THE COALITION FOR HEMOPHILIA B

SUMMER 2026

HEMOPHILIA B NEWS

NATIONAL NONPROFIT ORGANIZATION

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B THE VOICE
2026 NATIONAL ADVOCACY SUMMIT





HISTORIC GENE THERAPY RETREAT

2026 BEATS MUSIC PROGRAM

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THE COALITION FOR HEMOPHILIA

MISSION

To make quality of life the focal point of treatment for people with hemophilia B and their families through education, empowerment, advocacy, and outreach.

the Beats Strikes Chords of Music, Community, and Courage

By Renae Baker

The 8th Annual *The BEATS Music Program* took place July 15-18 at the Sheraton Music City Hotel in Nashville, and if participant feedback is any indication, it was an unforgettable success! Attendees described the experience as "beautiful," "thought-provoking," "uplifting," and "entertaining." More than a few participants declared it the best The BEATS program yet.

Those who arrived hoping to learn new musical skills or sharpen existing ones quickly found themselves having so much fun that they forgot to be nervous. Throughout the four days, music was everywhere. Between workshops, presentations, and performances, Renae Baker helped keep participants energized with vocal health exercises and a spirited new "B Chant" that quickly took hold.

The event began with music producer Adam Smith introducing this year's group recording project, *You Can't Always Get What You Want* by The Rolling Stones. Drawing on his extensive studio knowledge, Adam gave participants an inside look at the recording studio process and the elements involved in creating a professional track.

The educational sessions got underway with *Understanding Hemostasis: The Clotting Cascade*, presented by Cassandra Titus and Shelby Smoak of Sanofi. After a wildly successful icebreaker that had everyone participating, the presenters walked attendees through the science behind clotting, including factor VIII, factor IX, and von Willebrand factor.

Maintaining the momentum, Shelby then welcomed Rick Starks to the stage for the always-popular *Name That Tune* trivia game. The session encouraged participants to reflect on songs connected to meaningful memories from their lives. The room buzzed with energy as stories, laughter, and nostalgia filled the conversation, making it one of the week's most interactive sessions.

Even after a full day of activities, many participants stayed up for the first nightly *Jam Session and Group Hangout*, eager to keep the music and camaraderie going.

Day two started with one of The Beats most beloved traditions: Elec Simon's inspiring drum circle. With full participation and infectious enthusiasm, Simon had everyone making music together while building confidence and connection.

Next, attendees gathered for *The Beat of Life: Music and Hemophilia B*. Moderated by CSL's Myriam Lagomasino, the discussion featured Rick Starks and Domenic Catrine, who shared personal stories about living and









thriving with hemophilia B. As two mothers shared the challenges they've faced, Rick's heartfelt, empathetic response moved the room.

Lunch featured special guest Joe Turley, who captivated participants with stories from his remarkable career as an entertainer, including performing alongside legendary artists Dolly Parton, Waylon Jennings, Dr. Hook, and The Shirelles.

The afternoon kicked off with the first music workshops. Participants worked in their chosen classes: brass and woodwinds with Tessa Berger; drums with Rich Adams; guitar with Tiger Fitzhugh; piano with Sam Blizzard; strings with Hope Hamby; and vocals with Renae Baker, accompanied on piano by Greg Bieck. Each workshop provided a safe and encouraging environment where students stepped outside their comfort zones and developed new skills.

Daily Teen Talk sessions provided teens with opportunities to connect and support one another. It was heartwarming to watch friendships deepen as the week progressed.

After dinner, the events focused purely on fun. A lively line-dancing lesson filled the room with smiles, and then a Nashville-style *Writers' Round* featured five local singer-songwriters. In an intimate acoustic setting, each performer shared their original songs ranging from deeply emotional to side-splittingly funny. Participants were impressed and grateful for the authentic Nashville experience.

On day three, Max Feinstein moderated a discussion featuring Adam Smith and special guests Bryan Morelli and Kevin Finckle, who shared their experiences with musical training, jamming, and professional performance. Then, Shelby, Adam, and Max led *This Is Your Brain on Music*, a session exploring the cathartic power of music where participants worked in small groups on a songwriting exercise, creating hemophilia B-themed lyrics set to familiar melodies. When the groups performed their creations, the room alternated between awe and laughter.

On the final morning, Trish Amerson from CSL presented *A Blueprint for Confidence: Making Informed Choices in Hemophilia B With Idelvion*. She discussed the importance of maintaining factor levels and how even a single bleed can contribute to long-term complications. Participants appreciated the practical information and preparedness strategies she shared.

Following one final workshop session, it was time for the grand finale concert. The meeting room transformed into a theater, and students took the stage to share the music they'd been developing throughout the week.
















































* The Beats photos courtesy of Will Baldwin

A special kind of magic happens when the lights dim and a supportive audience waits expectantly. Shy students suddenly become performers. Musical expression becomes more intentional. Fear gives way to courage.

As each performance ended, the applause affirmed that something important had happened. Participants had challenged themselves, taken creative risks, and discovered strengths they hadn't realized they possessed. They left the stage triumphant, while Coalition families were inspired by the courage they had witnessed.

The Beats 2026 wouldn't have been possible without the generous support of its sponsors. We're grateful to CSL at the Director Level, Sanofi at the Conductor Level, CVS Specialty and Medexus Pharma at the Performer Level, and Novo Nordisk, whose grant support helped make the program possible. Thanks also to Music Director Bill Gati and the committee: Wayne Cook, Kim Phelan, Erica Garber, Shelby Smoak, Steve Long, Max Feinstein, Rick Starks, Adam Smith, and Renae Baker, and all of our volunteers.

The Beats 2026 was more than a music program. It celebrated creativity, connection, and personal growth!

Transformative Power of The Beats

By Max Feinstein

As someone living with hemophilia, I have had the privilege of teaching at the Coalition's *The Beats Music Program* for the past five years. The Beats lets me share skills I have spent a lifetime developing and use them to inspire and empower others.

I believe in The Beats more than literally any other program in the community. There is no 'almost.' This is the most unique program in the community. There is literally nothing else like it.

The Beats fills an important need by creating a bridge between the bleeding disorders community and the world beyond it. It opens the door to experiences beyond the traditional conference setting, giving participants opportunities to explore new interests, develop skills, express themselves, and discover possibilities they may never have considered.



What makes The Beats especially meaningful is that hemophilia does not have to be the center of the experience. We already know we live with a bleeding disorder. The Beats gives us the opportunity to be defined by our interests, our talents, our creativity, and the experiences we share together.

It is more than having fun. It is about building confidence, discovering possibilities, forming meaningful connections, and experiencing life beyond a diagnosis. This is why I believe so strongly in The Beats and why I am proud to have been part of this program for the past five years.

Soothing the Soul

By Pam Williams

***The Beats Music Program* returned to Nashville July 15 to 18. Now in its eighth year, The Beats brings together patients with hemophilia B, ages 13 and over, for four days of music instruction, studio recording, and live performance. No experience is required, and instructors meet every participant at their own skill level. This was my second year attending – I came home even more hooked than the first time.**







Last year, I often felt like a deer in the headlights. I hadn't played piano in 15 years, but I persevered and was thrilled to be selected to attend again.
I started learning piano at age five (many years ago!). Although I got by, I was never the best student. I managed. I could read and play music, but I wasn't exceptional. During high school, I accompanied the junior high choir, and in college, the men's choir. Playing piano was always my emotional outlet.

When I was a child, my grandparents had a piano in their front room, and my grandfather insisted that if you didn't know how to play, it was off limits. I would often go in, close the door, and play to my heart's content until my grandfather opened the door, insisting that music was meant to be heard, not kept secret. I loved that.

At The Beats, it was so much fun catching up with people I'd met last year and meeting new participants. Being from Ohio, I noticed a teenager wearing a Cincinnati Reds shirt, so I introduced myself to her and her mom. They were from Ohio as well. They had previously attended FamOhio, an annual conference in Columbus, and her mom recognized me from my time on the FamOhio board. Our conversation flowed easily.

For my piano performance this year, I chose Don McLean's *Vincent (Starry, Starry Night)*. I hadn't played it since my high school choir days and had only practiced once before arriving in Nashville.

Our piano group consisted of just two performers: Robert, a talented teenage pianist I'd met the year before, and myself. Our instructor, Sam Blizzard, whom we had also met last year, was wonderful. He consistently offered insightful advice to help songs flow better and was patient beyond words.

One of the most exciting and unforgettable sessions was the *Writers' Round* performance. Five songwriters gathered on stage to share their original songs and the stories behind them. Their inspirations and sense of humor kept the audience laughing and clapping for more.

We enjoyed plenty of delicious food, and on Thursday evening, we took part in an invigorating Nashville line dancing class. We learned a couple of dances, laughed, and moved to the beat, hopefully ending up facing the same direction!

Last year, the recording studio felt overwhelming. This year, I embraced the experience and put my heart into the piano part I was assigned. Robert and I were the last two attendees to record our sessions, and Adam Smith made a point of telling me how much I had improved since the previous year.

The final performance took place on Saturday afternoon. I was nervous, but I'd practiced my song all week, so I crossed my fingers and hoped for the best. I don't think I knocked it out of the park, but I did fine and managed to cover up a few mistakes. I sighed with relief when it was over.

Year two of The Beats is complete, and I'm already looking forward to the possibility of attending again next year.

If you've ever wanted to learn a new instrument or develop your musical talents further, consider applying to The Beats Music Program. You won't regret it, and you might just surprise yourself!

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Gene Path Weekend Retreat: *Real People. Real Experiences. Real Connection.*

By Matt Marlatt

For men living with hemophilia B, gene therapy represents far more than just a new treatment option; it is a life-changing decision. The journey is filled with questions about risks, expectations, hopes, possibilities, and uncertainties. The *Gene Path Weekend Retreat* was founded with a single goal: to ensure no one has to navigate this journey alone.

During the weekend of June 4-7, nestled in the rolling Osage Hills of Oklahoma, Postoak Lodge provided an immersive, peer-centered retreat designed for men aged 18 and older who were either considering gene therapy or had already undergone it. Instead of focusing solely on clinical education, Gene Path cultivated an environment where honest conversations, shared experiences, and meaningful connections were at the heart of the event.



Throughout the weekend, participants engaged in facilitated discussions, shared meals, small-group conversations, and quiet moments in nature. The relaxed setting encouraged everyone to slow down, reflect, and engage in conversations that often extend far beyond what can be covered during a medical appointment.



Matt Barkdull led an interactive session exploring the opportunities, questions, and personal considerations that often accompany gene therapy, while Bonnie Ben-Shmuel was available to answer questions about CSL and Hemgenix.

As evening settled over the lodge, campfire discussions guided by Wayne Cook offered a welcoming space for participants to openly share their decision-making journeys, the emotional impact of pursuing gene therapy, life after treatment, and the unexpected challenges and rewards encountered along the way.




























One of the retreat's most memorable moments came unexpectedly. When rain disrupted the planned schedule, Dr. Walsh led an impromptu discussion that became a defining experience of the weekend.

The informal atmosphere created a rare opportunity for the men considering gene therapy to ask candid questions of those who had already undergone treatment. These authentic peer-to-peer conversations emerged as one of the retreat's greatest strengths, providing insights and perspectives that no brochure, presentation, or clinical trial could offer.

The retreat concluded with a final wrap-up discussion facilitated by Wayne Cook, where the men still considering gene therapy reflected on their experiences and insights from the weekend. The conversation reinforced Gene Path's core strength: offering a safe, supportive, and pressure-free environment where individuals could explore questions, hear diverse experiences, and make decisions at their own pace.

Many shared that conversations with both clinical experts and men living successfully with gene therapy reduced their uncertainty and gave them greater confidence as they considered their next steps.

While educational sessions were invaluable, the weekend's lasting impact stemmed from the connections formed. The men consistently expressed how meaningful it was to spend time with others who truly understood the realities of living with hemophilia B and facing decisions about gene therapy. The retreat fostered a genuine sense of brotherhood that extended well beyond the scheduled sessions, creating friendships and support networks that many expect will continue long after the weekend ended.

The Gene Path Weekend Retreat illustrates that education alone is not enough. Making an informed






































decision about gene therapy requires more than clinical facts; it calls for honest dialogue, trusted relationships, and the reassurance that comes from hearing real experiences from those who have walked the same path. By uniting individuals with shared experiences in a spirit of openness and mutual support, Gene Path empowers participants to make decisions with greater confidence and clarity.

The Coalition for Hemophilia B extends sincere appreciation to CSL for its generous support of the Gene Path Weekend Retreat and its ongoing commitment to advancing education, connection, and support within the hemophilia B community.

CSL

Comments from Matt Barkdull:

- This historic event was the first of its kind, bringing together the largest number of gene therapy recipients at a single event.
- Very open discussions took place about gene therapy among the men. Subjects included reservations about gene therapy, strong plugs for gene therapy, people mentioning difficulties of no longer being a person with hemophilia due to “survival guilt,” and even those with PTSD symptoms. These discussions added to the very strong, bold, and raw, albeit respectful and supportive, comments that were made.

Dom's Journey:
Growing Up, Growing Strong, and Growing into Gene Therapy



By Renae Baker

Dom laughs easily, tells stories with warmth, and carries a kind of grounded gratitude that feels rare in someone his age. Born in 2004 and raised in New Jersey, Dom grew up with severe hemophilia B (less than 1% clotting factor), yet he talks about his childhood with a mix of humor, humility, and perspective that makes his story feel both deeply personal and universally human.

Childhood in a Helmet

Dom describes himself as "a little, clumsy kid," and his mother, like many parents of a child with a bleeding disorder, was especially vigilant. "I had to wear a helmet up until third grade," he recalls. Not in class, not walking around, but always for recess and gym. "It made my mom feel safe."

He resisted the knee and elbow pads his mother tried to put on him as a baby, but the helmet was non-negotiable. So, he made it his own. "I put SpongeBob stickers on it that I got from doctor visits." His classmates were accepting, especially after his teacher read a book about hemophilia that his mother brought to the class. "I knew I was different, but I never wanted to seem different. The helmet made that hard, but I still played tag, hide-and-seek, and climbed on the playground equipment."

