Amyloidosis Center WINTER 2019 | ISSUE 32 | bu.edu/amyloid

Dear Friends,

Best wishes from the Amyloidosis Center at Boston University School of Medicine and Boston Medical Center for upcoming 2019. I hope you had a blessed holiday season with friends and family. This newsletter describes the extraordinary successes of our faculty, trainees and staff.

We completed 2018 with record-breaking achievements in the growth of our clinical program, new research grant funding and publications in the peer-reviewed medical journals. Accomplishment at this level only occurs through collective efforts of all



members of the Center including you. We continue to hold ourselves accountable to the highest standards of excellence.

Our Clinical Program thrives to grow. Each week, 25 or more patients are evaluated by a multidisciplinary team of clinical experts and given opportunities

to participate in standard and novel therapies for AL and ATTR amyloidosis. We are able to offer innovative treatments tailored to each patient's need and disease type. This year marks a momentous period in the development and expansion of novel agents to treat hereditary TTR amyloidosis. We are proud to be in the forefront of this innovation.

Amyloidosis Center faculty and investigators pursue cutting edge research on the frontlines of basic laboratory research in the Alan and Sandra Gerry Amyloid Research Laboratory. The long tradition of medical research is exemplified by the collaboration with investigators across our university and at other medical centers. We are ecstatic to welcome and introduce Gareth Morgan, PhD. Dr Morgan joined our basic science team in October. His research will focus on translational research to understand the mechanisms of light chain misfolding and development of small molecules to prevent misfolding of the light chains as a possible therapeutic strategy for AL amyloidosis: the epitome of targeted personalized therapy for AL amyloidosis.

Eighteen members of our research and clinical teams attended and presented science at the XVIth

International Symposium on Amyloidosis in Kumamoto, Japan in March. It was a wonderful opportunity to discuss amyloid research with other investigators from around the world and to initiate scientific collaborations with our multinational colleagues.

This newsletter includes a personal and powerful pledge from Ms. Christine Stiefel, whose husband passed away from systemic amyloidosis in 2005. Through her foundation, The Wildflower Foundation, she has been supporting our groundbreaking research for many years. This year, the Wildflower Foundation, has committed a \$1 million multiyear pledge to the Amyloidosis Center, but it is contingent upon your support. Gifts from this foundation are dependent on other donors joining to support the mission of the Center. New or increased gifts will be matched by The Wildflower Foundation through the matching challenge. Please understand that your gift to the Amyloidosis Center is more than just a gift, it is an investment to make this lifesaving work possible.

I hope you enjoy reading this report and learning more about this past year. Our hopes for 2019 are high; progress is only possible with your generous and steadfast support. We send our best wishes for a happy, healthy and prosperous 2019 for you and yours. I take this opportunity to thank you for your support and friendship. It takes a team to conquer Amyloidosis!

Sincerely yours,

ali Sancherawala

Vaishali Sanchorawala, MD

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Amyloidosis Center

The Wildflower Foundation

Dear Supporters and Future Supporters,

I know many of you presently support the fight against this terrible disease amyloidosis, and I hope others will become new supporters.

Like me, you know the impact amyloidosis can have whether you are a patient or a caregiver. My husband, Bob, was diagnosed with kidney disease. He fought for years, went on dialysis and did everything right.



Bob and Christine Stiefel

In the early 2000s he received a kidney transplant and was doing well. Then he was diagnosed with amyloidosis. It was debilitating and extremely painful. Through his doctors and his own research, he found the Amyloidosis Center at Boston University School of Medicine. We planned a trip to Boston in June 2005, but he died on 31 May 2005.

The Amyloidosis Center at Boston University School of Medicine is one of the largest and oldest centers in the world devoted to laboratory and clinical research on systemic amyloidosis. When the Center began as a small research program, directed by Dr. Alan S. Cohen, 50 years ago, the nature of amyloid diseases was poorly understood, and in the early days there were no treatments available. Drs. Martha Skinner and David Seldin had the vision to establish a multidisciplinary program involving clinicians and scientists across the Boston University Medical Campus, allowing the Center to make tremendous progress in our understanding of these diseases and in diagnosis and treatment. And now, under the leadership of Dr. Vaishali Sanchorawala, the Center is entering an exciting new era of personalized and targeted therapies.

