



ANGIOMA ALLIANCE NEWSLETTER

because brains shouldn't bleed

2017: A Year of Care

Every day on social media and in primary care offices, cerebral cavernous angioma patients ask a simple question: How can I receive the best care? At Angioma Alliance, we ask the same question on behalf of our members, and we are working to provide answers.

We are proud to announce that the Angioma Alliance Clinical Care Consensus Guidelines will be published in a major peer-reviewed journal in May. The guidelines will appear online beforehand, perhaps as early as late March, and, thanks to your financial support, which allows us to pre-pay access, they will be freely available to everyone. The guidelines were developed over the course of the last three years by the clinical members of our Scientific Advisory Board and invited experts, with Dr. Amy Akers, our Chief Scientific Officer, as lead author.

While no document can answer every question about treating the illness, the guidelines offer evidence-based information and recommendations on genetics, neurology, neurosurgery, and radiology. This information is presented in simple language in our new patient booklet, which is already available on our website (see page 2 of this newsletter). The guidelines will increase consistency in care everywhere and will be used as the standard by which Centers of Excellence are recognized by Angioma Alliance.

Last fall, Angioma Alliance recognized its first Center of Excellence at the University of Chicago (see page 3). To be recognized as a Center of Excellence, a hospital must provide expert coordinated, multi-disciplinary care, and support a cavernous angioma research program. In 2017, we expect to recognize at least two additional Centers of Excellence, and plan to expand to eight Centers by the end of 2018. These Centers will provide our members, particularly those

with complicated forms of the illness, with confidence that their care is informed and consistent. As we move into clinical drug trials, these centers will also become participant enrollment sites, allowing trials to fill quickly so medications can be approved to reduce the risk of hemorrhage.

In this issue, you will also read about our groundbreaking program in New Mexico. Because of a specific genetic mutation, there are more families with cavernous angioma there than anywhere in the world. We will be finding families at risk and connecting them to each other, to better care, and to research. This is an ambitious and innovative program that you can read about on page 9.

On February 28, World Rare Disease Day, the New Mexico Congressional delegation introduced the CCM-CARE Act of 2017 (page 2). This bill requests federal support for improved care and increased research. We will need your help to move it forward and capitalize on the access having a bill provides. The CCM-CARE Act is our stepping stone to raising awareness of the illness in government where so much research funding originates.

We also need your help in raising public awareness of the illness and our efforts in improving care. This year, we are focusing our efforts on International Cavernous Angioma Awareness Month in June. Read more about how you can participate on page 5.

In 2017 and beyond, our families will see real improvements in the care they receive, and they will have better tools to make informed treatment decisions. This is only possible with your financial and volunteer support; through you, better treatments and a cure are within our reach.

Connie Lee



News

Call to Action: CCM-CARE Bill 2017 Introduced

On February 28, the CCM-CARE Bill of 2017 was introduced in both the United States Senate (where it is known as S. 475) and House of Representatives (where it is known as H.R. 1255). This bill was co-written by Angioma Alliance, reflecting our priorities over the coming years. The bill calls for:

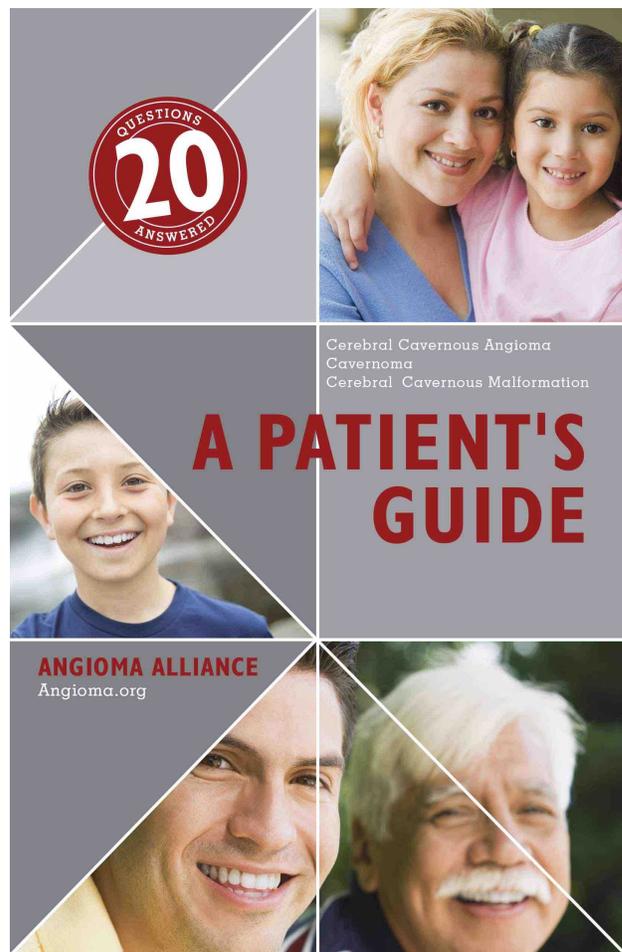
- Support for creation of additional Centers of Excellence that can coordinate drug trials as well as secondary clinical centers that can provide outstanding care;
- Increased funding for research at all levels: basic, translational, and clinical;
- Prioritization of CCM medications at the FDA since, unlike other illnesses, we do not yet have a single pharmacological treatment; and
- Collection and analysis of data on CCM by the Centers for Disease Control so we have a better understanding of how many people are affected by the illness and the ways in which they are affected.

