



# CENTERPOINTS

## A Big Hello!



**Nancy Berliner, MD,**  
Medical Director of the  
Boston Hemophilia  
Center

*By Nancy Berliner, MD*

I want to take this opportunity to introduce myself as the new Medical Director of the Boston Hemophilia Center (BHC) following the departure of Dr. Ellis Neufeld. It is a pleasure and an honor to take on the leadership of the BHC, the largest hemophilia treatment center in New England.

As Chief of the Division of Hematology at Brigham and Women's Hospital and a practicing clinician, I have a special interest in the clinical care of persons with bleeding disorders. I have worked closely with the skilled physicians in the Center: Drs. Aric Parnes, adult hematologist, and Stacy Croteau, pediatric hematologist. In addition, I have known the dedicated staff and have cared for many of the bleeding disorder patients and families over the years.

I look forward to leading and working with the BHC staff in this exciting time of medical and pharmaceutical advances. The future has never been so bright for persons with hemophilia and other bleeding disorders. The development of new factor products and more effective medical treatment has greatly increased the quality of life for our patients and we are committed to providing the excellent healthcare that you have come to expect. ❖

**Note:** Dr. Berliner is Chief of Hematology at the Brigham and Women's Hospital and a Professor at Harvard Medical School. She received her medical degree from Yale Medical School, and obtained training as a Resident in Internal Medicine, Internal Medicine Chief Resident, and Hematology Fellow at the Brigham and Women's Hospital in Boston, MA. She then spent over 20 years on the faculty of the Yale University School of Medicine, where she rose through the ranks to Professor of Internal Medicine and Genetics. She has broad clinical interests in both classical hematology and hematologic malignancies. She was President of the American Society of Hematology in 2009 and is the current Deputy Editor of the journal *Blood*. She is a member of many prestigious organizations, such as the National Academy of Medicine, the American Society for Clinical Investigation, and the Association of American Physicians. She is the author of more than 80 original articles and editor and author of more than 60 chapters and text books on hematology.

## On the Threshold



*By Aric Parnes, MD*

Sometimes, we find ourselves standing on the threshold of something big. The history of hemophilia has been there before and it will pass through similar doors again. In the 1960s, Dr. Judith Graham Poole realized that the sludge found at the bottom of thawing frozen plasma was loaded with factor VIII. Previously, treatment for hemophilia required plasma

infusions, but the factor concentration in plasma was so low that extreme volumes were needed to be infused to stop acute bleeds. Even fairly routine bleeds, such as those resulting from dental work, required large volumes that could take days to infuse and could lead

to persistent bleeding or even congestive heart failure. The working concept of cryoprecipitate was simple and the cost cheap. Suddenly, an easy and effective therapy became available to patients worldwide.

Of course, cryoprecipitate, although an immense leap in the right direction, was far from a perfect therapy. Firstly, home infusions were still a stretch, and cryoprecipitate, while brimming with factor VIII, could benefit from further purification. Ultimately, cryoprecipitate and other plasma-derived products were impure in other ways, unimaginable at the time. As we all know, plasma pools became contaminated with human immunodeficiency virus (HIV) and hepatitis viruses, which have caused the deaths of thousands of persons with hemophilia in the U.S. alone.

A few years before her discovery of cryoprecipitate, Dr. Poole also helped develop a laboratory test to measure the activity level

>>> continued on page 2

## On The Threshold <<< continued from page 1

of factor VIII. Tests like this became invaluable in not only diagnosing the disease, but determining severity, uncovering inhibitors, and establishing effective therapy plans.

Fast forward 50 years, and we find ourselves once again on the edge of our seats. New factor products are in pharmaceutical pipelines and some have been released for use. Emicizumab, previously called ACE910, is a bispecific antibody to factors IX and X. In other words, this drug was designed as a perfect mock-up of the real factor VIII. It is important to realize that emicizumab is not a perfect copy. It's not even a factor. It's an antibody, designed to have the same targets that factor VIII does. And because it is not a factor, inhibitors to factor don't see it, don't recognize it, and importantly, don't find it. Inhibitors do not touch emicizumab, which means that today's biggest complication in hemophilia may be on the verge of defeat.

Our excitement must be somewhat tempered because of the incidence of several blood clots in the clinical trials when patients with inhibitors were treated with 4-factor activated prothrombin complex concentrate (PCC), also known as FEIBA, during acute bleeds. The type of clotting that was seen is referred to as thrombotic microangiopathy (TMA), meaning that the blood clots are in small vessels and can damage organs, notably the kidneys.

Since then, the trials have been modified to encourage avoidance of the 4-factor PCC and to minimize dosing of activated factor VIIa (Novoseven) if absolutely needed. This problem has not occurred in patients without inhibitors. Despite this side-effect, emicizumab stands to be a major breakthrough for several reasons. One—treatment for patients with inhibitors, called bypass agents, has been poor in regards to efficacy and cost. These drugs are also known to sometimes cause blood clots.