Through my foundation, The Wildflower Foundation, I have been supporting the Center's groundbreaking research for many years. This year I want to increase my funding for the great plans already prepared by the Center. I have committed a \$1 million multiyear pledge to the Amyloidosis Center, **but it is contingent on your support.**

In order to encourage contributions to the Amyloidosis Center, I have made an initial gift of \$400,000 to support research—and will continue to provide support over the coming three years up to \$1 million. However, gifts are dependent on other donors joining with me to support the Center. **New or increased** gifts will be matched by The Wildflower Foundation through this matching challenge. **So please give now and give generously to make this lifesaving work possible.**

Gifts of all sizes are welcome and a response card has been included for your convenience. If you have questions about making a gift, please contact BUSM's assistant dean of development, Suzanne Maselli, at 617.358.9530, or <u>smaselli@bu.edu</u>. To make a secure gift online, please visit <u>bu.edu/</u> <u>amyloid/donate</u>.

Thank you in advance for your generosity. Sincerely,

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Christine Stiefel

Gerry Amyloid Research Laboratory Update

I am delighted to share some of this past year's news from the Alan and Sandra Gerry Laboratory of the Amyloidosis Center, a facility that serves as 1) an area for biochemical, biophysical, and cell-based investigations of amyloid proteins; 2) an international reference center for diagnostic testing of amyloidosis and determination of amyloid disease type; and 3) a repository for thousands of clinical specimens and de-identified patient data. The Gerry Amyloid Laboratory team is comprised of an outstanding group of dedicated research scientists, graduate and post-doctoral students, and laboratory technical assistants, all committed to discovering better treatments, improving diagnosis, and ultimately finding cures for these devastating amyloid diseases.

Highlights of 2018 have included expansion of our research group, receipt of vital funding from private and government sources, renewal of our CAP accreditation/CLIA approval for amyloid diagnostic testing, a number of publications detailing our basic science research findings and participation of our research group at the XVIth Amyloidosis Symposium, which was held in Kumamoto Japan from March 25-29, 2018.

We also have welcomed Gareth Morgan, PhD as a research assistant professor of medicine to our team. Dr. Morgan's research interest is AL amyloidosis and his experimental studies will be aimed at deciphering the mechanism of amyloid formation and developing small molecule stabilizers of amyloid-forming light chains as potential therapeutics.

Funding for our important basic science studies came from a variety of sources in 2018. Dr. Celia Torres Arancivia, a post-doctoral fellow in the Gerry Lab, received a grant from the Amyloidosis Foundation to study the biochemical properties of clusterin, a blood protein that plays a critical role in transthyretin-associated forms of amyloid disease (ATTRwt and ATTRm amyloidosis). Generous funding from the E. Rhodes and Leona B. Carpenter Foundation supported continuation of a project investigating the profile of blood proteins in patients with ATTRwt amyloidosis in an effort to discover disease biomarkers in an under-recognized cause of congestive heart failure in older adults. Drs. Haili Cui and Lawreen Connors participated in a sponsored research agreement with Proclara Biosciences to study the ability of compounds developed by the company to bind and remodel amyloid in archived heart tissue samples. Importantly, a four-year research study of renal AL amyloidosis pathobiology will be funded through generous support from the Wildflower Foundation, Inc. We are extremely grateful to Ms. Christine E. Stiefel and the Foundation for this and previous grant funding which has enabled our team, including Drs. Tatiana Prokaeva, Elena Klimtchuk and Andrea Havasi to make significant progress toward unlocking the how and why of immunoglobulin light chain amyloid disease.

This year, several key manuscripts detailing data from our experimental studies were published in peer-reviewed journals. Strong and successful integration of the clinical and research teams led to a report by Dr. Jacquelyn Sikora Hanson in Circulation: Heart Failure, describing the prognostic value of serum prealbumin measurement in transthyretin-associated cardiac amyloidosis. Dr. Elena Klimtchuk, a senior scientist on our team, presented the biophysical and biochemical features of a newly discovered, highly aggressive transthyretin mutant in the prestigious journal, PNAS. In June, a paper documenting a collection of ATTR amyloidosis patient-specific induced pluripotent stem cells available for research studies was published in Amyloid: Journal of Protein Folding Disorders by Richard Giadone, a doctoral graduate student exploring the use of patient-derived stem cells to model ATTR amyloidosis.

