



Pathways to a Cure

The CAA Newsletter

MESSAGE FROM THE DIRECTOR

Another productive year is coming to a close for us here at the Research Center! Our ongoing devotion to research is driven by your support, and we are happy to announce another milestone in the growth of the international CAA community.

This past September, I had the pleasure of traveling to London for the 4th International CAA Conference. The conference brings together investigators from around the world, allowing for valuable face-to-face interactions that launch new collaborations in CAA research. International partnerships are rapidly growing, and this networking enables research findings to be combined and compared across diverse populations. One goal of the International CAA Association is to promote large scale, comparable data sets around the world, allowing for more precise analysis of the disease and its components. This will lead us closer to an infrastructure that supports multicenter research studies and clinical trials of promising candidate treatments. The association also aims to educate healthcare providers, patients, and the public about CAA, ensuring that the disease does not go unnoticed in the medical-research community. The conference will continue to meet every other year in an effort to optimize communication and create a worldwide network of CAA experts. If you are interested in learning more about the International CAA Association, please visit www.internationalcaaassociation.org.

In this edition of *Pathways to a Cure*, we are also excited to provide you with a unique look at the array of follow-up research studies taking place at the J.P. Kistler Stroke Research Center. As always, we thank you for your continued interest in our newsletter!

Sincerely,
Steve Greenberg, MD, PhD



What's Happening in CAA Research?

Our Current Studies:

As we approach the two-year anniversary of *Pathways to a Cure*, we want to give our readers a snapshot of each of our active CAA research studies. While each of these studies is separate and unique, your participation in more than one makes the data collected from each more valuable. Try to imagine each study as a puzzle piece; we may not know exactly where every piece "fits" yet, but we do know that the more pieces we have in our hands, the closer we will be to completing the puzzle.

Clinical Evaluation of Florbetapir F18 (18F-AV-45) in Subjects with CAA: Participation in this study involves receiving a onetime injection of an FDA approved drug, called Florbetapir, paired with a 20-minute PET scan. Florbetapir is a radioactive drug that attaches to the beta-amyloid protein, which is known to build up in the blood vessels in the brain of individuals with CAA. This study aims to determine the effectiveness of using Florbetapir to diagnose CAA early and accurately.

Measurement of Biomarkers in

Cerebrospinal Fluid in CAA: Those who enroll in this study will undergo a one-time blood draw and a lumbar puncture (also known as a "spinal tap"), which can be uncomfortable, but typically not painful, and is safe – and many of our dedicated research subjects have generously volunteered for this study. Through this study we are trying to learn whether CSF contains markers which can be used to diagnose CAA.

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—What's Happening in CAA Research

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Advanced Neuroimaging to Detect CAA: This study was previously highlighted in the spring 2014 edition of Pathways to a Cure. Enrollment involves MRI scanning once per year for two or three consecutive years, and the option to have a onetime PET scan and/or MRI scan(s) on higher definition scanners. Additionally, we test for changes in memory, balance, and vision. Through this process, we hope to better identify trends in the natural course of CAA

PiB PET: If interested in the optional, one-time PET scan, an appointment is made at MGH where you will receive an injection of a radioactive drug called Pittsburgh Compound-B (C-11 PiB) and then lie in a PET scanner for 45-60 minutes. This portion of the study aims to reveal whether C-11 PiB PET can predict worsening of damage to brain tissues and clinical worsening such as new bleeds or development of dementia in CAA patients.

Ponezumab as a Potential Treatment Option for CAA: This treatment trial requires participants to come to the hospital for 11 visits over 8 months. It involves 3 infusions with the study drug (Ponezumab) or

placebo (looks like the drug but has no active ingredients) and extensive safety monitoring – most (two-thirds) of participants receive the actual drug rather than the placebo. Through this groundbreaking trial, we hope to better understand whether or not the study drug is effective at removing amyloid protein from the blood vessels in the brain.

