From the Director

Dear Friends,

Best wishes from the Amyloidosis Center at Boston University School of Medicine and Boston Medical Center for 2017. I am pleased to introduce you to this Newsletter on behalf of the clinicians, administrative staff and researchers. I take this opportunity to thank you for your support and friendship, and to update you on some the highlights of the last year.

The XVth International Symposium on Amyloidosis was held in Uppsala, Sweden from July 3-7, 2016. Twenty members of our research and clinical teams attended and presented research at this meeting. It was a wonderful opportunity to discuss amyloid research with investigators from around the world and to initiate collaborations. Several of the presentations were made by our students and trainees, and brief highlights of these and other contributions from our group are described in this report.

Our Multidisciplinary Amyloid Clinical Program continues to grow. Each week, 20 or more patients are evaluated by a multidisciplinary team of clinical experts and given opportunities to participate in standard and novel therapies for AL, AA, and ATTR amyloidosis. We are able to offer innovative treatments tailored to each patient’s need and disease type. This is truly a new era of expanding therapeutic landscape for systemic amyloidosis. Several new physicians have joined our clinical team this year and we are pleased to welcome them. Dr. Sabrina Browning, (internist), Dr. Omar Siddiqi (cardiologist) and Dr. Shayna Sarosiek (hematologist) have joined our high performing, dedicated team of clinicians to care for patients with amyloid diseases.

We continue to build our basic laboratory research programs and to collaborate with investigators across our university and at other medical centers. We are confident that the treatments of the future will be guided by basic research studies. For a report on our research activities, see Dr. Lawreen Connors’ column on page 3.

Last year, we wrote to you about the death of our beloved director, Dr. David Seldin. We continue to miss his leadership, but we go forward with the legacy he left us and our mission to develop better treatment and cures for amyloidosis. I am grateful and pleased to report to you that “David C. Seldin, MD, PhD Amyloid Research Training Fund” has been established in his memory and the first recipient of this will be a visiting scholar, Dr. Roberta Mussinelli, from Italy. She will participate in studies on cardiac amyloidosis under the mentorship of Dr. Frederick L. Ruberg in our Center. While we miss Dr. Seldin, we are grateful for your help in establishing the Training Fund as a lasting memory.

I am honored and delighted to accept the directorship of the Amyloidosis Center and my vision is to create a high performing future oriented center with better and continued international recognition. We are devoted to expanding diagnostic categories and developing therapeutics to target different stages of pathogenesis, staying tuned with the goal of shared passion for interdisciplinary research. The focus will be on excellence, innovation and collaboration.

I hope you enjoy reading this report and learning more about this past year. Our hopes for 2017 are high; progress is only possible with your generous and steadfast support. We send our best wishes for a happy and healthy and prosperous 2017 for you and yours.

Dr. Vaishali Sanchorawala

Dr. Lawreen Connors

Dr. John Berk

Dr. Shayna Sarosiek
ATTN and AA Amyloidosis

By John Berk, MD

Over the past year, we and approximately 45 patients partnered to participate in international clinical trials to develop new drugs for ATTN and AA amyloid diseases.

We are disappointed to report that Kictla (eprodostat), a glucosaminoglycan mimetic, did not show benefit. Finding and treating the underlying cause for the chronic inflammatory state driving the production of AA amyloidosis appears to slow progression of kidney injury just as well as Kictla. We await further direction from the Sponsor of the study, but at present have no information about additional studies involving Kictla.

Treatment for ATTN amyloidosis has progressed on 2 fronts: TTR stabilizers and TTR silencing agents. All clinical trials for gene silencing agents completed enrollment at the beginning of 2016. Final data collection and initial analyses should occur by the summer of 2017. Diffusil, a TTR tabilizer, remains the only available agent in the USA. While the Diffusil Trial demonstrated the drug dramatically inhibits the progression of polyneuropathy, the new TTR gene silencing drugs may prove to be even more successful in altering the course of ATTN disease. In addition, Pfizer is testing two different dosages of another TTR stabilizer, Tafamidis, in patients with ATTN amyloid cardiomyopathy. We anticipate additional studies could open in the coming year testing the effects of TTR gene silencing agents on ATTN amyloid cardiomyopathy and, possibly, an additional new agent to disrupt existing amyloid deposits.

We look forward to partnering with you to develop several new treatments for your benefit and to better the lives of future generations.