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Two—emicizumab is administered subcutaneously. Amazing! Does that mean no more IV's, no more blown and scarred veins? Not exactly. Emicizumab will be used only for prophylaxis and will transform severe patients into mild patients. That means acute bleeds will still need factor infusions. Hope is widespread that the need for factor

infusions will diminish. But will this hold up when patients, feeling healthy and strong, increase their activity levels and participate in more activities that put them at risk for bleeds? Time will tell.

The third stunning breakthrough with emicizumab is its increased half-life. Half-life is the time it takes for half (50%) of the drug to disappear from the body. Factor VIII concentrates have a half-life of roughly 12 hours leading to intravenous infusions for prophylaxis every 2-3 days. Extended-duration factor VIII products, approved in the last few years, delayed this process, so half-life improved to 14-20 hours. This gave patients one extra day off from self-infusing for prophylaxis. The clinical trials for emicizumab had patients injecting (subcutaneously!) once a week or once every other week. This regimen is conservative since the half-life of the product is 30 days (days, not hours) and undoubtedly, studies will need to follow how often patients really need to treat.

Medical advances excite us as lives are saved and the quality of life enhanced. There is a palpable sense that a true cure is on the horizon. For years, we have dreamt about gene therapy. Now this technology draws near and is rapidly approaching reality for hemophilia B. Multiple companies, scientists, and clinicians are participating in this endeavor and transferring the science into excitement and progress for gene therapy in hemophilia A. In addition, it seems that every aspect of the clotting system will soon be exploited to our advantage. Through my clinical practice, I feel that I am witnessing the resilience and spirit of my patients and finally their courage will pay off. ♦

## Who's Who at the Boston HTC: Mike Miller, Financial Manager, 340B Program



"This is Mike, our finance guy. He deals with, uh....numbers and money."

It would be a safe bet to say that, when referencing my role in the Boston Hemophilia Center 340B Factor Program, most people would say something along these lines, which is all well and good. But what exactly is it that I do?

On the front line of the BHC are, of course, the doctors, nurses, social workers and physical therapists. However, behind the scenes, there is a whole other group of people working to make sure that the Center runs smoothly so that patients receive the high-quality care that they deserve. This is where I fit in.

I monitor the day-to-day financial activities of the Factor Program. As we all know, factor costs money...and lots of it. When a

patient needs factor, our pharmacists at Eaton Apothecary place an order with one of our manufacturers, and subsequently, the factor is delivered to the patient. The patient's insurance company then reimburses us, based on a pre-determined rate, for this factor.

I run certain reports out of various internal systems to track this flow of money. Furthermore, since this is a 340B program, the revenue we earn from the sale of the factor enables the Hemophilia Program to operate as needed. Staff salaries, educational and patient/family events, and various operating expenses (e.g. office supplies, refrigerators to store factor, computer equipment, etc.) are all paid for with this revenue. I track all this activity using internal reporting tools.



>>> continued on page 3

# That's a Wrap!

## NEHA Golf Tournament

BHC sponsored a team of staff members in the NEHA golf tournament held on Monday, Sept 11, 2017, at Cyprian Keyes Golf Course in Boylston, MA. Our team included: Amanda Stahl, MSW, LICSW, Adult Hemophilia Social Worker, Aric Parnes, MD, Adult Hematologist, Deb McNamara, Division Administrator - Hematology, and Loren D'Angelo, PNP, pediatric nurse practitioner. The day was full of fun contests and golf activities, while raising funds for NEHA, our regional consumer chapter.

**Shown from left to right: Amanda Stahl, MSW, LICSW, Aric Parnes, MD, Deb McNamara, and Loren D'Angelo, PNP**

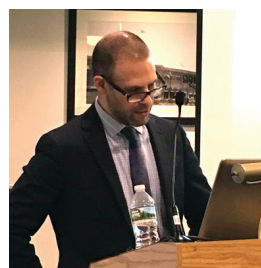
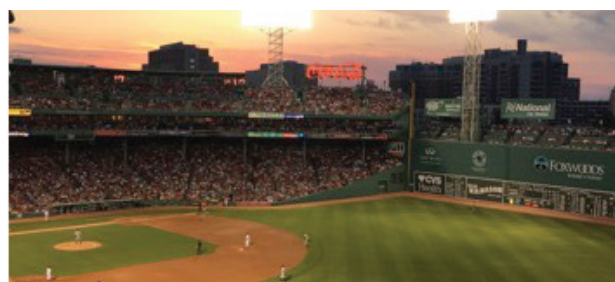


## New England Hemophilia Association (NEHA) Walk

Saturday, June 10th, turned out to be a beautiful summer day at Prowse Farm in Canton, MA. Hundreds of hemophilia patients, friends and family members converged on the farm to take part in NEHA's Walk to benefit the New England consumer chapter. Cliff Haas, the Operations Manager for BHC's 340B factor program, and Stacy Croteau, MD, pediatric hematologist, led the BHC's team, "Boston Strong", at this incredible community event.

