

2-year-old Matty Siegrist was admitted to Boston Children's Hospital in August for brain surgery to remove a hemorrhaging cerebellar cavernous angioma. The journey leading up to his successful surgery was fraught with fear and uncertainty. Matty's surgery came after months of medical advice from doctors who were not as familiar with the illness as they could be. Thanks to the community and resources at Angioma Alliance, his mother Jessie was connected to a neurosurgeon familiar with CCM. "I really think that reaching out to the Angioma Alliance community on Facebook saved Matty," Jessie says. "That's what put us in touch with Dr. Smith. Otherwise we wouldn't have had an MRI scheduled for a year and would have missed that the lesion in his cerebellum was growing fast. I am so thankful for this organization."



The Siegrist family is just beginning their journey with cavernous angioma. Matty has a hereditary form of the illness, and genetic testing determined that his father Tim and older sister Lydia are also affected. Matty himself had a 3-month post-surgical MRI recently and the family discovered he has had significant additional bleeding from other lesions. He may be facing another brain surgery in 2017.

As Angioma Alliance moves into its 15th year, I am still struck each time I learn of a young child or parent or grandparent receiving this life-changing diagnosis. Each family is unique, but the fears are the same. Your financial support of the work of Angioma Alliance is critical in helping our families manage the complexities of the illness and in fulfilling the promise that, soon, the fear will be a distant memory.



This year, together, we took the first step toward playing a leading role in improving patient care. Our Scientific Advisory Board and invited experts completed peer-reviewed clinical care consensus guidelines which will be published in the next weeks by a major medical journal. This is a great achievement: guidelines provide the knowledge base to train medical professionals around the country. As Matty's family knows, informed care can mean the difference between life and death. Also, the guidelines allow us to begin identifying centers that rise to the level of excellence. Our first Center of Excellence at the University of Chicago was recognized by Angioma Alliance and by the Chicago Mayor's Office, who declared October 26th Cavernous Angioma Awareness Day in Chicago. We plan for 2017 to be a Year of Care as we grow our Center of Excellence network, disseminate our clinical care consensus guidelines, and send our comprehensive new patient booklet across the country. We invite you to help us bring the Year of Care to a hospital near you.

You have made it possible for us to continue supporting drug development. At this year's Scientific Meeting, the largest ever, we learned of even more potential medications that could be useful to prevent hemorrhage. Seven drugs are moving through the pipeline — of course, more slowly than we'd like. We had hoped to begin trials of Lipitor (atorvastatin) late in 2016. Unfortunately, the National Institutes of Health turned down the request for trial funding from the University of Chicago. The University has scaled back the plan and resubmitted. Atorvastatin as well as one or two other drugs could begin enrolling trials by the end of 2017 — this would be very exciting for all our families. It will be wonderful when Matty and the thousands of children and adults like him could live without the terrifying interruption of brain surgeries and multiplying lesions. **We would like this illness to become an afterthought, not the focus of a family's life.** With your support, we are expediting trials

by matching collaborators, by starting a project to develop tools and measures of drug effectiveness, and by serving as primary consultants on trial design. Soon, there will be real treatments.

This year, your support has allowed us to continue offering free genetic testing to our members and their families, providing them with the knowledge they need to receive appropriate care. We awarded grants to two researchers: one for basic science and the other to help us understand the impact of genetic testing on families. Our DNA/Tissue Bank remains the major supplier of cavernous angioma tissue to labs around the world. Our Susan Sukalich Angioma Alliance International Patient Registry has grown by leaps and bounds, allowing us to answer questions asked by researchers and to be poised to fill drug trials when they begin. Next year, we will begin the process of upgrading this registry to let medical professionals to enter information directly. Finally, we are a recruitment site and research partner for a major natural history study, now in its 8th year.



Knowledge is power, and for our families, it is life. In addition to creating new printed materials for patients, we hosted two regional patient conferences. With your help, next year we will host a national conference. We revamped our one-on-one support program and are exploring ways to expand support to post-surgical members. As always, our website and online support groups provide the best information and strongest community to be found anywhere. And our families raise public awareness in walks around the country, at public sporting events, and at fundraisers large and small. With a higher profile, we gain public awareness and increased support, which can lead to faster diagnosis and better care.

Next year, all the work I described above will continue and expand. Your generosity guarantees this. Also, we will be collaborating with Congress to introduce federal legislation to increase funding for CCM research and to expand care facilities. We will count on you to contact your representatives to tell them how important this is. Also, we just learned we will receive a one-year \$50,000 grant award by the Julian Grace Foundation to expand our genealogy work and begin an outreach program in New Mexico. Because of a genetic mutation passed down since the first Spanish settlers, there are more people with the illness in New Mexico than anywhere in the world. We plan to find these families and nurture community so that **no one feels alone with their illness or is afraid to be diagnosed**. New Mexico is critical to the success of clinical drug trials for everyone – we welcome this opportunity to be able to give back.

There are no words to express my gratitude for what you have made possible for our families, for the future. I invite you this year to play an even bigger part in our mission to help thousands of children and families — like Matty and his parents — by making a generous donation at www.Angioma.org/Donate. Because of you, we are undertaking big projects with priceless long-term benefits. We look forward to a world where no child lives in fear of brain hemorrhage and its crippling effects, where no adult is disabled by seizure or paralysis. With your help, this vision is within our reach.



Warmly,

Connie Lee

Connie Lee, Psy.D.
President and CEO