In the spring, a majority of the Gerry Lab team traveled to Japan to attend the XVIth Amyloidosis Symposium. Basic science presentations included invited talks by Dr. Celia Torres and Ms. Eila Maenpaa who detailed recent studies of protein modifiers and genetic risk variants in age-related transthyretin amyloidosis (ATTRwt). Doctoral graduate students, Batbold Boldbaatar, Richard Giadone, and Nicholas Frame, each presented results from

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their research projects aimed at deciphering the mechanism and implications of amyloid (TTR and AA) formation, and testing new strategies to interrupt the process. Dr. Elena Klimtchuk showed comprehensive experimental data providing an explanation for the aggressive nature of a newly identified and unique TTR mutant protein identified in a patient seen at our center. Dr. Olga Gursky presented data in a keynote address showing how oligomers formed by serum amyloid A contribute to the pathogenesis of AA amyloidosis. In addition, travel awards were granted to several of our young investigators and we are most grateful to the Amyloidosis Foundation for this support.

by Lawreen Connors, PhD

Update on ATTR Amyloidosis

The past year has seen unparalleled development of new treatments for ATTR amyloidosis, culminating in three publications in the New England Journal of Medicine. Two of these medicine, Inotersen (Tegsedi[®], Ionis Pharmaceuticals/Akcea Therapeutics) and Patisiran (Onpattro®, Alnylam Pharmaceuticals), are designed to stop production of transthyretin (TTR) by the liver, effectively eliminating the protein that misfolds and deposits as ATTR amyloidosis. These two drugs constitute a new category of therapeutics for hereditary ATTR (hATTR) amyloid neuropathy: TTR gene silencers. Many of our patients participated in the global phase III studies that proved the effectiveness of Inotersen and Patisiran. Unlike the TTR protein stabilizing drugs, Diflunisal and Tafamidis, the TTR gene silencers can potentially improve sensory nerve function as seen in 37-56% of patients receiving Inotersen or Patisiran in the phase III studies. The FDA approved Patisiran (early August) followed by approval of Inotersen (early October) for treatment of hATTR amyloid neuropathy. Although secondary data suggested these drugs may treat hATTR amyloid cardiomyopathy, the FDA restricted approval to patients with hATTR amyloid neuropathy. Both of the TTR gene silencers should be available at community medical centers now or in the near future.

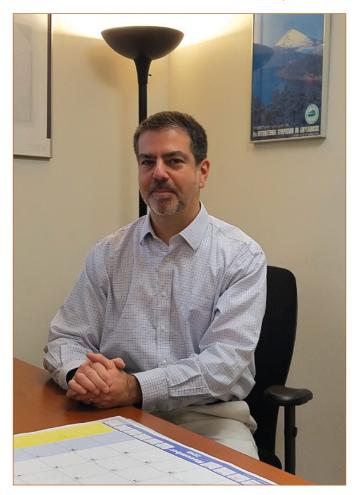
To add to the magic of new ATTR amyloid treatments, Pfizer Pharmaceuticals conducted a global phase III study examining the effect of **Tafamidis**, a TTR protein stabilizer, on ATTR amyloid cardiomyopathy both the hereditary and wild-type forms of disease. To the delight of all involved, Tafamidis extended survival and decreased the number of heart-related hospitalizations of patients receiving the drug. By the preliminary analysis, patients with mild to moderate wild-type ATTR amyloid cardiomyopathy appeared to benefit most. Pfizer plans to submit an application to the FDA by the end of 2018 for approval to market the drug. The FDA will have six months from receipt of the application to review the data and determine whether the drug merits approval to market. In the interim, Pfizer has expanded its open-label extension (OLE) study to new participants, making Tafamidis available to those who qualify. We are one of those sites conducting the OLE study.

