



Centerpoints

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Director's Corner

Rebalancing Blood Coagulation

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Medical Director

More prophylaxis options have been recently approved by the FDA for individuals (≥ 12 -years-old)

with more severe forms of hemophilia A or B including those with inhibitors. These two new medicines are the first licensed in a treatment class called **Rebalancing Therapies**. Normal blood clotting is a careful balance between proteins that promote clotting (coagulation factors) and proteins that prevent too much clotting (anti-coagulation factors). Previously, bleed prevention treatments (factor concentrates and emicizumab), improved blood clotting by increasing the effect of pro-coagulant proteins. In contrast, rebalancing therapies work to improve blood clotting by decreasing an anticoagulant protein.

Marstacimab (Hympavzi, Pfizer) and **concizumab** (Alhemo, Novo Nordisk) are both antibodies that interfere with an anti-coagulation protein called Tissue Factor Pathway Inhibitor (TFPI), thus these new medicines are called **TFPI inhibitors**. Both medications are injected subcutaneously (under the skin) rather than in a vein and are administered with pre-filled medication pens. Although these medicines are very similar to each other, the initial FDA approval for marstacimab is for HemaA/B (≥ 12 yo) without inhibitors; the initial FDA approval for concizumab is for HemaA/B (≥ 12 yo) with inhibitors.

During the clinical trials, participants on weekly marstacimab prophylaxis had an

(continued on page 2)



Empowering Women in the Bleeding Disorders Community Advocating for Your Health

By: Amanda Stahl, LICSW & Jackie Miranda LICSW

Women in the bleeding disorders community face many unique challenges, including delayed diagnoses and lack of access to specialized care. With the right tools and strategies, you can take charge of your health and advocate effectively for your health care needs. Whether you are managing a bleeding disorder yourself or advocating for a loved one, here are some steps to ensure your voice is heard:

Navigating the Healthcare System

Navigating the healthcare system can feel overwhelming, but preparation and self-advocacy can make a difference. At your appointment with your healthcare team:

- Keep detailed records of your symptoms and treatments, and discuss them with your care team
- Write down and bring any medical questions with you to your appointments
- Recruit a trusted friend or family member to accompany you for additional support and to act as a sounding board
- Utilize your hospital's patient portals as a way to easily access providers and your medical records
- Ask your medical provider to explain medical terms or treatment options until you fully understand

Remember, you are an expert in your own body and experiences—advocating for what you need is a crucial part of your care.

Build a Network

Finding a support network can help you navigate your care. Luckily, there are many ways to join the bleeding disorders community and no shortage of ways to connect. In New England, the New England Hemophilia Association offers a retreat for women in the spring – information can be found on their website. Spaces like this allow opportunities to connect with others who share similar experiences, exchange advice, and find encouragement.

(continued on page 2)

Resources for Advocacy

Advocating for change in the systems that impact bleeding disorder care can help you and others more smoothly navigate the healthcare system. Educational workshops and advocacy days offered by organizations like The National Bleeding Disorders Foundation (NBDF) can equip you with tools to influence policy and raise awareness. These programs often aim to address crucial and timely topics like insurance barriers and the importance of access to specialized care, like at hemophilia treatment centers.

Taking charge of your health and becoming an advocate may feel overwhelming at times, but there are resources and communities to support you. By staying informed, building connections, and confidently navigating your healthcare, you can make a meaningful impact for yourself and for the bleeding disorders community.

Women with bleeding disorders often have one or more signs or symptoms.

Heavy bleeding during menstruation (period) that can include:

- Bleeding that lasts longer than 7 days from the time bleeding starts until the time it ends
- Flooding or gushing of blood that limits daily activities, such as work, school, exercise, or social activities
- Passing clots that are bigger than the tip of your thumb
- Soaking a tampon or pad every hour or more often on the heaviest day(s)
- A diagnosis of "low in iron" or having received treatment for anemia
- Symptoms of easy or frequent bleeding that can include
 - Nosebleeds that occur for no apparent reason and last longer than 10 minutes or that need medical attention;
 - Easy bruising that occurs with no physical injury;
 - Excessive bleeding after a medical procedure or dental extraction; and
 - A history of muscle or joint bleeding with no physical injury.