Even then, Dom sensed he was growing up in a different era than the older men he now calls his "blood brothers." "I've learned that I was just born at the right time," he says. "In 2004, the medicine wasn't what it is now, but it was still great. It prevented a lot of spontaneous bleeds I probably would've had twenty years earlier."





Staying Active, Staying Safe

Dom's bleeds were always injury-related. His earliest memory is jumping into a kiddie pool that happened to be sitting on a rock. "I hit my knee and ended up using a walker for a while." Still, he stayed active, playing soccer from second to fourth grade, and then discovering his true love: karate, inspired by his obsession with the Teenage Mutant Ninja Turtles.

When his aunt married into a family that owned a karate studio, Dom finally got his chance. His doctors approved the activity, with limits. "As long as I wasn't sparring or doing anything too physical, I'd be fine." They even noted benefits: flexibility, stronger joints, better body awareness. Dom thrived. "I loved it. I still love it."

By then, he was on prophylaxis, first BeneFIX twice a week, then Idelvion once a week. "That was a game-changer," he says. His mom still infused him until a counselor at bleeding disorders camp asked, "You don't know how to infuse yet?" Dom learned that day. "I was a little scared, but everybody's gotta learn at some point."

He avoided joint damage thanks to effective medication, quick treatment, and physical therapy during a growth spurt when his joints struggled to keep up with his bones. "The medicine was so effective that I never had spontaneous bleeds," he says. And when he did get injured, his mom got him to the ER immediately.

A Young Man with an Old Soul

Dom speaks at events for CSL, and those experiences have shaped his understanding of the hemophilia community. He is acutely aware of the trauma older generations endured: the "bad blood"







era, the losses, the fear, the lack of treatment options. "Doing this has really shown me how blessed I am to be born when I was. I dodged all those bullets." He pauses, worried he sounds casual about it. He doesn't. He sounds grateful. "Those older guys... they lost a lot of people. It was the craziest time back then."

College, Independence, and the Routine that Slipped

Dom studied filmmaking at Montclair State University and graduated in May 2026. College brought independence, and with it the challenge of adhering to his infusion schedule. "Once I left home, the routine slipped through my hands. I'd go weeks without adhering to my treatment protocol."

He knew Idelvion could last longer for some people, though his doctor told him he wasn't one of them. "I was like, 'I'll figure it out myself.'" He didn't have spontaneous bleeds, but he did experience two injury-related ones: one from playing football in the dorms and another from a fractured ankle during a basketball game. Still, he says, "Other than that, the hardest part was just the routine and explaining it to people."



Hearing About Gene Therapy

Dom first heard about gene therapy in March 2024, when a nurse from Children's Hospital of Philadelphia (CHOP) called his mother. They thought he'd be a good candidate. At the consultation, he received folders of charts, trial data, and explanations.

"It definitely sparked my interest because of my irresponsibility in handling my prophylactic treatments properly." His only hesitation was the vector: once his body built immunity to it, another gene therapy might not be an option if this failed.

His grandfather helped him decide. "He said, 'Look, the rewards outweigh the risks.'" Dom trusted his CHOP team. He talked to friends, including one with sickle cell disease, and ultimately, he chose to move forward. He received gene therapy in May 2024, during summer break, giving him three months to manage the post-treatment blood work.

Life After Gene Therapy

"It's worked great!" Dom says. His factor level sits at 15–17%, putting him in the mild range. Some people he's met reached over 70%, but Dom is thrilled with his results. "The only thing I wanted was to do the things I regularly do and not worry about infusing." He tested that confidence recently. "I took my sister to a trampoline park and hit my ankle really hard. I thought I'd wake up with swelling or a bruise. Nothing."

He did have a bleed last May, which meant having to infuse for the first time in two years. He had been sparring in karate and pushed through the pain instead of resting. "It turned into a muscle bleed, which I'd never had before."

But overall? "The way I navigate life is different." He loves hiking. "Before gene therapy, if I hadn't taken my medicine and tried a fifteen-mile hike, I'd come back with swollen ankles."

Advice for Others Considering Gene Therapy

Dom doesn't sugarcoat it. "Really look at the facts, and really look into your soul," he says. Gene therapy requires responsibility, especially when it comes to liver care.

"You have to stay away from alcohol for a certain number of months. I was in college. My friends were drinking on weekends. I had to make sure I could say 'No.'" Even now, he's cautious. "If you damage your liver and it affects the gene therapy, your liver will heal, but your factor levels won't come back." There's also the irony of three months of blood work. "You get gene therapy so you don't have to get poked anymore, and then you're getting poked constantly," he laughs.

His hopes for the future? "Gene therapy for all types of hemophilia and being able to tell people with 100% certainty that it will last their whole life."

Would he do it again? "Absolutely. I went ATVing last summer. Pre-gene therapy, I would've had to treat beforehand. Instead, I just put my helmet on and went."

This time, it was a helmet he wanted to wear.

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MEDEXUS PHARMA

A Life That Hemophilia Never Defined: Remembering Richard Hiteshew Through Toni's Eyes



By Renae Baker

When Richard Hiteshew was born in 1941 with severe hemophilia B, the expectations for his future were limited by the realities of the time. Treatment options were not what they are today, and families living with hemophilia often faced fear, uncertainty, and a lack of clear answers.

Richard's projected life expectancy was only 35 to 40 years. Yet, he lived to be 82.

But the length of Richard's life is only part of the story. Most remarkable is how fully he lived it. Richard did not allow hemophilia to define him, limit him, or keep him from building a meaningful life rooted in family, work, service, humor, and generosity.

His wife, Toni Hiteshew, stood by him through much of that life. Married for 49 years, they raised a family and built a home together. Toni was also with Richard when he passed away at home on October 30, 2023. For those who knew him, Richard's story is not just about living with hemophilia B; it is about living with purpose.





Richard grew up in West Baltimore as one of fourteen children, in a family with an extraordinary history. He was one of five sons with hemophilia. The Hiteshew family became part of early genetic research, studied by renowned geneticist Dr. Victor McKusick, often called the father of medical genetics.

This family history connects Richard's life to the broader story of hemophilia B. It was a time when families contributed to the medical understanding of inherited bleeding disorders while navigating the personal realities of the condition.

After graduating from Baltimore Polytechnic Institute, Richard built a long career in insurance and investments. He became a registered investment advisor and the president of Chesapeake Financial Corporation. Working from a home office, he had a gift for connecting people, ideas, and opportunities. His work was not only about finances; it was about relationships. Over the years, he hired and mentored many young assistants, helping them launch their adult lives and careers.

His generosity extended far beyond his professional life.



Richard was deeply involved in his community. He served on the board of the Institute of Notre Dame and the Catonsville Emergency Food Ministries. Active in Rotary, he served as treasurer for both the Catonsville Sunshine Rotary and the Elkridge Rotary Club. In recognition of his service, he was named a Paul Harris Fellow, which is one of Rotary's highest honors, and received the *Martin Luther King Content of Character* award for his commitment to community service. He also helped start the Canopy Tree Project in Catonsville, leaving a legacy that will continue, quite literally, to grow for years to come.

Those who knew Richard remember his vast knowledge, sharp sense of humor, love of automobiles, and generous spirit. He was a caring father, loving husband, mentor, professional, volunteer, and friend. Hemophilia was part of his life, but never the whole of it.

That distinction truly matters.

For families affected by hemophilia B, especially those who remember earlier eras of treatment, Richard's story carries particular weight. Born into a world with limited medical options and uncertain expectations, he built a career, raised a family, served his community, and lived well beyond what many once thought possible. His life reflects not only the progress made in hemophilia care but also the resilience of the people and families who lived through decades before such progress was possible.

Toni's story is part of that legacy, too. Spouses and caregivers often carry memories that statistics cannot capture. They remember the daily realities, difficult moments, humor, problem-solving, hospital visits, family milestones, and the quiet strength required to keep moving forward.

Richard and Toni built a life together that included five children and six grandchildren. Their family reflects a life not shortened by fear, but expanded through love, service, and connection.

In remembering Richard Hiteshew, the Coalition for Hemophilia B honors more than one man's life. We honor a family's history, a community's resilience, and generations of people with hemophilia B who helped shape the world we know today.

Richard's life reminds us that hemophilia may be part of a person's story, but it does not write the ending.

In Richard's case, the story was long, generous, and deeply meaningful.



ADVOCACY NEWS



B THE VOICE ADVOCACY SUMMIT:
Turning Lived Experience into Advocacy

BY ERICA GARBER

The Coalition for Hemophilia B united individuals and families from across the nation for the 2026 *B the Voice Advocacy Summit*, held June 22–25 in Gaithersburg, Maryland, and Washington, D.C. Across four days of education and advocacy, participants strengthened their skills as advocates, deepened their understanding of policies impacting the hemophilia B community, and prepared to share their voices directly with lawmakers on Capitol Hill.



At the heart of B the Voice is the belief that people living with hemophilia B and their families are uniquely positioned to help policymakers understand the realities of life with a rare bleeding disorder. The Summit opened by empowering participants to recognize the value of their experiences. In *The Story Only You Can Tell*, advocates reflected on pivotal moments that shaped their lives and began learning to transform personal experiences into stories that educate others and inspire change. An evening rap session provided a welcoming space to openly discuss diagnosis, treatment, caregiving, relationships, resilience, and experiences that have often gone unheard.

The next morning, Dana Kuhn, Ph.D., connected that work to the broader history of bleeding disorders advocacy. In his session, *Advocacy Storytelling: Bridging the Past with the Present*, he revisited the extraordinary advocacy efforts of the 1990s, when community members confronted fear, stigma, discrimination, and profound loss to demand change. Their voices helped drive major advances in blood safety and public policy, demonstrating how personal stories can become a powerful force for collective action.

Participants then focused on the pressing issues facing the community today. Advocacy and policy experts provided

education on state and federal policy, and the priorities advocates would bring to Congress. These included safeguarding patient access through the HELP Copays Act, expanding Medicaid access to innovative treatments via the MVP Act, addressing gaps in diagnosis, research, and care for women and girls through the FED UP with Bleeding Disorders Act, and protecting federal investments in research, public health, rare blood disorders programs, and Hemophilia Treatment Centers.

During educational and policy sessions, the Health and Medicine Counsel of Washington (HMC) equipped advocates for their congressional visits by reviewing the legislative process and detailing the hemophilia B community's funding priorities. HMC reviewed how a bill becomes law, Congress's role in the annual appropriations process, and how approved funds are allocated to federal agencies and their respective bleeding disorder programs. Advocates were encouraged to get to know their respective Members of Congress, accentuating the importance of consistent constituent action and vocal congressional champions. Most importantly, HMC emphasized the value of the advocate's personal story and the impact that personal connection can have beyond statistics and policy details.

Detailed funding priorities for FY27 appropriations were outlined, including: $51.3 billion for the National Institutes of Health (NIH) to facilitate continued growth in rare disease research and advancement in hemophilia and rare blood disorders, and $11.6 billion for the Centers for Disease Control and Prevention (CDC) to bolster public health initiatives for the rare disease community. Although programs avoided cuts through the level-funded FY26 process, HMC explained that flat budgets limit new efforts and impede progress for existing programs. Advocates were urged to highlight the need for increased funding for research and innovative technologies, which can ultimately help reduce long-term costs.











With these issues in mind, participants turned their attention to preparing for Capitol Hill.

The interactive *Advocacy Day Practicum: Putting Your Voice into Action* allowed teams to participate in mock congressional meetings, refine their stories and legislative asks, and receive feedback before meeting with lawmakers the next day.

On Wednesday, 35 advocates from 22 states, accompanied by HMC team members, met with 58 House and Senate offices. The seven teams gathered in congressional offices, committee rooms, and even hallways. All offices received materials in advance, and health and legislative aides listened to advocates' stories, asked thoughtful questions, and noted key points for follow-up with Members. Advocates left the meetings energized and empowered to continue engaging with their elected officials.

Advocates' voices from Capitol Hill were also featured in a special episode of the Boundless B Podcast. Through interviews recorded during Advocacy Day, listeners can hear firsthand from

participants about their meetings with lawmakers, the issues they raised in Congress, and what it was like to put their advocacy training into action. The episode is available at hemob.org/podcast.

After a day of congressional meetings, participants returned to Gaithersburg for a final reflection, sharing memorable moments, lessons learned, and the significance of putting their training into practice. Feedback showed how much they accomplished in a short time. Many left with increased confidence in their ability to advocate for themselves and their community.

For many, the impact was deeply personal. One longtime community member described B the Voice as "One of the most exciting events in my 65 years living with hemophilia B," adding, "I learned so much about lobbying and healthcare reform at the national level." Another participant appreciated the opportunity to connect advocates at different stages of their journey: "Being able to come together as a group and inspire and encourage each other, with first-timers and seasoned professionals sharing our stories and learning from each other."

The impact of B the Voice reaches far beyond a single day on Capitol Hill. Participants left eager to continue building relationships with policymakers, strengthen their advocacy skills, and share their knowledge within their local communities. As one participant reflected, "Our voices do matter."



The Coalition for Hemophilia B gratefully thanks CSL, Pfizer, Sanofi, and Medexus Pharma for their generous support of the 2026 B the Voice Advocacy Summit.













Hemophilia Landscape *Updates*

By David Clark, Ph.D.

Some of the items below were presented at the International Society on Thrombosis and Haemostasis (ISTH) Congress, July 11-15, 2026, in Paris. Copies of the abstracts (summaries) for the presentations are available for free at https://isth.m-anage.com/programme?segment=abstracts&sort=short&view=list.

Real-World Unmet Treatment Needs

Two groups recently published articles on unmet needs in hemophilia treatment. Both used the Adelphi Real World Haemophilia Disease Specific Programme (DSP) database. The DSP is a survey of physicians and their patients with hemophilia (or caregivers of patients < 18 years). Data was collected in France, Germany, India, Italy, Japan, Saudi Arabia, Spain, the UK, and the U.S. from July 2023 to October 2024.

