ALLIANCE TO CURE CAVERNOUS MALFORMATION NEWSLETTER

Empowered Patients Drive Research

In 2023, the families of Alliance to Cure Cavernous Malformation will drive research. This year, as an international community, we will embark on the development of a Patient-Driven Research Roadmap. This Roadmap will guide the Alliance to Cure's research funding decisions, point toward new research directions and researchers to engage, and offer evidence of patient priorities in our conversations with government funders, companies that are working on treatments, and our academic researchers.

Our researchers are talented and compassionate, and we appreciate all they do for our community. However, research decisions are made in a flawed ecosystem. When was the last time a researcher asked your opinion on research priorities? Has the research community ever come together to listen to what patients most want? As the voice of patients, the Alliance to Cure certainly has input, but so far, there has been no structured way to capture the broader needs and priorities of our families.

In 2023, this will change. We are engaging in a year-long systematic effort to identify and prioritize patients' needs and clinicians' treatment questions. We will chart a course with our broader research community to address these. The Alliance to Cure and our researchers will use the information gained from patients and clinicians to determine where to focus our efforts. Once we have identified priorities, we can assess whether anything is standing in our way. For example, is there a tool or resource we can fund? Or, do we need to bring in expertise from a different field of research? With a roadmap in hand, we will be focused and can be proactive.

This effort will be international with the help of our sister organizations and their advisors. *We urge you to take the time to complete the surveys that come your way; the roadmap must reflect your needs.*

With a research roadmap in hand, we will guide researchers and raise funds to tackle the projects that patients and their loved ones believe will have the biggest impact. We will drive research.

Each year, the Alliance to Cure Cavernous Malformation selects a word to represent our intention for the year. In 2023, our word is **Empower**. As you'll read throughout this issue, our members are empowered to drive us to a cure.

In addition to creating a Patient-Driven Research Roadmap, what will our members be doing in 2023?

- We will attend conferences with scientists, sharing our faces and stories to motivate and inspire those working in labs worldwide (page 3).
- We will fund cutting-edge research projects exploring non-invasive cures (page 4). These high-risk/high-reward projects are opportunities to leapfrog toward a future without cavernous malformations.
- We will join clinical trials, understanding that our involvement is the only path to better treatments. The faster we step up, the sooner we will have solutions (pages 5 & 9).
- We will create resources for scientific discovery by enrolling in the new Alliance to Cure patient registry and providing samples to the biobank (page 8). The usefulness of resource is entirely dependent on our participation. One thousand registrations and one hundred biosamples is an achievable 2023 goal.
- We will stay informed about research findings and expand our self-care toolbox (pages 8 & 10). We will make good choices to stay as healthy as we can be.
- We will share our stories with other patients because our lived experiences can make their journeys easier and help guide their decisions (page 9).

In 2023, we are empowered, and the cure is in our hands.

2022 International CCM Scientific Meeting: Progress and Changes

In November 2022, over 100 CCM scientists from around the world met in person for the first time since 2019. The Washington Duke Inn in Durham, NC, was chosen this year as a special venue to mark our return to live meetings and to celebrate the 20th anniversary of the Alliance to Cure Cavernous Malformation.

The Washington Duke Inn abuts the Duke University campus. This location was also auspicious because it marked a transition in the Alliance to Cure's leadership. With our 20th Anniversary, Dr. Issam Awad, Chair of the Scientific Advisory Board (SAB) since our inception, moved up to Chair Emeritus. The new Alliance to Cure's SAB Chair is Dr. Douglas Marchuk, an original member. Dr. Marchuk is the James B. Duke Distinguished Professor of Molecular Genetics and Microbiology at Duke University. You can read more about him and our new SAB Vice Chair Dr. Brent Derry on page 6. The meeting also allowed us to introduce our new Medical Advisory Board and its Chair, Dr. Ed Smith, Boston Children's Hospital, and Vice Chair, Dr. Kelly Flemming, Mayo Clinic.