We need your help. We would love for you to contact your Senators and Representatives by phone or in person to let them know this bill is important to your family. We need more legislators to co-sponsor the legislation to increase its impact. We particularly need families from the following cities to reach out: Ann Arbor, Albuquerque, Baltimore, Boston, Chapel Hill/Durham, Chicago, Hampton, VA, Milwaukee, New Haven, Philadelphia, Phoenix, Rochester, MN, Rochester, NY, Salt Lake City, San Diego, and San Francisco. These are cities that have active research programs and/or university hospitals that could benefit from the legislation.

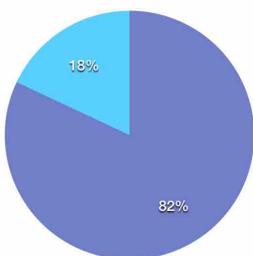
For a script and more information, please visit our website at www.Angioma.org/advocacy.

New Patient Booklet Available

Angioma Alliance has produced a new patient booklet with extensive valuable information about the illness and care options. The booklet mirrors the Angioma Alliance Clinical Care Consensus Guidelines that will be published in a peer-reviewed journal and on the Angioma Alliance website in the next few weeks. The patient booklet can be downloaded from our website at www.Angioma.org/documents/AA2016PatientBookletWeb.pdf.



Angioma Alliance 2016 Expenses



The 2016 numbers are in for Angioma Alliance, and we are doing a great job targeting your donations to help people affected by cavernous angioma. Best practices encourage organizations to spend 75% of their income on mission activities. In 2016, Angioma Alliance dedicated 82% of its income to informing, supporting and empowering those affected by cavernous angioma and driving research for better treatments and a cure.

University of Chicago Medicine Designated as First Center of Excellence for Treatment and Research of Cavernous Angiomas

Chicago Mayor Rahm Emanuel Proclaims Oct. 24 Cavernous Angioma Awareness Day

Angioma Alliance, the nation's only patient advocacy nonprofit for individuals impacted by cerebral cavernous angiomas, has designated the University of Chicago Medicine as its first Clinical Center of Excellence for treatment and research into the rare disease.

In conjunction with the designation, Mayor Rahm Emanuel proclaimed October 24 as Cavernous Angioma Awareness Day to raise awareness of the need for expanded research and treatment resources.

For the past 14 years, Angioma Alliance and Dr. Issam A. Awad, Director of Neurovascular Surgery at the University of Chicago, have worked together to serve the cavernous angioma patient community. Dr. Awad serves as Founding Chairman of the Angioma Alliance Scientific Advisory Board. He is a John Harper Seeley Professor of Neurosurgery and a leading cavernous angioma researcher. Angioma Alliance frequently refers patients to Dr. Awad's clinic. Dr. Awad will be the Medical Director of the new Cavernous Angioma Center of Excellence overseeing a multi-disciplinary team.

"My proclamation of Oct. 24 as Cavernous Angioma Awareness Day further acknowledges this partnership," says Emanuel. "I encourage the public and medical community to learn more about this devastating illness. Chicago is proud to have Dr. Awad and the University of Chicago Medicine play such a key role in researching potential treatments into CCM."

Criteria for certification of Centers of Clinical Excellence were created by Angioma Alliance with input from other patient organizations and the CCM patient community. Angioma Alliance plans to certify 7-8 Centers of Excellence and 20-25 Clinical Centers during the next five years. Both Centers of Excellence and Clinical Centers will offer multidisciplinary, wrap-around care for CCM patients who are often burdened with coordinating multiple appointments with

different medical specialists.

"The University of Chicago has provided for more than two decades of clinical evaluation, radiology, and surgery for hundreds of CCM patients, many traveling from other states and countries," Dr. Awad said. "The University has been at the forefront of research about CCM, discovering new knowledge, enhancing care, and teaching physicians and trainees about its care."