5/21/26 The first group comprised researchers from Novo Nordisk and from HTCs in Italy and Spain. They identified data from 283 physicians, reporting on 2227 male patients (1596 As, 631 Bs, including 37 (5.9%) Bs with an inhibitor) with moderate to severe hemophilia. Although 94% of patients were either fairly or fully compliant with their prophylactic treatment, their physicians were not completely satisfied with the results for 54% of the As and 59% of the B's. For the Bs, 29% were on prophylaxis with standard half-life (SHL) products and 67% were on extended half-life (EHL) products.

Among Bs on SHL products, 53% had joint problems, and 48% had target joints. Of those Bs on EHL products, 43% had joint issues, but only 28% had target joints. Physicians reported complete satisfaction with treatment for a higher proportion of Bs receiving EHL compared with SHL factor replacement. When physicians were not completely satisfied with prophylaxis, they most commonly cited administration/dosing and lack of efficacy. Quality of Life (QoL) outcomes suggest that patients with hemophilia have a poorer QoL, regardless of prophylaxis type.

The authors saw a high persistence of spontaneous bleeding episodes in patients on prophylaxis. This also highlights a therapeutic gap. "Even in the USA - where access to the most advanced therapies is high, 48% of patients on prophylaxis still reported at least one bleed in the past year, with 78% of all bleeds requiring factor treatment, highlighting the global nature of this residual burden." [Yuste VJ et al., Haemophilia, 32(4) 989-1001, 2026]

8/25/26 A group of researchers from Pfizer performed a similar study using the DSP database. They looked at data from 75 physicians reporting on 739 patients with hemophilia A and 131 with hemophilia B. For the Bs, 39.7% were on standard half-life (SHL) prophylaxis and 55% were on extended half-life (EHL) prophylaxis. 5.3% of the Bs had a historical or current inhibitor.

Patients still bleed. Among patients who had experienced bleeding within the last 12 months, the average number of bleeding episodes was 1.4 for both those with hemophilia A and those with hemophilia B. Among these patients, the most common type was a joint bleed, which occurred in 53% of As and 57% of Bs. The authors point out, "These findings highlight that despite prophylactic treatment, breakthrough bleeding, particularly within the joints, remains common, emphasizing the need for more effective therapeutic strategies." [Thakkar S et al., Blood Coagulation and Fibrinolysis online ahead of print 8/25/26].

A good conclusion for both studies is taken from the Novo article: "Although current therapies have reduced bleeding and improved QoL, many patients continue to experience limitations to their daily activities, productivity, and physical independence. These findings also underscore a key compromise for [people with hemophilia], namely the trade-off between realizing the benefits of prophylaxis and managing treatment burden."

Intracranial Hemorrhage Risk by Age

7/11/26 Intracranial hemorrhage (ICH, also known as a "brain bleed") is the most serious complication in hemophilia. A group from India presented data on 66 ICH incidents in 54 patients, who were divided into two groups: those under 18 years of age and those 18 or older. (Some patients had more than one ICH episode, since they were all treated on-demand.) In the pediatric group, the median age at ICH was 9 years (range 1 to 17). In the adult group, the median age at ICH was 28 years (range 18 – 40). The results were as follows:

- Six pediatric patients (15%) died, including one with hemophilia B; no adult deaths occurred.

- Three pediatric survivors developed neurological sequelae [after-effects]: all had hydrocephalus [accumulation of fluid in the brain] requiring a shunt [to drain the fluid], and one developed epilepsy. No adult survivors had sequelae.
- Seven pediatric patients (18%) were newly diagnosed with hemophilia during ICH presentation.
- Inhibitors emerged during ICH treatment in ten patients (nine hemophilia A, one hemophilia B); three died. Three hemophilia A patients developed inhibitors during a subsequent ICH episode.
- Seven patients required neurosurgical intervention; eight experienced recurrent ICH (two with three episodes each).
- Among survivors, 85% achieved favorable functional recovery at discharge and after 90 days. Most episodes (62/66) were spontaneous.

This study shows that ICH produced substantially worse outcomes in pediatric patients, including higher mortality, neurological after-effects (sequelae), inhibitor development, and need for surgical intervention. In comparison, adults experienced uniformly favorable recovery. A significant proportion of the pediatric patients were first diagnosed with hemophilia during their ICH treatment, suggesting that delayed diagnosis contributes to catastrophic bleeding.

The authors conclude, "Pediatric hemophilia-associated ICH represents a distinct high-risk clinical phenotype, underscoring the need for early recognition and proactive inhibitor surveillance, particularly in settings with limited prophylaxis access." [ISTH abstract PB1486]

New Treatments for Hepatitis B and Hepatitis Delta Infection

5/28/26 Many older hemophilia patients who used plasma-derived products before the late 1980s are infected with diseases like hepatitis B, hepatitis C, and HIV. With hepatitis B, most patients go on to recover from the disease, but a fraction develop chronic hepatitis B, which stays in their body for their lifetime. Hibsago (bepirovirsen), a treatment for hepatitis B, is under development by Ionis Pharmaceuticals and GlaxoSmithKline. It has already been approved in Japan and is under priority review by the U.S. FDA, which is expected to rule on its approval by the end of October 2026. The product is a weekly injection.

Hibsago was tested in more than 1838 patients in 29 countries. One in five (20%) patients in the study experienced a "functional cure" in which no virus was detectable after 48 weeks. No placebo patients were cured. [Ionis press release 5/28/26]

5/22/26 Hepatitis Delta virus (HDV) is a piggyback infection that only occurs in people already infected with hepatitis B. It is not infectious on its own. It exacerbates hepatitis B infection, leading to a more rapid progression, cirrhosis, liver failure, and potentially death. Gilead Sciences has developed Hepcludex (bulevirtide) to treat delta virus infections. It is a daily subcutaneous injection that was approved by the FDA under accelerated approval designation on 5/26/26. The approval is provisional pending additional studies by Gilead. Patients with cirrhosis are excluded, but those with compensated cirrhosis may be treated.

Gilead estimates that 2–4% of patients infected with chronic hepatitis B are co-infected with HDV. In the Phase III study, 100 patients received daily injections for 96 or 144 weeks (1.8 or 2.75 years). The product met its endpoints of HDV RNA reduction and normalization of ALT (liver enzyme) levels. [Gilead press release 5/22/26]

Comorbidities and Mortality in the Aging Hemophilia Population

7/11/26 Two studies at ISTH looked at comorbidities and death rates in older hemophilia patients. Comorbidities are other diseases or disorders a patient may have in addition to hemophilia. A group from Emory University reviewed U.S. patients using the Medicare claims information database.

They found 2058 people with hemophilia. Their average age at Medicare enrollment was 57.2 years; 83.1% were male, and 84.3% had hemophilia A. Less than 5% had inhibitors. They found that rates of common disorders, such as anxiety, depression, cancer, cardiovascular disease (CVD), and diabetes, were significantly higher in the hemophilia population than in the general population. In addition, various hemophilia-related disorders such as liver disease, HIV, and joint problems also occurred at a higher rate in the hemophilia population.

Interestingly, they also found that death rates were 12% higher in males with hemophilia, but not in females. The authors conclude, "the substantially higher comorbidity burden in the hemophilia population calls for more intensive prevention efforts and ongoing coordinated and multidisciplinary care." [ISTH abstract PB1450]

7/11/26 French researchers carried out a similar study using data from the FranceCoag registry, supplemented by patient questionnaires. They only looked at people with severe or moderate hemophilia. In that more restricted group, they also found a significantly higher death rate, almost twice as high as in the general population. The leading causes of death were cancer, hemorrhage (bleeding), and liver disease. For the

common morbidities, they found no increased risk compared with the general population. [ISTH abstract PB1449]

Definition of a Severe Bleeding Phenotype for Patients with Non-Severe Hemophilia

3/25/26 Current guidelines (WFH and ISTH) recommend prophylaxis for people with non-severe (mild or moderate) hemophilia who exhibit a severe bleeding phenotype. Phenotype here refers to the bleeding behavior observed in a patient, regardless of the patient's factor levels. We know that about 15% of hemophilia patients bleed differently than predicted by their factor levels. However, the guidelines don't define what a severe bleeding phenotype actually is.

A large group from the GTH (German, Swiss and Austrian Society of Thrombosis and Hemostasis Research) decided to rectify that situation using a Delphi consensus procedure. According to NIH, a "Delphi consensus process is a structured, iterative method designed to gather reliable agreement from a panel of anonymous experts through a series of questionnaires and controlled feedback."

According to their results, the major criteria are:

1. Life-threatening bleeding in critical regions or organs that occurred spontaneously or after minor trauma, such as intracranial, intraocular, retroperitoneal, intra-articular, pericardial, or muscle bleeding with compartment syndrome, or bleeding leading to a loss in hemoglobin ≥ 2.0 g/dL or a red blood cell transfusion.
2. Severe bleeding that occurs spontaneously, repeatedly, or after inadequate trauma, such as muscle bleeding or bleeding with consequences for function and structure.
3. Development of hemophilic arthropathy (joint damage).
4. Presence of chronic synovitis (joint inflammation).
5. Hb-relevant menstrual bleeding despite exhausting conservative measures such as tranexamic acid, DDAVP, and/or hormone therapy.

They also listed several minor criteria:

1. Repeated spontaneous extensive hematomas requiring substitution with factor concentrates.
2. Either of the following:
   a. A positive family history for a severe bleeding phenotype.
   b. A positive family history for life-threatening bleeding.
3. Repeated bleeding following inadequate trauma that does not impair function and structure but may indicate a potential structure-impairing bleeding.
4. The consensus confirmed that a severe bleeding phenotype can be defined by the presence of at least one major or two minor criteria. Additional details are available in the article, which is free on the Haemophilia journal website. Note that this is just a proposal and has not been accepted by any major bleeding disorder organizations. [Pfrepper C et al., Haemophilia, 32(3), 665-670, 2026]

HEMOPHILIC ARTHROPATHY

Joint Health in Inhibitor Patients, Women and Children with Mild Hemophilia

Three studies presented at ISTH examined joint health in inhibitor patients, women with hemophilia, and children. In severe hemophilia joint problems are obvious and can be treated. In mild and moderate hemophilia, including many women with hemophilia, joint degradation can accumulate quietly because it's not associated with noticeable bleeding episodes.

7/11/26 A group from the UK performed a qualitative (descriptive, not numerical) study of joint health in hemophilia patients with inhibitors. They found marked differences between those with hemophilia A and those with hemophilia B. Hemophilia A patients have better success with immune tolerance induction (ITI) to eliminate their inhibitors and also have access to Hemlibra, a factor VIII substitute that has been available for several years and performs well for many inhibitor patients. Meanwhile, hemophilia B inhibitor patients have poorer results with ITI and, until the recent approval of rebalancing agents, had no good treatment beyond activated factor VII bypassing agents.

In the words of the authors, hemophilia B inhibitor patients "faced allergic reactions, limited ITT [immune tolerance therapy] success, and only recent access to non-factor therapies. Children with [hemophilia B] experienced ongoing joint bleeds, synovitis, reduced mobility, often requiring wheelchairs and aids. Older adults reported significant joint damage, pain, and reliance on mobility aids, but those with HB had more persistent joint morbidity due to fewer effective treatment options." Hopefully, the rebalancing agents will help to rectify this situation. [ISTH abstract PB3333]

7/11/26 A group from Argentina looked at joint health in women with hemophilia using the HEAD-US method. HEAD-US (Hemophilia Early Arthropathy Detection with Ultrasound) uses ultrasound to image joints. This is similar to the ultrasound imaging used to look at babies in the womb. You can get a fairly detailed picture of a

joint that helps a physician understand what's going on. Because ultrasound is much cheaper and more widely available than MRI, HEAD-US has been getting a lot of attention.

In a group of 29 women with hemophilia (24 As, 5 Bs), they found no evidence of joint damage in 21 subjects (72.4%), but found signs of synovitis or inflammatory joint changes in eight (27.6%) of the women. In all of the eight women with joint abnormalities, the most affected joint was the knee. Seven subjects showed bilateral changes (both knees), while one subject had problems in only one knee. Most of the synovitis (inflammation of the joint capsule) scores were minimal, with no evidence of damage to the bones and cartilage.

The authors point out that most of the joint inflammation potentially comes from microbleeds, bleeds too small to be noticed, but which can cause cumulative joint problems over time. They conclude, "The combination of HEAD-US and serological tests [factor assays] allows for a comprehensive and early evaluation of joint involvement in this population, facilitating the prevention and timely management of complications." [ISTH abstract PB3342]

7/11/26 A group from Greece looked at joint status in children with mild hemophilia. One goal of hemophilia treatment is to identify joint damage as early as possible so it can be treated before it gets too bad. Mild hemophilia patients tend to have low bleeding rates, but recent data on mild adults have shown joint damage despite that.

The authors reviewed chart records for 14 boys with hemophilia. Their average age was 11.4 years (range 5–18) and their median factor level was 13.5% (range 5–33.6). Eight (57.1%) patients reported regular physical activity, ranging from football (soccer) to breakdancing. Their median number of reported joint bleeds was 0.7 (range 0–7), with nine patients (65.2%) reporting no joint bleeds.

Subjects were evaluated using the Hemophilia Joint Health Score (HJHS). Their median score was 0.2 (range 0–2), with 12 (85.7%) patients scoring zero, indicating no joint issues. Ultrasound scans showed mild synovitis in two patients who had normal HJHS scores. The authors conclude, "Nearly one third of pediatric patients with mild hemophilia experienced joint bleeding, and 14.3% demonstrated early joint changes detectable by ultrasound, despite zero or low reported bleeding rates. These findings highlight the importance of regularly evaluating joint health in children with mild hemophilia. Early identification of joint involvement is essential in developing strategies aimed at reducing long-term disease burden." [ISTH abstract PB3326]

Normal Values for Coagulation Factors in Men, Women and Neonates

7/11/26 One issue in looking at hemophilia in women may be that the normal levels (called "reference values") of factors VIII and IX for men might differ in women. The standard reference values for factor VIII and factor IX in blood are "50 to 150% of normal" for both. It's not clear where those numbers originally came from. Were they just statistical assumptions, or were they based on actual patient data? Did the studies include women, and which assays did they use? We now know that some factor assays give different results for men than for women.

