited to London for a day of arts and crafts and a visit from Santa, followed by a day at the London Wetland Centre.

Throughout 2017/18 CAUK have been selected as a charity by the Mayors of Richmond upon Thames in London and in its home town of Dorchester. Various activities have taken place and, whilst we do not expect any substantial financial input, it has been wonderful to raise awareness of cavernoma.

Ian Stuart



How You Can Help

Your contributions help fund our research initiatives toward a cure and our patient support programs. To donate, please send a check or money order in the enclosed envelope or visit our website at www.angioma.org to donate with a credit card.

Sponsorships can maintain essential programs or help us expand our support for the patient and research community. Sponsors are acknowledged with logo placement, naming opportunities, or appropriate other recognition. Sponsorships are available for the following:

Scientific Meeting - \$35,000 to \$1,000

Our scientific meeting offers a variety of opportunities to support and reach the research community, including travel awards and sponsored speakers, breaks, and meals.

Newsletter - \$10,000 to \$5,000/year

This newsletter reaches thousands of patients and donors both in print and online. It is the only patient-directed source of information for the cavernous angioma community. If you would like to reach this community and support our efforts, please contact us.

Website - \$10,000 to \$1,000/year

Our website has a global reach, and is always in the top three search results for cavernous angioma. It is the first place newly diagnosed patients look for information and support. In addition to being a patient resource, the website provides information to medical support staff, researchers and the general public.

Events - Range of opportunities

Angioma Alliance members host multiple events throughout the year, from Cavernous Angioma Awareness Night at major league sporting events to smaller Fun Runs and tournaments. Sponsorship opportunities are always available with varying levels of public exposure depending on the event.

DNA and Tissue Bank - \$20,000/year

The DNA and Tissue Bank is the major source of cavernous angioma biological samples for labs around the world, and we have provided the raw materials for several major published studies.

Contact Stephanie Alband at salband@angioma.org to learn more about these opportunities and valuable benefits for your company.



About Angioma Alliance

Angioma Alliance is a non-profit, international, patient-directed health organization created by people affected by cerebral cavernous angiomas (also known as cavernous malformations or CCM). Our mission is to inform, support, and empower individuals affected by cavernous angioma and drive research for treatments and a cure. We are monitored closely in our educational efforts by a Scientific Advisory Board comprised of leading cerebrovascular neurosurgeons, neurogeneticists, and neurologists.

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Twitter: @AngiomaAlliance

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: Angioma Alliance, 520 W 21st St STE G2-411, Norfolk, VA 23517, info@angioma.org. If you are a resident of one of the following states, you may obtain financial information directly from the state agency.

- 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.