## BHC Night at Fenway Park

It turned out to be a "Double Header" for Boston HTC patients and staff when they were able to enjoy Saturday, June 10, 2017, at the NEHA Walk during the day and then attended a Red Sox game that same evening! During this annual BHC event, the Boston Red Sox defeated the Detroit Tigers, 11 to 3. One hundred people attended this family sports event and greatly enjoyed the exciting win!



## Hepatitis Symposium

BHC sponsored an educational symposium at the Inn at Longwood Medical on Thursday, April 13, 2017. Coordinated by Aric Parnes, MD, Adult Hematologist at BHC, hemophilia patients, families and staff members attended the talk entitled, "An Update on Hepatitis and Liver Disease in Bleeding Disorders."

The Keynote Speakers were Paul E. Sax, MD, Professor of Medicine, Harvard Medical School, Clinical Director of the Division of Infectious Diseases and the HIV Program at Brigham and Women's Hospital, and Anna Rutherford, MD, MPH, Assistant Professor of Medicine, Harvard Medical School, Clinical Director of Hepatology at Brigham and Women's Hospital in Boston. Attendees enjoyed the informative talks which focused on the recent advances in hepatitis treatment and liver care for persons with bleeding disorders.

If you have ideas or requests for a medical educational program at BHC, please contact Peg Geary, Program Manager, at [mkgear@bwh.harvard.edu](mailto:mkgear@bwh.harvard.edu) or phone 617-732-8537.

## Who's Who <<< continued from page 2

Each year, our hospital goes through an annual budget process. The numerous departments, divisions and programs that make up Brigham & Women's Hospital are instructed to generate a detailed projection of their revenues and expenditures. If done correctly, each of these budgets should provide BWH with an accurate snapshot of its financial health for the upcoming fiscal year. I am heavily involved in completing the necessary templates during this budget process for our Program.

Of course, my days are constantly being filled with other ad hoc assignments and requests, ranging from ensuring that invoices are getting paid in a timely manner and reviewing contractual

reimbursement rates with our insurance companies, to playing housekeeper and being tasked with the honor of cleaning out the refrigerator in our kitchenette on a regular basis.

The Center is comprised of so many different and wonderful people, each working hard within his or her respective area of expertise — from the Nurse Practitioner, who is coming up with a treatment plan for a patient's recent bleed, to the Administrative Assistant, who is helping to schedule a patient's next visit.

I'm happy to be part of such a great team and play my part as the guy who deals with numbers and money. ♦

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## New Staff at BHC



**Nina Balan** recently joined the BHC team as a Clinical Research Coordinator. She is a recent graduate of Boston University, where she earned her B.S. in Health Science with a dual-minor in Business Administration & Management and Public Health. Outside of her studies, she served as the Hospital Relations Chairwoman for the BU Dance Marathon benefiting Boston Children's Hospital and as a volunteer on the hematology unit at Boston Children's Hospital. Nina has a great interest in population health management and development of low-resource healthcare settings; she has previously worked on projects with the UK National Health Service and the Global Health Initiative team at the Dana-Farber/Boston Children's Cancer and Blood Disorders Center. She was born in Russia, grew up in Miami, and now calls Boston home. In her spare time, she enjoys cycling, cooking, exploring New England, and travelling (her favorite destination being her family home in Tbilisi, Republic of Georgia). Nina can be reached at (617) 525-0033.



**Hoang Tran** joined the Boston Hemophilia Center Factor Program as a staff pharmacist in January, 2017. Hoang graduated from UMass Boston with a BS in Biochemistry in 2010 and then the Massachusetts College of Pharmacy and Health Sciences (MCPHS) University with his PharmD. Since 2009, Hoang has worked as a Pharmacy Technician and then a pharmacist with Eaton Apothecary, which is the 340B pharmacy for the Boston HTC. As such, Eaton's provides factor products to HTC patients, while funding the staff and services of the HTC. Hoang enjoys spending time with his family, on shopping adventures and traveling.

**Christine Ploski**, PT, MS, PCS, MAc, LicAc, joined the Hemophilia Team in January, 2017, as our Pediatric Physical Therapist. She received a BS in Physical Therapy from the University of Connecticut in 1976, and an MS in Physical Therapy from the Massachusetts General Hospital Institute of Health Professions in 1989. She is currently the Manager of Education in the Department of Physical Therapy and Occupational Therapy Services at Children's Hospital, Boston, where she has worked since 1980. Christine received a Master of Acupuncture from the New England School of Acupuncture in 2004 and she has been an adjunct faculty member at the Boston Conservatory of Music Dance Division, Bay State College, as well as a guest lecturer at several area Physical Therapy Programs. Christine has co-authored articles including "Transition Dance Class: Rehabilitation through Dance" and "Myelodysplasia: Habilitation from Infancy to Adulthood." She is currently a physical therapy evaluator for the Progeria Drug Trial at Children's Hospital, Boston. Her personal interests include dance and hiking.