Angioma Alliance President and CEO Connie Lee, Psy.D., expresses her enthusiasm for the certification and the Mayor's recognition. "The University of Chicago and Dr. Awad have set the bar for all future center certifications and for treatment of CCM patients. The Chicago clinic is the start of a new era of integrated treatment for CCM patients," Lee said. "Everyone impacted with cavernous angiomas appreciates Mayor Emanuel's effort to raise awareness about CCM and Angioma Alliance's mission. As more people are knowledgeable, we save lives and get closer to viable treatments."

Dr. Awad expressed his appreciation both to Angioma Alliance and for Emanuel's recognition. "We're honored by the Mayor's public acknowledgement to raise public awareness about the special needs of patients with cavernous angiomas," he said. "My partners at the University of Chicago—neurosurgeons, neurologists, geneticists, nurses and staff—are committed to delivering the best clinical care and access to novel therapies for this disease. We are committed to the discovery of new treatments and we will not rest until we identify an ultimate cure."



City Attorney and patient Tiffany Harris, Dr. Issam Awad, and Dr. Connie Lee

Angioma Alliance to meet with FDA

On May 2, Angioma Alliance Chief Scientific Officer Dr. Amy Akers and President and CEO Dr. Connie Lee will meet with Federal Drug Administration officials for a Critical Path Innovation Meeting (CPIM). The CPIM is a means by which the FDA and investigators from industry, academia, patient advocacy groups, and government communicate to improve efficiency and success in drug development.

In general, potential topics for a CPIM include, but are not limited to, the following:

- Biomarkers in the early phase of development and not yet ready for the Biomarker Qualification Program (BQP);
- Clinical Outcome Assessments in the early phase of development and not yet ready for the Clinical Outcome Assessment Qualification Program;
- Natural history study designs and implementation;
- Emerging technologies or new uses of existing technologies;
- Innovative conceptual approaches to clinical trial design and analysis.

The goals of the CPIM are to discuss a method or technology proposed by Angioma Alliance and for FDA to provide general advice on how this method or technology might enhance drug development. This discussion should relate to all drugs in development.

Dr. Akers has invited representatives from NIH, industry, and academia to join the meeting. It is the first time any CCM research group has met in person with FDA and we expect to learn a great deal from the experience, and hope to educate FDA about the specifics of our illness. We are thrilled to have moved far enough along the drug development path to hold such a meeting and feel privileged to be the primary requesters.

The morning with FDA will be followed by an afternoon visiting Senate and Congressional offices on Capitol Hill. See the article in this issue on the CCM-CARE bill for more information on our current legislation.

Measuring Quality of Life with Cavernous Angioma: A Critical Project on the Road to Drug Trials that Needs Your Participation

In the coming months, Angioma Alliance will be asking our community to participate in one of the most important projects we've undertaken. We have begun the process of creating a measurement tool that will be used in drug trials and in other studies. The tool is tentatively called the CCM Health Index, and it will measure the impact of cavernous angioma on an individual's quality of life.

We are working with the University of Rochester, experts in developing this type of instruments. In addition to getting this project started and funded, our role will be to recruit members who are willing to participate in interviews and complete trial versions of the index.

Why is this important? One of the biggest challenges associated with clinical research for cavernous angioma is the lack of a validated outcome measure. Academic researchers have been planning to rely on hemorrhage as one measure; a devastating event, but infrequent. A clinical trial only designed to measure hemorrhage rate would require very large patient numbers and long trials lengths (two years or more), making it too expensive to be practical. If we want efficient drug trials, we need additional outcome measures.

Cavernous angioma affects patients more than physically; it also affects quality of life. Even if a patient is minimally symptomatic, they may experience secondary consequences like emotional or financial distress. No tool has been validated to measure these effects in cavernous angioma patients. The CCM Health Index will be used to measure the impact of the illness and to determine whether an intervention, such as a medication, has a positive effect on the entire burden of disease and patient quality of life.

Your participation in this effort, as an affected individual, is critical to its success. Please look for recruitment announcements in the next months.

Events

June is Cavernous Angioma Awareness Month

Cavernous Angioma Awareness Month is an international effort by Angioma Alliance to raise awareness amongst the public and decision-makers about CCM and its impact on patients' lives. During this month, we ask everyone affected by CCM to stand up and show their support in the following ways:

- Reach out to local media to share your story on TV, radio, and in print. We can help you.
- During the month of June, add a twibbon to your social media profile picture to show your support of Angioma Alliance and CCM. A twibbon is a frame for your profile picture that will show your friends and followers that you are raising awareness. Go to angioma.org to find the twibbon link.
- Post a short video or picture on social media and share your story about your involvement in the fight against CCM. Use the hashtags #CareForCCM and #BrainsShouldntBleed, and share with us @angiomaalliance; this part is important to help us have the greatest impact.
- Make a donation online at angioma.org/donate or by mail to Angioma Alliance, 520 W 21st St STE G2-411, Norfolk, VA 23517.
- Start planning a fundraising event or Angioma Alliance Walk in your area to be held in 2017. We will give you the tools to ensure it is a success.