When to talk to your primary care:

If you have one or more of these signs and symptoms, talk with your doctor or other healthcare professional. You can also visit the National Bleeding Disorders Foundation's [Better You Know website](#) to take a bleeding disorder risk assessment to determine if you might be at risk for a bleeding disorder. **Bleeding disorders can be treated, but first you need to know if you have one.**



Did you know?



For our pediatric aged patients, the [Young Women with Bleeding Clinic](#) offers, an interdisciplinary clinic including the specialties of Hematology, Adolescent Medicine, and Gynecology.



Stacy E. Croteau, MD, MMS
Pediatric Hematology



Pamela J. Murray, MD, MHP
Adolescent Medicine



Jessica Shim, MD
Pediatric Gynecology

For our adult patients, [The Fish Center for Women's Health](#) offers primary care and specialties, including: Hematology and many more.



Arielle L. Langer, MD, MPH
Hematology

Reach out to your primary bleeding disorder provider to learn more!

Camp Corner:

Camp enrollment is officially open!! Please consider attending a camp session for a spring and summer filled with fun, friends and unforgettable memories. Let the countdown to adventure begin...

[New England Hemophilia Family Camp](#), June 25-June 28, 2025. Lake Winnepesaukee, in Moultonborough, NH. The website below has more information and an online application.

Applications close March 24, 2025.

[2025 Spring Family Weekends](#) start Friday, March 28 and are offered through Sunday, May 11!

[2025 Summer Programs](#) start Friday, June 20 and are offered through Wednesday August 20 and include focuses for siblings!



a seriousfun camp

(continued from Director's Corner)

average reduction in treated bleeds from 8/year on their routine factor concentrate prophylaxis to 5/year. For participants with inhibitors, use of daily concizumab prophylaxis reduced the average yearly bleed rate to 1.7/year compared to 10.6/year for those using an on-demand treatment strategy. Neither of these TFPI inhibitors have been tested in comparison to other newer therapies such as emicizumab (Hemlibra) or Altuviiro factor VIII concentrate.

While from a safety perspective these TFPI inhibitors seem to be generally well tolerated, there are risks. Due to the way these medications affect blood clotting, there is a risk of developing abnormal blood clots (thrombosis). This occurred in a few patients during the concizumab clinical trials. The TFPI inhibitors only serve as bleed prevention, so access to a clotting factor bleed treatment will still be important. The appropriate dosing and frequency of bleed treatments for those on a TFPI inhibitor will be different than our standard treatment approach to avoid blood clot risks. Close communication with your bleeding disorder team will continue to be essential. More information is being gathered about the use of TFPI inhibitors in children with hemophilia A or B.

Interested in learning more? Your bleeding disorder team is happy to discuss these new treatment options with you in more detail and explore whether or not they are a good fit for your hemophilia prophylaxis.

The 76th Annual Bleeding Disorders Conference Atlanta, GA - September 12-14, 2024

Our clinical team and center staff attended BDC for three incredible days of education and training, valuable networking opportunities, and exciting exhibits at the Georgia World Congress Center in downtown Atlanta.

With center support, one of our patients was able to attend and share their experiences with others, participate in lectures and further connect with the bleeding disorder community! **Speaking to their experience:** *"I'm appreciative of the connections I made and I now have plans to do more activities with the New England Hemophilia Association because of some great people that I met. Most of all I'm appreciative of my HTC for giving me the opportunity to go to the 2024 Bleeding Disorders Conference."*



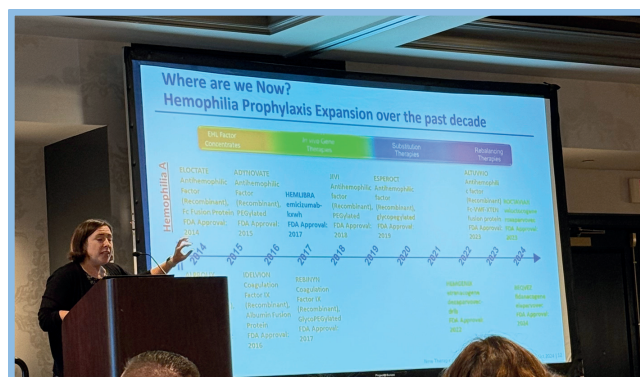
Fall Patient and Family Event: TopGolf Canton, MA - October 2024



With a focus on physical therapy and healthy movement, BBDC team members joined patients and their families to learn the finer points of proper movement and body mechanics from a professional golf coach. Our hope is to encourage everyone to move and exercise to the best of their ability. And these events are also an opportunity to mingle, network and connect with other members of our community. Keep an eye out for future events, as these are great opportunities to talk with staff about bleeding disorder awareness, and to have positive experiences with people just like you!