We are grateful to Dr. Awad for his 20 years of leadership, and the International Scientific Meeting allowed us to share our thanks. The patient and research community created an emotional tribute video with 16 minutes of thank you messages, six minutes of which were shown at the meeting; there wasn't a dry eye in the house. Then, Dr. Marchuk's keynote speech took us on a journey from the early days of CCM research to today, with a heavy emphasis on the role of patients and our organization in the field's progress.

After passing their mandatory daily COVID antigen test, attendees settled in for cutting-edge science and discussion. The agenda for this year's scientific meeting was a far cry from our last in-person event. In 2019, we had only two presentations about clinical trials, and only one of those trials, the Italian propranolol study, was underway. In 2022, we dedicated an entire session to clinical trials, with one trial completed (propranolol), one trial fully enrolled (atorvastatin), and two trials nearly enrolled (the CARE Trial and the REC-994 Sycamore Trial).

Our agenda is split between studies involving molecular or animal models and clinical studies, that include humans. In 2019, we could fill only two sessions with clinical research. In 2022, we dedicated an entire day of the plenary, with sessions on natural history, biomarkers, trial readiness, and trials, totaling 17 presentations. What does this mean? This heavier focus on people rather than molecular or animal models indicates that our disease is becoming well-understood and that the focus has moved on to treatment. In three short years, amid a pandemic, this has been a seismic shift for our patients. Momentum has built, and we expect more good news each coming year.

You can see the full agenda for the meeting on our website (rebrand.ly/2022CCMScienceMtgAgenda). A video of Dr. Marchuk's keynote address, which includes Dr. Awad's tribute, is available on our YouTube channel (youtu.be/iXk2r-nxZm8).

We look forward to meeting again in Miami this summer!

Join Us! Alliance to Cure Family Conference 2023 – July 7-8

Where can you meet the researchers and clinicians, share stories with other families, and offer your kids the opportunity to make new friends for life? The Alliance to Cure Cavernous Malformation Family Conference! Please join us on Friday and Saturday, July 7-8, at the InterContinental Hotel in Miami, Florida, for a very special event.

For the first time since 2015, this year's conference includes a children's program, and our kids will have the opportunity to meet and form friendships that can last well beyond the conference. The siblings of affected children and the children of affected adults are also welcome. At past conferences, it has been life-changing for our kids to learn that they aren't alone.

For adults, the conference is held concurrently with the International Scientific Meeting, and patients and researchers share sessions and meals. You don't need to be a parent to attend; everyone is welcome and will have the same opportunity to learn and share.

Registration is open now. Visit our Registration Page at rebrand.ly/CCMConference2023 for more information. Registration includes breakfast and lunch on Friday and Saturday. Please note the early and late registration dates.

- Early registration (through May 1) is $60 for adults and $40 for children.
- Registration from May 2 - June 16 is $80 for adults and $60 for children.

Hotel reservations must be made by June 12th to receive the discounted room rate. If you'd like to make a vacation of it, your family can check in as early as July 5 and still receive the discount. You can find the hotel information at rebrand.ly/Conference2023Hotel.

Limited financial assistance with lodging and registration is available. Please check the registration form for details.

Cutting-Edge Research You Are Funding

Our organization has contact with every CCM research lab in the world. We know each lab's strengths and have access to pre-published information from their work. We also have staff with broader scientific knowledge who are laser-focused on our disease. No other entity is in the same position to identify and evaluate the best research opportunities for funding. Rather than putting out a call for proposals, we define a project, directly select a partner, and collaborate on the research plan and budget. At this time, we do not accept unsolicited grant applications.

Currently Funded Projects

Focused Ultrasound

The Alliance to Cure Cavernous Malformation has awarded $87,000 to the laboratory of Dr. Petr Tvrdik at the University of Virginia to fund research using focused ultrasound and sonosensitizers as a treatment for CCM. Focused ultrasound has several mechanisms of action. Like laser surgery, it can work through ablation by heating and destroying tissue. It can also be used with a substance that becomes caustic when exposed to focused ultrasound. This is called sonodynamic therapy and requires a minimal increase in temperature. The initial work is being conducted in cultured human cells and mouse models of CCM.

Gene Therapy

The Alliance to Cure has awarded $18,400 to Dr. Angela Glading at the University of Rochester to support her work developing gene therapies for CCM.

