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Centerpoints

Issue 26/Fall 2025

Director's Corner



Shifting Expectations in Bleeding Disorders Care

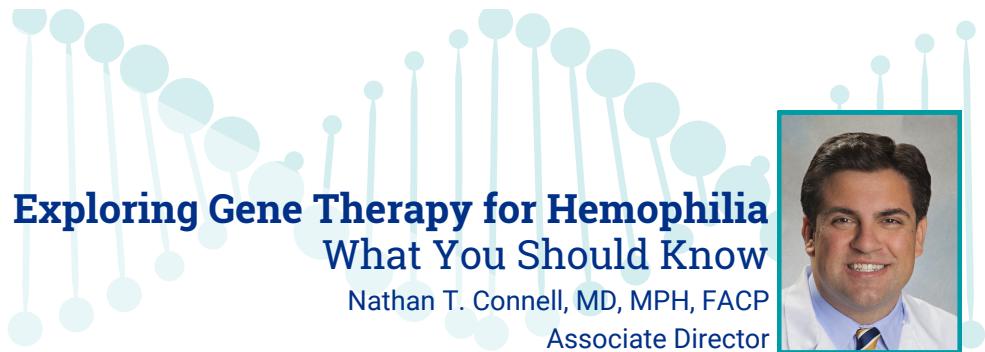
Stacy E. Croteau, MD, MMS
Medical Director

As we prepared this issue of Centerpoints, I took some time to look back over the 25 prior installments and reflected on all the changes that have taken place since our first issue in 2009. Bright and youthful faces of our youngest patients filled many pages as they participated in our education and family events and summer camps. Many of these faces have a much more mature look now and have been transitioning to the adult side of the BBDC ready to take on their independence and new adventures.

Fostering readiness for this transition from our pediatric program to the adult program has continued to be a focus of our center's quality improvement efforts. We strive to bolster patient and caregiver preparedness and make our own processes more seamless. Ongoing feedback from all of you (those preparing for transition as well as those who have transferred care) regarding our processes, age/skill specific education, and transition readiness assessments is critical to help both the pediatric and the adult care teams continue to refine and improve the experience of this life milestone.

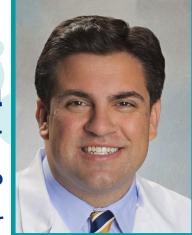
As I looked at the photos of our young patients and their families, I was also struck by how dramatically my initial "what to expect" conversations with parents have changed over the years, particularly for those with severe bleeding disorders.

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Exploring Gene Therapy for Hemophilia: What You Should Know

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Treatment options for hemophilia have expanded significantly over the past few decades. Today, people with hemophilia A and B have access to a range of therapies that help prevent bleeding and improve quality of life. One of the most exciting developments is gene therapy—a promising approach that may reduce the need for regular factor infusions.

Gene therapy works by delivering a working copy of the gene responsible for producing clotting factor directly to the liver. This allows the body to begin making its own factor, potentially reducing bleeding episodes and the need for ongoing treatment.

The first gene therapy approved for hemophilia was for people with hemophilia B. The U.S. Food and Drug Administration (FDA) approved it for individuals with very low levels of factor IX—typically those with severe or moderate hemophilia B—who are on regular prophylaxis or have experienced life-threatening bleeds. More recently, gene therapy has also been approved for hemophilia A.

If you're considering gene therapy, the process begins with a conversation with your hemophilia treatment team. Together, you'll review your bleeding history, factor usage, and personal goals. Your team will also discuss the benefits, risks, and possible side effects of gene therapy, including the use of medications like corticosteroids, which may be needed after treatment to support liver health.

Before receiving gene therapy, several tests are performed. Bloodwork is used to check liver function and to ensure there are no inhibitors (antibodies) that could interfere with treatment. Additional testing may assess whether your immune system will accept the delivery method—called a vector—that carries the gene to your liver. In some cases, imaging such as an ultrasound or a consultation with a liver specialist may be needed to confirm that your liver is healthy enough for therapy.

Insurance approval can take time, and your care team will help guide you through the process to ensure everything is in place before your scheduled infusion. On the day of treatment, a nurse will place an IV, and the gene therapy will be infused over one to two hours. While allergic reactions are possible, they are rare, and most people tolerate the infusion well. You'll be monitored for a few hours afterward before going home.