Please be on the lookout for our next patient event coming in the spring!

Hemophilia Alliance: Fall Members Meeting New Orleans, Louisiana - October 2024



The Boston Bleeding Disorders Center team participated in the 2024 Hemophilia Alliance - Fall Member's Meeting in New Orleans, Louisiana where they were celebrating its 25th anniversary! These meetings focus on the relationships between HTC's and other local organizations; give an opportunity to provide updates and education about important advancements in patient care; and discuss the advocacy work done in Washington D.C. on behalf of the bleeding disorders community.

Upcoming Events:

Bleeding Disorders Awareness Month! | March 2025

We're excited to launch a mini-series where we'll share the powerful, personal stories of women and girls with bleeding disorders. In this series, you'll hear firsthand about their journey, challenges, and triumphs as they navigate life with conditions that often go unrecognized. Through their experiences, we hope to raise awareness, foster understanding, and inspire others in the community.

Hemophilia Federation of America (HFA) 2025 Symposium | March 27-29, 2025 - San Diego, CA

Explore the latest products and services available to make life with a bleeding disorder more manageable. It is a great place to hear from individuals who have overcome obstacles and show strength in the face of adversity. If you are interested, please check out their website: <https://www.hfasymposium.org/>

World Hemophilia Day | April 17, 2025

Today, women and girls with bleeding disorders (WGBDs) are still underdiagnosed and underserved. Through recognition, diagnosis, treatment, and care, the quality of life of women and girls will improve, and the bleeding disorders community will become stronger. Learn more: <https://wfh.org/world-hemophilia-day/>

Bleeding Disorders Conference | August 21-23, 2025 - Aurora, Colorado

The National Bleeding Disorders Foundation hosts the 77th Bleeding Disorders Conference! Join other patients, families, and healthcare professionals to learn from each other and network. Registration is NOW OPEN and will be available through to the start of the conference.

Early registration ends on Friday March 14, 2025. For more information, please check out their website: <https://events.bleeding.org/website/80813/home/>

Join Our Patient Advisory Group – WE NEED YOU!

The Boston Bleeding Disorders Center is looking for 8-10 patients to join our Patient Advisory Group meeting to be held on Zoom in the Spring. This group is a space for patients to share their experiences, provide feedback, and help guide quality improvements in patient care. Our next meeting will focus on patient engagement—including how we communicate with patients, the events we hold, and ways to enhance involvement in care. Your input will help shape how we connect with and support our community.

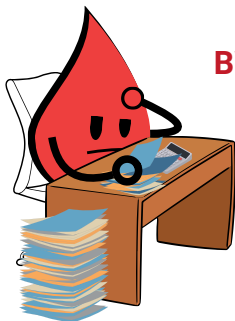
If you'd like to participate, please RSVP by April 18th by emailing bostonhtc@bwh.harvard.edu. We look forward to hearing from you!



Institutional/HTC Resources:

BCH Corner (Pediatric Patients)

- [Young Women with Bleeding Clinic](#)
- [Patient Financial Services](#)



BWH Corner (Adult Patients)

- [Patient Financial Services](#)
- [Fish Center for Women's Health](#)

Feedback Helps!

We want to hear from you! Please send us your ideas for educational presentations or events.

We enjoy hearing about and sharing your meaningful life experiences and adventures especially those within the bleeding disorders community or helping to increase awareness of the bleeding disorders community. Please share your stories or photos with us.

Your comments and feedback help us to improve our bleeding disorders clinical and educational programs to ensure we are meeting your needs.

How can you share your ideas or suggestions? Email our team at bostonhtc@bwh.harvard.edu or use this QR code to complete an anonymous comment card.



Stay in the know! Check out our BBDC website or follow us on Facebook.
Web: www.childrenshospital.org/BBDC | Facebook: <https://www.facebook.com/bostonhemophilia/>