Treatment for cerebral cavernous malformation (CCM) is currently limited to surgery, though several drugs are in pre-clinical development or clinical trials. While these new therapies give hope that we will one day be able to manage the disease, they do not represent a cure. Dr. Glading's lab is looking at gene therapy as a cure for CCM. This project aims to develop novel tools that could be used to rectify the mutations that underlie CCM development in patients of any age.

This grant will fund efforts to target gene therapy to brain endothelial cells directly rather than to every cell in the body. This would make gene therapy a safer option for our patients. Our funding will cover a small but critical portion of this work, synthesizing AAV particle production.

Upcoming Projects for 2023

CD5-2

In 2020, the Gamble lab at the University of Sydney's Centenary Institute published *Targeting miR-27a/VE-cadherin interactions rescues cerebral cavernous malformations in mice*, in which they explored the use of CD5-2, a target site blocker (TSB) that prevents the mRNA interaction of miR-27a/VE-cadherin in mice bred with CCM mutations. From their abstract:

In a neonatal mouse model of CCM disease, CD5-2 normalizes vasculature and reduces vascular leakage in the lesions, inhibits the development of large lesions, and significantly reduces the size of established lesions in the hindbrain.

There were two limitations to this experiment: the mice used in the experiment were very young, and they didn't have established lesions when the treatment began. Additionally, individual lesions were not monitored over time, because Centenary did not have mouse models that survived into adulthood and they did not have the technology for mouse MRI. The Alliance to Cure Cavernous Malformation is funding the next step in this research: testing CD5-2 in adult mice with established lesions and following the development (or regression) of these lesions over time using MRI. To our knowledge, this is the first time sequential MRI will be used to see treatment effects in a mouse model. A formal announcement regarding our partner in this research will be made in the Spring.

Targeted Drug Delivery Using Nanoparticles

With your help, we are funding the exploration of advanced drug delivery methods to get treatments directly to lesions. This work goes hand-in-hand with the recent discovery of the cancer-like biology of CCM. If we need to resort to cancer drugs to shrink a lesion, we want to isolate the drugs' activity to the lesion itself to prevent side effects. Targeted drug delivery research and implementation are already well underway in other diseases. It's time for us to figure out how to make it work for CCM. In 2023, the Alliance to Cure Cavernous Malformation will fund targeted delivery research using nanoparticles. Please expect an announcement of a research partner this summer.

Exciting Clinical Trials Updates

If you follow us on social media, you may have seen our video series "Clinical Trials: Myths vs. Facts," or have learned about a patient's enrollment experiences in Recursion's REC-994 trial. As a community, we are participating in accelerating the cure through clinical trials, and we're ensuring that everyone has the information they need to make decisions. We continue progressing on trials, and trial enrollment opportunities exist in the United States, the UK, and Ireland.

The Research and Clinical Trials section of our website provides additional information, and our interview with Amy Chick, a participant in a clinical trial, on page 9, offers a first-hand account.

Recursion's REC-994 Sycamore Trial

Recursion's trial is in Phase 2, intending to enroll 60 symptomatic adult US patients to determine whether the medicine is safe for CCM patients, and whether there is enough effect in reducing symptoms and stabilizing lesions to warrant an expanded trial. There are currently 13 open trial sites, and most have a few slots available. To learn more about the trial, visit the REC-994 page in the Research and Clinical Trials section of our website. Contact Connie Lee at clee@alliancetocure.org if you'd like to join the trial.

Propranolol Trial

Results of the Phase 2 propranolol trial were published in Lancet Neurology in November. This was a small 2-year trial of 79 adult patients with familial CCM at several sites in Italy. The trial demonstrated that propranolol appears safe for our patients. Results of the trial also appear to indicate that propranolol slows the development of new lesions in familial CCM. This trial is seeking funding to expand so that we can have a more definitive answer about the effectiveness of the medicine.