AL Amyloidosis

By Anthony C. Shelton, RN, OCN

Amyloidosis center has been at the forefront of clinical trials for AL Amyloidosis for many years. Widely used treatments such as Bortezomib and Lenalidomide were first administered in clinical trials to patients who were willing to participate without assurance of benefit. Fortunately, these novel treatments are beneficial and helping patients with AL amyloidosis based on these pivotal clinical trials that were conducted here. Other investigational medications such as Bendamustine, Carfilzomib, and Pomalidomide, have now provided important information regarding side effects, dosing, and efficacy.

Today, the Cancer Clinical Trials Office, which administers AL Amyloidosis studies, continues to provide trials to search for answers to the question of effective treatment for this disease. Currently, there are several clinical studies available for enrollment with ixazomib, NEOD001 anti-fibril antibody, Idelalisib and a combination of Pomalidomide and Bortezomib. A new clinical trial with Daratumumab (monoclonal antibody with a great promise in myeloma) will start enrollment in January 2017.

We are excited to have two new Research Nurses join our team this year. Mary Ellen Migre, RN, and Meredith Mistark, RN will be joining Anthony Shelton, RN in coordinating the care of patients participating in clinical trials for AL Amyloidosis. Please ask your doctor about our currently enrolling clinical trials.

I am delighted to introduce you to the newer members of the Gerry Amyloid Laboratory and share some of this past year’s news from our group. Basic science research aimed at understanding the why and how of amyloid deposits is a cornerstone of the Boston University Amyloidosis Center and our entire team of investigators is strongly committed to accomplishing this goal. The studies that are currently underway in our laboratory (and those of several collaborators) have all been designed to help improve our understanding of this devastating disease.

Within the last year, our research group has been expanded and now includes a dedicated and dynamic group of students and trainees who are at various stages in their careers.

- Dr. Celia Torres Arancivia joined us as a post-doctoral fellow in May of this year. She received her PhD in Biochemistry from the City University of New York after completing a study of several cell membrane proteins implicated in Alzheimer’s disease. Results from her thesis research have been published in several peer-reviewed journals. Celia is now investigating the biochemical properties of clusterin, a blood protein that we feel plays a critical role in transthyretin-associated forms of amyloid disease (ATTRwt and ATTRm amyloidosis).

- Ms. Aparna Bhukar is a second year graduate student in the Boston University Bioinformatics MS program. She has an MS in Electrical Engineering from Columbia University and extensive experience with datasets from various disciplines. Aparna is interning with us this summer and her work involves developing and implementing updated versions of our immunoglobulin light chain platform (ALbase) and amyloid patient database.

- Mr. Hassan Aboofahla is a graduate student enrolled in the Boston University Graduate Medical Sciences program. In his second and final year, Hassan has completed his course work and opted to perform bench top research over the literature review for fulfillment of the MS degree requirements. His project involves studying the structure and stability of a very rare and unique transthyretin protein (TTR-p.Glu51_Ser52dup).

• Mr. Hassan Abdullahi is a graduate student enrolled in the Boston University Graduate Medical Sciences program. In his second and final year, Hassan has completed his course work and opted to perform bench top research over a literature review for fulfillment of the MS degree requirements. His project involves studying the structure and stability of a very rare and unique transthyretin protein (TTR-p.Glu51_Ser52dup).

- In mid-September, we welcomed Ms. Amber Polia to the laboratory. Amber recently graduated from Boston University with a BS in Neuroscience which she managed to complete in less than 4 years. She is rapidly becoming familiar with the techniques and instrumentation used in the laboratory to conduct our biochemical, biophysical, and histologic studies.

- While not a new addition to our research group, Ms. Jacqueleen Sikora Hanson, nevertheless, is well worth mentioning as she is about to complete her PhD degree requirements with the defense of her thesis research on Monday, November 21st. Over the past 5 years, Jacqueleen has been studying genetic variation and disease associations in ATTRwt amyloidosis, trying to help us understand why men far outnumber women in patients diagnosed with this amyloid disease. Her genetic study is the first such investigation of a large, well-characterized ATTRwt group and the initial results have been published in the journal, Human Genetics.

