



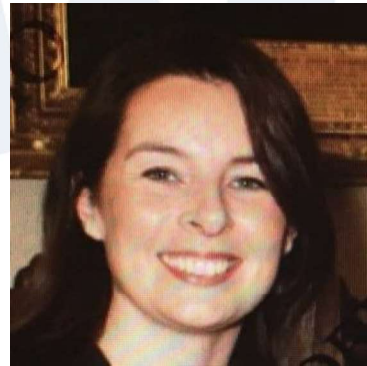
Rodney H. Falk, MD
Director, Brigham Amyloidosis Program



Giada Bianchi, MD
Associate Director, Brigham Amyloidosis Program



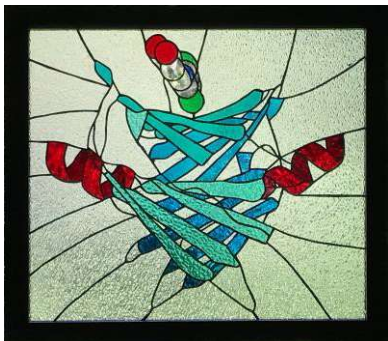
Sharmila Dorbala, MD, MPH
Director of Nuclear Cardiology, Brigham and Women's Hospital



Sarah Cuddy, MD
Clinician-Researcher, Brigham Amyloidosis Program

THANKS TO YOUR SUPPORT, the Amyloidosis Program at Brigham and Women's Hospital is transforming the diagnosis and treatment of amyloidosis. Under the direction of **Rodney H. Falk, MD**, in collaboration with oncologist **Giada Bianchi, MD**, imaging expert **Sharmila Dorbala, MD, MPH**, and clinician-researcher **Sarah Cuddy, MD**, this world-renowned program is bringing new hope to patients in Boston and beyond. As you read the following updates, we hope you take great pride in the vital work made possible by your philanthropy.

In the past year, Drs. Falk, Bianchi, Dorbala, and Cuddy have made great strides advancing clinical trials for new amyloidosis treatments, collaborating with centers worldwide to improve patient care, and fostering the next generation of leaders. We are pleased to share highlights of their pioneering work.



A grateful patient of Dr. Falk's who is also a scientist and an artist creates stained-glass images of cells and other organisms to illuminate scientific concepts. Above, the artist's rendition of transthyretin protein, which has been implicated in amyloidosis.

Evaluating New Treatments for Amyloidosis

“Silencer” drugs for cardiac amyloidosis. Drs. Falk and Cuddy are leading three clinical trials to evaluate the efficacy of drugs that slow or halt the progression of cardiac amyloidosis by reducing levels of transthyretin (TTR) protein in the body. An overproduction of TTR can lead to amyloid deposits in the heart and other organs.

- A total of 32 patients have now been enrolled in a phase 2 trial of inotersen (Ionis Pharmaceuticals) and will receive the drug over the next two years. Early results have been promising, showing that the drug lowers TTR protein by 70 to 80 percent.
- Recruitment recently began for the vutrisiran phase 3 multicenter placebo-controlled randomized trial, HELIOS-B. Vutrisiran (Amylin Pharmaceuticals) is only administered once every three months, a clear benefit for patients.
- Recruitment is about to start for the CARDIO-TTRansform study, which will evaluate whether monthly doses of the drug AKCEA-TTR-LRx can slow or halt disease progression.

An antibody for light-chain (AL) amyloidosis. Dr. Bianchi is a lead investigator for the Cardiac Amyloid Reaching for Extended Survival (CARES) phase 3 clinical trial. CARES is evaluating the efficacy of the antibody CAEL-101 in dissolving amyloid deposits in the heart of patients with AL amyloidosis, a blood-based disorder related to multiple myeloma. In earlier trials, CAEL-101 proved safe when administered on its own and in combination with chemotherapy (a standard AL amyloidosis treatment), and it extended overall survival for patients with advanced disease. The study aims to enroll about 370 patients from multiple sites around the world.



A renowned imaging expert. Dr. Dorbala is the 2020 President of the American Society of Nuclear Cardiology and was recently elected to the Council of Distinguished Investigators of the Academy of Radiology and Biomedical Imaging Research.

Developing Amyloidosis Centers of Excellence Around the World

Dr. Dorbala continues to advance research to improve noninvasive imaging techniques, which are enabling earlier and more accurate diagnosis of amyloidosis. In 2019, she chaired a panel of international experts to develop guidelines for the use of diagnostic imaging in cardiac amyloidosis. Building on that work, Dr. Dorbala is now spearheading efforts to help major medical centers around the world develop amyloidosis programs of their own. In collaboration with centers in Kuwait, India, and South Africa, she has applied for a grant from Pfizer to launch a quality improvement project. The grant will support diagnostic imaging costs for centers that do not have funds for these critical scans. Guided by the Brigham's amyloidosis expertise, the centers aim to work together to improve their abilities to identify patients at risk for amyloidosis, interpret diagnostic images, and optimize patient care. Ultimately, the goal is for each center to be able to host their own national meetings, teach their peers, and—over time—evolve into amyloidosis centers of excellence.

Fostering the Next Generation of Leaders

Fellows in the Amyloidosis Program participate in comprehensive clinical training that helps them become exemplary caregivers while offering the opportunity to pursue innovative research. Highlighted below is a recent graduate of the program who has now joined the program faculty as well as our current fellow.



SARAH CUDDY, MD

Physician, Division of Cardiology

After excelling as a fellow in the Amyloidosis Program, Dr. Cuddy joined the faculty as an

attending physician in the Division of Cardiology in spring 2020. In addition to caring for patients with amyloidosis, Dr. Cuddy aims to further her research on cardiac amyloid imaging that she began during her fellowship under the mentorship of Drs. Falk and Dorbala. Highly skilled in cardiac MRI and other imaging technologies, Dr. Cuddy has been invited to present at several international conferences.



VASVI SINGH, MD

2020–2021 Fellow, Amyloidosis Program

A rising star in the field, Dr. Singh received the first American Society of Nuclear Cardiology/Pfizer

Young Investigator in Cardiac Amyloidosis Research Fellowship Award. The award recognizes an early-career researcher whose work has the potential to advance the science of cardiac imaging. Her winning research project will investigate whether loss of muscle mass in adults 65 years or older is an early sign of amyloidosis. Dr. Dorbala will serve as mentor on the project.



Dr. Falk reviews patient data with a colleague.

YOUR GENEROSITY has played a vital role in the growth and global influence of the Brigham's Amyloidosis Program. With your help, Drs. Falk, Bianchi, Dorbala, and Cuddy are advancing groundbreaking clinical trials for new treatments and diagnostic approaches, while mentoring outstanding fellows who are on track to shape the future of the field. On behalf of our entire team, as well as the many patients and their loved ones who will benefit from this work, thank you for your support and partnership. Together, we are improving lives and transforming the future of medicine.