Thank you for supporting Cavernous Angioma Awareness Month. Contact Stephanie Alband at salband@angioma.org to find out more, and for help crafting a press release and reaching out to media.

MadoroM Auction a Star-Studded Success

Ian Somerhalder and Nikki Reed attended the MadoroM Wine Auction this year to support Angioma Alliance. They are pictured with Angioma Alliance Board member Liz Neuman, who drives our participation in this wonderful event. The MadoroM Wine Auction, hosted by Andy and Marissa Amador, has raised more than \$700,000 for Angioma Alliance since its inception twelve years ago.

3rd Annual Cincinnati Reds Cavernous Angioma Night

The Cincinnati Reds and Angioma Alliance will hold its third annual Cavernous Angioma Night on May 8 as the Redlegs take on the Yankees in this rare interleague game. The event is likely to sell out and will serve as an amazing awareness opportunity, with over 40,000 fans in attendance and millions more watching from home. The pre-game ceremony will include CCM patients and supporters on the field, our video will play on the Jumbotron, and Joe Price, retired Red and Angioma Alliance family member, will throw out the first pitch.

In conjunction with the Reds game, a free patient conference will also be held on Monday, May 7. Please check our website or Facebook page for conference details.

The event is chaired by Tony and Cari Meyer. In 2012, their one-year-old son Dylan suffered a seizure, which led to his diagnosis of CCM, two subsequent surgeries, and the possibility of future surgeries.

Sponsorships, donations, and ticket sales are currently available. \$6 of every ticket sold benefits Angioma Alliance. Tickets will be on sale shortly at www.reds.com/angiomaalliance. To donate directly, please visit angioma.org/donate.



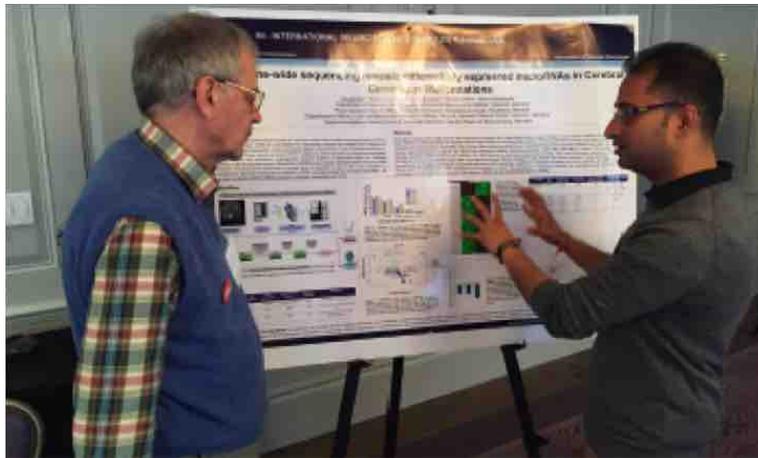
The 12th Annual Angioma Alliance CCM Scientific Meeting



President and CEO Connie Lee, Psy.D., kicks off the meeting with an overview of the progress made towards a cure in 2016.



The Angioma Alliance Board of Directors enjoys dinner with the meeting's largest attendee group ever, including over 80 scientists and researchers eager to learn the latest updates in the field of CCM. Pictured are (left to right, front to back): Tony Mayer, Julie DeMichiel and sponsor Tony Varano; Karen Buck, Liz Neuman, and Tracy Brown.



A post-doctoral student from Germany, who attended the conference thanks to a select group of scholarships awarded, explains his research on microRNA markers serving as indicators of future development of CCM mutations.



The attendees of the 12th Annual CCM Scientific Conference pose for a group shot outside the Dupont Circle hotel in Washington, D.C. To contrast, when the Scientific Conference began only 10-15 scientists and researchers came together to begin the field of CCM research. Now, each year, medical professionals from around the world gather to exchange information to promote research in the field of cavernous angiomas and further progress for a cure.

Interview: Amy Akers, Chief Scientific Officer

Connie Lee: I'm here today with Dr. Amy Akers, who is the Chief Scientific Officer of Angioma Alliance. Can you tell me a little bit about how you became interested in science and specifically in cavernous angioma research?

Amy Akers: Sure. It all goes back to being a little kid and having a really intense interest in science and nature and animals. I always loved bugs. For example, when I was a little kid I had a butterfly collection and we'd pin them on the walls. We still have butterflies in our bathroom. I'm fascinated by them and fascinated by science in general.