GOCHA: Genetics of Cerebral Hemorrhage with Anticoagulation: Participants are asked for a one-time blood draw for genetic analysis and a short interview. This study provides a unique opportunity to link an individual's genetic sample to data collected in the above studies. We hope that this study will help us identify whether or not there are genetic biomarkers that may be used in learning about CAA.

As you can see, we stand to learn something unique from each one of these studies. Furthermore, your participation in multiple studies provides us a much more in depth understanding of CAA as a whole.

If you're interested in learning more, or simply have questions, please contact Research Coordinator Grace Riley at (617) 643-2782 or at griley1@mgh.harvard.edu.

Meet Grace

Grace Riley is our new Research Coordinator here in the J.P. Kistler Stroke Research Center. She holds a Bachelor of Arts degree in global health, and recently started a pre-med post baccalaureate program with the intention of pursuing medical school. Prior to joining the Kistler Center team, Grace worked for the International OCD Foundation where she helped those living with obsessive compulsive disorder (OCD) find local support and effective treatment. Grace has taken over our CAA research studies, and looks forward to meeting many of you in the near future!



PATIENT SPOTLIGHT:

This edition of *Pathways to a Cure* features one of our longtime research participants, John Lavelle, a 58 year old gentleman who lives in upstate New York. John has worked as a lawyer for over 20 years, and he and his wife have recently established a thoroughbred horse breeding farm. John has participated in many of our studies, including Advanced Neuroimaging with PiB-PET, Lumbar Puncture, Flortetapir, GOCHA and he recently completed our drug trial study, Ponezumab.

How did you come to find out about your CAA?

I had a bleeding stroke in November 2010. At a follow up visit with my local stroke center here in NY, the head of the practice casually mentioned the word “amyloidosis”. Having never heard of it, I looked it up and was terrified. I took the initiative to go to the Amyloidosis Center in Boston, and after extensive testing, they ruled it out. Instead, they said I had another disease – CAA, and sent me to your team.

What did you find to be the most challenging part of the diagnosis?

The lack of information, the relative rareness of the disease, and the absence of treatment options were all pretty intimidating. I can't tell you how comforting it was to find that there was actually a center trying to do something about this disease. Until you find that, you feel very much alone.

What inspires you to be so active in research?

Despite my personal squeamishness about all things medical, when I learned that I was probably among the youngest and otherwise healthiest of diagnosed patients, I felt a real calling to help out.

In what way has your life changed since being diagnosed with CAA? Have there been any “silver linings” that you can share with us?

When all I knew was that I had survived a very dangerous form of stroke, I was trying to get back to normal with no real idea of what the future held. Once diagnosed, although it was unnerving to have such an unknown disease, thanks to your center's work, there was suddenly both a path to and hope for a better future.

What advice would you give to others who have CAA?

Be active in seeking help, utilize the resources of the MGH stroke research team and try to participate in making a brighter future. There is hope now, and the more you know, the more comfortable you can be living with CAA.

If you are interested in speaking with John Lavelle, he is willing to speak with groups interested in learning about living with CAA, or anyone who would just like to reach out to another with CAA. To

arrange communication, please call Clinical Research Coordinator Grace Riley at (617) 643-2782, or email griley1@mgh.harvard.edu



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www.angiopathy.org

Contact Information:

Please send your comments, questions
and recommendations to:

pathwaystoacure@partners.org.

For more information on our CAA research and
for FAQs, please visit: www.angiopathy.org

In the spirit of giving this holiday season,
please consider making a donation to the
MGH Stroke Research Center in honor
or in memory of someone special. All
contributions directly support research on
CAA and bleeding strokes.

Donations can be mailed to the MGH
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100 Cambridge St., Ste. 1310 | Boston, MA
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visiting the “Support” section of our website
angiopathy.org. Happy Holidays to you and
yours from all of us at the MGH Stroke
Research Center!



**“Thank you for your commitment to
CAA research!”**

From all of us at the J. Philip Kistler MGH Stroke Research Center