Atorvastatin Trial

This is a single-site trial of adults with a recent CCM hemorrhage being conducted at the University of Chicago. The trial is trying to determine whether atorvastatin (at relatively high doses) is safe for our patients and whether it shows sign of reducing the chance of additional hemorrhages. The trial completed enrollment in 2022, and participants are expected to remain on the medication for two years. Interim safety results may be published in the following months, with final results in 2024.

Neurelis NRL-1049

Neurelis received Investigational New Drug (IND) approval from the Food and Drug Administration for its ROCK inhibitor, NRL-1049. If you have been a member for a few years, you may remember that this medication was developed by BioAxone specifically for CCM and called BA-1049. Neurelis will now move the medicine forward into clinical trials. Approval of the IND means that Neurelis can begin a Phase 1 trial, testing the medicine in healthy adults.

CARE Trial

In the UK and Ireland, a trial comparing surgery, radiosurgery, and standard of care (no surgery) is enrolling. As of this writing, the trial is very close to its goal of 60 patients with an enrollment deadline of April 30th. This proof-of-concept trial enables researchers to see whether it could be possible to enroll sufficient patients for a full trial if there is additional funding and expansion to Europe. The full trial, if feasible, aims to answer the question of which treatment is the best for patients and under which circumstances. UK and Irish patients are encouraged to enquire at CARE.Trial@ed.ac.uk.

Additional Trials Seeking Funding

Researchers are excited about the potential for low dose rapamycin as a treatment for aggressive lesions and have received approval from FDA to launch a trial. However, funding has yet to be secured. In the UK, there is talk of starting a trial of low-dose aspirin to prevent hemorrhage with the hypothesis that hemorrhage results from clotting. Finally, we hope to find funding for expanded trials of atorvastatin and propranolol and, perhaps, combined use of the two. Some of these trials could be performed using a platform trial structure in which multiple international trials utilize the same placebo group.

Meet the New Scientific Advisory Board Leadership

As you read in our story about our International CCM Scientific Meeting, the Alliance to Cure's Scientific Advisory Board (SAB) has new leadership. We are grateful to Dr. Issam Awad for his 20 years of service to the SAB and are pleased that he is remaining as Chair Emeritus.

The SAB's new Chair is Dr. Douglas Marchuk, the James B. Duke Professor of Medical Genetics and Microbiology and Director of The Division of Human Genetics at the Duke University Medical Center. Dr. Marchuk has been a scientific leader in the CCM field for many years. For example, his lab identified the CCM1 and CCM2 genes in 1999 and 2002, respectively. Currently, his team uses human genetic studies and mouse models to understand the mechanisms behind cavernous malformation formation. He also collaborates with Dr. Issam Awad's study team to investigate drug treatments and gene therapy in mouse models of cavernous malformation. He is an original member of the SAB has been a co-organizer of our annual International CCM Scientific Meeting since its inception in 2005. We are excited to welcome him into this new role.

The SAB's first Vice Chair, Dr. Brent Derry, is a Senior Developmental and Stem Cell Biology Scientist at the University of Toronto, SickKids Hospital. He serves as the University of Toronto's Canadian Research Chair of Genetic Models of Human Disease. His research interests primarily focus on using C. elegans (nematode worms) as a model for human disease and studying the molecular mechanisms for the normal process of programmed cell death. This line of research led him to discover a unique function in the worm versions of CCM1 and CCM3 and has demonstrated how worms can be a fantastic research tool for learning about the basic biology of a disease system. Dr. Derry will be hosting the 2024 International CCM Scientific Meeting in Toronto.

Alliance to Cure Cavernous Malformation Receives Major Recognition

The National Organization for Rare Disorders (NORD) has awarded the Alliance to Cure Cavernous Malformation this year's prestigious Abbey S. Meyers Leadership Award. There are 2,000 rare disease organizations, 330+ of which have been accepted as members of NORD. Each year since 2010, NORD has recognized one organization for its leadership in rare disease advocacy. The 2023 Abbey S. Meyers Leadership Award will be granted to the Alliance to Cure Cavernous Malformation at the National Portrait Gallery on May 4 at its Rare Impact Awards ceremony. We are being recognized, specifically, for creating the Baca Family Historical Project and Breaking Barriers for Black Health Empowerment which serve as models for outreach to under-resourced and frequently underserved patient communities.