Two ISTH papers give us some new information. A group from China examined the levels of several clotting factors in healthy Chinese adults [ISTH abstract PB3129], while a group from Brazil examined factor levels in neonates (newborns) [ISTH abstract PB2225]. The table below shows the results.

In the table, the first column lists the measured clotting factors. The next column, "Overall," shows the factor-level values for all subjects, both men and women, combined. For instance, for factor II, the range was 68.0 to 119.2% of normal. The range shown is not the actual range measured. The actual measured values were fit to a statistical model, and the range shown is between the 2.5th and 97.5th percentiles. That gives us 95% confidence that all of the data fall between those limits. For instance, if we measured factor II in 100 subjects, we would expect 95 of those people to fall within the range shown. That's not an exact description of 95% confidence, but it's good enough for our purposes here.

In parentheses under each range are the mean value and the span. The mean (average) value is just the midpoint of the range in this case. (That's approximate, but good enough for this discussion. It depends on whether the data are "normally distributed," which we don't know.) The span is the difference between the maximum and minimum values.

The next two columns labeled "Men" and "Women" show the same information broken down by gender. The next column, labeled "Mean: Men – Women," shows the difference between the mean value for men and the mean value for women.

The last column contains the data from the Brazilian study of factor levels in neonates. The Brazilian study used a different statistical interval, the interquartile range. This is the range from the 25th percentile to the 75th percentile, so it gives us an approximately 50% confidence level. If we measured a factor level in 100 neonates, approximately half of the result values would

fall within the range shown. Again, the mean value is assumed to be the midpoint of the range.

Because the two studies use different statistical intervals, the ranges are not directly comparable. However, we can compare the mean values.

Therefore, you can compare the mean values for neonates with the mean values for adults in the Chinese study to get an approximate idea of the differences. For instance, for factor II, the adult mean value is 93.6% while the neonate value is 44.7%. That suggests that factor II levels in neonates are about half of what we see in adults.

All of the Chinese data below are from subjects without known bleeding disorders. Depending on the factor measured, each group included 168 to 204 men and 206 to 244 women. Samples came from 374 to 448 individuals per factor. The Brazilian data came from 92 neonates (48 males, 44 females), also without known bleeding disorders.

Gender Differences:
Looking at the Chinese study data (middle four columns of the table), the expected mean value for each group is 100%, because the "unit" of measurement for each factor is defined as the activity of that factor in 1 mL of blood. Most results fall within about ±10 % of that value (90–110%). There are two prominent outliers.

Table: Clotting Factor Levels in Chinese and Brazilian Subjects

Clotting Factor	Factor Level as % of Normal	Factor Level as % of Normal	Factor Level as % of Normal	Factor Level as % of Normal	Factor Level as % of Normal
Clotting Factor	Chinese Study 2.5^{th} – 97.5^{th} Percentiles (Mean, Span)	Chinese Study 2.5^{th} – 97.5^{th} Percentiles (Mean, Span)	Chinese Study 2.5^{th} – 97.5^{th} Percentiles (Mean, Span)	Chinese Study 2.5^{th} – 97.5^{th} Percentiles (Mean, Span)	Brazilian Study Interquartile Range (Mean)
Clotting Factor	Overall	Men	Women	Mean: Men - Women	Neonates
I	-	-	-	-	208 – 290 mg/dl (249)
II	68.0 – 119.2 (93.6, 51.2)	60.6 – 115.8 (88.2, 55.2)	80.9 – 119.9 (100, 39.0)	-12.4	38.4 – 51.0 (44.7)
V	63.9 – 122.5 (93.2, 58.6)	64.0 – 122.4 (93.2, 58.4)	64.0 – 122.4 (93.2, 58.4)	0.0	51.2 – 81.5 (66.4)
VII	65.2 – 125.8 (95.5, 60.6)	63.5 – 125.6 (94.6, 62.1)	68.6 – 125.8 (97.2, 57.2)	-2.6	45.2 – 88.0 (66.6)
VIII	58.1 – 157.5 (108, 99.4)	54.5 – 161.0 (108, 106.5)	60.3 – 156.5 (108, 96.2)	0.0	54.0 – 88.0 (71.0)
IX	70.5 – 152.1 (111, 81.6)	62.2 – 152.0 (107, 89.8)	79.5 – 151.3 (115, 71.8)	-8.0	41.5 – 54.8 (48.2)
X	63.7 – 117.6 (90.6, 53.9)	65.0 – 119.1 (92.0, 54.1)	63.6 – 114.8 (89.2, 51.2)	3.0	37.0 – 46.8 (41.9)
XI	68.2 – 123.8 (96.0, 55.6)	66.1 – 123.4 (94.8, 57.3)	74.0 – 124.8 (99.4, 50.8)	-4.6	36.0 – 56.0 (46.0)
XII	45.3 – 145.1 (95.2, 99.8)	46.5 – 140.6 (93.6, 94.1)	45.0 – 145.1 (95.0, 100)	-1.4	34.0 – 53.3 (43.6)
XIII	-	-	-	-	62.7 – 76.6 (69.6)
vWF antigen	-	-	-	-	115.9 – 188.2 (152)
vWF activity	-	-	-	-	79.2 – 111.7 (95.4)

The mean level of factor II (prothrombin) in men is 88%, and the mean level of factor IX in women is 115%. The mean factor X level in women is borderline at 89.2%. Interestingly, factor VIII levels are the same in both men and women, at 108%.

The largest differences between men and women are for factor II and factor IX, where women have substantially higher levels than men. The next largest difference is in factor XI; factor XI activates factor IX, so it is also important in hemophilia. It has been a mystery why women have been observed to bleed with factor IX levels in the 60 – 70% range, but this data suggests that the lower level of "normal" for women is about 80%, not the 50% used in the U.S. or the 40% used internationally.

We can also see some interesting information in the span values. The span suggests how much patient-to-patient variability there is in the results. A low span suggests patients have similar factor levels, while a large span suggests much more patient-to-patient variability. The span results for most factors are in the 50–60% range, but some are notable outliers. In the Overall column, the spans for factors VIII and XII are both near 100%, while the span for factor IX is also high at about 82%. This suggests more patient-to-patient variation for those factors.

We can also examine differences in spans between men and women. The largest difference is in factor IX, where men have a span of 89.8%, and women have 71.8%, a difference of 18%. The next highest difference is for factor II at 16.2%, followed by factor VIII at 10.3%. Interestingly, women seem to have the tighter spans for those factors than men. Thus, hemophilia factors VIII and IX both show large differences between men and women. However, we can't say much about the significance of those differences.

Levels in Neonates:
The samples for the Brazilian data were collected on day 2 or 3 post-birth. All patients received 1 mg of vitamin K in the delivery room. Vitamin K jump-starts the production of the vitamin K-dependent (VKD) clotting factors II, VII, IX, and X, plus the anticoagulants protein C and protein S. Even so, these factors are about half the levels found in adults. Most of the other factor levels are low as well. The key point is that these levels differ from those in adults. Therefore, care should be taken to avoid misdiagnosis of bleeding disorders.

The results in both studies should not be taken as universal values for all patients. Values can vary depending on the specific testing laboratory methods and the genetic diversity of local populations. The authors recommend that every laboratory develop ranges for their specific local populations. However, the current results provide valuable data for comparison between women and neonates.

Other Observations:
One of the big mistakes we make in thinking about hemophilia is the assumption that except for factor IX (or VIII for hemophilia A), all the rest of everyone's factors are at approximately normal levels. This data shows that assumption is faulty. A person without hemophilia could have factor levels quite far from the mean and still fall within the "normal" range. For instance, a person with all their factor levels near the maximum end of the range would be expected to clot faster than a person with all their factor levels near the minimum end of the range, even though both would be considered "normal."

Researchers have speculated that differing levels of the other clotting factors could explain why some patients don't fit the severity category associated with their factor IX level. For instance, if a person with severe hemophilia B (factor IX level <1%) had high levels of all the other clotting factors, they might retain enough clotting activity for their bleeding to appear moderate rather than severe.

Another issue for women is that their factor levels likely vary depending on their hormone levels, especially estrogen. Thus, their observed factor levels could depend on when in their monthly cycle their blood sample is taken. Estrogen rises during ovulation and then falls during menstruation. Studies have shown that estrogen increases the body's production of factors II, V, VIII, IX, X, XI, and XII, and also reduces the production of the anticoagulants protein S and protein C.

The decrease in clotting factor levels during menstruation is probably intended to inhibit clotting so menstrual blood can flow freely, wash out the uterus, and prepare it for the next monthly ovulation. Thus, the time of the month a blood sample is taken probably affects the factor level measured. When we talk about factor levels in women, we should define a specific sampling time point.

Besides affecting clotting factor levels, some evidence suggests estrogen may affect the clotting cascade. We don't know whether it is a direct effect in which estrogen molecules enter the clotting factor reactions or an indirect effect in which estrogen affects something else that then affects the clotting cascade. [Stanczyk FZ, et al., Frontiers in Endocrinology, 16:1617731 (2025)]

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It is not known if Alhemo® is safe and effective in people while receiving ongoing Immune Tolerance Induction (ITI).

It is not known if Alhemo® is safe and effective for hemophilia A and B with and without inhibitors in children younger than 12 years of age.

Do not use Alhemo® if you are allergic to concizumab-mtci or any of the ingredients in Alhemo®.

Before using Alhemo®, tell your healthcare provider about all of your medical conditions, including if you:

- have a planned surgery. Your healthcare provider may stop treatment with Alhemo® before your surgery. Talk to your healthcare provider about when to stop using Alhemo® and when to start it again if you have a planned surgery.
- are pregnant or plan to become pregnant. It is not known if Alhemo® may harm your unborn baby. **Females who are able to become pregnant:**
  - Your healthcare provider may do a pregnancy test before you start treatment with Alhemo®.
  - You should use an effective birth control (contraception) during treatment with Alhemo® and for 7 weeks after ending treatment. Talk to your healthcare provider about birth control methods that you can use during this time.
- are breastfeeding or plan to breastfeed. It is not known if Alhemo® passes into your breast milk. Talk to your healthcare provider about the best way to feed your baby during treatment with Alhemo®.

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Know the medicines you take. Keep a list of them to show your healthcare provider and pharmacist when you get a new medicine.

How should I use Alhemo®?

- Use Alhemo® exactly as prescribed by your healthcare provider.
- Your healthcare provider will provide instructions for stopping (discontinuing) your current treatment when switching to Alhemo®.
- Inject Alhemo® 1 time a day.
- **Your healthcare provider should show you or your caregiver how to use Alhemo® before you use it for the first time.**
- Alhemo® is given as an injection under the skin (subcutaneous injection) by you or a caregiver.
- Ask your healthcare provider if you need to use a different injection technique. For example, children and people who are lean may need to inject into a pinched fold of skin to avoid injecting too deep (into the muscle).
- Change (rotate) your injection site with each injection. **Do not** use the same site for each injection.
- You will inject a larger dose (a loading dose) of Alhemo® on your first day of treatment. Then your healthcare provider will prescribe a dose to inject 1 time a day until your maintenance dose is established.
- To determine the right maintenance dose for you, your healthcare provider will do a blood test to check the amount of Alhemo® in your blood. Your healthcare provider may do additional blood tests during treatment with Alhemo®.

How should I use Alhemo®? (cont'd)

- Your healthcare provider will prescribe your dose based on your weight. If your weight changes, tell your healthcare provider.
- Your healthcare provider will provide information on the treatment of breakthrough bleeding during your treatment with Alhemo®.
- Do not share your Alhemo® pens and needles with another person, even if the needle has been changed. You may give another person an infection or get an infection from them.
- **If you miss a dose of Alhemo® during the first 4 weeks of treatment,** contact your healthcare provider right away. Your healthcare provider will tell you how much Alhemo® to inject.
- **If you miss a dose of Alhemo® after your daily maintenance dose is established:**
  - For 1 missed dose, continue your daily maintenance dose.
  - For 2 to 6 missed doses, give 2 doses as soon as you remember. Then continue your daily maintenance dose the next day.
  - For 7 or more missed doses, contact your healthcare provider right away as you will need to receive a new loading dose before continuing your daily maintenance dose.
  - If you are unsure about how much Alhemo® to inject, contact your healthcare provider.

What are the possible side effects of Alhemo®?

Alhemo® may cause serious side effects, including:

- **Blood clots (thromboembolic events).** Alhemo® may cause blood clots to form in blood vessels, such as in your arms, legs, heart, lung, brain, eyes, kidneys, or stomach. You may be at risk for getting blood clots during treatment with Alhemo® if you use high or frequent doses of factor products or bypassing agents to treat breakthrough bleeds, or if you have certain conditions. Get medical help right away if you have any signs and symptoms of blood clots, including:
  - swelling, warmth, pain, or redness of the skin
  - headache
  - trouble speaking or moving
  - eye pain or swelling
  - sudden pain in your stomach or lower back area
  - feeling short of breath or severe chest pain
  - confusion
  - numbness in your face
  - problems with your vision
- **Allergic reactions.** Alhemo® can cause allergic reactions, including reactions that can be serious. Stop using Alhemo® and tell your healthcare provider right away if you have redness of the skin, rash, hives, itching, and stomach-area (abdominal) pain. Stop using Alhemo® and get emergency medical help right away if you develop any signs or symptoms of a severe allergic reaction, including:
  - itching on large areas of skin
  - trouble swallowing
  - wheezing
  - pale and cold skin
  - dizziness due to low blood pressure
  - redness or swelling of lips, tongue, face, or hands
  - shortness of breath
  - tightness of the chest
  - fast heartbeat

The most common side effects of Alhemo® include:
- bruising, redness, bleeding, itching, rash, or lump at the injection site
- headache
- hives

These are not all the possible side effects of Alhemo®.

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

How should I store Alhemo®?