A highlight of this past year includes the participation of our research group at the XVth Amyloidosis Symposium which was held in Uppsala, Sweden from July 3-7, 2016. All 12 abstracts reporting results from many of our basic science studies were accepted for presentations at the meeting. In addition, travel awards were granted to 3 of our young investigators and we are most grateful to the Amyloidosis Foundation and the Boston University Division of Graduate Medical Sciences for this support. Manuscripts detailing each of these studies have been submitted for publication or are in preparation.
Saije and Calli Beatman and their family were special visitors last summer. Their beloved grandmother had died from amyloidosis soon after her diagnosis and before she could seek a treatment center. The girls wanted to help find a cure and put their talents to work in memory of their grandmother. Saije competed and was a winner in the McGee Middle School Berlin, CT Essay Contest on why she wanted to support amyloid research. The students at McGee had been hosting bake sales, penny drives, hoop challenges, and other fund raising efforts as part of the school’s emphasis on supporting philanthropy. Big sister, Calli Beatman, a high school student ran her own fundraiser, a mini-golf tournament. Using social media advertisement and engaging the mini-golf center’s support, Calli gathered friends and family for a successful tournament that netted more than $5,000 for amyloid research. How proud their grandmother would be of the philanthropic awareness, the enterprising nature of the fundraising events, and the hard work of Saije and Calli!

The mother and sisters of Eileen Cochran were special visitors from Arizona last summer. Eileen died of amyloidosis very soon after her diagnosis at the young age of 50 in 2003. Her sisters started a fundraiser, Walk for an Angel, in her memory and over the years they have raised more than $110,000 for amyloid research. Eileen has not been forgotten! We had a wonderful visit with Eileen’s mother, Celina Valenzuela, and sisters, Deborah Baxter, Elva Dillavou, Cindy Arino, Shelly Cordova and Yolanda Valenzuela, when they came to tour the Baxter, Elva Dillavou, Cindy Arino, Shelly Cordova

Eileen’s mother, Celina Valenzuela, and sisters, Deborah Baxter, Elva Dillavou, Cindy Arino and Shelly Cordova

One our aims here at the Center is to be a resource to scientist, physicians and patients for information on amyloidosis. The Amyloidosis Center clinic hosted 2 visiting clinicians this year.

Dr. Esther Gonzalez, MD, Ph.D., was here in the U.S. on sabbatical at Brigham & Women’s Hospital in October 2016. Dr. Gonzalez was visiting from Universitario Puerta de Hierro Majadahonda in Madrid, Spain. She is a faculty member of the Heart Failure and Inherited Cardiac Diseases Unit as well as working in the Myocardial Pathophysiology area at CNIC (Centro Nacional de Investigaciones Cardiovasculares) also in Madrid.

Johana R. Almansa, MSN, ANP-BC CHPN, from the department of Cardiology at Johns Hopkins Hospital in Baltimore, MD visited in September 2016. Johana works in the Heart Failure Bridge Clinic under the direction of Dr. Daniel Judge. Her primary interest is in facilitating the management of Cardiac Amyloidosis patients. Under the guidance of Dr. Judge and with the valuable information she gathered during her experience at our center, they were able to create a clinical pathway to facilitate the diagnosis of cardiac amyloidosis at the outpatient heart failure clinic at Hopkins. The clinical pathway will allow the nurse practitioners who are seeing patients after a routine hospital discharge to screen for cardiac amyloidosis and initiate the diagnostic workup when indicated. Their goal is to identify amyloidosis early in the process and hopefully improve their chance to receive treatment and survive this awful disease. This clinical pathway will be implemented at Hopkins in February 2017.

We are very grateful for your help in establishing the David C. Seldin, MD, PhD Amyloid Research Training Fund in memory of our beloved former director. Our goal was $100,000 and with your generosity we received more than $130,000 to establish an endowed fund. This Fund will be used to help support the training of young investigators in amyloid research, a passion close to Dr. Seldin’s heart.

I am delighted to report that the first recipient of a David C. Seldin, MD, PhD Amyloid Research Training Award will be visiting scholar, Dr. Roberta Mussinelli, from Pavia, Italy. She will join the Amyloidosis Center clinical program from January through June, 2017. While here she will participate in research studies on cardiac amyloidosis under the mentorship of Dr. Frederick Ruberg.
The Amyloidosis Center at Boston University School of Medicine is pleased to recognize the generosity of its many donors whose support has assisted us in enhancing and continuing our progress in discovering for amyloidosis. We thank our donors for their ongoing participation and commitment. This donor list recognizes individuals who have made gifts totaling $250 or more to the Amyloidosis Center between January 2013 and October 2015. We have made every effort to provide a complete and accurate list, but may have made an error in any area that may have been the result of a typographical error. While space constraints prevent us from listing the names of donors of gifts under $250, we very sincerely appreciate the support of those many donors.

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