NORD is the first and largest umbrella organization for rare diseases; its focus is legislative advocacy and patient organization development. Abbey S. Meyers founded NORD in the early 1980s and was a true pioneer in patient advocacy, leading the organization until her retirement in 2009. Her efforts led to the creation and passage of the Orphan Drug Act of 1983, which incentivizes the development of treatments for rare diseases. We are direct beneficiaries – Recursion's REC-994 was awarded orphan drug designation by FDA, making the path to approval somewhat easier.

"We are humbled to receive the Abbey S. Meyers Leadership Award. Abbey remains a role model who blazed a path for all of us. This award increases our resolve to work creatively and strategically to bring better care and cures to all," said Alliance to Cure CEO Connie Lee.

Looking Back: Patient Conference 2022: A Unique Event

While the scientists were meeting, our patients and their caregivers came together for their own outstanding conference. This year, our attendees were treated to special opportunities.

Attendees toured Duke University's CCM research lab, an outing led by Dr. Marchuk, where they were treated to an inside view of where the science magic happens. Our attendees appreciated Dr. Marchuk's explanations of the role of mouse CCM models in finding treatments. Brave tour participants glimpsed the brain of a mouse and the specialized equipment used to understand it.

The Duke University CCM Center of Excellence award ceremony was conducted during the meeting. This allowed attendees to meet the Medical Co-Directors, Dr. David Hasan and Dr. Wayne Feng, and hear about the Center's technology and programs.

The meeting included a screening of the *Eloquent* documentary before its general release to the public (now at eloquent.cbo.io). Watching the documentary in a room of similarly affected families brought home the reality of the illness and its impact. In a discussion after the documentary, patients emphasized the importance of belonging to our community and learning as much as possible about the illness. We are stronger together.

Patients were invited to participate in blood biomarker research and be the first to register into the Alliance to Cure's soon-to-be-announced, upgraded patient registry. Each patient attendee donated a blood sample that was separated into plasma and serum for this important work.

Finally, patients had time to talk with researchers who attended the patient conference, and to talk with each other. These events emphasize connections as we are joined together in our work toward a cure.

Read about our upcoming Family Conference in July in Miami on page 3. This will be the first conference since 2015 that includes a children's program. Like past conferences, attendees will meet and hear from CCM scientists and doctors, and share stories and support. Some travel assistance is available for those in need.

The New Patient Registry Needs You!

The Alliance to Cure Cavernous Malformation is upgrading our International Cavernous Malformation patient registry to serve research better. As part of this upgrade, we need every patient to re-register (or register for the first time if you aren't in the old registry). You can find the new registry at www.ccmregistry.org.

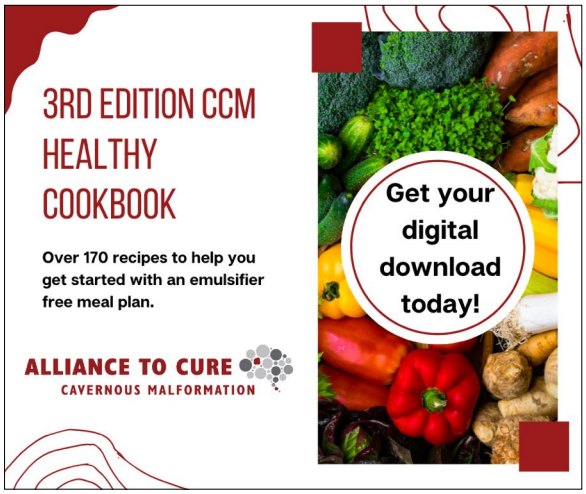
You will see several differences in our new registry. First, you'll be asked to read and sign an informed consent form before participating. The protection of patients in the new registry is overseen by an external Institutional Review Board, which ensures that the way we use your data meets the highest protection standards. You are given the IRB contact information and can reach out to them if you feel we have breached your trust or violated the consent you gave us. Our old registry met the highest standards but did not have an external review board. This external review allows anonymous data from our registry to be incorporated into research. Our old registry was used to generate hypotheses and to describe our patient population, but researchers had to enroll patients and collect new data if they wanted the information to be used in a publication. The new registry removes this burdensome extra step.