Expanding the drug armamentarium for ATTR amyloid neuropathy and cardiomyopathy is exciting and fulfilling, but it is just the beginning. In collaboration with our patients, we hope to develop drugs for patients with limited treatment options.

by John Berk, MD

SCAN-MP Project

ATTR cardiac amyloidosis is an emerging disease. Not emerging in the sense that patients are contracting new cases (as is seen with viral outbreaks), but rather that new cases are being identified with increasing frequency. In fact, at our Center, ATTR amyloidosis represents 35% of new cases of cardiac amyloidosis



referred for evaluation over the past 7 years. This compares to only 13% in the prior time frame. Other referral centers have seen a similar influx of new cases of ATTR amyloidosis. Why is this so, when doctors are still taught that amyloidosis is a rare disease?

What has become clear is that awareness of amyloidosis is increasing, and with increased awareness comes increased identification. This results from a number of concurrent advances in the diagnosis and treatment of ATTR amyloidosis that work synergistically to prompt clinicians to consider the disease. First, about five years ago, a decades-old cardiac imaging test approved to identify myocardial infarction was discovered to selectively identify ATTR cardiac amyloidosis. This imaging test is called technicium pyrophosphate (PYP) imaging (in Europe the radiotracers technicium DPD or HMDP are used). These radiotracers are taken up by bone and, it turns out, the heart in the context of ATTR amyloidosis. There is no diagnostic PYP uptake seen in other causes of heart failure, permitting the test a high degree of accuracy. The scans are available nearly everywhere, inexpensive, safe with very low radiation dose, and easy to interpret. Multiple studies have demonstrated the accuracy of these scans as compared to invasive heart biopsy, scan reproducibility, and capacity to identify undiagnosed ATTR amyloidosis in populations of patients with evidence of congestive heart failure. At the BU Amyloidosis Center, we have participated in many of these critically important studies. Importantly, given this new evidence, cardiology-based medical societies such as the American Society of Nuclear Cardiology and Heart Failure Association of America have embraced PYP imaging and ATTR amyloidosis as important educational areas meriting specialized symposia and continuing medical education activities. Such attention serves to increase awareness and guide physicians in the application of these new imaging tests. Now ATTR amyloidosis can be accurately diagnosed without the need of an invasive heart biopsy.

Second, pharmaceutical companies have identified ATTR amyloidosis as a disease space for drug development. This past summer, three large clinical trials of three new pharmaceuticals were published in the prestigious New England Journal of Medicine each showing benefit in ATTR amyloidosis. Again, physicians scientists and patients at the BU Amyloidosis Center participated in each of these trials. Data from these trials were sufficient to permit FDA to approve both patisaran or Onpattro[™] (Alnylam Pharmaceuticals) and inotersen or Tegsedi[™] (Ionis/ Akcea Therapeutics) as the first treatments for ATTR

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amyloidosis. Both drugs were approved for hereditary ATTR (hATTR or ATTRm) amyloidosis with evidence of neuropathy. It is important to note that neither of these drugs was approved for ATTR amyloidosis with cardiomyopathy and neither was approved for ATTR wild-type (ATTRwt) amyloidosis, while evidence suggests that ATTRwt cardiac amyloidosis is likely to be by far the most common amyloid type. In addition, the most commonly encountered hATTR mutation is Val122IIe, seen in 3.4% of African Americans. While it is unknown how many African Americans with this genotype develop hATTR amyloidosis, this form is most often not associated with neuropathy. While at our Center we utilize the oral drug diflunisal as a means to slow ATTR amyloidosis progression, this drug, while effective and inexpensive, is not tolerated by many patients. Thus, we await FDA action on the 3rd new drug reported this summer, tafamidis (Vyndagel[™], Pfizer, Inc.) which will seek approval for ATTR amyloidosis with cardiomyopathy and provide needed treatment for this population.