- **Before first use:**
  - Store unused Alhemo® pens in the refrigerator between 36°F to 46°F (2°C to 8°C).
- **After first use:**
  - Store the Alhemo® pen in the refrigerator between 36°F to 46°F (2° C to 8°C) or at room temperature below 86°F (30°C) for up to 28 days.
  - Write the date of first use in the space provided on the carton.
  - Throw away (discard) the Alhemo® pen 28 days after first opening even if some medicine is left in the pen.
- Store Alhemo® with the cap on and keep it in the original carton to protect from light.
- Do not store Alhemo® in direct sunlight and keep away from direct heat.
- When stored in the refrigerator, do not store the pen directly next to the cooling element (the part that cools the refrigerator).
- Do not freeze Alhemo®.
- Do not use Alhemo® if it has been frozen or if it has been stored above 86°F (30°C).

Keep Alhemo® and all medicine out of the reach of children.

More detailed information is available upon request.

Available by prescription only.

For information contact: Novo Nordisk Inc., 800 Scudders Mill Road, Plainsboro, New Jersey 08536, USA, 1-888-668-6444

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At: Novo Nordisk A/S, Novo Allé 1, 2880 Bagsværd, Denmark

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novo nordisk®

Hemophilia Landscape
Emerging Therapies

By David Clark, Ph.D.

Summer 2026 With the recent approval of several new products for hemophilia B, including rebalancing agents and gene therapy, product development is now in a lull. Much of the current research focuses on how these new therapies perform in the real world. Current hemophilia B products can be separated into three categories: 1) improved factor products, 2) rebalancing agents, and 3) gene therapy. These updates are divided into those three categories. Within each category, the entries are generally listed in order of the names of the organizations developing the products.

Some of the items below were presented at the International Society on Thrombosis and Haemostasis (ISTH) Congress, July 11-15, 2026, in Paris. Copies of the abstracts (summaries) for the presentations are available for free at https://isth.m-anage.com/programme?segment=abstracts&sort=short&view=list.

IMPROVED FACTOR PRODUCTS

These are improved versions of the factor products most people with hemophilia B currently use, including inhibitor treatment products.

EHL versus SHL Products for Treatment of Mild-to-Moderate Patients Undergoing Surgery

7/11/26 A group from Italy compared extended half-life (EHL) factor products with standard half-life (SHL) products in patients with mild-to-moderate hemophilia undergoing surgery. Data on using EHL products in surgery for patients with severe hemophilia are good, but much less so for non-severe patients. They looked at 24 surgeries managed with EHL products in 21 patients (As and Bs). This included two patients who had undergone the same surgery twice, once with SHLs and once with EHLs. The results show reduced factor infusions and usage with EHL products. All 24 surgeries were managed with a single pre-operative infusion of EHL product, and post-surgical infusions never exceeded one per day. There were no safety issues.

The two patients (one mild B and one moderate B) with the same surgeries on both SHL and EHL products showed substantial decreases in factor usage and infusions. The mild patient had five infusions of SHL factor IX with a total factor consumption of 200 IU/kg. In the second surgery on EHL factor IX, that patient only required two infusions and used 67 IU/kg. The moderate patient had 15 infusions of SHL factor IX with a total factor consumption of 925 IU/kg. On EHL factor IX, the patient required 10 infusions and used 650 IU/kg. The authors conclude that EHL factor products appear safe and effective for surgery in mild-to-moderate hemophilia. [ISTH abstract PB2152]

A Modified Factor X for Inhibitor Treatment

5/8/26 A group from Children's Hospital of Philadelphia and the Universities of Ferrara and Careggi in Italy are exploring a modified factor X as a bypassing agent (BPA) for patients with inhibitors. Although many inhibitor patients benefit from new therapies like rebalancing agents, BPAs will still be needed for those for whom rebalancing agents are ineffective, as well as for treatment of breakthrough bleeds.

Factor X is a key central participant in the clotting cascade. It is activated to factor Xa by both the factor VII/tissue factor pathway and the factor VIII/IX pathway. Activated factor X goes on to convert prothrombin (factor II) to thrombin, which then converts fibrinogen (factor I) to fibrin. Fibrin sticks to itself and to damaged tissue to form a clot. Current BPAs contain activated factor VII, which activates factor X, so there has long been a desire to develop a factor X-based BPA as a more direct path to clotting in inhibitor patients.

There are two major problems. First, factor X is toxic in that too much factor X can lead to dangerous thrombosis (unwanted clotting). Factor X overload was probably the problem with early factor IX complex products, which contained factor X as well as factors II, VII, and IX. Those products could treat bleeds on demand but could not be used for prophylaxis or surgery because of the risk of thrombosis. The second problem is that activated factor X has an extremely short half-life of less than two minutes.

The authors developed a modified factor X, FXBP4b, that is activated by factor XIa, the same factor that activates factor IX. Thus, it bypasses the need for the factor VIII/IX step in the clotting cascade. Laboratory studies show that FXBP4b is a promising BPA with broad applicability not only for hemophilia inhibitor treatment but also for other bleeding disorders. [Tarantino R et al., Journal of Thrombosis and Haemostasis, 24(8), 2789–2800, 2026]

REBALANCING AGENTS

Rebalancing agents tweak the clotting system to restore the balance so the blood clots when it should and doesn't clot when it shouldn't. The clotting cascade is a complex system of clotting factors that promote clotting plus anticoagulants that inhibit and control clotting. In a person without a bleeding disorder, clotting and anticoagulant activities are balanced, so the system produces clots as needed but doesn't clot when it shouldn't. In hemophilia, the loss of some clotting factor activity unbalances the system; anticoagulant activity becomes too high, keeping the blood from clotting. Rebalancing agents mainly reduce or inhibit anticoagulant activity in the system. Most of these agents help restore clotting in people with hemophilia A or B, with or without inhibitors, and will likely find application for other bleeding disorders.

Alphamab Publishes Results for Armocibart (KN057) in Patients with Hemophilia or vWD, Type 3

6/4/26 Suzhou Alphamab, a Chinese company, is developing Armocibart (KN057) as a rebalancing agent for treatment of patients with hemophilia A or B or with Type 3 von Willebrand disease. KN057 is a subcutaneous monoclonal antibody that inhibits tissue factor pathway inhibitor (TFPI), an anticoagulant. The product is currently in Phase III studies in China with a projected completion in mid-2027. Alphamab also recently licensed the product to Nanolek, a Russian company, for future distribution in Russia, Azerbaijan, Uzbekistan, and other Eurasian countries. [Ju M, et al., Haemophilia, online ahead of print 6/4/26 and Nanolek press release 6/17/26]

Why are we talking about developments in China and Russia? Because that may be our future. With the U.S. government's cutback of research support, we are rapidly losing our standing as the most prolific supplier of scientific knowledge. In 2024, China overtook the U.S. in publishing scientific studies in prestigious journals. They are now the world's leader in science. [Quincy Institute for Responsible Statecraft, https://quincyinst.org/research/chinas-historic-rise-to-the-top-of-the-scientific-ladder/#] Other countries are also producing excellent research. Europe, Japan, and Australia continue as they have always done, while Canada has made great strides in research on hemophilia in women.

GC Biopharma Reports Results from Phase Ib Study of MG1113

7/11/26 GC Biopharma, a Korean company, is developing MG1113, a rebalancing agent for treatment of patients with hemophilia A or B. MG1113, a weekly subcutaneous monoclonal antibody product that inhibits tissue factor pathway inhibitor (TFPI), an anticoagulant. At ISTH, they reported results from their Phase Ib safety and dose-finding study. In 15 subjects (14 As, 1 B), they found MG1113 was well tolerated, with no serious adverse events and no signs of thrombogenicity. They found a 92% reduction in annualized bleeding rate (ABR), with 60% of patients experiencing no bleeds. [ISTH abstract PB2111]

Note that GC Biopharma (formerly Green Cross) is another non-U.S. up-and-coming pharmaceutical company focusing on hemophilia. When Catalyst Biosciences sold its hemophilia portfolio, GC Biopharma bought two of their hemophilia assets. Dalcinonacog alfa is a subcutaneous extended half-life (EHL) factor IX that Catalyst advanced to Phase II. Marzeptacog alfa activated is a subcutaneous EHL factor VIIa product for inhibitor treatment that reached Phase III. GC Bio has not announced the future of these two products, but its work on MG1113 suggests it is serious about the hemophilia market.

Novo Presents Results from Pediatric Study of Alhemo

7/11/26 Novo Nordisk markets Alhemo (concizumab) as a rebalancing agent for treatment of patients with hemophilia A or B, 12 years or older, with or without inhibitors. Alhemo is a daily, subcutaneous monoclonal antibody product that inhibits tissue factor pathway inhibitor (TFPI), an anticoagulant. At ISTH, they reported on their ongoing study of Alhemo in children with inhibitors under 12 years of age. In 24 subjects treated with Alhemo, they saw an average ABR of 2.08 compared with an ABR of 11.51 for the same group on on-demand treatment. There were no new safety concerns. [ISTH abstract OC 02.1]

Novo Presents Results on Binding of Alhemo to TFPI in Platelets and the Extracellular Matrix

7/11/26 TFPI, the target of both Alhemo and Hympavzi, exists in two forms: TFPI-α, which exists in the extracellular matrix (ECM) and inside platelets, and TFPI-β, which binds to endothelial cells on the blood vessel wall. The ECM is a collection of proteins and other molecules that surround cells to provide structural and biochemical support. Alhemo binds to both types of TFPI, but Novo also wanted to look at the various places in the body where Alhemo and TFPI could interact.

Platelets are small blood cells that are part of the clotting system. When clotting is initiated, platelets become activated and stick to each other and to the blood vessel wall around the injury site. They form a "platelet plug" that is reinforced by the network of fibrin strands that arise from the actions of the clotting factors in the coagulation cascade. It is because of platelets that people with hemophilia and other factor-based bleeding disorders eventually stop bleeding.

Even with poor fibrin network development (the "clot"), the platelet plug can eventually stop the bleeding. Platelets contain a number of clotting factors, anticoagulants, and other molecules both inside and on their surfaces. Platelets release many of these clotting molecules when they become activated, and they then participate in various clotting reactions.

Alhemo was found to be taken up by platelets but did not associate with the TFPI-α inside the platelets until the platelets became activated. However, as soon as platelets became activated and secreted TFPI-α into the bloodstream, Alhemo antibodies bound to it and neutralized TFPI. Alhemo directly bound to TFPI-α and TFPI-β in the ECM and on the walls of the endothelial cells lining the inside of blood vessels. Thus, Alhemo was seen to interact with TFPI in all of the various reservoirs of TFPI in the body. The study authors point out that this widespread binding of Alhemo to TFPI probably contributes to the product's efficacy as a prophylactic treatment for hemophilia. [ISTH abstract PB2085]

Pfizer's Hympavzi Approved for Use in Children and Inhibitor Patients

6/8/26 Pfizer markets Hympavzi (marstacimab) as a rebalancing agent for treatment of patients with hemophilia A or B. Hympavzi is a weekly, subcutaneous, monoclonal antibody product that inhibits tissue factor pathway inhibitor (TFPI), an anticoagulant. Hympavzi has now been approved for all patients with hemophilia A or B aged six years and older, with or without inhibitors.

Hympavzi is now the only rebalancing agent indicated for children between six and twelve. [Pfizer press release 6/8/26]

Pfizer Presents Data on Hympavzi in Children and Plans for Study in Children Under Six Years Old

7/11/26 At ISTH, Pfizer presented interim results from their ongoing pediatric study of Hympavzi. As of the data cutoff, 5/26/25, they had enrolled 92 subjects (median age 10.0; 31 with hemophilia B). Among the 14 inhibitor patients who completed the study, they found an average ABR of 1.44 on Hympavzi compared with 18.92 on-demand treatment with bypassing agents. For the 36 non-inhibitor patients, the ABR was 1.84 on Hympavzi compared with an ABR of 3.55 on factor prophylaxis. The results were consistent with those seen in adult patients. [ISTH abstract LB 02.1]

Sanofi's Qfitlia™ and Hepatobiliary Events

7/11/26 Sanofi markets Qfitlia (fitusiran) as a rebalancing agent for treatment of patients with hemophilia A or B, with or without inhibitors. Qfitlia is a bimonthly, subcutaneous injection that reduces the body's production of antithrombin, an anticoagulant. Early data (from 2023) had shown instances of liver enzyme elevation and hepatobiliary disorders (HDs) in 3.8% of patients (3.1% of hemophilia B patients). "Hepatobiliary" refers to the organs and structures related to the liver, gallbladder, bile ducts, and sometimes the pancreas. Although the number of patients experiencing HDs was small, many potential Qfitlia patients have been concerned. At ISTH, Sanofi presented 120 days of additional data from the 286 study subjects.

In the 120-day update, the researchers saw no significant increase in HDs. The only change was that one hemophilia B subject had an increase in liver enzymes. Thus, when adjusted for exposure-days, the incidence of HDs went down. Sanofi will continue to follow these patients in their extension study. [ISTH abstract PB2099]

Safety and Efficacy of Qfitlia by Age

7/11/26 Sanofi has continued to analyze the results of its clinical studies of Qfitlia. This is a common practice among manufacturers. Although efficacy and safety are the "primary endpoints," studies are expensive, and manufacturers don't want to miss the opportunity

to learn more about their product in a patient group. A key principle in the pharmaceutical industry is that "the manufacturer should know more about their product than anyone else in the world."

To that end, Sanofi examined the effect of patient age on the safety and efficacy of Qfitlia. The 286 participants in their Phase III study were divided into three age groups: adolescent (12–17 years), adult (18–64 years), and elderly (65 years and older). They found no differences in efficacy among the age groups, with a median ABR of 3.7 for both the adolescents and adults. Safety findings were equivalent across the three groups.

One of the 60 adolescents, three of the adults, and none of the elderly experienced thrombotic events. 40% of the adolescents and 28.9% of the adults had no bleeds. The elderly group had lower ABRs and fewer bleeds, but with only three subjects, no definitive conclusions could be made. [ISTH abstract PB2126]

Shanghai RAAS Developing SR604, a Rebalancing Agent Against Activated Protein C

7/11/26 Shanghai RAAS Blood Products Co. is developing SR604 as a rebalancing agent for treatment of patients with hemophilia A or B. SR604 is a biweekly, subcutaneous, monoclonal antibody that inhibits activated protein C (APC), an anticoagulant. Bayer previously developed HAPC1573, a precursor to SR604, but it did not appear to inhibit APC strongly enough in preclinical animal studies. SR604 was developed by modifying HAPC1573 to have a higher binding affinity and thus a stronger effect on clotting.