The new registry also allows us to respond more quickly to researcher requests by enabling us to create and launch surveys without extra cost or vendor assistance. When you register, you'll see more surveys in your portal than before; don't worry, you don't have to complete them all right away. You may also receive short follow-up surveys that explore new research questions when researchers ask us questions not included in the original data we collect. With the new registry, we can answer these questions answer quickly. We can communicate with you about new research findings and opportunities.

The patient registry continues to be the way you let us know that you are interested in free clinical diagnostic genetic testing, a service that we offer to members with multiple cavernous malformations that can't be explained by the presence of a DVA. Please take advantage of this if you think you have the familial form of the illness, but your family doesn't know their mutation.

Finally, the new registry is connected to our Biobank. We have moved our repository of biological samples to a new facility at Texas Heart Institute. In addition to the tissue and blood used for DNA extraction, the new biobank facility allows us to collect more sample types than we have in the past, including plasma and urine for biomarker research. You'll be hearing more about this move in a future edition of the newsletter. You can consent to join our biobank directly through the patient registry portal.

Joining the patient registry is one of the most important ways you can help get better treatments and a cure. Every ongoing or planned clinical trial has used the information in the registry to help with trial design. It is critical for all clinical research recruitment. Enrolling in the registry takes 20-30 minutes and offers a priceless resource to our community.



CCM Healthy Cookbook, 3rd Edition

The 3rd Edition of the CCM Healthy Cookbook is now available for purchase as a digital download in our online store at shopalliancetocure.bigcartel.com. This edition includes even more emulsifier-free recipes, an updated explanation of the research connecting gut health to cavernous malformations, a research-based emulsifier scorecard, and a list of safer swaps for common emulsifiers.

While you're in our online store, check out this year's gear, featuring our word of the year, EMPOWER. With your new gear, the world will see your strength and resolve as a member of the Alliance to Cure.

Grabbing the Opportunity to Participate in a Clinical Trial

When Amy Chick first discovered that she had cavernous malformations, it was already a trying time in her life. "When I was diagnosed, it had been just after my father had died from this disease. It fractured my family in an irreparable way. I was alone for a long time without any information about how to combat the disease or even what it was all about until I found the Alliance in 2010. I was able to connect with Connie and all of the wonderful people. It was eye-opening to me to see how many big structures of support people have." Amy has a CCM2 mutation and has had three brain surgeries.

Amy's involvement with the Alliance has been ongoing. She has been active in our online discussion groups and has formed close relationships with other members. She participates in several research projects. In 2022, she decided to take her participation to the next level: she reached out to the Recursion REC-994 Sycamore trial to see whether she could qualify.

When asked why she stepped up, she explained, "I'm not a big fundraising person. I'm not really a big go-out-in-public kind of person. So, this is the way to give back in a smaller way, by giving my time and my data. And did you know that you can also sign a little waiver that will let you give your data to future research studies? So, it's the gift that keeps on giving."

This is not to say that she did not have any hesitations, but they were not what one might think. She shared, "I'm kind of an anxious person in general, so the idea of going and doing a whole bunch of things with new people and talking about my disease with a whole other cast of characters did not appeal to me very much."

How did Amy manage this? "The way that I was able to get past that was by looking for a study site that was near me that had things that I look forward to going and seeing. I'm on the West Coast, so I chose Stanford, which is right by San Francisco. They have the Golden Gate Bridge and the Monterey Bay Aquarium not too far away. I was able to incorporate going to see some of those into some of my visits, which makes it really worthwhile. It's something that I look forward to."

Amy is now six months into her time in the trial. She feels the challenges have been minor and not too hard to overcome. "I have to say, I'm more of a night person. Being up at eight in the morning and ready to go, having all these people ask these questions, and four people talking to me all at once is kind of overwhelming. But after that's all over – it's thankfully only a small part of my day – I get to go back to the hotel and nap. After, I go and do my own thing for a little bit and then travel home, so it's not that bad."