As an undergraduate, I really thought I would pursue a medical career. I thought medicine was the path for me. I started volunteering in a hospital and at a nursing home and found out that it wasn't right for me. But what was right for me... I did a research experience at the vet school at Cornell where I did my undergrad and this changed my plan. It opened so many doors for me, and I just became so excited by the research portion of science that I decided to pursue a Ph.D. in human genetics.

I went to Duke University into their program of genetics and genomics. I really wanted to work in a genetics lab where I could have some human disease experience so I could marry that medical school desire with basic science.

At Duke, I joined Doug Marchuk's lab. Doug is an expert geneticist who studies mouse models of a variety of cardiovascular diseases, including cavernous angioma. I started working on the projects there. My project was focused on understanding the genetic mechanisms of lesion genesis: what is happening at the genetic level in those endothelial cells that is causing lesions to form. Importantly, the project required using CCM tissue from patients. This was an issue because my boss, my advisor, was not a surgeon, and we didn't have surgical tissue around. It's a very hard thing to access. But fortunately, Angioma Alliance runs a

wonderful DNA and Tissue bank and had tissue we could use. I completed my Ph.D. and had some really cool findings.

During my days in the lab, I got to know Connie very well because of the need for tissue and, of course, she was running the Scientific Meeting at the time. I gained a true appreciation for advocacy organizations like Angioma Alliance. After I finished my Ph.D., I was ready to move out of the lab. I still wanted to stay in science but didn't want to pursue a traditional scientific career. I jumped on the opportunity to join the Angioma Alliance team, and that was more than 7 and a half years ago. It's been an absolutely wonderful opportunity and experience.

CL: What is it about your position that brings you the most fulfillment?

AA: The position is really cool. The intellectual stimulation and opportunity to learn are just absolutely fantastic. No two days are ever the same.

For example, I'm talking to you today, which is really fun. It's very satisfying to work on a variety of different projects. For example, I still include some basic research, to which I'm accustomed, but also had the opportunity to work with the New Mexico legislative office of Senator Udall

to develop and introduce the CCM-CARE bill. This is a piece of legislation intended to increase awareness and funding for CCM research and drug development and to support the development of our CCM Clinical Centers of Excellence.

Another very different project I worked on recently is to develop the clinical care guidelines for CCM, a project I'm very proud of. Within the next month or so a comprehensive guideline for cavernous angioma clinical management will be published in a peer-reviewed journal and on the Angioma Alliance website. I'm the lead author on this paper and I'm really grateful for the contributions of clinical members of our Scientific Advisory Board and other experts to



bring this project to fruition. I think it will have a huge impact on the patient community. At the end of the day, that's what it's all about.

The most rewarding part of the position is knowing all the work I do has a direct impact on the CCM patient community. Without a doubt, my work and the work of Angioma Alliance has and will continue to push the frontier of CCM research and bring us closer and closer to clinical trials and non-invasive drug treatments.

CL: You aren't in a lab anymore doing bench science, but do you consider yourself to still be doing medical research?

AA: Absolutely, I'm the lead investigator on two research studies right now, one of which is our biobank that I mentioned before. The biobank is a study where we collect DNA and tissue and imaging and medical records that can be used for research: research within Angioma Alliance itself or we can share those resources in a de-identified manner with other laboratories. I'm very much involved in that – recruiting patients and the research end of the biobank project.

Also, I am the lead investigator for one of the sites for the Brain Vascular Malformations Consortium. This is a project that studies three different vascular illnesses. I'll just talk about the CCM portion of it. There are four sites: University of New Mexico, University of California San Francisco, the Barrow Neurological Institute, and Angioma Alliance. It's a genetic study. We're collecting DNA samples and clinical information to look at the variability of this illness. This illness is highly variable. Even in families where they have the same genetic mutation, you'd think they'd all show the same disease spectrum. They don't. There are some people who are symptom-free and others who, unfortunately, are not. We are trying to figure out what other effects are modifying the symptomology of CCM. Those are two clear examples where I am definitely still doing research.

In a broader sense, I am facilitating research on a regular basis. I'm involved in supporting research collaboration bringing people together at the CCM meeting, bringing new industry involvement into the Angioma Alliance fold.

BioAxone is a great example. They are a small biopharmaceutical company out of Cambridge, Massachusetts that is studying rho kinase inhibitors. Rho kinase inhibitors are one of the big drugs we're

looking at for clinical trials down the road. They [BioAxone] were studying them for a different illness. I reach out to them and said, "CCM science is really interesting and there might be some cross-fertilization here." They came to our scientific meeting and have now started these private-public research partnerships with some of our academic researchers to push forward the drug that they are working on. That's not direct research but it's still a very important thing, bringing people together.