At ISTH, Shanghai RAAS presented the results of their Phase IIb clinical studies. Three groups of 12 patients each were dosed every four, six, or eight weeks. The subjects included both hemophilia A and B patients, with or without inhibitors. At week 24, median ABRs were 2.2, 5.4, and 6.5 in the three dose-interval groups, respectively, compared with pre-treatment median ABRs of 40.0 in all three groups. The safety profile was also favorable across the three dose groups. The company is now planning a Phase III study. [ISTH abstract LB 02.2]

Tangram Reports Preclinical Results for TGM-148 for Hemophilia and vWD

7/11/26 Tangram Therapeutics (formerly e-Therapeutics), a British company, is developing TGM-148 (formerly ETX-148), a rebalancing agent for treatment of patients with hemophilia A or B, with or without inhibitors, and for treatment of von Willebrand disease (vWD). TGM-148 is a quarterly subcutaneous injection that reduces the production of Protein Z-dependent protease inhibitor, an anticoagulant.

In mice and non-human primates, TGM-148 was well-tolerated and showed no signs of thrombogenicity. It also did not increase thrombogenicity when co-administered with BeneFIX or NovoSeven, suggesting little trouble treating breakthrough bleeds. Tangram is planning to submit an application to begin clinical studies this year. [ISTH abstract PB2133]

CELL AND GENE THERAPY (CGT)

Gene therapy is the process of inserting new, functional factor IX genes into the body so it can produce its own factor IX. Cell therapy is the transplantation of whole cells that have been modified with a new gene to perform a specific function such as producing factor IX.

Be Bio Ends Cell Therapy Study for Hemophilia B

6/17/26 Be Biopharma has ended its development of BE-101, a B cell-based cell therapy for hemophilia B. Few details have been reported beyond the fact that the discontinuation was not due to safety issues but was a "strategic business decision." They had already enrolled five patients for their Phase I/II study, but it is unclear whether any have been treated yet. [https://www.biospace.com/drug-development/be-bio-bins-hemophilia-b-cell-therapy-trial-the-biotechs-lead-program]

Belief BioMed Reports Long-Term Follow-Up for Dalnacogene Ponparvovec Gene Therapy

7/11/26 Belief BioMed (BBM), a Chinese company, has developed BBM-H901 (dalnacogene ponparvovec), a gene therapy for hemophilia B. BBM-H901 uses a proprietary AAV vector (AAV843) to deliver the Padua factor IX gene variant to the liver. China approved it in April 2025 as the first hemophilia gene therapy approved for a Chinese company.

At ISTH, they reported on the long-term safety and efficacy from their clinical studies. In 32 subjects, they found an average factor IX level of 61.3% and a median of 33.4% after two years. The ABR was 0.48 after one year, with 84.4% of subjects experiencing zero bleeds at

the two-year point. The average number of target joints decreased from 1.1 before treatment to zero after two years. No drug-related adverse events, no thrombotic events, no inhibitor development, and no signs of cancer were observed. [ISTH abstract OC 21.2]

Belief BioMed also reported a literature study comparing BBM-H901 with CSL's Hemgenix and Pfizer's discontinued Beqvez. That encompassed 229 subjects from 16 studies. After three years, BBM-H901 patients had an average factor IX level of 65.3% compared with 42.0% for Hemgenix and 39.4% for Beqvez. The authors state, however, that because of the small number of patients, the results require "cautious interpretation." [ISTH abstract PB2059]

Note that BBM has apparently been planning to bring BBM-H901 to the U.S. and Europe, as they have received Breakthrough Therapy Designation, Orphan Drug Designation, and Rare Pediatric Disease Designation from FDA, as well as Advanced Therapy Medicinal Product Classification from the EU. However, considering the business situation for the product (see below), it's not clear whether that will happen.

Belief BioMed Ends Collaboration with Takeda

8/27/26 Belief BioMed and Takeda China have ended their collaboration on BBM-H901. BBM will retain the commercial rights to the product. The two companies called it a "mutual strategy-driven decision." One industry observer on X has claimed that the product had not had a commercial patient in over a year and had cut the price by over 50%. This seems similar to the situation for gene therapies in the U.S. In addition, Takeda has been stepping away from cell and gene therapy for several years. [Endpoints News Briefing 8/27/26 and comment on X by Minhua Chu 8/25/26]

CSL Update on Temporary Shortage of Hemgenix

7/24/26 CSL markets Hemgenix, a gene therapy for hemophilia B that is delivered by an adeno-associated virus (AAV) vector and uses the Padua high-activity factor IX gene. In March 2026, CSL announced it was experiencing a product shortage due to manufacturing difficulties.

In April 2026, CSL reestablished a limited supply of Hemgenix, which treated 22 additional patients, both commercial and clinical study patients. They have now treated over 100 patients commercially. [CSL press release 7/24/26]

UCL/St. Jude Explores Factor Expression after Gene Therapy

7/11/26 University College London (UCL) and St. Jude Children's Research Hospital performed some of the earliest successful hemophilia gene therapy studies. In ten hemophilia B patients, they have observed continued factor IX production with no long-term toxicity for up to 13 years. They have also been conducting the GO-8 study of factor VIII gene therapy for hemophilia A. Their clinical studies are research studies to better understand hemophilia gene therapy, rather than studies toward approval of a new gene therapy treatment.

At ISTH, they presented results on factor expression by transformed liver cells in five subjects (4 As, 1 B) treated for up to 128 months (10.7 years). They are trying to understand the varying responses in individual patients as well as the durability (longevity) of the response. One marked response seen in the four hemophilia A patients was a decrease in factor VIII production over time. The four hemophilia A subjects had initial factor VIII levels of 36, 10, 57, and 18% of normal, which declined to 1, <1, 35, and 4%, respectively.

In contrast, the single hemophilia B patient has maintained steady factor IX production at 6% for 128 months. The decline is similar to findings in several other factor VIII gene therapies. The reason isn't yet understood, but this study sheds some light on it.

Using liver biopsies, the authors determined that a median of 24% of liver cells (range 10-73%) retained their new genes, but less than 25% of those were actively producing factor. The new factor genes in the remaining cells appeared to have been silenced by epigenetic changes. Epigenetic changes refer to chemical tags placed on genes that can increase or decrease the production of their protein product. In other words, the new genes are still there in the transformed liver cells, but those genes have been "turned off" by the cell. They found that certain compounds can reverse silencing by turning the genes back on, as can targeting certain gene regulatory pathways in the nuclei of the cells.

These changes may explain differences in factor production among patients. They offer promising targets to improve the uniformity and durability of gene therapy outcomes. [ISTH abstract OC 21.3]

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women & girls with hemophilia



articles to support, educate, and empower

Meet Gareth:
Former In-N-Out All-Star, but now "Mom" is Her Most Favorite Management Role of All

By Shelly Fisher

Amid the happy Saturday sounds filling her home, and between hugs from her children, Gareth shared part of her weekend with me to talk about being a mom, crafting, burgers, and hemophilia B. Judging from the way her face lit up each time a family member entered the room, it was clear which one of her many roles meant the most to her.

With an easygoing demeanor and calm spirit, Gareth is the kind of person who reminds you not to take life too seriously, and her natural people skills didn't go unnoticed by her employer either. As an all-star employee for In-N-Out Burger, she has helped open no fewer than 50 locations in the United States as a cook and trainer, and coincidentally, met her husband while opening one particular store. With a grin, she explained more. "I met my husband Daniel when they put him on the grill with me, and he was too slow, so I kicked him off, and they put him on traffic."



Gareth never missed a beat when we talked about future goals and dreams. "Right now, my goal is just to be the best mom I can be. I've always wanted to be a mom, and now that I've got them here, I just want to make sure they grow up knowing who they are, that they are loved, and know where they came from."

When she gets the house to herself, Gareth grabs her crochet hook, yarn, and the television remote. "With only one TV in the house, I don't ever get control of the remote unless I am here alone," she shared with a huge smile. She also taught herself to refurbish some vintage sewing machines her grandmother left her, and she plans to leave one to each of her children.



When our conversation turned to hemophilia B, Gareth shared that she is an obligate carrier. Though her father experienced a bleeding episode during the removal of his wisdom teeth in the '80s, he didn't seek a diagnosis until many years later when he had knee surgery in 2015.

Her older sister became symptomatic after a surgical procedure in 2009, and it was then that Gareth considered the possibility that hemophilia B might be responsible for the constant bruising in her hips and pain in her hands that she had experienced for

most of her life. Gareth received her diagnosis as a carrier in 2009, but her sister struggled to receive the appropriate diagnosis and factor she needed until 2019.

Frustrated by her sister's continued challenges to obtain the clotting factor that she needs even after her diagnosis, Gareth joined the Lone Star Bleeding Disorders Foundation in 2024. She then connected her sister with a liaison who provided the support she needed. Gareth benefited from her association with the foundation because she learned about the Coalition for Hemophilia B there last year.

In addition to her sibling's diagnosis, all but one of Gareth's children were also diagnosed at birth, or shortly after, with mild hemophilia B, and the youngest currently has a low-clotting factor, but has not been formally diagnosed, nor has she had any bleeding episodes. "When we go in for doctors' visits, it's all of us at once, and it's a wild room!" While she is grateful their levels aren't lower, she stays watchful and ready to act if needed.

Gareth's gratitude for the Coalition for Hemophilia B symposiums runs deep. "I want my boys to know that they are not alone. It was important to us for them to meet other people like them."

For Gareth and her husband Daniel, the sessions provided to new families meant the most. Listening to other parents talk about navigating sports and life in general was invaluable, and they also enjoyed a nutrition class. A session on relationships rounded out their first symposium, as they learned how to become closer through different seasons of life through small acts like touch, kindness, and service.

Her advice for anyone newly diagnosed? "It's not going to hinder you in life, and science has come a long way. You will have a huge support in the CHB community. Your family is not alone." Her sons, Everett and Ryett, agreed whole-heartedly and added, "You can come with us to the big party (symposium)!"

Gareth's personal inspiration comes from 1 Corinthians 16:14, and she tries to live it out: everything you do and say should be done in love. She mentioned her husband specifically as someone who exemplifies this verse because he works hard for their family, reads the Bible with their family, and teaches Sunday school at church. "He's the rock in our family."

In addition to her husband, Gareth is especially grateful for Cienna, the nurse who has supported her family since her oldest son was born. Gareth shared that Cienna's knowledge and experience make them feel cared for, and her support has made all the difference in their lives.





Thank you, Gareth, for sharing your journey with the CHB. Your family's light shines brightly, and we can't wait to see you at future symposiums!

Gettin' in the Game Junior National Championship 2026



By Shelly Fisher

On May 1-3, 2026, 98 junior athletes and their families converged on Henderson, NV, to "get in the game" thanks to the longstanding partnership between the Coalition for Hemophilia B and CSL.

The only national sports competition designed for the bleeding disorders community since 2002, the Gettin' in the Game Junior National Championship (GIG JNC) was designed for youths ages 7 to 18 to learn the fundamentals of basketball, baseball, golf, and swimming from professional athletes who have also been diagnosed with a bleeding disorder.

In addition, participants and their families also attend educational seminars about staying physically active while managing hemophilia B and von Willebrand disease, but perhaps the most exciting part of this yearly event developed by CSL is the competitions themselves. Peers compete in friendly attempts to vie for a national championship title, and of course, bragging rights back home.

Jackson was one such contender. A baseball player since he was three years old, he learned about the GIG JNC from a social worker at the clinic where he obtains his clotting factor. His expertise as a catcher and first baseman helped him win the essay.

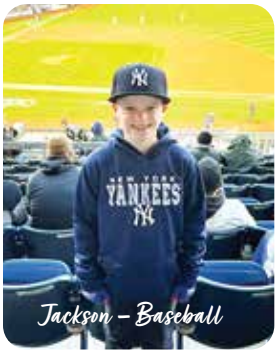
He remembers being super excited the day his dad told him he had been invited to the GIG JNC, and he recalls meeting Jesse Schrader as one of the weekend's highlights. Jackson would highly recommend this experience to anyone with hemophilia B because "you get to meet other people with hemophilia that play the same sport you do." With plans to play in high school and college, we might just see Jackson at a GIG JNC in the future as a coach!



Theo's mom saw an Instagram post with GIG JNC info. As an avid swimmer, Theo "jumped right in" and began writing his essay on the sport he loves. Like Jackson, he too enjoys competing with friends and traveling to new places. His winning essay stated, "My swimming with hemophilia helps people know that they can still do hard stuff even if they have to face a challenge."

No stranger to the pressure that precedes a heat, Theo was ready to dive into the competition. He shared, "I am pretty fast." And he's got the medals to show for it. When he arrived at the GIG JNC, it was more than he anticipated. "It was a lot bigger a deal than I thought, with more kids, and fun things like swimming with a bungee cord on." With plans to keep swimming in school competitions, Theo might one day also coach at future GIG JNCs!

Since its inception, the JNC has taken place in more than 30 cities across the United States and has hosted more than 1000 youths and their families. Participants learn how to stay active and manage their condition effectively while meeting other families and athletes who share the same interests.