Exactly how common ATTR amyloidosis is remains unclear with conservative estimates of 100,000 to 200,000 patients with ATTRwt based on extrapolation from smaller studies. The proportion of heart failure caused by hATTR amyloidosis from Val122IIe is also unclear. Finally, how PYP imaging might fare as a screening or early diagnosis test in a population of patients in whom amyloidosis is not at all suspected also remains unknown. Given these important knowledge deficits, a team of investigators at our Center working in close collaboration with Columbia University Medical Center announce a new study called SCAN-MP or Screening for Cardiac Amyloidosis with Nuclear Imaging in Minority Populations. Led by Frederick Ruberg, MD at BU/BMC and Mathew Maurer, MD at Columbia, the SCAN-MP Study will be a multicenter, prospective, cohort study of 800 older Black and/or Hispanic adult patients with congestive heart failure that will determine the prevalence of transthyretin (ATTR) cardiac amyloidosis. The fiveyear, \$7 million study is funded by the National Heart, Lung and Blood Institute (NHBLI) of the National Institutes of Health (NIH) and is being conducted under the auspices of an IND (Investigational New Drug) for PYP imaging issued by the Food and Drug Administration, Division of Medical Imaging Products (DMIP). The overall goal of SCAN-MP is to facilitate earlier diagnosis of patients with ATTR cardiac

amyloidosis at time when administration of emerging pharmacotherapies would be most beneficial. New cases of ATTR amyloidosis identified will be referred to the respective treatment center in Boston or New York. SCAN-MP will also answer important questions regarding sex-based differences previously reported in ATTR amyloidosis (male predominance), explore whether hATTR or ATTRwt have different rates of progression, and test the performance of a newly developed clinic-based prediction tool that would permit doctors to determine the likelihood a patient they are seeing has ATTR amyloidosis. Important insights into the mechanism of TTR misfolding that precedes amyloid fibril formation and deposition will also be gained through collaboration with Dr. Jeffery Kelly at The Scripps Research Institute. Collaborating recruitment sites will include Harlem Hospital in New York, and community health clinics associated with Boston Medical Center. SCAN-MP will be posted on ClinicalTrials.gov (NCT number pending at present time) and should start enrolling participants in early 2019.

We are extremely excited to undertake this important project which will elucidate the burden of disease related to ATTR amyloidosis borne by African Americans and Caribbean Hispanics who comprise the plurality of patients seen at BU/BMC and Columbia University Amyloidosis Center. The study will establish and longitudinally follow an important new cohort of minority patients with congestive heart failure that will permit exploration of amyloid and non-amyloid based questions. The answers to these questions should directly impact the health outcomes of minority patients with ATTR amyloidosis and heart failure.

by Frederick Ruberg, MD

Dr. Carl O'Hara - In Retirement

Late one afternoon I sat down with Dr. Carl O'Hara, our amyloidosis center hematopathologist, to discuss his contributions to the Amyloidosis Center in light of his recent retirement announcement.

After graduating from Georgetown Medical School, Dr. O'Hara trained at New England Deaconess Hospital in the surgical pathology department. After training he began his career working as a member of the Anatomic & Clinical Pathology department.



In 1991, Dr. O'Hara was recruited to the Boston University Mallory Institute/Department of Anatomic Pathology as the Associate Professor and Chief of Surgical Pathology & Hematopathology by Dr. Michael O'Brien, who was the Chief at the time. In addition, he lectured to second-year medical and dental students. In 2006 he was promoted to Professor, and in 2012 he became Chief of Laboratory Medicine.

In the mid to late 1990's Dr. O'Hara received a call from then Director of the Amyloid Treatment and

Research Program, Dr. Martha Skinner, inquiring if he could be more descriptive with the details of the amyloid bone marrow biopsies for research. At that time the only way to capture whether or not there was amyloid deposition was the Congo red stain. Dr. O'Hara began working with Dr. Skinner to explore other method of amyloid deposition detection as well as typing of amyloidosis. He became the director of the Pathology Core of our NIH Program Project Grant in 2001. Soon thereafter, the Amyloid Treatment and Research Program was recognized by Boston University as a Center of Excellence (The Amyloidosis Center) and Dr. David Seldin became the Director. Dr. O'Hara was an integral part of the Center and its research and clinical program. He attended the International Society of Amyloidosis Symposium in Woods Hole, MA in 2006 where he made a major presentation. He really enjoyed the collaboration between Pathology and the Center. He investigated other methods of typing including immunogold, immunohistochemistry and electronmicroscopy, that defined accurate typing of amyloid fibrils. The laboratory expanded and became CLIA approved in 2009 for testing samples sent to us from around the world. This is an important and prestigious accreditation.