We also have developed great relationships with the NIH [National Institutes of Health] and we're starting to develop a relationship with FDA [Food and Drug Administration] as we prepare for clinical trials. I think relationship-building is a huge part of what we do.

Angioma Alliance has a lot of other resources that can support research. For example, study design consultation for clinical drug trials – talking with investigators about the best way to design clinical trials. What are the best outcomes for our patients? Will patients actually participate in a trial that requires this, this, and that? Those are conversations we're involved in.

Study recruitment is huge for us. Our patient registry is a fantastic tool for study recruitment: angioma.org/registry. We can reach out to people interested in being part of research studies and really increase enrollment, both for basic research studies that are happening now and for clinical trials down the road. It's just a connector between the patient community and the research community who don't have that direct contact.

Even now, we're helping researchers with preliminary data, sending out surveys to our members, asking questions about medications they've taken or experiences they've had that pertains to their symptomology. That's really helpful preliminary data for some of our basic researchers to use to obtain funding for new studies.

CL: Thank you. We're going to have a second chat later on about the specific medications that are under development and the Angioma Alliance role in bringing them to patients, as well as some of the obstacles and how we're trying to overcome them. I'm glad we had this bit of an introduction first, and I look forward to speaking to you in the future.

Watch this interview with Dr. Akers on the Angioma Alliance YouTube channel.

The Baca Family Historical Project: Building a CCM Community in New Mexico



New Mexico is known as the Land of Enchantment but, because of a founder mutation called the Common Hispanic Mutation, it also has the the largest population of families affected by cerebral cavernous malformations (CCM), in the world.

The Common Hispanic Mutation has been passed down through at least 14 generations beginning with the earliest Spanish settlers in the late 1500s. We estimate that there are as many as 30,000 patients in northern New Mexico with this illness. In the northern half of the state, one person in every 50 may be affected. That equates to 6-10 kids in every elementary school and 35 affected individuals per square mile in towns like Santa Fe and Farmington. Most are undiagnosed, and the number of affected individuals is growing.

New Mexico is a geographically large and rural majority-minority state with poor educational attainment and health literacy. It is considered medically underserved; physicians' awareness of CCM is variable, and there are few neurologists. As a result, access to diagnosis and quality care is uneven, and is often determined by geography, language, insurance status, and financial resources.

Angioma Alliance has been active in New Mexico, but we have found a vicious cycle perpetuating care deficits. Families' mistrust of the medical system removes hope for better treatments and increases reluctance to participate in efforts to learn about the illness or to obtain genetic testing. Individuals remain out of sight, leading physicians to place low priority on learning more about the illness. This maintains poor care, which again turns away patients.

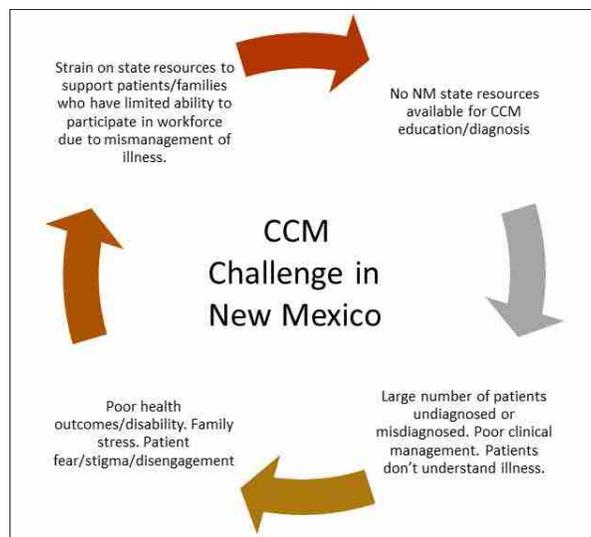
A secondary result of invisible and undiagnosed

patients is that research participation is unacceptably low. Among a population totaling up to 30,000 patients, the total pool of research subjects in University of New Mexico studies is approximately 300. Finally, invisible patients cannot support each other, increasing stigma and isolation.

The key to increasing diagnosis and improving care is increasing patient and family engagement. Engaged patients learn about their illness, support each other, and participate in research for better treatments. Through Angioma Alliance's 12 years of working in New Mexico, we have discovered that approaching patients and families with direct medical intervention does not work. In fact, the mistrust, fear, and stigmatization described above often provoke the opposite response to what we are hoping to achieve. We propose an innovative approach to establishing positive relationships within this very extended family: the Baca Family Historical Project. The mission of this project is to foster a cohesive community in the under-served Hispanic population at risk for CCM. This community will be equipped to provide mutual support in the face of the challenges of a CCM diagnosis, thereby reducing stigma and fear, and increasing the likelihood of medical and research engagement.

