



SPRING 2017

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Pathways to a Cure

The CAA Newsletter

MESSAGE FROM THE DIRECTOR

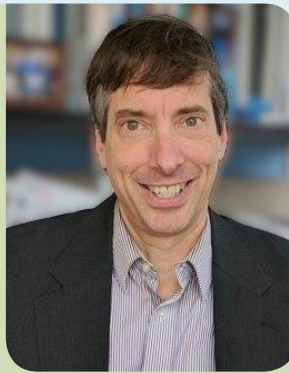
People who study neurological disease often talk about biomarkers which are indicators of presence and prognosis of a disease. Biomarkers are typically checked by relatively simple tests like blood draws and brain scans. The reason biomarkers are so valuable is that they can provide windows into how/why a disease appears and progresses and what steps might be taken to delay/halt that progression to determine whether a trial is succeeding.

The search for biomarkers has been a major focus of our CAA research center and more broadly in the field of small vessel diseases of the brain that cause stroke and cognitive impairment. The National Institutes of Health recently recognized the importance of biomarkers by establishing a consortium of leading US medical centers to identify (validate) biomarkers for small vessel disease that can contribute to cognitive impairment. This new consortium is one of the biggest projects funded by the National Institute of Neurological Disorders and Stroke (NINDS) this year, reflecting the neurology community's understanding of the importance of these diseases and the potential impact of finding better biomarkers. MGH's J. Philip Kistler Stoke Research Center is honored to be selected as the coordinating center for this 5-year initiative.

As of now, our sleeves are rolled up as we begin to transform NINDS' vision into powerful steps forward for the small vessel disease field. We see this as a tremendous opportunity to move toward more and better clinical trials aimed at helping patients with CAA and other forms of small vessel disease. We are happy to keep you posted on our progress as things hopefully move ahead.

In the next few pages you will read about "microbleed-only" CAA and how it's possible to have CAA without having had a stroke. Additionally, you'll hear from a former research participant about his choice to participate in research and more recently his decision to make a philanthropic contribution that will have a lasting impact on the future of CAA research.

Sincerely,
Steve Greenberg, MD, PhD



I've never had a bleeding stroke. Could I still have CAA?

The most dramatic symptoms of CAA occur when the buildup of amyloid causes a blood vessel to break open, resulting in bleeding into the brain tissue. This can cause sudden paralysis, difficulty speaking, or other signs of stroke. However, through our ongoing research we have learned that the disease often manifests in much more subtle ways. Most common are changes in memory and cognitive performance. This is particularly the case in people who have only had tiny bleeds (referred to as "microbleeds" or "microhemorrhages") and have never had a large hemorrhagic stroke (also called a "macrobleed"). "Microbleed-only" CAA may in fact be more common than CAA-related macrohemorrhages, and may be just as serious. Microbleed-only CAA is identified based on the presence and location of these microscopic bleeds as seen on MRI scans.

Our research center is particularly interested in better understanding the role microbleeds play in determining a person's risk of future bleeding in the brain. We are also interested in being able to
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— I've never had a bleeding stroke

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better assess severity and progression of the disease based on criteria like microbleeds. For instance, our research has shown that those who have “microbleed-only” CAA will often stay that way – meaning they are at lower risk of developing a macrohemorrhage in the future when compared to those who have already suffered a large brain bleed. As such, our ongoing studies are aimed at looking for new biomarkers that may help us identify which people with “microbleed-only” CAA are at the highest risk of future bleeding.

While there has been a lot of attention on microbleeds and their significance in CAA, there are also lesser known biomarkers that we've identified through years of imaging data made possible by our many generous research participants. One of these is superficial siderosis, which

results from repeated bleeding into the subarachnoid space – the thin layer just outside the brain itself. The term “superficial siderosis” is often used for old bleeding events and “subarachnoid hemorrhage” when it is recent. While there is more to understand about these biomarkers, we do know that they independently indicate an increased risk for early and repeated “macrobleeding” and may also provide additional insights into the mechanisms of repeat bleeding in patients with CAA.

We are hopeful that findings from our research will ultimately enable us to offer personalized treatment and a more accurate picture of how the disease progresses in the future. Having a consultation with one of our stroke neurologists may help you better understand your risk for developing brain bleeds, which could help you handle future medical situations.

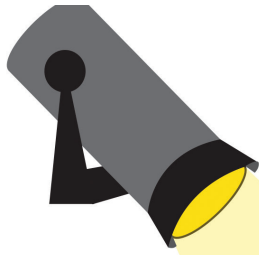
Update!