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Back 2 School Corner

Heading back to school can be exciting (and a little overwhelming). Here's a quick guide to help ensure a smooth start for children and teens living with a bleeding disorder:

Create a School Health Plan

If your child with a bleeding disorder is entering school for the first time, or changing schools, your bleeding disorders nurse can provide written information and/or set up a zoom meeting with the school nurse to provide additional education if needed. Just let your team know!

Pack Smart

Your child's backpack should include:

- Emergency contact card and any documentation requested by their school
- Any necessary medication (if approved by the school)
- A small first-aid kit

Check with your school whether you can send in school medications and related medication orders via backpack or if these need to be dropped off in person by a parent/guardian.

(Continued from, Exploring Gene Therapy for Hemophilia...)

Following the infusion, you'll continue your regular factor prophylaxis while your body begins producing its own clotting factor. Weekly lab tests will monitor your liver and factor levels. Once your team determines your level is producing enough factor on its own, you may be able to stop regular infusions. This transition typically takes several weeks.

Some people may experience changes in liver enzyme levels during this time. If that happens, your team may prescribe corticosteroids—like prednisone—to reduce inflammation. Everyone responds differently to gene therapy, and each product has its own data on expected outcomes. Your team will help you understand what to expect based on your individual situation.

For many, the goal of gene therapy is to move factor levels from the moderate or severe range, into the mild range—reducing spontaneous bleeding and the need for regular infusions. However, factor may still be needed for injuries, procedures, or surgeries.

Throughout the process, your hemophilia team will support you and your family, answering questions and helping you make informed decisions. Resources from the World Federation of Hemophilia and the National Bleeding Disorders Foundation are available online to support shared decision-making.

Gene therapy is a rapidly evolving field, and ongoing research may lead to new delivery methods or improved outcomes. Some people may choose to wait for additional data or future therapies. Others may explore clinical trials if current FDA-approved options aren't the right fit.

There's no one-size-fits-all answer. The decision to pursue gene therapy is personal and should be made in partnership with your hemophilia treatment team.



Gene Therapy Resources

[National Bleeding Disorders Foundation](#)

[World Federation of Hemophilia](#)



(continued from Director's Corner)

With very limited therapies, even just 10 years ago, it was commonplace for our youngest patients to not initiate prophylaxis until 1 year of age or older. The conversation around signs and symptoms of bleeding, likelihood of bleeding for infants and young children has changed significantly as new therapies have been licensed, permitting earlier initiation of prophylaxis and better bleed protection. The experience of our newest parents is proving to be very different from those raising young children with severe hemophilia even 6-7 years ago. We will likely soon need to assemble The Gift of Experience 3!

The past decade has truly seen amazing innovation in bleeding disorder therapies. Many of our past issues highlighted the new categories of medications, primarily for individuals on prophylaxis, extended half-life (EHL) factor concentrates, on factor VIIIa mimetics (emicizumab), on gene therapy, and most recently on rebalancing therapies. In this issue we continue to expand on experiences with these newer therapies selecting bleed prevention/prophylaxis approaches to meet the needs of an individual rather than prescribing a "one-size fits all" plan for everyone.

Despite all of this progress, awareness and education about bleeding disorders and the importance of access to care and to these life-saving therapies remains essential. Opportunities to come together and learn from one another such as at summer camps continue to be a valuable resource for personal growth, exploration, and fun. We are excited to share more of these photos and stories with you all as well.

Fall Wellness Tips

It's important to stay on-top of how the seasons affect us!

To keep yourself and your family healthy this fall, here are some quick tips:

Get Vaccinated

Protect yourself and your loved ones with a flu shot and consider asking your provider about updated COVID boosters.

Routine Matters

As school and work schedules fill up, don't forget to maintain your regular medical check-ins and medication routines.

Stay Safe

Take time now to prepare for icy sidewalks by stocking up on salt or sand, checking outdoor lighting, and making sure walkways are clear before the winter weather hits.

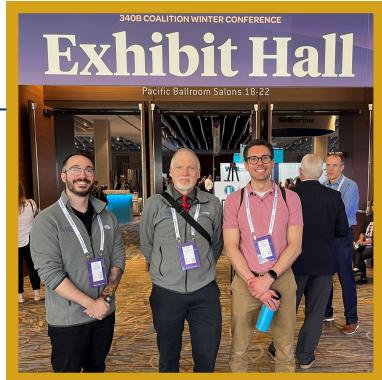
Planning ahead for shorter daylight hours and slippery conditions can help you stay healthy, safe, and stress-free all season long!