Our first task is to find affected families and to interest them in connecting. In the search, we are helped by genealogical research. Joyce Gonzales, amateur genealogist and patient, began researching the Common Hispanic Mutation family tree more than 10 years ago. Joyce has compiled significant data, leading

to stories in the New York Times in 2007 and on NPR in 2013, but her work is not nearly complete. With funding from Global Genes, we have hired Joyce on a part-time basis to continue her research so we can pinpoint where potentially affected families live. Professional genealogists at the NM Genealogical Society have offered to assist her as consultants. Now, we know of affected families around the state. However,



these families don't know each other, often including those living in the same town. Joyce's research will help to grow our understanding of the populations in these places and may point to other important areas not yet on our radar.

Perhaps more than anywhere in the country, Hispanics of northern New Mexican descent connect to their heritage. Geographically isolated for hundreds of years, many families have remained in place since the 1600s. Ancestral stories are part of everyday conversation. This passion is an opportunity for connection. With funding from the Julian Grace Foundation and Cassie Wright, we have hired Nora Chavez as a Community Engagement Specialist. She will begin a campaign of connecting the descendants of Cristóbal Baca and Ana Maria Pacheco Ortiz, believed to be parents of the first affected individual, and any other families identified. She will facilitate connection through Facebook, through in-person events, at community gatherings, and by interfacing with community leaders like clergy, non-profit administrators, and city officials in our target cities. Initially, we are celebrating heritage and creating opportunities for extended families to meet and get to know each other.

As families become acquainted and a community forms, we will begin introducing discussions of health issues. Angioma Alliance has created a patient booklet with information about CCM (see page 2), which provides better information than any rural doctor can offer as it reflects our expert CCM Clinical Care Consensus Guidelines. We will also begin to introduce University of New Mexico providers to the community we've fostered. UNM neurologist and Angioma Alliance Scientific Advisory Board member Dr. Leslie Morrison, along with other UNM staff, are available to offer presentations about the illness at community meetings in our target areas.

Long range, with additional funding, Angioma Alliance plans to open a formal Outreach Center in New Mexico to increase physician education and facilitate genetic testing, improved care, and research participation. We are grateful for the opportunity to begin this innovative project that has the potential to improve the lives of thousands in New Mexico and around the world.

Meet our new team members

Stephanie Alband, Director of Development

Stephanie has dedicated her career to fundraising for various nonprofits, including the Huntington's Disease Society of America, American Cancer Society, United Way, and the Breast Cancer 3-Day. Stephanie has over 17 years of fundraising experience, and she is excited to help Angioma Alliance grow.

Prior to joining Angioma Alliance, Stephanie was Assistant Development Director for the Pacific Southwest Region of the Huntington's Disease Society of America, where her work involved organizing galas, golf tournaments, grassroots walks, and annual giving drives as well as developing relationships with individual major donors, corporations, and foundations.

If you are considering organizing an event, or have connections who you think might be interested in supporting our work, please reach out to Stephanie at salband@angioma.org for her support.

Nora Chavez, New Mexico Community Engagement Specialist

Nora Chavez is our first New Mexico Community Engagement Specialist, funded by a grant from the Julian Grace Foundation. Nora has a background as a Communication Specialist and a Hispanic & African American Communities Liaison with NM CARES Health Disparities Center/UNM HSC, and as an Assistant Development Director at the New Mexico Community Foundation. She will create our New Mexico outreach program, initially focusing on finding families at risk for cavernous angioma based on genealogy. Nora will be connecting these families to each other and to resources.

Joyce Gonzales, Staff Genealogist

Joyce has been a volunteer with Angioma Alliance for many years, spearheading our efforts in New Mexico, where a founder mutation among the original Hispanic population has resulted in the largest population of families affected by cavernous angioma in the world. In January, thanks to funding from Global Genes, Joyce joined us as a part-time genealogist, working to complete the family tree that starts with the descendants of Cristóbal Baca and Anna Maria Pacheco Ortiz. This will allow us to find additional at-risk families around New Mexico and other Southwestern states.

International News

Cavernoma Alliance UK

The closing months of 2016 saw both exciting developments and losses at CAUK. After an extensive search, CAUK appointed Debbs Urch as community worker for the South of England and Sian Oliphant as administrator. These appointments came after Angie Yeomans and Sophie Eldridge left the charity three years of hard and exemplary work. But Sian and Debbs have made a fantastic start providing CAUK with a new energy and vibrancy.