Dr. O'Hara is still training young investigators and enjoys teaching; currently he teaches medical students weekly and has included instruction in amyloidosis diagnosis.

In retirement, he will continue teaching part-time and he plans to learn to play golf.

by Janis Johnson, MPH

Welcome Dr. Gareth Morgan

It's a real privilege for me to join the Amyloidosis Center and work with talented and dedicated physicians and scientists. I came from the Scripps Research Institute where I was working with Dr. Jeffery Kelly. I am a basic scientist with a background in structural biology and biophysics. I have studied



amyloid diseases for several years, most recently concentrating on AL amyloidosis, which will continue to be the focus of my research at BU. AL amyloidosis is such a challenging disease to understand and treat because every patient has a single, unique antibody light chain protein that forms amyloid deposits. An

important challenge is to understand why only some light chains do this, and to what extent the properties of a particular light chain drive disease progression. If we can identify properties that distinguish light chains that cause amyloidosis from others, we may be able to design treatments that prevent amyloid fibrils from forming.

My work with Professor Kelly suggested that stabilizing light chains reduces their ability to form amyloid, in a similar way to how transthyretin can be stabilized by diflunisal and tafamidis. The clinical samples and data at the Amyloidosis Center will allow us to develop molecules that could become drugs, as well as identifying which patients might benefit from this approach. We have a long way to go before we can think about testing this strategy in patients, but the Amyloidosis Center has some of the best resources in the world to move this project forward.

Another aspect of my research will be applying recent advances in gene sequencing to AL amyloidosis. Scientists have learned a lot about multiple myeloma, which is a cancer that is closely related to AL amyloidosis, by sequencing the genomes of the cancer cells. Using similar approaches for AL amyloidosis could help physicians decide on the best treatments for individual patients and help with monitoring patients' response to treatment. The data and samples at the Amyloidosis Center will allow us to ask whether particular genetic changes are associated with a different prognosis or response to treatment.

There is a lot of work to do. So I am delighted to be starting work in the Gerry Research Laboratory, and excited to contribute to the mission of the Amyloidosis Center.

by Gareth Morgan, PhD

Genetic Counseling

Students enrolled in the Master of Science degree program in Genetic Counseling at Boston University School of Medicine spent time this semester observing clinics at the Amyloidosis Center. During their first year in graduate school, our students spend one to two days per week observing in various clinics throughout Boston Medical Center and the Greater Boston area. Our goal for our students during these observations is to broaden their understanding of the role of genetics in healthcare, learn how different providers engage with patients and to integrate these experiences into their future professional development. As part of their ongoing clinical education, we ask our students to reflect on each experience, focusing on the lessons learned and ways they will integrate the experience into their future training. The following excerpts have all been shared with permission from first-year Genetic Counseling students.

Our students were impressed by how much of a team-based approach is employed by the Amyloidosis Center providers: "The best part of this experience for me was exposure to the team-based management for disease. I had never been exposed to this before, so it was great to see clinicians in different fields working hand in hand." – Jake

Several other students remarked on how much the families enrolled in this clinic impacted their education: "Being able to put faces and personal stories and experiences to my academic studies has been an invaluable addition to my education that you cannot get by reading a textbook." – Jason

For the hereditary amyloidosis patient encounters, students learned that an individual's concerns may extend far beyond their own care and treatment: "Across all three patients [I observed], there was concern about whether or not they may have passed their illness on to their children. Dr. Libbey explained the genetic testing process, who genetic counselors are and the pros/cons for their children in terms of insurance, psychosocial effects, etc." – Quinn