Community Events Recap

340B Coalition Winter Conference

San Diego, CA - February 24-26, 2025

BBDC staff lead and engaged in discussions on the latest challenges facing hemophilia treatment centers' Factor Program. HTC Factor Programs are a critical part of providing quality clinical care and education resources for patient care and support. We pride ourselves to help our bleeding disorders community.



Hemophilia Alliance Spring Conference

Salt Lake City, UT - March 10-11, 2025

The BBDC team joined other bleeding disorder centers for the Hemophilia Alliance's annual spring meeting. At this conference, we were able to get valuable updates on changes in the federal government that may impact our community, discuss ongoing efforts to improve upon access to- and quality of care for individuals with bleeding disorders and collaborate with other HTCs to the benefit of our mission.

NEHA Medical Symposium

Groton, MA - March 15-16, 2025

The BBDC Factor Program and Pharmacy team attended the New England Hemophilia Association (NEHA) Medical Symposium. The Medical Symposium was created to demonstrate the current and developing medical advancements to treat people with bleeding disorders. Our team had it's own table to share more information about our HTC, attend information sessions, and most importantly interact directly with our community. This annual symposium is open to the public! Keep an eye on NEHA's website for information on how to register for next year's!



NBDF Bleeding Disorders Conference

Aurora, CO - August 21-23, 2025

The BBDC team attended the National Bleeding Disorders Foundation's 77th annual Bleeding Disorders Conference in Aurora, Colorado. The conference offers a packed agenda that includes educational lectures, networking and advocacy opportunities for individuals with bleeding disorders, their families and medical providers. We encourage everyone to keep an eye out for next year's conference in Orlando, Florida from August 13-15th, as it is a great event for the bleeding disorders community!



NEHA 35th Annual Golf Tournament

Boylston, MA - August 4, 2025

NEHA held their annual golf tournament and raffle at the Cyprian Keyes Golf Club in early August. Our BBDC team members Amanda Stahl, Chris Baer, Kristen Benya and Alex Kireilis were part of 144 golfers who participated in the event that included community members from all over New England which helped to raise more than \$75k! This event helps to bring awareness to our community and provide an opportunity for some fun in the sun!



Camp Corner

Camp season is here!

Every fall, The Hole in the Wall Gang offers weekend programs and family weekends. There, families will be able to join in fun activities and enjoy the company of others who understand and share the circumstances they face. Camp weekends run from October through November, so there are plenty of opportunities to jump in! Talk with your care team about attending and they will be happy to help get everything set up!



"We have been going to family camp weekends and two of our sons go for a week in the summer for the last five years, Hole in the Wall camp has been an amazing experience for all of us.

It has given us a safe and welcoming place for our entire family. The staff, councilors and volunteers are great. Camp has given us a way to meet others and make life bonds in the bleeding community.

It's a safe place that we all feel comfortable going to and being ourselves."



This year's summer camp attendees were made up of children with bleeding disorders from the ages of 7 to 15. There were sessions for siblings without bleeding disorders too! Breakfast would start the day and help fuel everyone for adventuring including wall climbing and rope courses, archery, fishing, boating and swimming. There was arts and crafts, music and theater and even woodshop for kids to flex their creative muscles! Campers come from all over New England and everyone who attends has a medical diagnosis, with staff on hand at all times to make sure everyone stays safe.

Counselors stay with campers in their cabins and are often people who are veteran campers from previous years. We are so fortunate to have had so many happy and active patients from our HTC join the Hole in the Wall Gang, and we love hearing about their time there! See above and below for just a few quotes from happy campers.



Want to join? You're in luck!

Again, if you're interested in attending a camp, The Hole in the Wall Gang Camp hosts Family Weekends throughout the fall!

To learn more, check out their website: [The Hole in the Wall Gang Family Weekends](http://www.holeinthewallgang.org)

If you would like to participate in a summer camp next year, please be on the lookout for applications released in early 2026 by The Hole in the Wall Gang and NEHA.



Institutional & HTC Resources

BCH Corner (Pediatric Patients)

- [Boston Bleeding Disorders Center](#)
- [Patient Financial Services](#)

BWH Corner (Adult Patients)

- [Boston Bleeding Disorders Center](#)
- [Patient Financial Services](#)

We want to hear from you!

We are always trying our best to improve our patient care and wider community outreach and education programs. We aim to provide the best care possible to our patients, and hearing from you will help us do just that!

So if you have any suggestions or comments on how you think we might do that, please don't keep it to yourself. Let us know!

And how might you do that? 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/>