Amy does not live in San Francisco and must fly to her appointments. She talked about travel arrangements. "I'm a bit more of an independent person when it comes to making travel accommodations. They have a concierge program that you can enroll in, and I have been enrolled in it, but I have not used it so much. I found that it actually takes more time to sit there and explain what you need and want for your travel accommodations versus going online and just doing it yourself. Then you're reimbursed afterward versus having the concierge pay for it all at first. It's just better for me if I do it myself."

Amy understands how others might worry about clinic visits, and she offers reassurance. "I totally understand it. I understand the worry. I am such a worrywart and an anxious individual. The funny thing is, is that the more often I do this, the stronger I get. It's going back to the same place. It's not like you're going to different places all the time. You go back to the same airport, you take the same flights, and you can take the same hotel if you want. It becomes like a little routine so it doesn't seem that scary." Amy can have support at her appointments. "They will pay for a travel companion, so you don't have to do this alone if you don't want to."

It's common to have concerns about safety during a trial, but these haven't been an issue for Amy so far. "Do I feel safe taking this medication? Absolutely. The side effects they expect from taking this medication are headaches, which, come on, everybody has like a billion of those every day anyway. It's part of our thing. And then a little bit of nausea, which I have when I take it, and I think it's mostly because you're supposed to take it with a full glass of water. So, it's nothing. It's like taking any other pill."

Amy encourages patients who can participate to take the step. "This has the potential to help so many people. You've got to put all the little things that might be holding you back on the back burner if you think this is a possibility of something that you can do. It pays forward to all of these groups that just can't do it because of their family or job obligations. If you're somebody who has the time, please give it. It's just phenomenal that we're at this step right now. I've been waiting for years to be able to be a part of something like this. It's just so exciting to be able to finally do it. So please do."

Now that she is six months into the trial, Amy shared her feelings about her experience so far. "It is going really well. I'm actually sad to learn that I'm almost halfway through the whole experience. But I'm really looking forward to everything, like finding out how it works out for everybody and knowing if we can move forward with this medication. It's given me a good reason to have this disease, which is so bizarre. It gives me a reason, looking forward, to be hopeful for this disease, which is new. It's kind of unheard of. And it's amazing to be a part of it."

This phase of the REC-994 trial has only a few slots remaining, and we would like to see them filled quickly. Please see our Clinical Trials article on page 5 for updates on this and other trials. We appreciate all the volunteers who participate to move us toward better treatments!

How You Can Help

Volunteer: Share your talent and time in any number of ways including Community Alliances, peer support, events, legislative advocacy, and much more: www. alliancetocure.org/care-community/community/volunteer/.

Donate: 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.alliancetocure.org to donate with a credit card.

Sponsor: 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. Contact us at info@alliancetocure.org to learn more about these opportunities and valuable benefits for your company.

Our Mission and Goals

It is our mission to inform, support, and mobilize those affected by cavernous angioma and drive research for better treatments and a cure. We do this by developing and executing strategic, creative, high-return interventions as a model for rare diseases:

1) **Facilitate and participate in cavernous malformation research to achieve a complete understanding of the disease** and facilitate clinical drug trials and other treatment improvements. We do this through our Accelerating Cures program, Scientific Meetings, patient registry and biobank, genetic testing program, research collaborations, and outreach to special populations.

2) **Provide disease and resource information** to educate and improve the lives of people affected by cavernous malformation, caregivers, health professionals, researchers, policymakers, the media, and the general public. We achieve this through our website, publications, webinars, conferences, and media appearances.

3) **Foster and promote a caring community to provide support**. We offer live and online support opportunities and broad international outreach.

4) **Get people involved in advocacy and active participation toward a cure**. Involvement can include activities like research participation, Community Alliances, our upcoming Patient-Expert Certification, legislative advocacy, and public events.

5) **Build and sustain a broad base of funding sources to support our mission and goals**. We count on you!

About Alliance to Cure Cavernous Malformation

Alliance to Cure Cavernous Malformation is a non-profit, international, patient-directed health organization created by people affected by cerebral cavernous malformations (also known as cavernous angiomas or CCM). Our mission is to inform, support, and empower individuals affected by cavernous malformation 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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