Jackson – Baseball




Theo – Swimming





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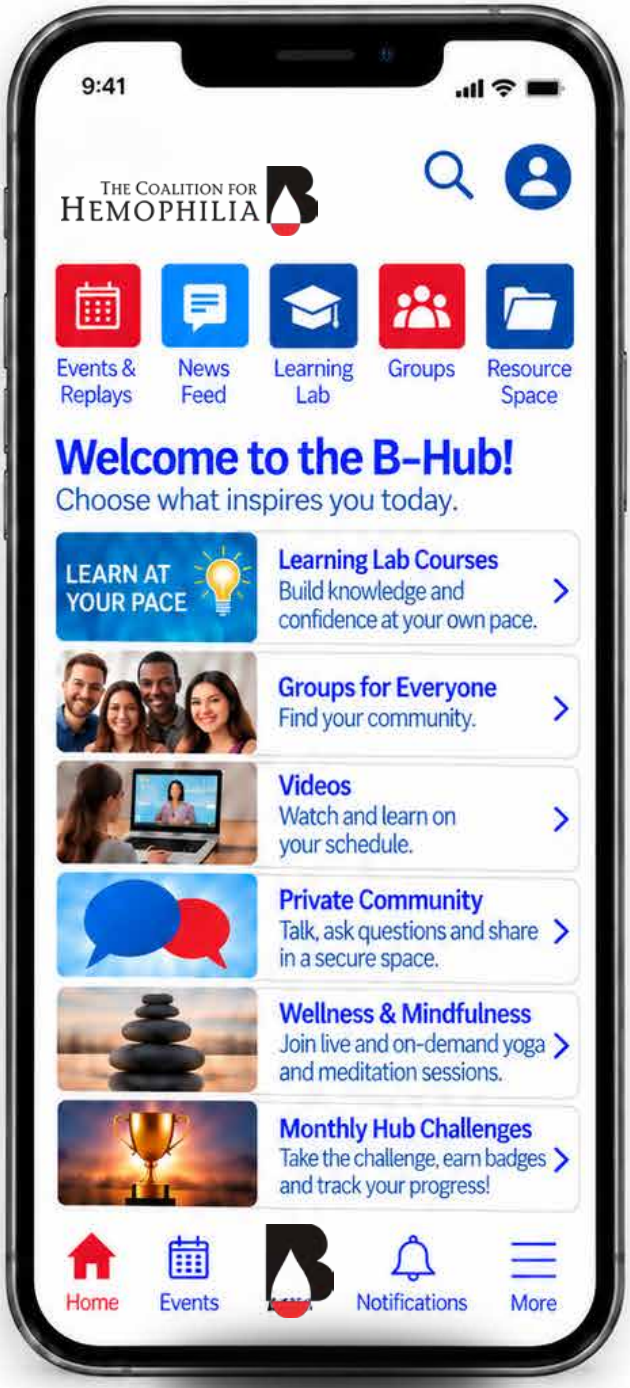
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Meetings on the Road

By Lisa Hensley, Ph.D., M.S.P.H., with contributions by Fel Echandi

Baltimore



























There is something special about a Coalition for Hemophilia B *Meetings on the Road*. The Annual Symposium brings us together as a large community, but these smaller regional meetings feel different.

They give us time to sit across the table from one another, talk, listen, ask questions, and get to know the people behind the stories. This June, we did that twice on back-to-back weekends, in Baltimore on June 6 and in Kansas City on June 13.

For me, these meetings are personal. I am a scientist by profession, but I am also the mother of a child with hemophilia B. Scientific advances matter enormously, but information becomes truly useful when families can understand it and consider what it means for their own lives. And sometimes what a family needs most isn't another presentation. It's the chance to talk with a parent who has faced the same decision, meet an adult who grew up with hemophilia B, or hear someone say, "We went through that, too."

Both days began with Robert Friedman's *Rhythm & Rise*, a mix of simple beats, light movement, and laughter that made it nearly impossible to remain a passive observer. In Baltimore, he also guided the group through a relaxation exercise that helped everyone settle in.

Next came one of my favorite parts of any Meetings on the Road: *Voices that Matter*, a community rap session designed as a conversation rather than a lecture. In Kansas City, I facilitated it with Leisa MacDougall. No one had to speak; simply being present and listening mattered just as much. People talked about hard things, and also about victories, children growing up, changing treatments, school, work, and care teams. Someone inevitably offered an idea another family had never considered.

Each city brought its own character to that conversation. Baltimore was an intimate group of five families, and most attendees were women who infuse. Discussion quickly turned to questions specific to women with a bleeding disorder.

Gene therapy was a central topic in both cities. In Kansas City, Amber Campbell brought years of experience caring for people with bleeding disorders, while Chip Hailey shared the perspective of someone who received gene therapy through the Phase 3 HOPE-B clinical trial in 2019. People with hemophilia B have more treatment choices than

Baltimore and Kansas City Highlights!

Kansas City

ever, which also means more decisions. Our role as a community is not to tell anyone which choice to make, but to make sure people have good information, know what questions to ask, and feel confident participating in their own care.

That theme carried into *Health is More than a Doctor's Appointment*. In Kansas City, Joseph Wilner's message was refreshingly practical: step out of crisis mode and focus on small, sustainable habits, including care, routines, planning, and attention to physical, mental, and emotional health. Dale Bodyk shared how he stayed active growing up with hemophilia by making thoughtful choices, preparing well, and still finding joy.

By mid-afternoon, everyone was on their feet again for *All Together in Motion*, a community dance experience designed for everybody. In Baltimore, a Hawaiian dancer taught the group how dance can communicate through hand gestures, a joyful reminder that connection takes many forms.

Meanwhile, our younger attendees had adventures of their own, bouncing through trampolines and obstacle courses at Sky Zone in Baltimore with volunteer Madison Smith, and heading to Urban Air in Kansas City. For a child who may be the only person with hemophilia in his school, meeting another kid who understands infusions, clinic visits, and bleeds can be incredibly powerful. They get to just be kids together, and friendships grow from that.

Late in the day, *Our History, Our Story* traced hemophilia from ancient records to modern treatment, including darker chapters like the AIDS crisis and the long failure to recognize hemophilia in women. It is an important reminder that today's options came from decades of research, advocacy, and families willing to push for better care. *B-Amazing: We Are In This Together* closed the program with games, laughter, and time to reflect on what we were taking home, before families gathered for a community education dinner.

Kansas City added a little extra excitement courtesy of the weather, as we narrowly missed the worst of some significant storms. Maybe it was fitting. Living with a bleeding disorder teaches families a great deal about changing plans and adapting when necessary.

















That is why these meetings matter. I hope people left with the name and phone number of someone they can call, a question to ask at their next appointment, a little more confidence, a new friend, or simply the reassurance that there are people who understand. That is the real strength of the Coalition for Hemophilia B. We keep showing up for one another.

We are grateful to everyone who joined us, to Laura and Fel Echandi for leading the Baltimore meeting, to all the speakers and volunteers who shared their time and expertise, and to CSL, whose support made these meetings possible. Because none of us should have to navigate hemophilia B alone.

CSL

Continuing to Deepen: A Gene Therapy Support Network Update

By Robert (Bobby) Wiseman Jr.

Our Gene Therapy Support Network has deepened, not just in topic complexity, but in how people show up, listen, and participate. It's more than a meeting; it's a space people return to.

Our monthly virtual gatherings continued this summer from June through August, making consistency part of the program. Some are exploring gene therapy, others are adjusting after receiving it, and some have chosen a different path. All these experiences meet at the same table.

A highlight has been the growing voice of our younger members, or "nephews." They're more active in chat and conversation, and in August, a 22-year-old shared his story openly. The "uncles" bring history; the "nephews" bring emerging realities. Wisdom now flows both ways.

Each gathering brings "aha" moments: someone shares an appointment, and another realizes, "I need to ask my team about that." The conversations are more practical, shifting from "Can I receive gene therapy?" to "What would it require, and what happens after?"



Community is growing beyond our meetings. This summer, participants attended a first-person talk by one of our "uncles" and reached out to support each other, unprompted, simply remembering one another.

Looking ahead to fall, our goal isn't to add more, but to nurture what's already growing. This network gives people access not just to information, but to each other.

We thank CSL for sponsoring the Gene Therapy Support Network and making this space possible.

CSL

Foro Latino: Empowering Our Spanish-Speaking Community Through Education

By Danelie Rivera

Foro Latino was established with a clear and meaningful mission: to serve the Spanish-speaking community by delivering accessible, relevant information about bleeding disorders in Spanish. This initiative emerged from actively listening to our community, understanding their concerns, and identifying the topics they wish to explore further.

We held virtual meetings on June 1 and July 13. These conversations guided the development of educational sessions that empower individuals with knowledge, confidence, and the tools to make informed decisions.

One topic that has generated significant interest is Section 504 of the Rehabilitation Act of 1973, a federal civil rights law in the United States and its territories that prohibits discrimination against individuals with disabilities in programs and institutions receiving federal funding, including public schools. Understanding these rights is especially important for families affected by a bleeding disorder, as it helps them recognize the resources, accommodations, and support available to ensure an equitable educational experience.



By addressing important topics in Spanish, Foro Latino bridges information gaps and strengthens our Latino community. More than just an educational forum, it is a welcoming space where families can learn, ask questions, share experiences, and deepen their understanding of bleeding disorders in the language that feels most comfortable to them.

We are very thankful to CSL for sponsoring Foro Latino!

CSL

Caregiver Thread: Caring for the Caregivers

By Leisa MacDougall

This summer, the Coalition for Hemophilia B launched the Caregiver Thread Support Network, a space where caregivers can come together, connect, and talk openly about the challenges that often come with caring for others.

Our first virtual rap session on June 15 tackled an important and often-overlooked topic: compassion fatigue. Lucretia Jelks guided participants through the emotional, mental, and physical toll that can develop when we continually care for others through ongoing challenges. Caregivers explored how to recognize the signs and symptoms, the difference between compassion fatigue and burnout, and practical strategies for self-care, resilience, and recovery. The message was one we so often forget: we deserve the same compassion and care that we so freely give to others.

On August 6, the conversation turned to preventing burnout. Our expert speaker, Lakiesha, offered practical strategies to protect well-being, recognize when support is needed, and build confidence in asking for help. Through real-life role-playing scenarios, caregivers practiced responding to challenging situations before they lead to exhaustion. Participants were encouraged to take charge of their energy and prioritize their own well-being, with a clear reminder that caring for ourselves is not selfish. It is essential to being able to continue caring for others.



Both sessions closed with a roundtable discussion led by Melissa P. and Leisa M., giving community members the chance to share experiences, ask questions, offer encouragement, and learn from one another. Together, these first two Caregiver Thread rap sessions have laid the foundation for something our community has long needed: a place where caregivers are reminded that they matter, too, and that seeking support is part of the journey.

Innovation Lab Recap: Understanding the Future of Hemophilia B Care

By Kelly Ireland

The pace of innovation in hemophilia B has never been greater, and this year's Innovation Lab was designed to help our community keep up. For over eight weeks starting in July, Dr. David Clark led an educational series on how hemophilia B treatment has evolved, where it stands today, and what may be coming next. Each 30-minute session paired a clear, approachable presentation with time for questions and discussion.

Sessions covered the history of hemophilia B care, extended half-life factor therapies, inhibitors, rebalancing therapies, cell and gene therapy, emerging research, and the unique needs of women with hemophilia B. The final session took a closer look at HYMPAVZI®, Pfizer's once-weekly treatment option for patients with hemophilia A or B, with or without inhibitors.

Whether you joined live or are just hearing about the series, you can find every Innovation Lab recording in the B-Hub. Watch at your own pace, revisit a session that stuck with you, or catch up on the topics most relevant to your care.



Understanding your options is the first step toward confident, shared decision-making with your healthcare team.

Many thanks to Pfizer for generously sponsoring this educational series!

(Watch the Innovation Lab recordings in the B-Hub)

Strength in Connection

By Carl Weixler

On June 11, Wayne and I led Strength in Connection, our gathering for CHB community members ages 50 and over, and about 25 people joined us.

We kicked things off as always, with an icebreaker and a meet-and-greet. This is a lively group, and the conversation flows freely. Before long, we were connecting over shared life experiences, the kind that only people who have lived with hemophilia B for decades truly understand.

Near the end, we wrapped up with some hemophilia trivia, which brought out plenty of friendly competition and laughter. Everyone who joined also had a chance at food vouchers and raffle prizes.

What I value most about this group is simple: I'm glad I don't have to do it alone. I truly feel like I have a hemophilia family, people who share ideas and support each other through whatever life brings. To me, that's what real strength looks like.



National efforts like *Aging Untold* have put a spotlight on growing older, and CHB has been doing the same for just as long in the hemophilia space, supporting the B community. Kudos to CHB for supporting us!

Living B Recap: Real Talk, Real Connection

By Kelly Ireland

This summer, Living B: The Men's Collective brought together men living with hemophilia B and dads raising the next generation for two sessions built around one idea: doing this alone isn't how it's meant to be.

On June 30, speaker, coach, and Air Force veteran Seth Miller led *Men's Health: Investing in Your Well-Being,* presented in partnership with *Balancing Life's Issues*. Seth named the habits many men learned early: push through pain, stay busy, put yourself last. He asked a simple question. If you keep up with oil changes for your car, why not checkups for yourself? The discussion gave guys a chance to talk openly about why taking care of themselves can be so hard.

On August 31, community co-host Carl started the conversation on joint health by sharing his own story. At 65, he has made swimming and aquatic exercise a key part of keeping his joints healthy. Others then shared what has worked for them, and the talk soon grew into a wide-ranging rap session among men who understand the day-to-day.



As Carl put it, "If you attend, you won't regret it."

Thank you to Sanofi for supporting Living B: The Men's Collective.

sanofi

Putting Ourselves First: Voices of Her Bloodline Explores Women's Health & Well-Being

By Jennifer DeGlopper

On July 10, women and girls from the hemophilia B community gathered for Voices of Her Bloodline: Women's Rap Session, *Women's Health: Investing in Your Well-Being*.

Conversation guide Carlisa Johnson, MPH, a public health strategist, health communications specialist, and founder of *K.A.M.U. Collective: Know the Art of Making You*™, opened the session by exploring the physical, emotional, and lifestyle factors that shape women's health across the lifespan. Drawing on more than a decade of experience supporting national public health initiatives, including work with the CDC, she offered practical insight into why so many women put their own health last while caring for others.

From there, the conversation belonged to the participants. Women shared experiences and asked questions about finding quality care, advocating for themselves in medical settings, managing stress, and building sustainable habits that support lifelong wellness. For women and girls who have or carry hemophilia B, whose symptoms are too often dismissed, these conversations matter deeply.