Following Angie's very successful summer 2016 residential for CaverFamilies, CAUK and BBC Children in Need provided a creative afternoon for young people at the Ronald McDonald House near the Royal Manchester Children's Hospital. Members and 40 neurologists attended a keynote talk by Dr. Ed Smith from Boston Children's Hospital entitled "Treatment Advances for Paediatric Cavernous Malformations - Big Ideas for Little Vessels." Sponsored in part by the Hospital Saturday Fund (HSF) a charity founded in 1873 for the "relief of sickness and suffering," Dr Smith's lecture formed part of a wonderful afternoon of talks by the whole Manchester Multi-Disciplinary Team comprising a neurosurgeon, neurologist, geneticist and a neuropsychologist.



Throughout its twelve year history, CAUK has been committed to research into cavernoma. But now with the support of the CaverCommunity and the HSF, CAUK has increased its momentum. 2016 saw the publication of David White's "Priorities for Research into Cavernoma". (An interactive PDF of the report is available at www.cavernoma.org.uk/wp-content/uploads/2016/05/PSP-booklet-2016-linksB.pdf)

David and Ian presented this set of guidelines to clinicians in poster form at the Angioma Alliance Scientific Workshop in Washington DC in November. Our medical and scientific advisers now hope to take this research forward by acquiring funds to continue working on the first priority identified by both patients, carers and clinicians, "does treatment (with neurosurgery or stereotactic radiosurgery), or no treatment, improve outcome for people diagnosed with brain or spine cavernoma?"



During this time, CAUK members continued to raise funds for our work. From cake sales to jumping from moving aircraft, swimming the Sicilian Channel to marathons and even successfully staging a play at a London theatre, our members form the very backbone of all that we do.

CAUK anticipates a lively 2017. With its new staff on board, CAUK has organised a group to go to the House of Commons on Rare Disease Day, and a programme of five events for Brain Awareness Week; Debbs is busy organising a CaverCentre in Brighton and Bristol, Sian is having to cope with the largest growth of new members in our history, and June sees the 11th international CAUK Forum in York where our special guest, Dr. Connie Lee, will update us on recent research developments in North America.

Ian Stuart

CCM Italia

Researchers' Night is an event fostered by the European Commission to share scientific culture. Its goal is to raise people's awareness of the importance of the role of researchers in solving the great challenges of the future, such as health and wellness, new technologies, sustainable development, and knowledge of natural phenomena and our cultural heritage. It is an important scientific (but popular) event that together with the research community, showing that "the researcher is an ordinary person doing extraordinary things."

The 2016 edition of the Researchers' Night in Tuscany (Italy) was called BRIGHT (Brilliant Researchers Impact on Growth Health and Trust in research), and took place on September 30, 2016, simultaneously with 300 towns across 24 European countries. Passionate and enthusiastic researchers crowded and animated streets, squares, and historic places of the 12 different Tuscan towns involved in the project, with live experiments and exhibitions, educational seminars, conferences, and shows, giving people the possibility to get to know them and the "secrets" of their work.

In Siena, the Researchers' Night project involved 200 researchers organizing 66 different events distributed in 18 different places in the town. CCM Italia and AIAC (Associazione Italiana Angiomi Cavernosi) were present with a pavilion in the renaissance cloister of the San Francesco Basilica, together with AIMAKU (Italian

Association of Alkaptonuria patients) and LND Famiglie Italiane (Italian Association of Lesch-Nyhan patients), to raise awareness of rare diseases.

The CCM Italia/AIAC pavilion was organized and led by members of the Siena Unit of the CCM Italia network: Lorenza Trabalzini and Irene Schiavo (Department of Biotechnology, Chemistry and Pharmacy), and Stefania Battistini and Claudia Fabbri (Department of Medicine, Surgery and Neuroscience). With the aid of two catchy and graphically creative slogans ("Rare but not alone" and "Turn on the light on uncommon genetic diseases"), posters and videos set up by Fabio Zanchetta, a graphics and visual communication expert, they explained with very simple terms about CCMs are, the symptoms of the disease, and how the disease is diagnosed and treated. They emphasized the important role played by the CCM Italia multidisciplinary research network and the AIAC patients' organization in promoting research networking both at the national and international level, and raising awareness of CCM disease among the Italian population and health care institutions.

Numerous people, including families, students, and groups of pupils with their teachers, stopped at the CCM Italia/AIAC pavilion to see posters and videos and ask for information. Flyers, bookmarks, brochures, cards, balloons, and candies were available to all those

who came. A special gift to the youngest visitors came from Marco Ottavi, AIAC partner and a famous rapper in Siena, also known as Zatarra, who performed live to make the experience enjoyable.

*Lorenza Trabalzini and
Francesco Retta*



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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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.