And for all individuals affected by this condition, our students recognized the powerful impact this

condition has on the entire family: "The Amyloidosis Center observation influenced my training this Fall by showing me the extent to which this condition affects the whole family. Everyone is involved with care and many patients came with aid and support in the form of family members who were very attentive." - Jason Genetic counselors are healthcare professionals with advanced training in medical genetics and counseling to guide and support patients seeking information about their genetic health. Genetic counselors work in a variety of clinical settings including prenatal, preconception, pediatrics, cancer, cardiovascular and neurology genetics. Genetic counselors also work in research, biotech, laboratory and patient advocacy spaces. The primary objective of the Master of Science degree program in Genetic Counseling is to educate graduate students in the core concepts of human genetics and counseling. Our focus is to provide students with the appropriate knowledge, experience and mentoring to become competent, sensitive and motivated genetic counselors. To learn more about Genetic Counseling, visit the National Society of Genetic Counselors website at www.nsgc. org. To learn more about our program at Boston University, visit our website at: bumc.bu.edu/gms/ genetic-counseling

by Lillian Sosa, MS, CGC

Let's Get Social 🛛 🖬 😏

The Amyloidosis Center is on social media! Last year, we launched our official Facebook and Twitter pages. Follow us:

Facebook ► BU Amyloidosis Center Twitter ► @Amyloidosis_BU



Cannot wait for our next newsletter? By establishing a social media presence we hope to keep patients, doctors, and colleagues informed with the latest Amyloidosis Center news and updates while continuing to spread awareness on Amyloidosis. We also hope that our social media platforms can serve as a resource for visitors who would like to know more about Amyloidosis as well as the Amyloidosis Center. In particular, our website has information on the following aspects:

- Amyloidosis Types: Signs and Symptoms
- Our evaluation process: How initial evaluation works and arrangement
- Research: Our Laboratory and Publications
- Diagnostics and Genetic Testing

Live Tweet Event

SAVE THE DATE: Tuesday February 5, 2019 | #AskBUAmyloid

What is the difference between AL and AA Amyloidosis? What are the symptoms of ATTRwt Amyloidosis? Where in my body can Amyloidosis occur? If you have general questions about Amyloidosis and would like to hear from a doctor, this is the event for you! We are excited to announce our first-ever Live Tweet event happening on Tuesday, February 5, 2019 from 10:00am-4:00pm EST.

Here's how it works:

- 1. Log into your Twitter account, or start a Twitter account if you do not have one already, it's easy.
- 2. Press the "Tweet" button on the upper right hand corner and enter your question (in 280 characters or less). Be sure to include #AskBUAmyloid in your tweet, as this is how we track and reply to your tweet!
- 3. Press the "Tweet" button to submit your Tweet!

Dr. Vaishali Sanchorawala, our director, will be personally replying to your tweets. Dr. Sanchorawala is a renowned hematologist and is also the Director of the Stem Cell Transplantation Program. She has been affiliated with the Amyloidosis Center since 1994 and is one of the pioneers in the field of clinical research in AL Amyloidosis.

Please submit your tweets within the allotted time period, as we will not be able to reply to tweets outside of the event period. If you have any unanswered questions, please refer to our website, our social media pages, or give us a call at 617.358.4750. We look forward to tweeting with you!



Earlier this year Boston University upgraded the telephone system on the medical campus. As a result the Amyloidosis Center has a new telephone and fax number.

Telephone 617.358.4750 Fax 617.358.4735



Carl O'Hara, MD and John L. Berk, MD



Elizabeth Pelletier, MA



Lisa Mendelson, NP



Dr. Andrew Staron



Seated (left to right): Brian Spencer, MA, Lawreen Connors, PhD, Gareth Morgan, PhD, Lisa Mendleson, NP Standing (left to right): Celia Torres, PhD, Tatiana Prokaeva, MD, PhD, Batbold Boldbaatar, MD and PhD candidate, Elena Klimtchuk, PhD, Andrew Staron, MD, Camille Edwards, MD



Elena Klimtchuk, PhD



Rainbow over the Boston University Medical Campus



Amyloidosis Center Donor List 2016-2018

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 a cure 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 2016 and November 2018. We have made every effort to provide a complete and accurate list. We apologize in advance for any errors that may have been made. 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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\$100,000 and above

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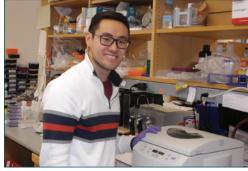
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