The first ever trial for CAA – Ponezumab – fulfilled its enrollment goal in 2016. Pfizer, the sponsoring company, is actively analyzing the results from this trial. We look forward to being able to share the results with you once they are publicly released. Thank you to all who participated or expressed interest in this important trial.

Emily Benson Clinical Research Coordinator

Emily joined the J. Philip Kistler Stroke Research Center in May of 2016. Emily holds a Bachelor's degree in Psychology and double minor in Education and Women's Studies. At JPK, Emily coordinates Dr. Anand Viswanathan's study examining the progression of “microbleed-only” CAA and mild cognitive impairment in subjects over the age of 55 who have probable CAA and memory concerns. In her role, Emily enjoys getting to know study participants and helping them navigate their research activities, which can include MRI and PET scans, memory and attention span testing or lumbar punctures. If interested in learning more about this study, contact Emily at ebenson3@mgh.harvard.edu





PATIENT SPOTLIGHT:

This edition of Pathways to a Cure features Mr. James Noel of Maryland. He recently retired after 35 years of federal service with the U.S. Government Accountability Office, where he was dedicated to improving government functioning. Mr. Noel, his wife Rachel, and his children have all been avid supporters of the MGH Stroke Research program. Mr. Noel has generously traveled to Boston several times to participate in imaging and biomarker studies. In this edition of our Patient Spotlight series, we asked Mr. Noel about his interest and experience participating in research, and were again amazed at the generosity and motivation of yet another wonderful research participant!

How did you come to find out about your CAA?

I had my first cerebral hemorrhage in 2013 and was treated at my local hospital. The neurologist there was unfamiliar with CAA and put me on medications that were more appropriate for an ischemic stroke diagnosis. I sought out a second opinion at John Hopkins Hospital. After examining me, reviewing my brain scans and other records, and comparing these with the “Boston Criteria,” they told me that I had probable CAA and adjusted my medication.

What did you find to be the most challenging aspect of receiving this diagnosis?

Given that there is not yet a cure for CAA, it was hard to imagine how this diagnosis would affect me. However, one doctor at Johns Hopkins told me I should simply, “live my life and enjoy it,” so that is what I have done.

What inspired you to be so active in research?

Since retiring from a very demanding job, it has become possible to dedicate time to a new cause. Now I am able to do something for a different group of people by participating in CAA research at Mass General Hospital. I would welcome the opportunity to participate in future studies.

Was there anything about participating in research at MGH that surprised you or was it really

different in any way from what you’d expected it to be?

What struck me the most was how patient and kind everyone was during the research visits. I felt that the staff took good care of me and even though the research involved going to various buildings all over Boston, the staff was with me at every step.

What advice would you give to others who have CAA?

As I said earlier, just “live your life and enjoy it.” I have a lot in my life to be thankful for— three wonderful grandkids, my daughter is getting married this June, my wife and I got married last year, and we have several trips planned for this summer. While I often get tired— one way CAA has affected me—I think about my daughter’s wedding and the summer trips we have planned.

Why did you decide to donate to CAA research at Mass General?

Since I have good retirement income, I thought, “why not help myself and others with CAA?” I started by giving \$100 per month through my credit card and plan to continue to do that until a cure is found. My wife and my son have also given to CAA research, so it is a cause near to our hearts.

What do you hope to accomplish with your philanthropic support of CAA research?

I thought that philanthropic support would be a good way to extend my range of giving. Using an annuity-type of donation approach gave me a nice tax break for 2016. It will also continue to give my wife and me a small amount of income throughout our lives. By writing MGH into our estate plan, we became members of “The Phillips Society,” and were given a very nice pin. But in the end, my donation was just another way to support CAA research.



The Noels

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Contact Information:

Please send your comments, questions and suggestions to:
pathwaystoacure@partners.org.
For more information on our CAA research and for FAQs, please visit: www.angiopathy.org

Looking to support CAA Research?
Many patients and families have lent their time and heartfelt dedication to finding a cure for CAA by helping raise funds for our clinical research program. The CAA Research Team at MGH encourages your interest in hosting a charity event or fundraising among friends, family and colleagues, and appreciate the efforts many of you have already made to this end!

If you would like to learn more about how to support CAA research at MGH, please visit this link: <https://giving.massgeneral.org/crowdfunding-community-fundraising/>

Individual donations can also be mailed to:
**MGH Development Office c/o
Shawn Fitzgibbons
125 Nashua Street, Suite 540
Boston, MA 02114**

*Please make checks payable to Mass General Hospital, memo: #1200-028184



Hello from the 2017 International Stroke Conference