Community co-hosts Kim Phelan and Jennifer DeGlopper helped guide the discussion, which reflected the heart of the Voices of Her Bloodline series: a supportive space to learn from one another and build meaningful connections.

Thank you to Carlisa and to everyone who joined us!

Adulting Unlocked Recap: Say It, Own It, Live It

By Kelly Ireland

On July 30, young adults in the CHB community gathered for the latest Adulting Unlocked webinar, *Say It, Own It, Live It: Find Your Voice with the People Who Matter Most*, presented in partnership with *Balancing Life's Issues* and led by Kristen Lee Picard, MSW, ASW, RMT.

Kristen's message was simple: for young adults managing hemophilia B, communication is not just a soft skill. It is a survival skill. And there are conversations many of us have been avoiding. The session walked through four zones where finding your voice matters most: family and friends, where speaking up about your needs builds deeper support; work, where you can confidently request accommodations without oversharing; your CHB community, where connection fosters strength; and yourself, where learning to name what you feel is the first step.

Participants also practiced essential listening skills, including active paraphrasing and asking permission before offering advice, and learned the *60-Second Reset*, a quick pause-and-breathe practice for choosing a thoughtful response instead of a reactive one.



The session ended with a challenge: identify one conversation you have been avoiding and commit to having it this week.

As Kristen reminded everyone, "You're not bad at this. You're just out of practice."

A Weekly Reset for Mind, Body, and Soul

By Kelly Ireland

Prioritizing mental health is easier when you don't have to do it alone. That's the heart of our ongoing live-streamed series in the B Education Hub, led by somatic healing practitioner Renk Koçtürk. Each 60-minute session offers practical tools and calming moments to help you care for your mind, body, and soul.

Renk brings a rich background to this work. She's a movement therapist, herbalist, and trauma-informed educator trained in dance therapy, yoga therapy, and somatic experiencing. Her sessions are inclusive, body-positive, and rooted in her belief that movement is medicine, accessible to all.

In a recent session, Renk guided participants through a relaxation meditation that began by noticing where their bodies connected with the ground. She then invited them to bring awareness to each part of the body, moving slowly upward. The practice closed with gratitude and a gentle reminder to make time for mindfulness, meditation, and ourselves.



This fall, join Renk live every Thursday at 7 pm ET / 4 pm PT in the B-Hub, or catch the replay whenever it fits your week.

This series is proudly sponsored by CVS Specialty.

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The Weekly Reset

Every Thursday
7 pm ET, Virtual
Sponsored by CVS Specialty

Join Renk Koçtürk in the B-Hub every Thursday for our ongoing series, featuring practical tools and calming moments designed to help you take care of your mind, body, and soul.



Foro Latino

Oct 19, 2026
8–9:30 pm ET, Virtual
Sponsored by CSL

Esta serie está diseñada para la comunidad latina de hemofilia B, proporcionando un espacio dedicado a conectar, aprender y discutir temas clave que más te importan.



Understanding a Rebalancing Therapy Option for Hemophilia

Oct 20, 2026
8–9:30 pm ET, Virtual
Sponsored by Pfizer

Join us for an educational session exploring a rebalancing treatment option for hemophilia.



Seen. Believed. Women & Girls with Hemophilia B

Oct 21, 28 and
Nov 4, 11, 18, and 24, 2026
8–8:30 pm ET, Virtual

Seen. Believed. is a B-Hub Learning Lab created for women and girls with hemophilia B, and their families, to better understand bleeding, health, and treatment through every stage of life.

Topics:

Oct 21 – Treat the Bleeding, Not the Label: Understanding Your Treatment Options

Oct 28 – The Whole Body: Hemophilia B & Your Changing Health

Nov 4 – Infusion 101: Tips, Tricks and Building Confidence

Nov 11 – Could Prophylaxis Be Right for Me?

Nov 18 – Caregiving for Girls and Young Women with Hemophilia B

Nov 24 – Periods and Hemophilia B: The Good, the Bad & the Bloody



Men's Education & Empowerment Retreat

Oct 22–25, 2026
Orlando, FL
Sponsored by Pfizer & Sanofi

A unique 3-day experience where men with hemophilia B, fathers of children and partners of women living with hemophilia B are given a safe space to share and gain support. Connect, laugh, learn, and talk with other guys navigating similar experiences in a supportive, judgment-free environment.



Living B, The Men's Collective

Nov 5, 2026
8–9:30 pm ET, Virtual
Sponsored by Sanofi

A space built for men living with hemophilia B and fathers of children with hemophilia B.
A supportive conversation where men can share experiences, ask questions, learn from one another, and build meaningful connection.



Meetings on the Road

Nov 7, 2026|Redondo Beach, CA
Sponsored by CSL

Connect with your hemophilia B community! This family-friendly event is educational, supportive, and free for all community members in attendance. The event features a full day of exciting activities and interactive learning sessions. Gas and toll expenses will be reimbursed, and meals will be provided.

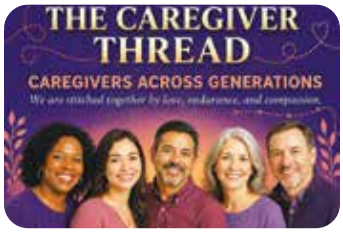


Gene Therapy Support Network

Nov 10 and Dec 8, 2026
8–9 pm ET, Virtual
Sponsored by CSL

Have had, or are considering gene therapy? Join our private group discussion to share firsthand experiences and have questions answered. Ages 18+. Dinner vouchers provided.





Caregiver Thread

Nov 13, 2026
8–9:30 pm ET, Virtual

Whether you're a parent, partner, or family member, this is a place to connect, share, and feel supported by others who truly understand.



Voices of Her Bloodline

Nov 16, 2026
8–9:30 pm ET, Virtual

For women living with hemophilia and those still on the journey to diagnosis. Event supportive conversation where women can share experiences, ask questions, learn from one another, and build meaningful connections.



Adulting Unlocked

Nov 19, 2026
8:00 pm ET, Virtual

Addressing the realities of adulthood head-on, building confidence and tools to thrive.



Gingerbread House Decorating & Trivia

Dec 5, 2026
4–5:30 pm ET, Virtual
Sponsored by Medexus Pharma

Join us as we gather to decorate gingerbread houses, share laughs, and enjoy a few games!

Let's Play IX Golf Outing

March 17, 2027
Orlando, FL

Let's Play Nine golf outing and scholarship fund makes it possible for youth with hemophilia B to participate in a fun and healthy lifestyle option they can continue to participate in as they age.



2027 Annual Symposium

March 18–21, 2027
Renaissance Orlando at SeaWorld; Orlando, FL

Our yearly educational symposium brings together families from across the USA to learn, share, and support one another. For over 3½ days, you'll find warmth, friendship, and valuable sessions on every aspect of life with hemophilia B. We look forward to seeing you!

Applications due Jan 4, 2027
Apply today!
https://www.hemob.org/annual-symposium

The Factor Nine Holiday Fund

The Coalition for Hemophilia B recognizes that some families in our community may experience financial hardship this holiday season. In addition to the Coalition's own contribution, we ask our more fortunate Factor Nine Families to donate to the "Holiday Fund." Your generosity will help bring gifts to children living with hemophilia B!



To donate, visit **hemob.org**, click "Donate" (top right), click on "Donate" again (center), scroll down and select "Factor IX Holiday Fund," or mail a check payable to:

CHB "Holiday Fund"
880 Third Avenue, 5th Floor; New York, NY 10022

Please respond by **November 17, 2026**, so Factor Nine Santa can load his sleigh with holiday gifts for all good girls and boys!



For families in our community in need of a little holiday cheer, we would love to help make the holidays special for your children. Fill out this form and send it to Santa's special elf, Kim, at the "East" Pole. Please return the form by **November 17, 2026**, as Factor Nine Santa has a busy schedule. Your name and information will be kept strictly confidential.

Mail this form to:

CHB "Holiday Cheer"
880 Third Avenue, 5th Floor; New York, NY 10022



We wish you all a beautiful holiday season filled with love, happiness and good health!

Name: _____

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City: _____ State: _____ Zip Code: _____

Phone: _____ Email: _____

* For children up to age 18. Gifts will be sent to your home.
* Please give an exact description of your child's wish item.
* Please note which child is affected by hemophilia B.

Child's Name and Age:	Child's Name and Age:	Child's Name and Age:
_____	_____	_____
Wish List:	Wish List:	Wish List:
_____	_____	_____
_____	_____	_____
_____	_____	_____

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> SHANNON L. ALDER

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Selene is Making Her Mark on the World

By Shelly Fisher

On a sunny day in Seattle, Selene settled in to talk about Beetlejuice, art, hemophilia B, and a snake named Dashel Cornington Russo I Esquire. Those topics alone would rank her among the most interesting 8th graders at her middle school, but there's more.



A self-proclaimed scary movie buff, Selene shared that her favorite movie, Beetlejuice, was going to be the theme for her birthday, and she was dressing up as the main character because, "He's the ghost with the most!" Then, in a flourish of feet, she displayed socks with Zero on one foot and Jack on the other, confirming her love for The Nightmare Before Christmas as well as all Tim Burton movies in general.

Selene also shared that she "likes art," but her exceptionally detailed and dimensioned drawing of a skeleton belied the fact that she does more than like it. However, when I asked Selene if she wanted to pursue illustration as a career, her answer surprised me. "I want to be a mortician. A lot of people are too scared to do the job, and there needs to be a lot more people in that field. Maybe I will draw comics on the side."

On weekends, she confided that you can find her scrolling Pinterest and hanging out with family, including three snakes, Dashel, Andromeda, and Houdini, a cockroach, a puppy named Daisy, a hamster named Ember, and a snail. Houdini was aptly named, but I was assured no pets were harmed during his brief breakout.

Diagnosed at 9 months with mild hemophilia, Selene revealed that she gets upset when someone calls her a carrier. "It's sexist thinking that women can only be carriers. It's like we don't have hemophilia." After attending several symposiums organized and produced by The Coalition for Hemophilia B, she found her voice and felt a special connection to the B the Voice Advocacy Summit event. "I enjoyed speaking to senators, congressmen, and other politicians about hemophilia because bleeding groups are marginalized."



Selene would like to thank her dad for teaching her to infuse, and how to use humor to get through the hard times. In fact, she included him in her advice to anyone newly diagnosed. "Your life isn't over. You can get through this. You've got this. Have your parents talk to my parents."

It should come as no shock at all that this Gen Z teen's favorite movie line and life mantra comes from Beetlejuice's Lydia Deetz character. The line "I myself am strange and unusual" fits Selene well, but it should also be added that she is incredibly talented, humble, and driven to make her mark on the world – whether it's with a pencil or a trip to Capitol Hill, and that's just "fire" to me.







Mateo Hits Grand Slams on the Field and the Hill



By Shelly Fisher

After finishing his homework, Mateo sat down with me to talk about what matters most to him: family, baseball, and a future in politics.

After a summer of baseball to "stay in the rhythm," Mateo was eager to start his upperclassman year. Humble yet proud, he explained he bats cleanup and made varsity as a freshman. When I mentioned that cleanup hitters are expected to deliver, he grinned and said, "That's me. I've hit some grand slams, but doubles are my specialty." Thankfully, he's gone a year without a baseball-related bleed, something that hasn't always been the case for this dedicated first baseman.

"In March of my freshman year, I got a bad knee bleed, which really set me back. In the first game of the season, I got my first varsity hit and was rounding third base when my knee popped inward. I missed half the season due to the internal bleed. I came back in the middle of May." It was determined that prophy infusions were in order from that day on. Although a challenging time, Mateo feels it contributed to who he is to this day.







Mateo is working on college applications and focusing on school. This drive began after attending B the Voice Advocacy Summit in D.C., where he visited House and Senate offices to advocate for legislation supportive of the bleeding disorder community. The experience sparked an interest in government, and now he dreams of earning a law degree, something that feels closer as he visits nearby universities.

It's no surprise that Mateo's favorite classes are AP government and U.S. politics. He's vice president of his junior class, secretary of the humanitarian service club, and active in the Spanish club. "I represent everyone, and I'm open about my hemophilia B. It's a big part of my life."

When he's not cracking a bat or planning to leverage legislation, Mateo is a typical high schooler: "I sleep in a bit, eat my mom's famous egg quesadillas, hang out with friends, hit the gym, and catch a movie." He credits his mom for always being there, his dad for being his number one fan and coach, his sister as a role model, and friends Eli and Sam for their steady support since third grade.

Mateo's mom and grandmother were especially important when he was diagnosed with mild hemophilia at 10 months old. After a fall and a hit to his head, his mother instinctively knew something was wrong. She was repeatedly turned away from the emergency room until Mateo's head began to swell. Although he was finally admitted, the hospital staff insisted on merely draining the blood that had accumulated. His mother refused and demanded further testing. Mateo received clotting factor and remained in the hospital for a week. His mother's strong stance came at the urging of Mateo's now 101-year-old grandma, who had watched her own son experience bleeds in the '60s as a rancher on the family farm.

Mateo credited his experiences at multiple CHB symposiums with helping him embrace and take pride in his disorder. He wants to share this mindset, teach peers to advocate for themselves, and stay involved by volunteering and attending future B the Voice Advocacy Summit events.



His advice to someone newly diagnosed? "Don't let it hold you back. Go enjoy. Go live. Do all you can." That's why his favorite movie is Pixar's *Soul*: "It reminds you to live each moment with appreciation." Thank you, Mateo, for reminding us all to enjoy every moment.

B inspired!

Stories and artwork from teens in the Hemophilia B Community

Summer 2026

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Meet Selene!



Meet Mateo!

WANTED: TEEN CONTENT CREATORS!



Calling all content creators! If you have a heart for tweens/teens and a drive for content creation, then we would love for you to volunteer your time and talents with us. The Coalition for Hemophilia B is currently accepting volunteers to collaborate on a new section of the newsletter just for those special 11- to 18-year-olds in our community.

No experience required as we have a team ready to polish your brilliant ideas for publication. If you have ideas for topics, events, and new sections, let's work on this together – reach out to MatthewM@hemob.org for your next steps!